

ACHILLION PHARMACEUTICALS **ANNUAL REPORT**

# ACH



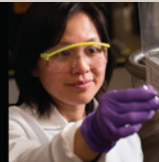
**STRATEGIC  
ANTIVIRAL  
PIPELINE**



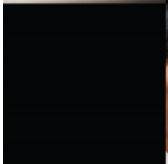
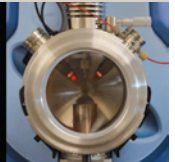
**5-YEAR  
NASDAQ  
ANNIVERSARY**



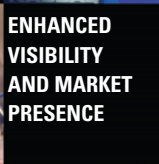
**INDEPENDENT  
BIOPHARMA  
LEADER**



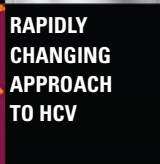
**STRONG AND  
NIMBLE  
INNOVATOR**



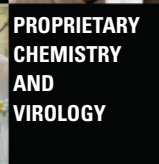
**ENHANCED  
VISIBILITY  
AND MARKET  
PRESENCE**



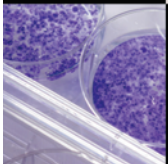
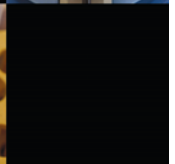
**RAPIDLY  
CHANGING  
APPROACH  
TO HCV**



**PROPRIETARY  
CHEMISTRY  
AND  
VIROLOGY**



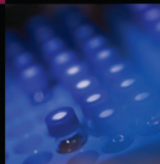
**OPTIMIZED  
NS3 AND NS5A  
INHIBITORS**



**POTENTIAL  
BEST-IN-CLASS  
COMPOUNDS**



**PROVIDING  
VALUE TO HCV  
COMMUNITY**



**UNMET  
GLOBAL  
NEED**



**2011**

# 2011

**A YEAR OF MILESTONES A FUTURE OF ADVANCEMENTS**

# ACHILLION 2011

ACHILLION HAS THE STRATEGIC PIPELINE AND LEADERSHIP NEEDED TO SUCCEED IN AN EVOLVING MARKET.



## INNOVATIVE CHEMISTRY AND VIROLOGY

Achillion focuses on identifying and advancing novel compounds to treat HCV and resistant bacterial infections. Founded in 2000, Achillion has traded on The NASDAQ Global Select Market since 2006. In-house expertise in chemistry, virology and drug development has resulted in a pipeline of small molecules being evaluated in clinical trials worldwide.



## ADDRESSING KEY ASPECTS OF HCV TREATMENT

Achillion has generated a proprietary portfolio of NS3 protease inhibitors and NS5A inhibitors that are being developed as an all-oral, interferon-free approach to treating HCV. Achillion's target product profile focuses on advancing best-in-class compounds that may offer once-daily drugs with pan-genotypic activity, improved barrier to resistance, and that are safe, well-tolerated, and efficacious with a low potential to interact with other medications.



## HCV—GLOBAL DISEASE

An estimated 170 million people worldwide are infected with HCV, including approximately 5 million or more individuals in the US. The prevalence of HCV is nearly twice that of HIV. Three-fourths of the HCV patient population is undiagnosed making it a "silent epidemic" and a major global health threat. A growing patient pool and significant unmet need means tremendous opportunities for new treatment options.

## ACHILLION PIPELINE

NS3 PROTEASE INHIBITORS		NS5A INHIBITORS	
ACH-1625	PHASE 2	ACH-3102	IND SUBMITTED
ACH-2684	PHASE 1	ACH-2928	PHASE 1

**SO MUCH CAN BE SAID ABOUT ACHILLION'S GROWTH** and maturation over the past eleven years, and 2011 was truly a pivotal point for employees and shareholders alike. Achillion celebrated its fifth anniversary as a NASDAQ-listed public company. Operationally, we achieved numerous milestones that advanced Achillion from a discovery company to one that is firmly entrenched as a clinical development organization. Most importantly, we are on track to advance our most promising hepatitis C drug candidates through clinical trials and toward commercialization.

As a company with a remarkably productive history of internal discovery and development, the Achillion team worked overtime during 2011 to advance what we believe is an exceptional portfolio of home-grown and potentially best-in-class compounds, including our NS3 protease and NS5A inhibitor programs for the treatment of the hepatitis C virus (HCV). Several years ago, we made a conscious decision, based upon the evolving science in HCV, the needs of HCV patients and prescribers, and the most expedient development strategies, that Achillion would focus on these two mechanisms with the goal of achieving an all-oral, interferon-free regimen to cure HCV.

A recent major shift in the field of HCV therapy is the clear movement away from interferon, a poorly tolerated injectable drug that has been historically used as part of the treatment regimen for HCV. It now appears, as was predicted several years ago, that the future of HCV treatment will feature a combination approach involving multiple orally-administered direct-acting antiviral agents. We believe that Achillion's portfolio, in its design and development, is poised to address this future treatment approach and to establish us as one of the companies that will usher in the new wave of novel oral therapies for HCV.

During the past 12 months, Achillion's therapeutic portfolio expanded from a single compound in clinical development to a portfolio that can boast four unique compounds: ACH-1625 and ACH-2684, protease inhibitors, and ACH-2928 and ACH-3102, our first-generation and second-generation NS5A inhibitors.

During 2011, we successfully achieved all of our pre-specified milestones. ACH-1625, our most advanced protease inhibitor, was launched into Phase 2 clinical development. The program successfully demonstrated that 12-weeks of triple therapy consisting of ACH-1625 with pegylated interferon and ribavirin could achieve up to 100% cEVR, or continued early virologic response, wherein patients have undetectable virus after 12 weeks of treatment. Data from this trial are continuing to be collected, and we look forward to reporting SVR, or clinical cures, later in the year. We have also achieved a number of milestones in other components of the ACH-1625 development plan. We have developed a tablet formulation that enhances bioavailability approximately 2-fold versus the first-generation study preparation, and have completed a relative bioavailability study in humans that will enable us to begin using the new tablet formulation in subsequent clinical trials. Additionally, all of the nonclinical toxicology work on ACH-1625, including 28-day, three-month and nine-month toxicology, has been completed. The profile of ACH-1625 continues to excel, highlighted by the genotype 1 and pilot genotype 3 clinical activity we have

reported, the robust program of nonclinical preparation that has been completed and the development of a commercially-feasible tablet formulation.

Further, Achillion leveraged its extensive chemistry and virology expertise to build upon the growing scientific knowledge surrounding the NS5A protein in order to rapidly advance a second-generation NS5A inhibitor, ACH-3102. The efficient manner by which ACH-3102 was discovered, nominated, and ultimately prepared for clinical development is a case study in effective pharmaceutical development. Over the course of 10 months, ACH-3102 was synthesized, rigorously evaluated across the necessary nonclinical studies, manufactured in quantities sufficient for clinical development, and prepared for an IND filing, all of which was achieved by March 2012.

More broadly, the story of ACH-3102 highlights the ability and culture that exists within Achillion, which has enabled us to assess the scientific and competitive landscape and tailor our development efforts in order to advance potentially best-in-class compounds for HCV. This spirit has been cultivated over our history, and fosters a very unique environment for drug discovery and development.

Looking forward, there are three key milestones we anticipate achieving during 2012 and into 2013. With regard to ACH-1625, our belief that this protease inhibitor is potentially best-in-class is supported by (a) robust interim Phase 2 cEVR data in a tough genotype 1 patient population, (b) its safety and tolerability profile to date, (c) its activity against genotype 3, and (d) its improved barrier to resistance. During 2012, we will continue to expand on the clinical profile of ACH-1625, not only by pursuing additional dosing and development work on genotypes beyond genotype 1, but by initiating a number of drug interaction studies throughout the year. Lastly, and most importantly we will continue to evaluate creative study arrangements, alternative trial designs, and approaches that could facilitate combination development with this unique compound.

With regard to ACH-3102, we have successfully filed an IND for this compound with the FDA and look forward to initiating Phase 1 during the second quarter of 2012, with safety and proof-of-concept data to be reported during the third quarter. The Phase 1 program with ACH-3102 will also set the stage for us to begin combination development of our proprietary regimen consisting of ACH-1625 and ACH-3102 in a therapeutic trial for the treatment of genotype 1 HCV during the fourth quarter of 2012, with data to be reported beginning in 2013.

This is truly an exciting time at Achillion. As we continue to mature as an organization, with our discoveries advancing through clinical trials and the potential to help cure HCV, we thank our shareholders for their support and encouragement and look forward to sharing these significant milestones with you throughout 2012.

Sincerely,



**MICHAEL D. KISHBAUCH**  
PRESIDENT & CHIEF EXECUTIVE OFFICER

# A FUTURE OF ADVANCEMENTS



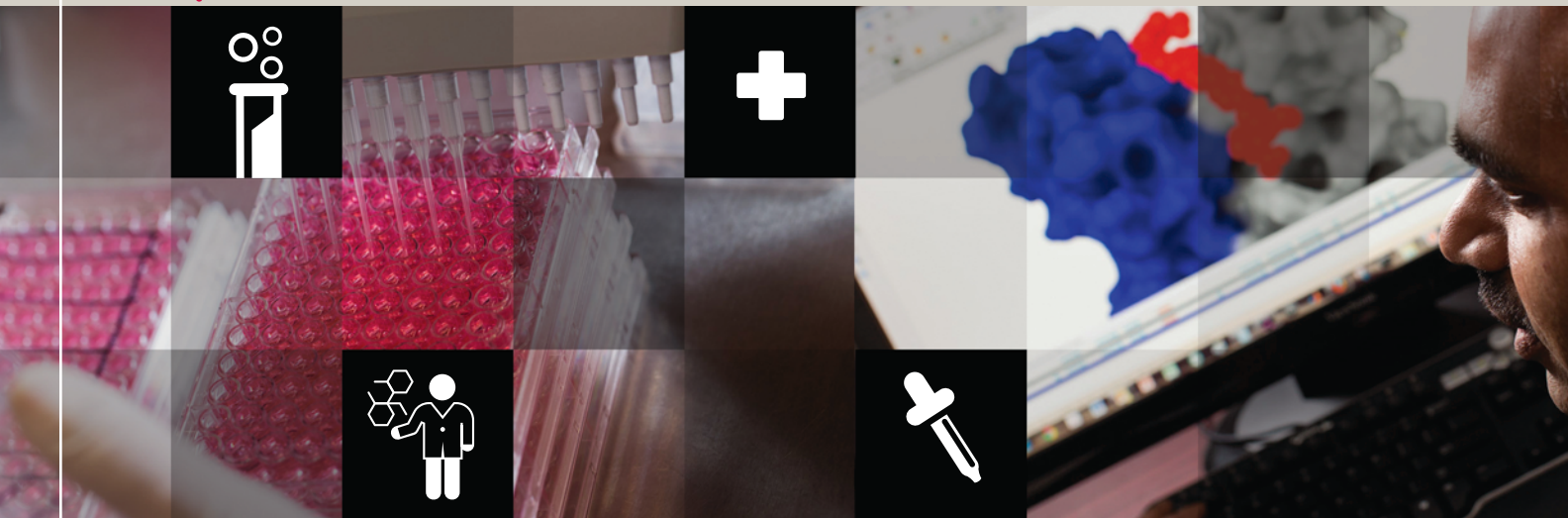
## DIVERSE PIPELINE

Achillion is working to optimize key aspects of HCV treatment by improving: efficacy, safety, dosing, combinability, pan-genotypic activity and barrier to resistance. By developing potentially best-in-class compounds, Achillion is poised to provide differentiated treatment options for HCV.



## TREATMENT RESISTANCE

A growing number of HCV patients are non-responders or resistant to currently available therapies. Achillion's compounds were designed to address this unmet medical need for an improved profile against resistant HCV mutants. An improved barrier to resistance could possibly offer improved treatment outcomes.



## COMBINING ASSETS

Achillion has proprietary compounds, which inhibit NS3 protease and NS5A. These compounds target different stages of the HCV lifecycle, affect formation of the replicase complex, and potentially suppress and eradicate HCV both in the liver and throughout the body. These assets could therefore form an optimal treatment regimen and become the cornerstone of a combination all-oral regimen to cure HCV.



## PHASE 2 NEXT-GENERATION PROTEASE INHIBITOR

ACH-1625, a pan-genotypic protease inhibitor, was evaluated in a Phase 2 clinical trial for patients with treatment-naive genotype 1 HCV for 12 weeks in combination with pegylated interferon and ribavirin. Interim results suggest that 100% of patients achieved complete early virologic response with undetectable HCV virus after 12 weeks of therapy. Additional results from this study, including sustained viral response are expected to be released throughout 2012.

**INDEPENDENT INNOVATORS, WE CONTINUE TO MAKE STRIDES IN RESEARCH & DEVELOPMENT, STRENGTHENING OUR VISIBILITY AND MARKET PRESENCE.**



### 2015: HCV CURE TO MARKET

Achillion has embarked on an aggressive, and what it believes to be achievable, development plan to advance a proprietary all-oral regimen consisting of ACH-1625 and ACH-3102 to market. A Phase 2 combination trial is scheduled to begin during the fourth quarter of 2012. Achillion expects the regimen could be approved during 2015.



### GROWING WORLDWIDE INCIDENCE

Over 170 million people are thought to be infected with HCV around the globe. In China and India, the number of people infected with HCV is estimated to be 40 million and 12 million, respectively. In the US, more than 5 million people are thought to be infected with HCV, making the disease more than twice as widespread as HIV.



### SECOND GENERATION NS5A INHIBITOR

A novel pan-genotypic NS5A inhibitor that exhibits potent activity *in vitro* against HCV was developed by Achillion. ACH-3102 is designed to maintain potency against the most common resistant mutants. Phase 1 development will begin during the second quarter of 2012 with proof-of-concept anticipated during the third quarter of this year.

**Achillion is a strong and nimble innovator with a proprietary pipeline and an experienced management team. We believe that this combination should allow us to capitalize on the dynamic HCV market and achieve the following milestones during 2012:**

#### ACH-1625

- Transition to a commercially feasible tablet formulation during 2012.
- Initiate multiple drug-drug interaction studies between ACH-1625 and methadone, atorvastatin or oral contraceptives during 2Q/3Q12.

#### ACH-3102

- Initiate Phase 1 clinical trial in healthy subjects and HCV-infected patients during 2Q12.
- Announce Phase 1 proof-of-concept results during 3Q12.

#### Combination Study

- Initiate an all-oral therapeutic trial evaluating ACH-1625 in combination with ACH-3102 in HCV genotype 1 patients in 4Q12.
- Announce proof-of-concept results during 1Q13.

# POISED TO MEET GLOBAL DEMAND



## 5 MILLION+ INFECTED IN THE US

Recent estimates suggest that more than 5 million individuals in the United States may be chronically infected with HCV. The risk for baby boomers, those born between 1946 and 1961, is even higher approaching approximately 1 in 33 individuals. Despite the high prevalence of HCV, it is estimated that 75% of patients are unaware they are infected. HCV is twice as prevalent as HIV and is responsible for more deaths than AIDS.



## STOPPING THE SPREAD

HCV is transmitted through direct contact with blood or blood products contaminated with HCV. After the discovery of HCV in 1989 routine screening began in 1992, however individuals who received a transfusion, organ transplantation or even dialysis before 1992 are at risk of being infected with HCV. Due to similar risk factors for the spread of the disease, approximately 25% of individuals diagnosed with HIV or AIDS are co-infected with HCV.



### HCV – A Global Market

Beginning in approximately 2015 when the first all-oral, interferon-free treatment regimens to treat genotype 1 HCV are expected to be approved for use, the overall HCV market will expand significantly from where it stands today. Independent models and those generated by Achillion predict that by the year 2020, peak sales of HCV therapies may exceed \$20 billion USD annually in the US and major EU nations. The substantial size of the market is expected to support a number of potential oral therapies, ranging from four to six marketed regimens, which will be used to treat HCV.

Achillion is well positioned to advance potentially best-in-class compounds, including protease inhibitors and NS5A inhibitors, that may form a cornerstone of treatment. Looking beyond the market peak, it is estimated that the demand for HCV therapies will remain strong into the year 2030, where it is estimated to be nearly three to four times the size of the market in 2010.



## NEED FOR NEW TREATMENTS

HCV is the leading cause of liver disease in the US. Currently, more than half of all patients infected with HCV will develop chronic liver disease, approximately 15% will develop cirrhosis and more than 1% of patients ultimately develop liver cancer. Liver diseases caused by HCV are the most common reason for liver transplantation in the US, and without improved screening and treatment the *Centers for Disease Control and Prevention* predict that deaths from HCV could double or triple over the coming decades.



**2011 FORM 10-K**





**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
Washington, D.C. 20549**

**FORM 10-K**

**ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the fiscal year ended December 31, 2011

OR

**TRANSITION REPORT PURSUANT TO SECTIONS 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the transition period from \_\_\_\_\_ to \_\_\_\_\_

Commission File Number 001-33095

**ACHILLION PHARMACEUTICALS, INC.**

(Exact name of registrant as specified in its charter)

**Delaware**  
(State or other jurisdiction of  
incorporation or organization)

**52-2113479**  
(I.R.S. Employer  
Identification No.)

**300 George Street, New Haven, CT 06511**  
(Address of principal executive offices) (Zip Code)

**Registrant's telephone number, including area code: (203) 724-6000**

**Securities registered pursuant to Section 12(b) of the Act:**

Title of Class

Name of Exchange on Which Registered

**Common Stock, \$0.001 par value per share**

**NASDAQ Global Market**

**Securities registered pursuant to Section 12(g) of the Act: None**

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes  No

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. Yes  No

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes  No

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§ 232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes  No

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K is not contained herein, and will not be contained, to the best of the registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K.

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, or a smaller reporting company. See definitions of "large accelerated filer," "accelerated filer," and "smaller reporting company" in Rule 12b-2 of the Exchange Act. (Check one):

Large accelerated filer   
Non-accelerated filer   
(Do not check if smaller  
reporting company)

Accelerated filer   
Smaller reporting company

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes  No

The aggregate market value of the voting stock held by non-affiliates of the Registrant on June 30, 2011 was approximately \$260,829,258 based on the closing price of such stock as reported by the NASDAQ Global Market on June 30, 2011.

As of March 1, 2012, the registrant had 70,567,538 shares of Common Stock, \$0.001 par value per share, outstanding.

**DOCUMENTS INCORPORATED BY REFERENCE**

Items 10, 11, 12, 13 and 14 of Part III (except for information required with respect to our executive officers, which is set forth under "Part I, Item 1—Business—Executive Officers of the Registrant") and the information required by Item 5 relating to our equity compensation plans have been omitted from this report, as we expect to file with the Securities and Exchange Commission, not later than 120 days after the close of our fiscal year ended December 31, 2011, a definitive proxy statement for our annual meeting of stockholders to be held on June 5, 2012. The information required by Items 10, 11, 12, 13 and 14 of Part III and the information required by Item 5 relating to our equity compensation plans, which will appear in our definitive proxy statement, are incorporated by reference into this report.

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*This Annual Report on Form 10-K contains forward-looking statements within the meaning of Section 21E of the Securities Exchange Act of 1934, as amended, that involve risks and uncertainties. All statements other than statements relating to historical matters (including statements to the effect that we “believe,” “expect,” “anticipate,” “plan,” “target,” “intend” and similar expressions) should be considered forward-looking statements. There are a number of important risks and uncertainties that could cause our actual results to differ materially from those indicated by forward-looking statements. These risks and uncertainties include those inherent in pharmaceutical research and development, such as adverse results in our drug discovery and clinical development activities, decisions made by the U.S. Food and Drug Administration and other regulatory authorities with respect to the development and commercialization of our drug candidates, our ability to obtain, maintain and enforce intellectual property rights for our drug candidates, our dependence on alliance partners, our ability to obtain any necessary financing to conduct our planned activities, and other risk factors. Please refer to the section entitled “Risk Factors” in Part I – Item 1A of this report for a description of these risks and uncertainties. Unless required by law, we assume no obligation to update these forward-looking statements to reflect events or circumstances that arise after the date hereof.*

## **PART I**

### **ITEM 1. BUSINESS**

#### **Overview**

We are a biopharmaceutical company focused on the discovery, development and commercialization of innovative treatments for infectious diseases. Within the anti-infective market, we are currently concentrating on the development of antivirals for the treatment of chronic hepatitis C infection, or HCV, and the development of antibacterials for the treatment of resistant bacterial infections. We are currently focusing our efforts on developing the following drug candidates for the treatment of HCV:

- ACH-1625, a NS3 protease inhibitor for the treatment of HCV, currently being tested in a phase IIa clinical trial;
- ACH-2684, a NS3 protease inhibitor for the treatment of HCV, currently being tested in a phase I clinical trial;
- ACH-2928, a NS5A inhibitor for the treatment of HCV, currently being tested in a phase I clinical trial; and
- ACH-3102, a NS5A inhibitor for the treatment of HCV, currently being prepared for investigational new drug, or IND, filing and initiation of a phase I clinical trial during the first half of 2012.

In addition, we have established a pipeline of certain antibacterial product candidates for which we are currently seeking appropriate collaborative partners, but to which we are not devoting significant resources at this time. These product candidates include ACH-702 for the treatment of dermatologic and ophthalmic infections and ACH-2881 for the treatment of serious resistant bacterial infections, including methicillin-resistant staphylococcus aureus, or MRSA.

We have established our current drug candidate pipeline through our internal discovery capabilities. Through these efforts we have identified and are developing the following drug candidates and programs:

- **ACH-1625, a NS3 Protease Inhibitor for Chronic HCV Infection.** We recently announced interim data from an on-going phase IIa clinical trial conducted in both the United States and Europe to assess ACH-1625’s safety, tolerability, pharmacokinetic properties and efficacy in treatment-naïve genotype 1HCV-infected subjects. In this interim analysis, ACH-1625 was demonstrated to achieve a complete early virologic response, or cEVR, in 100% of patients receiving ACH-1625 at doses of 200mg, 400mg and 800mg once-daily in combination with pegylated interferon alpha and ribavirin, or P/R. Mean viral

load, a measurement of the amount of virus in the blood stream, was reduced in HCV-infected patients by 4.59 log<sub>10</sub> to 5.12 log<sub>10</sub>, or reduction of over 99.9% of the virus. ACH-1625 continued to be safe and well-tolerated with no significant drug-related adverse events. Further, ACH-1625 was demonstrated to show efficacy in a pilot study of treatment-naïve patients infected with genotype 3 HCV. In addition, treatment with ACH-1625 has been shown to be effective in suppressing resistant variants of HCV including mutations R155, A156 and D168, viral mutations commonly associated with protease inhibitor therapy. The phase IIa clinical trial is currently being completed and full 12 week treatment data is anticipated late in the first quarter of 2012. In December 2011, ACH-1625 was granted Fast Track status by the United States Food and Drug Administration, or FDA.

- **ACH-2684, a NS3 Protease Inhibitor for Chronic HCV Infection.** In preclinical studies, ACH-2684 has demonstrated excellent potency, as well as good pharmacokinetic and safety profiles. Pharmacokinetics refers to the way in which the compound is taken into, moves through, and is eliminated from the body. The potency and virology profiles of ACH-2684 demonstrate that it effectively suppresses a broad range of natural variants of HCV, and may be effective in the prevention and treatment of emerging resistant variants. This compound also retains potent *in vitro* activity against all known HCV genotypes 1-6. In preclinical studies, ACH-2684 was effective in combination with other HCV inhibitors, and *in vitro* is synergistic with NS5B nucleoside polymerase inhibitors. We recently announced that in phase I clinical studies, ACH-2684 reduced viral load by a maximal 4.63 log<sub>10</sub> in genotype 1 HCV patients and by a maximal 2.03 log<sub>10</sub> in genotype 3 HCV patients. The compound was found to be safe and well-tolerated. This phase I clinical trial for ACH-2684 is on-going to further explore additional doses and viral pharmacokinetics.
- **ACH-2928, a NS5A Inhibitor for Chronic HCV Infection.** In preclinical studies, this compound demonstrated excellent potency against HCV replication, as well as good pharmacokinetic and safety profiles. ACH-2928 is highly active and potent against HCV genotypes 1a and 1b, as well as across other genotypes. We believe the high potency of ACH-2928, in the picomolar range, and its favorable pharmacokinetic properties, strongly suggest once-daily dosing. Importantly, NS5A inhibitors have been demonstrated in clinical trials to be highly effective in combination with NS3 protease inhibitors, and in *in vitro* studies to be highly effective in combination with NS5B polymerase inhibitors, interferon and ribavirin. In phase I clinical studies, ACH-2928 was demonstrated to reduce viral load by a maximal 4.86 log<sub>10</sub> and was safe and well-tolerated. This phase I clinical trial for ACH-2928 is on-going.
- **ACH-3102, a NS5A Inhibitor for Chronic HCV Infection.** In preclinical studies, ACH-3102 has demonstrated potent pan-genotypic activity, meaning activity against HCV subtypes referred to as genotypes 1 through 6, including excellent activity against both the 1a genotype and known mutant variants of genotype 1 HCV. We have completed IND-enabling preclinical testing for ACH-3102 and are preparing to file an IND with the FDA and to initiate phase I human clinical studies in the first half of 2012.

We were incorporated on August 17, 1998 in Delaware. Since our inception, we have spent substantial research and development funds to develop our product pipeline and expect to continue to do so in the future. We incurred approximately \$35.4, \$20.5 million and \$18.4 million in research and development costs for the years ended December 31, 2011, 2010 and 2009, respectively.

We intend to continue to focus on the discovery and development of new drug candidates through our extensive expertise in virology, microbiology and synthetic chemistry. Although significant additional funding and research and development will be required to support these efforts, we believe our drug discovery capabilities will allow us to further expand our product candidate portfolio, providing us with strong growth potential and, over time, reducing our reliance on the success of any single drug candidate.

## **Background**

Infectious diseases are caused by pathogens present in the environment, such as viruses, bacteria and fungi, which enter the body through the skin or mucous membranes and overwhelm its natural defenses. Some infections affect the entire body, while others may be localized in one organ or system within the body. The severity of infectious diseases varies depending on the nature of the infectious agent, as well as the degree to which the body's immune system can fight the infection. According to World Health Organization reports, infectious diseases, including HCV and drug-resistant bacterial infections, represent a significant cause of morbidity and mortality worldwide.

The market for anti-infective drugs can be divided into three main categories: antivirals, antibacterials (often referred to as antibiotics) and antifungals. To date, we have focused on the research and development of products for the antiviral and antibacterial markets.

The widespread use of anti-infective drugs has led to a significant reduction in morbidity and mortality associated with infectious diseases. However, for many infectious diseases, current treatment options are associated with suboptimal treatment outcomes, significant drug-related adverse side effects, complex dosing schedules and inconvenient methods of administration, such as by injection or infusion. These factors often lead to patients discontinuing treatment or failing to comply fully with treatment dosing schedules. As a result, physicians are often required to modify therapy regimens throughout the course of treatment.

Moreover, in recent years, the increasing prevalence of drug resistance has created ongoing treatment challenges for antiviral and antibacterial therapies. The ability of both viruses and bacteria to adapt rapidly to these treatments through genetic mutations allows new strains to develop that are resistant to currently available drugs. In addition, a patient's failure to comply fully with a treatment regimen both accelerates and exacerbates drug resistance.

As a result of these treatment challenges, the industry is focused on developing anti-infective drugs that delay the emergence of drug resistance, improve patient compliance and improve treatment responses in infections associated with drug-resistant pathogens.

We believe there are significant business advantages to focusing on the development of drugs to treat infectious diseases, including the following:

- the emergence of drug resistance creates a continuing need for new drugs to combat infectious diseases, thus creating a large and growing business opportunity;
- infectious disease research and development programs generally have shorter development cycle times when compared to various therapeutic areas such as oncology, cardiovascular and central nervous system disorders; and
- evidence suggests systemic anti-infectives have a higher clinical success rate compared to various therapeutic areas such as oncology, cardiovascular and central nervous system disorders.

### ***Viruses***

Viruses are submicroscopic infectious agents consisting of an outer layer of protein surrounding a core of genetic material comprised of deoxyribonucleic acid, or DNA, or ribonucleic acid, or RNA. Viruses require living host cells to grow and multiply. In many cases, the body's immune system can effectively combat the viral infection. However, in certain viral infections, the body's immune system is unable to destroy the virus, and the infection becomes chronic. In chronic infections, persistent viral replication and subsequent infection of healthy cells, over time, may lead to the deterioration or destruction of the infected cells, resulting in disease. Antiviral drugs are utilized to assist the body's immune system in combating or eliminating the infection. Reduction in viral replication as the result of anti-viral therapy slows disease progression and generally results in improved

prognosis. The effect of therapy with antiviral drugs is typically measured by the reduction in circulation of the virus in the blood stream of infected patients. In the case of HCV, the amount of viral particles in circulation is measured in log scale, wherein a reduction of over  $2 \log_{10}$  is generally equivalent to reduction of 99% of the viral RNA in a given blood sample.

The development of resistance to antiviral drugs is a major challenge for the treatment of life-threatening viral infections such as HCV. The ability of viruses to mutate spontaneously during replication allows drug-resistant viral strains to emerge when patients are on treatment regimens that do not completely inhibit viral replication. Resistance occurs because viruses continually make billions of copies of themselves, some of which will contain mutations in their genetic material. Mutations that confer a replication advantage in the presence of a suppressive antiviral drug will give rise to viral strains that are resistant or partially resistant to that antiviral drug. These mutated viruses, while initially found in low numbers, will eventually become the predominant strain in an infected patient. Once this occurs, the treatment benefit of the antiviral drug diminishes or disappears, which may result in treatment failure and create a need for an alternate therapy with new drugs.

Antiviral drug resistance is clinically managed by the administration of one or more potent direct-acting antiviral, or DAA, drugs and/or by enhancing the body's immune system through treatment with an immune response modifier to apply the highest possible level of suppression against viral replication. These direct acting antiviral drugs prevent viral replication by disrupting processes that are essential for completion of a viral infection cycle. The most effective disruption generally results from the use of multiple drugs that have different mechanisms of action.

### ***Bacteria***

Bacteria are unicellular, self-propagating microorganisms that multiply through growth in bacterial cell size and the subsequent division of the cell. Bacteria can be broadly classified into two categories based upon the composition of their cell walls: Gram-positive or Gram-negative. Many antibacterial drugs that are effective against Gram-positive bacteria are less effective or ineffective against Gram-negative bacteria, and vice versa. Antibacterial drugs that are active against a large number of both classes of bacteria are often referred to as "broad-spectrum" antibacterials.

Bacteria adapt remarkably well to their surroundings due to the high level of variation found within bacterial DNA and the ability of bacteria to reproduce rapidly. Replication of bacterial DNA is often error prone and can result in a high frequency of mutations. Because the bacterial reproductive cycle is very short, ranging from minutes to several days, a mutation that helps a bacterium survive exposure to an antibiotic drug may quickly become dominant throughout the population. Additionally, bacteria can acquire segments of DNA from other bacteria and organisms, which can also convey drug resistance.

Currently marketed antibacterials have historically proved highly successful in controlling the morbidity and mortality that accompany bacterial infections. The first antibacterials, introduced over 60 years ago, were highly effective in limiting or completely inhibiting bacterial reproduction, and thus were considered miracle drugs. A majority of the antibiotics currently in use were developed and introduced into the market before 1980. However, due to the widespread use of antibacterials over time and the ability of bacteria to develop drug resistance, many of these antibiotics now have diminished or have limited antibacterial activity. This problem is particularly acute in the hospital setting, where approximately 70% of certain types of serious infections are associated with multi-drug-resistant bacteria. The inability to effectively treat serious infections caused by drug-resistant bacteria has led to increased mortality rates, prolonged hospitalizations and increased health care costs. The rate at which bacteria are now developing resistance to multiple antibacterials, and the pace at which those multi-drug-resistant bacteria are spreading, represent significant medical challenges.

## Our Strategy

Our objective is to become a leading infectious disease-focused biopharmaceutical company. In order to achieve our objective, we intend to:

- *Advance the Development of Our HCV Drug Candidates.* In the next twelve months, we plan to:
  - continue clinical development and further characterize the attributes of ACH-1625, ACH-2684 and ACH-2928;
  - file an IND and initiate human clinical studies of ACH-3102;
  - select the most appropriate combination of NS3 protease inhibitor and NS5A inhibitor and initiate clinical testing of a proprietary combination regimen; and
  - identify and progress additional drug candidates and/or drug combinations.
- *Accelerate Growth Through Selective Collaborations.* We may establish strategic collaborations where we believe that in doing so we can accelerate the development or maximize the value of our drug candidates by (i) accessing additional drug candidates that may be combinable with our drug candidates for the future treatment of HCV infection, or (ii) utilizing the financial, clinical development, manufacturing and/or commercialization strengths of leading biotechnology, pharmaceutical companies or regional institutions. For example, in the past we have entered into collaborations with Gilead Sciences, Inc., or Gilead, to develop and commercialize certain of our HCV compounds demonstrating a mechanism of action we call NS4A antagonism, and with GCA Therapeutics Ltd., or GCAT, to develop and commercialize elvucitabine in China. We are seeking appropriate development partners for ACH-702 for dermatologic and ophthalmic uses and for ACH-2881 for serious resistant bacterial infections. We have established a subcommittee of our Board to consider and evaluate business development, financing and other strategic transactions presented to us. We may also seek to accelerate program development through affiliations with governmental, educational or other not-for-profit funding sources.
- *Expand our Infectious Disease Portfolio.* We intend to leverage our expertise in synthetic chemistry, virology and microbiology to quickly and efficiently discover and develop additional anti-infective compounds. Our research team has discovered multiple clinical candidates in multiple infectious disease programs. For example, in our HCV protease program, we discovered both ACH-1625 and ACH-2684. In our HCV NS5A program, we discovered ACH-2928 and ACH-3102. And in our antibacterial program, we discovered ACH-702 and ACH-2881.

## Our Drug Candidates

The following table summarizes key information regarding our drug candidates:

<u>Drug Candidate/ Indication</u>	<u>Mechanism</u>	<u>Stage of Development</u>	<u>Current Status</u>	<u>Current Marketing Rights</u>
<b>Core Assets:</b>				
<b>ACH-1625</b> <i>Chronic Hepatitis C Infection</i>	HCV NS3 protease inhibitor	Phase II	Phase IIa clinical trial on-going with complete 12-week data expected during the first quarter of 2012 (In December 2011, ACH-1625 was granted Fast Track status by the FDA)	Achillion
<b>ACH-2684</b> <i>Chronic Hepatitis C Infection</i>	HCV NS3 protease inhibitor	Phase I	Phase I clinical trial on-going for further dose exploration	Achillion
<b>ACH-2928</b> <i>Chronic Hepatitis C Infection</i>	HCV NS5A inhibitor	Phase I	Phase I clinical trial on-going for further dose exploration	Achillion
<b>ACH-3102</b> <i>Chronic Hepatitis C Infection</i>	HCV NS5A inhibitor	Preclinical	Preparing IND application for initiation of phase I human clinical trials during the first half of 2012	Achillion
<b>Non-Core Assets:</b>				
<b>ACH-702</b> <i>Resistant Bacterial Infections</i>	Triple target of gyrase, topoisomerase IV, and DNA primase	Preclinical	Seeking collaboration partner	Achillion
<b>ACH-2881</b> <i>Resistant Bacterial Infections</i>	Triple target of gyrase, topoisomerase IV, and DNA primase	Preclinical	Seeking collaboration partner	Achillion

## Overview of HCV Market

The hepatitis-C virus is a common cause of viral hepatitis, which leads to inflammation of the liver. HCV infection is contracted by transmission through the blood of an infected person. Hepatitis due to HCV can result in an acute process in which a person is affected for only several months and then the virus is cleared from the body. However, the Department of Health and Human Services Centers for Disease Control, or CDC, estimates that 75% to 85% of newly infected individuals become chronically infected following exposure. HCV disease progression then occurs over a period of 20 to 30 years during which patients are generally asymptomatic, meaning they exhibit no symptoms of the disease. Chronic hepatitis can lead to permanent liver damage, which can result in the development of liver cancer, liver failure or death.

Until mid-2011, the standard of care for patients with chronic HCV infection consisted of treatment with a combination of long-acting, pegylated forms of interferon alpha, a modified version of a protein that occurs naturally in the human body and boosts the immune system's ability to fight viral infection, administered through weekly injections, coupled with daily, oral doses of ribavirin, together referred to as P/R. The duration of treatment for patients infected with non-genotype 1 virus is six months and results in undetectable viral load and



normalization of liver function markers in up to 80% of patients receiving a full course of treatment. However, in individuals infected with the genotype 1 virus, the standard of care calls for 12 months of treatment and is only successful in approximately 40-50% of patients receiving a full course of treatment.

Treatment with P/R is further complicated by significant adverse side effects, including flu-like symptoms, anemia, depression, fatigue, suicidal tendencies and abnormal fetal development. Since HCV, with the exception of late-stage disease, is generally asymptomatic, the nature and extent of the treatment-related adverse side effects make patients feel sicker than they were prior to treatment. As a result of these treatment-related adverse side effects, many patients require dosage adjustments, and many may discontinue therapy altogether. In addition, current treatments are administered by injection, which is inconvenient, painful, and particularly problematic for patients who are afraid of needles.

We believe the lessons learned from the treatment of HIV infection, specifically the improved antiviral response achieved through the use of combination therapies, are relevant for the treatment of HCV due to its rapid replication and high frequency of mutations. One common approach to the discovery of new therapies to treat HCV focuses on the inhibition of viral proteins essential to the completion of the HCV replication cycle. Many drug developers have focused on three of the HCV proteins: protease or NS3, polymerase or NS5B, and more recently, another protein, NS5A. The goal of HCV drug development is to discover and develop molecules that have a high affinity for binding to these enzymes thereby inhibiting enzymatic activity and, in turn, inhibiting viral replication. Each of these inhibitor types have demonstrated in clinical trials a significant viral load reduction in infected patients. Many experts believe that these drugs, if approved, will need to be used in combination with other drugs in order to improve upon the efficacy obtained with the current standard of care.

In mid-2011, two DAA protease inhibitors were introduced to the market. These compounds, Victrelis (boceprevir) and Incivek (telaprevir), were approved only for the treatment of patients with HCV genotype 1, and are dosed in combination with P/R. This treatment regimen for HCV offers improved sustained viral response, or SVR, rates for those genotype 1 patients who can tolerate the triple combination therapy. However, a majority of individuals with HCV are unable to be treated with this regimen due to contraindications to one or more of the drugs used, such as advanced liver disease or psychiatric conditions. Further, the occurrence of side effects, both from P/R and the newly marketed DAAs, some of which can be serious and dose-limiting, combined with the inconvenient treatment regimen can result in many patients being non-compliant with their therapy or not completing therapy at all.

The less than optimal antiviral efficacy, potential for dose-limiting side effects, contraindications and inconvenient dosing regimen of the currently available P/R/DAA combination therapy illustrate the unmet medical need of the HCV patient population. Therefore, important goals for new HCV therapies are to:

- improve efficacy against the genotype 1 virus, particularly the more-challenging genotype 1a, and to develop all oral treatments for patients infected with HCV genotypes 2, 3, 4, 5 and 6;
- offer interferon-free therapies;
- offer a treatment response in patients who have failed a P/R-containing regimen;
- offer a treatment response in patients who have failed a telaprevir or boceprevir-containing regimen;
- offer therapies to which patients do not develop drug resistance;
- reduce the magnitude of treatment-related adverse side effects; and
- offer a more convenient, orally available, treatment option.

We believe our NS3 protease inhibitors can be used in combination with our NS5A inhibitors for the combination treatment of HCV patients, and that this combination therapy can address these treatment goals.

## Protease Inhibitors for Chronic Hepatitis C Infection

Our HCV protease inhibitors, ACH-1625 and ACH-2684, were discovered by our internal research team. The compounds have demonstrated strong *in vitro* potency and good safety profiles in animals. In recently announced clinical trials completed to date, the compounds have demonstrated efficacy and safety in human subjects infected with HCV.

### *Achillion Approach: HCV Protease Inhibitor ACH-1625*

We believe combination therapy for the treatment of chronic HCV infection will benefit from drugs that inhibit HCV replication through complementary mechanisms of action. Therefore, we have leveraged our experience in HCV drug discovery to identify NS3 protease inhibitors, NS4A antagonists and NS5 inhibitors that are distinct in their mechanism of action. The first of our NS3 protease inhibitors is ACH-1625.

We believe ACH-1625 has the following benefits:

- *Potency and Specificity.* Data obtained in the standard laboratory assays used to determine anti-HCV activity against the genotype 1 virus, the most common HCV virus subtype found in the United States, demonstrated that ACH-1625 has several times greater potency *in vitro* than either the Victrelis (boceprevir) or Incivek (telaprevir), HCV protease inhibitors recently approved. In addition, in preclinical studies, ACH-1625 demonstrated no cross resistance with other classes of inhibitors in development, meaning that ACH-1625 could ultimately be dosed in combination with those other classes of drugs. In human clinical studies, ACH-1625 was demonstrated to reduce viral load by up to 5.12 log<sub>10</sub> and achieve 100% cEVR in patients dosed over 12 weeks in combination with P/R.
- *Safety and Tolerability.* In laboratory and animal studies, ACH-1625 has demonstrated high safety margins, meaning the amount of drug exposure in animals is many times higher than the concentrations required to inhibit the HCV virus, and has minimal dose-related side effects. In human clinical trials, ACH-1625 was demonstrated to be safe and well-tolerated over multiple dosing periods up to 12 weeks duration.
- *Durability.* A clinical virology analysis revealed that treatment with ACH-1625 does not give rise to certain viral mutations commonly seen with treatment with other protease inhibitors and patients did not demonstrate rebound of viral load or breakthrough during treatment. For this reason, we believe ACH-1625 can provide a more durable treatment option for HCV patients.
- *Pharmacokinetics.* In laboratory and animal studies, ACH-1625 is rapidly and extensively partitioned to the liver, the organ of infection in HCV. After oral dosing, the liver concentration of ACH-1625 at the twenty-four hour time point exceeds the EC<sub>50</sub> observed in the replicon assay, the standard analysis used to determine the amount of drug necessary to inhibit a viral pathogen. Based upon these data, we designed clinical trials to test once daily oral doses of ACH-1625. Clinical studies subsequently confirm that ACH-1625 can be successfully dosed once-daily.
- *Potential for Combination Treatment.* Because ACH-1625 is a member of a known and extensively studied drug class, we believe ACH-1625 is well positioned for evaluation as a treatment for HCV in combination with the current standard of care and/or in combination with other direct acting antivirals. Further, ACH-1625 demonstrates *in vitro* synergy with our NS5A compounds.

### *Clinical Development History*

In June 2009, we initiated dosing in a randomized, double-blind, placebo-controlled phase Ia/Ib clinical trial to investigate the safety, tolerability, pharmacokinetic profile and antiviral activity of ACH-1625 after single and multiple ascending oral doses in healthy volunteers and oral repeat doses for 5 days in subjects with hepatitis C infection. The trial was conducted in Europe and dosed 83 subjects, including both healthy volunteers and HCV-infected patients.

In September 2009, we announced positive results from the phase Ia, healthy subject segment of the study. Subjects in the phase Ia single ascending dose (SAD) segment of the study received single doses of ACH-1625 ranging from 50 mg to 2000 mg. Subjects in the phase Ia multiple ascending dose (MAD) segment of the study received 5 days of ACH-1625 up to a maximal dose of 2000 mg per day. Preliminary data from the SAD and MAD trial segments demonstrated ACH-1625 was well tolerated at all doses and there were no serious adverse events, no clinically significant changes in vital signs, electrocardiograms (ECGs), or laboratory evaluations. All reported adverse events were classified as mild or moderate, were transient and showed no apparent dose relationship.

In December 2009, we announced proof-of-concept data from the phase Ib segment of this study. Subjects in the first dosing cohort of HCV-infected patients received doses of 600 mg twice daily (n=9, randomized to 6 active drug, 3 placebo). Preliminary results showed that a mean reduction in viral load of 3.94 log<sub>10</sub> was achieved in the treatment group, as compared to a mean reduction of 0.22 log<sub>10</sub> in the placebo group. All subjects in the treatment group had viral load decline between 3.0 and 4.5 log<sub>10</sub>, and two subjects reached undetectable levels of HCV RNA. Safety results from this dosing group were similar to those observed in the phase Ia segment of the trial. There were no serious adverse events, no clinically significant changes in vital signs, ECGs, or laboratory evaluations. All reported adverse events were classified as mild or moderate, were transient and showed no apparent dose relationship. Furthermore, all patients had viral loads that remained suppressed for at least 7 days after dosing was completed, maintaining a mean reduction of more than 2.0 log<sub>10</sub> from baseline through day 12, the last day of viral load measurement in the study.

In January 2010, we announced additional results from the phase Ib clinical study of ACH-1625. HCV-infected subjects in this second dosing cohort (n=9, randomized to 6 active drug, 3 placebo) received doses of 500 mg twice daily of ACH-1625. Preliminary results showed that a mean reduction in viral load of 4.25 log<sub>10</sub> was achieved in the treatment group, as compared to a mean reduction of 0.29 log<sub>10</sub> in the placebo group. Safety results from this dosing group were similar to those observed in both the phase Ia segment of the trial and in the first dosing cohort of HCV-infected subjects. Sustained viral suppression was also similar to the first dosing cohort, with patients maintaining a mean reduction of more than 3.0 log<sub>10</sub> from baseline through day 12, 7 days after dosing was completed, and the last day of viral load measurement in the study. We also completed four additional dose cohorts under the protocol, examining the drug's efficacy at lower doses, without food, and once-daily. We noted similar safety and efficacy results as were found in other cohorts.

In September 2010, we initiated dosing in a phase IIa clinical study of ACH-1625 in combination with P/R. The trial is comprised of two segments, the first testing three once-daily doses of ACH-1625 over 28-days (200 mg, 400 mg or 800 mg). Subjects were randomized and stratified by IL28B genotype, including CC, which indicates a normal or expected level of response to interferon based therapies, CT and TT, which are markers of a patient's diminished response to interferon. Results from the first segment of the trial were announced in March 2011 and demonstrated that ACH-1625 reduced mean maximal viral load in patients dosed over 28 days from 4.63 log<sub>10</sub> to 4.96 log<sub>10</sub>. Safety measures were the same as those noted in previous clinical trials. In December 2011, we completed a clinical virology analysis of patient samples obtained during this trial segment, examining the resistance mutation profile following treatment. Results indicated that following 28 days of treatment with ACH-1625 the presence of highly resistant variants were not detected, particularly those at positions 155, 156 and 168, the mutations commonly seen with treatment with other protease inhibitors.

In June 2011, we initiated a second segment of this ongoing Phase IIa trial testing three doses of once-daily ACH-1625 (200 mg, 400 mg or 800 mg) in combination with P/R over 12 weeks of therapy in patients with treatment-naïve HCV genotype 1. Subjects were randomized and stratified by IL28B genotype.

In January 2012, we announced that 100% of patients who reached week 12, across all dose groups, reached an undetectable viral load, a measure called complete early virologic response, or cEVR. Further, the compound continued to be safe and well-tolerated with no serious adverse events attributed to the drug. The below table summarizes the results from this segment of the trial:

<u>Segment 2: 12-week treatment duration assessments</u>	ACH-1625		
	200 mg N=12	400 mg N=11	800 mg N=12
Week 4 RVR: Subjects with HCV RNA < 25 IU/mL . . . . .	(8/12) 67%	(8/10) <sup>1</sup> 80%	(12/12) 100%
Week 12 cEVR: Subjects with HCV RNA undetectable < 25 IU/mL . . . . .	(11/11) <sup>2</sup> 100%	(8/8) <sup>3</sup> 100%	(12/12) 100%
CC . . . . .	(4/4) 100%	(3/3) 100%	(4/4) 100%
CT or TT . . . . .	(7/7) 100%	(5/5) 100%	(8/8) 100%
Mean maximum HCV RNA decline through Week 12 (log <sub>10</sub> ) . . . .	4.79	5.12	4.59

(1) One patient discontinued before week 4. Three patients discontinued treatment after week 4 but before week 12 of treatment including (2) one patient in the 200 mg group and (3) two patients in the 400 mg dose group.

In July 2011, we also initiated a pilot study to assess the use of ACH-1625 in the treatment of patients with genotype 3 HCV infection. A total of seven patients infected with HCV genotype 3 were enrolled and treated with monotherapy consisting of 400 mg ACH-1625 twice daily for 4.5 days. In January 2012, we announced the results of this exploratory study. ACH-1625 was safe and well-tolerated and the maximum HCV genotype 3 RNA viral load reduction achieved was 3.68 log<sub>10</sub> among the six out of the seven patients that achieved an antiviral response.

These results are based on a small number of patients in an early-stage clinical trial and are not necessarily predictive of results in later-stage clinical trials with larger and more diverse patient populations.

***Achillion Approach: Protease Inhibitor ACH-2684***

In another proprietary program against HCV infection, we are developing ACH-2684, also a NS3 protease inhibitor. In preclinical studies, ACH-2684 demonstrates excellent potency in the picomolar range, as well as good pharmacokinetic and safety profiles. The compound’s profile demonstrates that it very effectively suppresses a broad range of natural variants of the hepatitis C virus, and may be effective in prevention and treatment of emerging resistant variants. Importantly, ACH-2684 retains potent activity against all genotypes in the replicon assay.

The very high potency of ACH-2684 was achieved by designing the compound to optimize the way in which it binds with NS3 protease. We have demonstrated *in vitro* that ACH-2684 can be used in combination with other HCV inhibitors, and that it is synergistic with NS5B nucleoside polymerase inhibitors and NS5A inhibitors. We believe ACH-2684 can have the following advantages:

- *Potency.* Data obtained in the standard laboratory assays used to determine anti-HCV activity against the genotype 1 virus demonstrate that ACH-2684 has potency at inhibitory concentrations less than 100 picomolar and is 3000-fold more potent than telaprevir.
- *Pan-genotypic potency.* Our *in vitro* testing indicates that ACH-2684 is potent against all genotypes of HCV virus. Our clinical testing to date has indicated that ACH-2684 is effective against genotype 1 HCV and, to a lesser degree, genotype 3 HCV. Additional dose-ranging studies are on-going to further explore and optimize the ability of ACH-2684 to address all genotypes.
- *Resistance profile.* The *in vitro* potency and virology profile of ACH-2684 demonstrates that it effectively suppresses a broad range of natural variants of the hepatitis C virus, so it may be effective in prevention and treatment of emerging resistant variants of the HCV virus including mutations R155, A156 and D168.

### ***Clinical Development History***

In July 2011, we initiated a Phase I clinical study to investigate the safety, tolerability, pharmacokinetic profile and antiviral activity of ACH-2684. We tested healthy volunteers in a single ascending dose (SAD) segment with doses ranging from 10 mg once daily to 300 mg twice daily. The first cohorts of HCV-infected patients were enrolled and treated with ACH-2684 administered as 400 mg twice daily for 2.5 days.

In January 2012, we announced that ACH-2684 was safe and well tolerated at all doses. Proof-of-concept was achieved with ACH-2684 in HCV genotype 1 demonstrating a maximum HCV RNA viral load reduction of 4.63 log<sub>10</sub>. Antiviral activity with ACH-2684 in HCV genotype 3 was seen with a maximum HCV RNA viral load reduction of 2.03 log<sub>10</sub>. Additional cohorts of patients with either HCV genotype 1 or HCV genotype 3 are currently being enrolled to further explore doses and viral kinetics for ACH-2684.

### ***Preclinical Development History***

In preclinical studies, we have demonstrated that ACH-2684 is efficacious *in vitro* against all genotypes of HCV at very low concentrations of less than 100 picomolar. In 14-day preclinical studies, ACH-2684 demonstrated high safety margins in animals with minimal dose-related side effects in both single ascending dose and multiple dose trials. The compound is metabolically stable and is rapidly and extensively partitioned in the liver, the organ of infection in HCV patients. Therefore, we believe ACH-2684 can be dosed once-daily.

### **NS5A Inhibitors for Chronic Hepatitis C Infection**

In another proprietary program against hepatitis C infection, we have discovered and developed two potent inhibitors of the HCV NS5A protein. The NS5A protein serves multiple functions at various stages of the viral life cycle including involvement in virion production, interacting with host proteins and is implicated in interferon-resistance. Inhibition of NS5A is a clinically validated mechanism of action.

#### ***Achillion Approach: NS5A Inhibitor ACH-2928***

*In vitro*, ACH-2928 demonstrates potency at picomolar concentrations in both genotypes 1a and 1b, the genotypes most prevalent in the United States. Other NS5A inhibitors have been challenged to show potency against the difficult-to-treat genotype 1a. The compound is also effective against all other known genotypes (2, 3, 4, 5 and 6.) In addition, ACH-2928 operates synergistically with both NS3 protease and NS5B polymerase inhibitors.

### ***Preclinical Development History***

The following table shows the relative potency of ACH-2928, as measured by the concentration required to effectively inhibit the virus, therefore, lower concentrations reflect great potency, compared side by side to a leading compound under clinical development in this class by Bristol-Myers-Squibb:

	<u>EC50 (pM) in Replicon Assay</u>	
	<u>Genotype 1b</u>	<u>Genotype 1a</u>
<b>ACH-2928</b> .....	2.1	46
<b>BMS-0052</b> .....	7.7	33

In 14-day preclinical studies, ACH-2928 has demonstrated high safety margins in animals with minimal dose-related side effects in both single ascending dose and multiple dose trials.

### ***Clinical Development History***

In July 2011, we initiated a Phase I clinical study to investigate the safety, tolerability, pharmacokinetic profile and antiviral activity of ACH-2928. We tested healthy volunteers in a single ascending dose (SAD) segment with doses ranging from 10 mg once daily to 500 mg once daily. During the oral repeat doses segment in subjects infected with HCV, a total of 10 patients were enrolled with 2 patients (genotype 1a) receiving placebo and 8 patients (7 genotype 1a and 1 genotype 1b) treated with 3 doses of 60 mg ACH-2928 administered once daily. No serious adverse events were reported and there were no patient discontinuations during treatment. The mean maximum HCV RNA decline during therapy was 3.68 log<sub>10</sub> compared to a 0.54 log<sub>10</sub> decline for patients receiving placebo. There were no viral breakthroughs observed during ACH-2928 monotherapy.

### ***Achillion Approach: NS5A Inhibitor ACH-3102***

In another proprietary program against hepatitis C infection, we have discovered and developed a next-generation NS5A inhibitor, ACH-3102, which demonstrates improved efficacy against HCV genotype 1a, as well as an improved resistance mutation profile.

*In vitro*, ACH-3102 demonstrates potency at picomolar concentrations in both genotypes 1a and 1b, the genotypes most prevalent in the United States. Other NS5A inhibitors have been challenged to show continued potency against the difficult-to-treat genotype 1a. The compound is also effective against all other known genotypes (2, 3, 4, 5 and 6). ACH-3102 also operates synergistically with both NS3 protease and NS5B polymerase inhibitors.

The following table shows the relative potency of ACH-3102 compared side by side to a leading compound under clinical development in this class by Bristol-Myers-Squibb:

	<b>EC50 (pM) in Replicon Assay</b>	
	<b>Genotype 1b</b>	<b>Genotype 1a</b>
<b>ACH-3102</b> .....	5.1	26
<b>BMS-0052</b> .....	2.9	60

Importantly, ACH-3102 has demonstrated ten to one hundred fold improvement in efficacy against the common resistance mutations compared to BMS-0052.

In 14-day preclinical studies, ACH-3102 has demonstrated high safety margins in animals with minimal dose-related side effects in both single ascending dose and multiple dose trials.

An IND for ACH-3102 is currently being prepared for filing and we anticipate initiation of human clinical trials in the first half of 2012.

### **ACH-702 and ACH-2881 for Drug Resistant Bacterial Infections**

ACH-702 is a preclinical candidate with potency against a broad spectrum of bacterial pathogens including methicillin-resistant staphylococcus aureus, or MRSA. We have determined that the compound is most suited for dermatologic and ophthalmic use and use in medical biofilms. Due to resource constraints, at this time, we do not anticipate moving into clinical development of ACH-702 for these indications and we do not expect to invest significantly in the future development of this compound without a collaboration partner or other external funding source.

In our research on compounds similar to ACH-702 for systemic use against MRSA, we discovered ACH-2881, a hydroxythienoquinolone, or HTQ. We believe ACH-2881 shares the potency and broad-spectrum of ACH-702, but can be dosed systemically in both IV and oral forms. In April 2010 we were awarded an SBIR grant for the further study of these compounds for the treatment of drug-resistant tuberculosis.

### ***Achillion Approach: ACH-702***

We believe ACH-702 has the following benefits:

- *Broad-Spectrum Potency.* ACH-702 has a novel target profile against bacterial DNA replication enzymes and potent broad-spectrum activity. We have established potent activity of ACH-702 against multi-drug-resistant bacteria in a laboratory evaluation of recent clinical isolates obtained from infected patients, as well as in preclinical models of infection. The spectrum of activity includes inhibition of the DNA replication enzymes: gyrase, topoisomerase IV and primase.
- *Bactericidal Mechanism of Action.* ACH-702 has demonstrated bactericidal activity against multi-drug-resistant MRSA. A number of the other drugs currently used to treat MRSA infections are bacteriostatic, meaning they are able to prevent the growth of new bacteria, but have a limited effect on the bacteria existing at the time of treatment.
- *Dosing.* We believe the properties of ACH-702 support the potential for administration through a variety of formulations.

### ***Achillion Approach: ACH-2881***

We believe ACH-2881 has the following benefits:

- *Broad-Spectrum Potency.* ACH-2881 shares ACH-702's novel target profile against bacterial DNA replication enzymes and has potent broad-spectrum activity. The compound demonstrates potent activity against multi-drug-resistant bacteria in a laboratory evaluation of recent clinical isolates obtained from infected patients, as well as in preclinical models of infection. ACH-2881 inhibits the DNA replication enzymes gyrase, topoisomerase IV and primase.
- *Bactericidal Mechanism of Action.* ACH-2881 demonstrates bactericidal activity against multi-drug-resistant MRSA as opposed to the limited bacteriostatic activity of other drugs.
- *Dosing.* We believe ACH-2881 can be dosed in both oral and IV forms to treat a number of systemic bacterial infections.

## **Drug Discovery Programs and Capabilities**

We have successfully advanced six drug candidates into human clinical trials, with three additional drug candidates in late-stage preclinical studies. We discovered seven of these eight drug candidates in house by applying our deep understanding of virology, microbiology and synthetic chemistry. We intend to continue to capitalize on our internal drug discovery and development capabilities to expand our product candidate portfolio.

From early lead identification through clinical candidate selection, we have coupled our knowledge base in genomic replication targets with an integrated drug discovery infrastructure to aid in the rapid advancement of our discovery programs.

### ***Target Selection and Assay Development***

We are focused on addressing unmet medical needs in infectious diseases, with an emphasis on inhibiting viral and bacterial proteins essential for genomic replication. We select targets for our drug discovery programs based upon the relevance of the target to key steps within the viral or bacterial replication cycle, our ability to develop appropriate assays for early assessment of potency, selectivity and safety and have confidence in our ability to identify small molecules that can be optimized within a reasonable time period to become drug candidates. We have developed proprietary assays for identification and optimization of small molecule inhibitors of viral and bacterial genomic replication.

### ***Compound Synthesis, Hit Identification and Lead Optimization***

Our focused compound library contains a diverse set of molecules that have been synthesized for the principal purpose of inhibiting genomic replication in viruses and bacteria. We have developed the following discovery tools that enable us to manage our compounds efficiently and advance our discovery programs:

- AACP (Achillion Automated Chemistry Platform) is a proprietary software that facilitates synthesis of thousands of small molecules in parallel by automating several cumbersome steps involved.
- ACE (Achillion Cheminformatics Engine) is a software interface which provides access to commercially available compound libraries and their physicochemical properties, assists in designing new compound libraries for synthesis, and displays new and database compounds in 3D. ACE is integrated with computational chemistry tools and a database of more than two million compounds.
- CART (Compound Acquisition and Repository Tracking) streamlines our scientists' ability to select and acquire compounds for lead identification.
- CHEM-ACH is a data mining software that allows analysis of Achillion's proprietary compounds and their biological activities. Such analysis helps in studying the structure-activity relationships and designing and synthesizing compounds for lead optimization.
- CIDM (Competitive Intelligence & Data Mining) is a web application. It analyzes publicly available information to display competitive information including clinical and preclinical development activities, intellectual property and scientific literature.
- PSTS (Preclinical Study Tracking System) is a web interface which is used for accessing the details of our preclinical studies. It allows scientists to enter, modify, and query preclinical study documents.

### ***Preclinical Candidate Selection***

A cornerstone of our approach to drug discovery and development is the early assessment of the drug-like properties associated with optimized lead compounds. Potency and activity against a given target are necessary but not sufficient predictors of eventual successful clinical development of a new drug. In order to perform an early assessment of the potential for successful development, prior to progression of a compound into late-stage preclinical studies in support of clinical trials, we aggressively evaluate compounds in numerous tests relating to safety, metabolism, pharmacokinetic properties and physical properties associated with the feasibility for an oral formulation.

### **Competition**

Our industry is highly competitive and subject to rapid and significant technological change. All of the drugs we are developing, if approved, would compete against existing therapies. In addition, we believe a significant number of drug candidates are currently under development and may become available for the treatment of chronic hepatitis C and bacterial infections. The key competitive factors affecting the commercial success of these drugs are likely to be efficacy, safety profile and reliability, convenience of dosing, price and reimbursement.

Many of our potential competitors, including many of the organizations named below, either alone or with their collaborative partners, have substantially greater financial, technical and human resources than we do and significantly greater experience in the discovery and development of drug candidates, obtaining FDA and other regulatory approvals of products and the commercialization of those products. Accordingly, our competitors may be more successful than we may be in obtaining FDA approval for drugs and achieving widespread market acceptance. Our competitors' drugs may be more effective, have fewer negative side effects or be more effectively marketed and sold, than any drug we may commercialize and may render our drug candidates obsolete or non-competitive before we can recover the expenses of developing and commercializing any of our drug candidates. We anticipate that we will face intense and increasing competition as new drugs enter the



market and advanced technologies become available. These organizations may also establish collaborative or licensing relationships with our competitors. Finally, the development of a cure or new treatment methods for the diseases we are targeting could render our drugs non-competitive or obsolete.

***ACH-1625, ACH-2684, ACH-2928 and ACH-3102 for HCV***

If approved, our protease inhibitors, ACH-1625 and ACH-2684, and our NS5A inhibitors, ACH-2928 and ACH-3102, would compete with drugs currently approved for the treatment of HCV, i.e., the interferon-alpha-based products from Roche (Pegasys and Roferon-A) or Merck (Intron-A or Peg-Intron), the ribavirin-based products from Merck (Rebetrol), Roche (Copegus) or generic versions sold by various companies, as well as recently-approved protease inhibitors telaprevir (Incivek) by Vertex and boceprevir (Victrelis) by Merck. In addition, our HCV compounds may compete with the interferon- and ribavirin-based drugs currently in development such as Valeant’s ribavirin analog (Viramidine) and Human Genome Sciences’ Albuferon, and with other products in development in multiple classes including protease inhibitors, polymerase inhibitors (nucleoside and non-nucleoside), NS5A inhibitors, toll-like receptor inhibitors and cyclophilin inhibitors also under development for the treatment of HCV by companies such as Abbott, Astra-Zeneca, Boehringer Ingelheim, Bristol-Myers Squibb, Celgene, Enanta, Gilead, GlaxoSmithKline, Human Genome Sciences, Idenix, Johnson & Johnson, Presidio, Medivir, Merck, Novartis, Pfizer, Roche, Valeant and Vertex.

**Intellectual Property**

Our strategy is to pursue patents, developed internally and licensed from third parties, and other means to otherwise protect our technology, inventions and improvements that are commercially important to the development of our business. We also rely on trade secrets that may be important to the development of our business.

Our success will depend significantly on our ability to:

- obtain and maintain patent and other proprietary protection for the technology, inventions and improvements we consider important to our business;
- defend and enforce our patents;
- preserve the confidentiality of our trade secrets; and
- operate without infringing the patents and proprietary rights of third parties.

We hold issued patents and pending patent applications in the United States, and in foreign countries we deem appropriate, covering intellectual property developed as part of our research and development programs.

Our hepatitis C patent portfolio currently includes the following:

	<u>Issued Patents</u>	<u>Provisional Patent Applications</u>	<u>Pending Non-Provisional Applications</u>	<u>Pending PCT Applications</u>
<b>U.S.</b> .....	10	8	13	—
<b>Foreign</b> .....	28	—	115	—

These patents and patent applications, if issued, will expire between 2024 and 2027. The patent applications contain claims directed to classes of compounds, methods of use, mechanism of action, and research assays. Our HCV patents and patent applications are filed in 25 different countries, with the majority of them in Australia, Brazil, Canada, China, Europe, Japan, New Zealand and the United States.

In connection with a 2004 license agreement with Gilead, we granted a worldwide exclusive license to Gilead for past, present and future patents, patent applications and patent filings with claims directed to our first NS4A antagonists and chemically related compounds, any additional compounds which inhibit HCV via a mechanism similar to that of NS4A antagonism and intellectual property relating to the mechanism of action. In the first quarter of 2012, that agreement was terminated and the related patents and patent rights returned to us.

In addition, we have obtained non-exclusive licenses to HCV drug discovery patents and patent applications owned by Apath, L.L.C., and ReBlikon, GmbH.

Our antibacterial patent portfolio currently includes the following:

	<u>Issued Patents</u>	<u>Provisional Patent Applications</u>	<u>Pending Non-Provisional Applications</u>	<u>Pending PCT Applications</u>
<b>U.S.</b> .....	4	—	5	—
<b>Foreign</b> .....	39	—	34	1

These patents and patent applications, if issued, will expire between 2024 and 2026. The patent applications contain claims directed to classes of compounds, methods of use, and processes for synthesis. Our antibacterial patents and patent applications are filed in 42 different countries, with the majority of them in Australia, Canada, Hong Kong, Japan, South Korea, New Zealand and the United States.

Our HIV patent portfolio currently includes the following:

	<u>Issued Patents</u>	<u>Provisional Patent Applications</u>	<u>Pending Non-Provisional Applications</u>	<u>Pending PCT Applications</u>
<b>U.S.</b> .....	8	—	—	—
<b>Foreign</b> .....	30	—	12	—

We either own or hold exclusive worldwide licenses from Yale University and Emory University to these patents and patent applications. The patents and patent applications, if issued, will expire between 2014 and 2025. The issued U.S. patents contain claims directed to elvucitabine chemical compound, method of use, synthesis, and formulation. Our HIV patents and patent applications are filed in 33 different countries with the majority of them in Australia, Brazil, Canada, Europe, India, Japan and the United States.

We rely on trade secrets to protect our technology, especially where we do not believe patent protection is appropriate or obtainable. However, trade secrets are difficult to protect. In order to protect our proprietary technology and processes, we also rely in part on confidentiality and intellectual property assignment agreements with our corporate partners, employees, consultants, outside scientific collaborators and sponsored researchers and other advisors.

We are party to a number of licenses that give us rights to third-party intellectual property that is necessary or useful for our business. In particular, we have obtained licenses from Yale University and Emory University with respect to elvucitabine. We may enter into additional licenses for third-party intellectual property in the future. Our success will depend in part on the ability of our licensors to obtain, maintain and enforce patent protection for their intellectual property, in particular, those patents to which we have secured exclusive rights.

## **Collaborations and Licenses**

### ***Gilead Sciences, Inc.***

In November 2004, we entered into a research collaboration and license agreement with Gilead pursuant to which we agreed to collaborate exclusively with Gilead throughout the world to develop and commercialize compounds for the treatment of chronic hepatitis C which inhibit HCV replication through a novel mechanism of action targeting the HCV NS4A protein. In September 2009, we and Gilead amended the license agreement so that we may continue to develop certain NS4A antagonist compounds, including ACH-1095, independently while Gilead retained the right to rejoin in the development of certain compounds after clinical proof-of-concept, as defined. In February 2012, following on-going discussions between us and Gilead, Gilead provided a notice of termination of the collaboration as neither party was devoting significant time to advancing the compounds under the agreement. We retain the right to develop ACH-1095, although we do not have current plans to do so.

### ***GCA Therapeutics, Ltd.***

In February 2010, we entered into a license agreement for elvucitabine with GCA Therapeutics, Ltd. (GCAT) for the treatment of both HBV and HIV infection. The agreement was amended and restated in March 2010. The exclusive license grants GCAT the right, through its Chinese joint venture with Tianjing Institute of Pharmaceutical Research, or TIPR, to clinically develop and commercialize elvucitabine in mainland China, Hong Kong and Taiwan. Under the terms of the agreement, GCAT, through a sublicense agreement with its Chinese joint venture, T & T Pharma Co., Ltd., formed with TIPR, will assume all development and regulatory responsibility and associated costs for elvucitabine, and Achillion will be eligible to receive development milestones and royalties on net sales in those territories.

The Agreement may be terminated by either party based upon material breaches by the other party, effective 90 days after providing written notice to the breaching party, if the breaching party fails to cure its material breach.

We may terminate the Agreement upon 30 days written notice in the event GCAT fails to meet any of the development or commercialization diligence milestones by the deadlines specified in the agreement, or upon 90 days written notice in the event of a change of corporate control. In the event of a change of control, as defined in the agreement, we will pay GCAT termination fees, in an amount determined based upon specified progress milestones.

### **Manufacturing and Supply**

We currently rely on contract manufacturers to produce drug substances and drug products required for our clinical trials under current good manufacturing practices (cGMP), with oversight by our internal managers. We plan to continue to rely upon contract manufacturers and collaboration partners to manufacture commercial quantities of our drug candidates if and when approved for marketing by the FDA. We currently rely on a limited number of manufacturers for the preclinical or clinical supplies of each of our drug candidates and do not currently have relationships for redundant supply or a second source for any of our drug candidates. We believe that there are alternate sources of supply that can satisfy our clinical trial requirements without significant delay or material additional costs.

### **Sales and Marketing**

We intend to establish our own sales and marketing capabilities if and when we obtain regulatory approval of our drug candidates. In North America and Western Europe, patients in the markets for our drug candidates are largely managed by medical specialists in the areas of infectious diseases, hepatology and gastroenterology. Historically, companies have experienced substantial commercial success through the deployment of these specialized sales forces which can address a majority of key prescribers, particularly within the infectious disease marketplace. Therefore, we expect to utilize a specialized sales force in North America for the sales and marketing of drug candidates that we may successfully develop. We currently have no marketing, sales or distribution capabilities. In order to participate in the commercialization of any of our drugs, we must develop these capabilities on our own or in collaboration with third parties. We may also choose to hire a third party to provide sales personnel instead of developing our own staff.

Outside of North America, and in situations or markets where a more favorable return may be realized through licensing commercial rights to a third party, we may license a portion or all of our commercial rights in a territory to a third party in exchange for one or more of the following: up-front payments, research funding, development funding, milestone payments and royalties on drug sales.

## **Regulatory Matters**

### ***Government Regulation and Product Approval***

Government authorities in the United States, at the federal, state and local level, and other countries extensively regulate, among other things, the research, development, testing, manufacture, labeling, record keeping, packaging, promotion, storage, advertising, distribution, marketing and export and import of products such as those we are developing. Our drugs must be approved by the FDA through the new drug application, or NDA, process before they may be legally marketed in the United States.

In the United States, drugs are subject to rigorous regulation by the FDA under the Federal Food, Drug and Cosmetic Act, or FDCA, and implementing regulations, as well as other federal and state statutes. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local, and foreign statutes and regulations require the expenditure of substantial time and financial resources. Failure to comply with the applicable United States requirements at any time during the product development process, approval process or after approval, may subject an applicant to administrative or judicial sanctions. These sanctions could include the FDA's refusal to approve pending applications, license suspension or revocation, withdrawal of an approval, a clinical hold, warning letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, civil penalties or criminal prosecution. Any agency or judicial enforcement action could have a material adverse effect on us. The process required by the FDA before a drug may be marketed in the United States generally involves the following:

- completion of preclinical laboratory tests, animal studies and formulation studies according to FDA's Good Laboratory Practice regulations;
- submission of an investigational new drug application, or IND, which must become effective before human clinical trials may begin and which must include approval by an institutional review board, or IRB, at each clinical site before the trials are initiated;
- performance of adequate and well-controlled human clinical trials according to FDA's Good Clinical Practice regulations to establish the safety and efficacy of the proposed drug for its intended use;
- submission to, and acceptance by, the FDA of an NDA;
- satisfactory completion of an FDA inspection of the manufacturing facility or facilities at which the drug is produced to assess compliance with cGMP regulations to assure that the facilities, methods and controls are adequate to preserve the drug's identity, strength, quality and purity; and
- FDA review and approval of the NDA.

### ***United States Drug Development Process***

Once a pharmaceutical candidate is identified for development it enters the preclinical testing stage. Preclinical tests include laboratory evaluations of product chemistry, toxicity and formulation, as well as animal studies. Prior to beginning human clinical trials, an IND sponsor must submit an IND to the FDA. The IND sponsor must submit the results of the preclinical tests, together with manufacturing information and analytical data, to the FDA as part of the IND. Some preclinical or nonclinical testing may continue even after the IND is submitted. In addition to including the results of the preclinical studies, the IND will also include a protocol detailing, among other things, the objectives of the first phase of the clinical trial, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated, if the first phase lends itself to an efficacy evaluation. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA, within the 30-day time period, raises concerns or questions about the conduct of the trial. In such a case, the IND

sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. The FDA may, at any time, impose a clinical hold on ongoing clinical trials. If the FDA imposes a clinical hold, clinical trials cannot commence or recommence without FDA authorization and then only under terms authorized by the FDA.

Clinical trials involve the administration of the investigational new drug to healthy volunteers or patients under the supervision of one or more qualified investigators in accordance with FDA's Good Clinical Practice regulations. Clinical trials must be conducted under protocols detailing the objectives of the trial and the safety and effectiveness criteria to be evaluated. Each protocol must be submitted to the FDA as part of the IND. Further, an institutional review board, or IRB, at each institution participating in the clinical trial must review and approve each protocol before any clinical trial commences at that institution. All research subjects must provide informed consent, and informed consent information must be submitted to the IRB for approval prior to initiation of the trial. Progress reports detailing the results of the clinical trials must be submitted at least annually to the FDA and more frequently if adverse events or other certain types of other changes occur.

Human clinical trials are typically conducted in three sequential phases that may overlap or be combined:

- *Phase I:* The drug is initially introduced into healthy human subjects or patients with the disease and tested for safety, dosage tolerance, absorption, metabolism, distribution and excretion. In the case of some products for severe or life-threatening diseases, especially when the product may be too inherently toxic to ethically administer to healthy volunteers, the initial human testing is often conducted in patients.
- *Phase II:* Involves studies in a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance and optimal dosage.
- *Phase III:* Clinical trials are undertaken to further evaluate dosage, clinical efficacy and safety in an expanded patient population, typically at geographically dispersed clinical study sites. These studies are intended to establish the overall risk-benefit ratio of the product and provide, if appropriate, an adequate basis for product labeling.

Phase I, phase II and phase III testing may not be completed successfully within any specified period, if at all. The FDA or an IRB or the sponsor may suspend a clinical trial at any time on various grounds, including a finding that the research subjects or patients are being exposed to an unacceptable health risk.

Concurrent with clinical trials, companies usually complete additional animal studies and must also develop additional information about the chemistry and physical characteristics of the drug and finalize a process for manufacturing the product in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the drug candidate and, among other requirements, the manufacturer must develop methods for testing the identity, strength, quality and purity of the final drug. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the drug candidate does not undergo unacceptable deterioration over its shelf life.

### ***United States Review and Approval Processes***

FDA approval of an NDA is required before marketing of the product may begin in the United States. The NDA must include the results of product development, preclinical studies and clinical studies, together with other detailed information, including information on the chemistry, manufacture and composition of the product. The FDA has 60 days from its receipt of the NDA to review the application to ensure that it is sufficiently complete for substantive review before accepting it for filing. The FDA may request additional information rather than accept an NDA for filing. In this event, the NDA must be resubmitted with the additional information. The resubmitted application also is subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth substantive review. The submission of an NDA is also subject to the payment of user fees; a waiver of such fees may be obtained under certain limited circumstances. Further, the

sponsor of an approved NDA is subject to annual product and establishment user fees. The approval process is lengthy and difficult and the FDA may refuse to approve an NDA if the applicable regulatory criteria are not satisfied or may require additional clinical or other data and information. Even if such data and information is submitted, the FDA may ultimately decide that the NDA does not satisfy the criteria for approval. The FDA may also refer applications for novel drug products or drug products which present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved. The FDA is not bound by the recommendation of an advisory committee. The FDA reviews an NDA to determine, among other things, whether a product is safe and effective for its intended use. Before approving an NDA, the FDA will inspect the facility or facilities where the product is manufactured to determine whether its manufacturing is cGMP-compliant to assure and preserve the product's identity, strength, quality, purity and stability.

The FDA has various programs including Fast Track, priority review and accelerated approval that are intended to expedite or simplify the process for reviewing drug candidates, and/or provide for approval on the basis of surrogate endpoints. Even if a drug candidate qualifies for one or more of these programs, the FDA may later decide that the drug candidate no longer meets the conditions for qualification or that the time period for FDA review or approval will not be shortened. Generally, drug candidates that may be eligible for these programs are those for serious or life-threatening conditions, those with the potential to address unmet medical needs or those that offer meaningful benefits over existing treatments.

Fast Track is a process designed to facilitate the development, and expedite the review of drug candidates to treat serious diseases and fill an unmet medical need. Priority review is designed to give drug candidates that offer major advances in treatment or provide a treatment where no adequate therapy exists an initial review within six months as compared to a standard review time of ten months. Although Fast Track and priority review do not affect the standards for approval, the FDA will attempt to facilitate early and frequent meetings with a sponsor of a Fast Track designated drug candidate and expedite review of the application for a drug candidate designated for priority review. Accelerated approval provides an earlier approval of drugs to treat serious diseases and that fill an unmet medical need based on a surrogate endpoint, which is a laboratory measurement or physical sign used as an indirect or substitute measurement representing a clinically meaningful outcome. As a condition of approval, the FDA may require that a sponsor of a product receiving accelerated approval perform post-marketing clinical trials.

If the FDA evaluation of the NDA and inspection of manufacturing facilities are favorable, the FDA may issue an approval letter or an approvable letter. An approvable letter generally contains a statement of specific conditions that must be met in order to secure final approval of the NDA. If and when those conditions have been met to the FDA's satisfaction, the FDA will typically issue an approval letter. An approval letter authorizes commercial marketing of the drug with specific prescribing information for a specific indication. As a condition of NDA approval, the FDA may require post approval testing, including phase IV trials, and surveillance to monitor the drug's safety or efficacy and may impose other conditions, including labeling or distribution restrictions which can materially impact the potential market and profitability of the drug. Once granted, product approvals may be withdrawn if compliance with regulatory standards is not maintained or problems are identified following initial marketing.

If the FDA's evaluation of the NDA submission or manufacturing facilities is not favorable, the FDA may refuse to approve the NDA or issue a not approvable letter. The not approvable letter outlines the deficiencies in the submission and often requires additional testing or information in order for the FDA to reconsider the application. Even after submitting this additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval. With limited exceptions, the FDA may withhold approval of a NDA regardless of prior advice it may have provided or commitments it may have made to the sponsor.

### ***Post-Approval Requirements and Considerations***

Once an approval is granted, the FDA may withdraw the approval if compliance with regulatory standards is not maintained or if problems occur after the product reaches the market. After approval, some types of changes to the approved product, such as adding new indications, manufacturing changes and additional labeling claims, are subject to further FDA review and approval. In addition, the FDA may require testing and surveillance programs to monitor the effect of approved products that have been commercialized, and in some circumstances the FDA has the power to prevent or limit further marketing of a product based on the results of these post-marketing programs.

Any drug products manufactured or distributed by us pursuant to FDA approvals are subject to continuing regulation by the FDA, including, among other things, record-keeping requirements, reporting of adverse experiences with the drug, providing the FDA with updated safety and efficacy information, drug sampling and distribution requirements, notifying the FDA and gaining its approval of certain manufacturing or labeling changes, and complying with certain electronic records and signature requirements. Certain changes to the product, its labeling or its manufacturing require prior FDA approval and may require the conduct of further clinical investigations to support the change. Such approvals may be expensive and time-consuming and, if not approved, the product will not be allowed to be marketed as modified. FDA also regulates the promotional claims that are made about prescription drug products. In particular, a drug or biologic may not be promoted for uses that are not approved by the FDA as reflected in the product's approved labeling. In addition, the FDA requires clinical substantiation of any claims of superiority of one product over another, including that such claims be proven by adequate and well-controlled head-to-head clinical trials. For anti-infective drugs, *in vitro* superiority taken alone is generally not sufficient to permit promotional claims of product superiority. To the extent that market acceptance of our products may depend on their superiority over existing therapies, any restriction on our ability to advertise or otherwise promote claims of superiority, or requirements to conduct additional expensive clinical trials to provide proof of such claims, could negatively affect the sales of our products or our costs. Drug manufacturers and their subcontractors are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMP regulations and other laws.

We rely, and expect to continue to rely, on third parties for the production of clinical and commercial quantities of our products. Future FDA and state inspections may identify compliance issues at the facilities of our contract manufacturers that may disrupt production or distribution, or require substantial resources to correct.

Once a new drug application is approved, the product covered thereby becomes a listed drug that can, in turn, be cited by potential generic competitors in support of approval of an abbreviated new drug application, or ANDA. An approved ANDA provides for marketing of a drug product that has the same active ingredients in the same strength, dosage form, and route of administration as the listed drug and has been shown through bioequivalence testing to be therapeutically equivalent to the listed drug. There is generally no requirement, other than the requirement for bioequivalence testing, for an ANDA applicant to conduct or submit results of non-clinical or clinical tests to prove the safety or effectiveness of its drug product. Drugs approved in this way are commonly referred to as generic equivalents to the listed drug, are listed as such by the FDA, and can often be substituted by pharmacists under prescriptions written for the original listed drug.

From time to time, legislation is drafted, introduced and passed in Congress that could significantly change the statutory provisions governing the approval, manufacturing and marketing of products regulated by the FDA. In addition, FDA regulations and guidance are often revised or reinterpreted by the agency in ways that may significantly affect our business and our products. It is impossible to predict whether legislative changes will be enacted, or FDA regulations, guidance or interpretations changed or what the impact of such changes, if any, may be.

### ***Foreign Regulation***

In addition to regulations in the United States, we will be subject to a variety of foreign regulations governing clinical trials and commercial sales and distribution of our products. Whether or not we obtain FDA approval for a product, we must obtain approval of a product by the comparable regulatory authorities of foreign countries before we can commence clinical trials or marketing of the product in those countries. The approval process varies from country to country and the time may be longer or shorter than that required for FDA approval. The requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary greatly from country to country.

Under European Union regulatory systems, we may submit marketing authorization applications either under a centralized or decentralized procedure. The centralized procedure, which is compulsory for medicines produced by certain biotechnological processes and optional for those which are highly innovative, provides for the grant of a single marketing authorization that is valid for all European Union member states. For drugs without approval in any Member State, the decentralized procedure provides for a member state, known as the reference member state, to assess an application, with one or more other, or concerned, member states subsequently approving that assessment. Under this procedure, an applicant submits an application, or dossier, and related materials, including a draft summary of product characteristics, draft labeling and package leaflet, to the reference member state and concerned member states. The reference member state prepares a draft assessment and drafts of the related materials within 120 days after receipt of a valid application. Within 90 days of receiving the reference member state's assessment report, each concerned member state must decide whether to approve the assessment report and related materials. If a member state cannot approve the assessment report and related materials on the grounds of potential serious risk to public health, the disputed points may eventually be referred to the European Commission, whose decision is binding on all member states.

### ***Reimbursement***

Sales of pharmaceutical products depend in significant part on the availability of third-party reimbursement. It is time consuming and expensive to seek reimbursement from third-party payors. Reimbursement may not be available or sufficient to allow us to sell our products on a competitive and profitable basis.

The passage of the Medicare Prescription Drug and Modernization Act of 2003, or the MMA, imposed new requirements for the distribution and pricing of prescription drugs for Medicare beneficiaries, which may affect the marketing of our products. The MMA also introduced a new reimbursement methodology, part of which went into effect in 2004, and a new prescription drug plan, which went into effect on January 1, 2006. While the MMA applies only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own payment rates. Any reduction in payment that results from the MMA may result in a similar reduction in payments from non-governmental payors.

In addition, in some foreign countries, the proposed pricing for a drug must be approved before it may be lawfully marketed. The requirements governing drug pricing vary widely from country to country. For example, the European Union provides options for its member states to restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. A member state may approve a specific price for the medicinal product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market.

There have been and we expect that there will continue to be frequent federal and state proposals to impose governmental pricing controls or cost containment measures for prescription drugs. While we cannot predict whether such legislative or regulatory proposals will be adopted, the adoption of such proposals could have a material adverse effect on our business, financial condition and profitability.



## Segment Reporting

We are engaged solely in the discovery and development of innovative anti-infective drug therapies. Accordingly, we have determined that we operate in one operating segment.

## Employees

As of March 1, 2012, we had 48 full-time employees and 2 part-time employees, 20 of whom hold doctoral degrees. Approximately 35 of our employees are engaged in research and development, with the remainder engaged in administration, finance and business development functions. We believe our relations with our employees are good.

## Available Information

Our internet address is [www.achillion.com](http://www.achillion.com). We are not including the information contained in our website as part of, or incorporating it by reference into, this annual report on Form 10-K. We make available free of charge through our web site our annual reports on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K and amendments to these reports filed or furnished pursuant to Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended, or the Exchange Act, as soon as reasonably practicable after we electronically file such materials with the Securities and Exchange Commission.

## Executive Officers of the Registrant

<u>Name</u>	<u>Age</u>	<u>Position</u>
Michael D. Kishbauch . . . . .	62	President and Chief Executive Officer
Milind S. Deshpande, Ph.D. . . . .	55	President of Research and Development and Chief Scientific Officer
Mary Kay Fenton . . . . .	48	Senior Vice President and Chief Financial Officer
Elizabeth A. Olek, D.O. . . . .	47	Senior Vice President and Chief Medical Officer
Gautam Shah, Ph.D. . . . .	55	Senior Vice President and Chief Compliance Officer
Joseph Truitt . . . . .	47	Senior Vice President and Chief Commercial Officer

*Michael D. Kishbauch, President and Chief Executive Officer.* Prior to joining Achillion in July 2004 as our President and Chief Executive Officer, Mr. Kishbauch founded and served as President and Chief Executive Officer of OraPharma, Inc., a publicly traded, commercial-stage pharmaceutical company focused on oral health care, from September 1996 to July 2004. OraPharma was acquired by Johnson & Johnson, a pharmaceutical company, in 2003. Prior to OraPharma, Inc., Mr. Kishbauch held senior management positions with MedImmune, Inc., a biotechnology company. Mr. Kishbauch holds an M.B.A. from the Wharton School of the University of Pennsylvania and a B.A. in biology from Wesleyan University.

*Milind S. Deshpande, Ph.D, President of Research and Development and Chief Scientific Officer.* Prior to joining Achillion in September 2001, Dr. Deshpande was Associate Director of Lead Discovery and Early Discovery Chemistry at the Pharmaceutical Research Institute at Bristol-Myers Squibb, a pharmaceutical company, from 1991 to 2001, where he managed the identification of new clinical candidates to treat infectious and neurological diseases. From 1988 to 1991, he held a faculty position at Boston University Medical School. Dr. Deshpande received his Ph.D. in Organic Chemistry from Ohio University, following his undergraduate education in India.

*Mary Kay Fenton, Senior Vice President and Chief Financial Officer.* Prior to joining Achillion in October 2000, Ms. Fenton, a certified public accountant, held various positions within the Technology Industry Group at PricewaterhouseCoopers LLP, an independent registered public accounting firm, from 1991 to 2000, most recently as Senior Manager responsible for the life sciences practice in Connecticut. Prior to 1991, Ms. Fenton

was an economic development associate in the nonprofit sector. Ms. Fenton is on the Board of Directors of Connecticut Business and Industry, a representative business organization. Ms. Fenton holds an M.B.A. in Finance from the Graduate School of Business at the University of Connecticut and an A.B. in Economics from the College of the Holy Cross.

*Elizabeth A. Olek, D.O., Senior Vice President and Chief Medical Officer.* Prior to joining Achillion in December 2007, Dr. Olek served as Global Brand Medical Director and Clinical Research Physician in the Infectious Disease, Transplant and Immunology Group at Novartis Pharmaceuticals Corporation, a pharmaceutical company, from January 2005 through November 2007. Between August and December 2004, Dr. Olek was employed as a clinical research consultant at the Avidia Research Institute, a biotechnology company. Between January 2003 and July 2004, Dr. Olek served as a Director of Clinical Research at InterMune Inc., a biotechnology company. From September 1998 through December 2002, Dr. Olek was a Director of Clinical Research at Genetics Institute/Wyeth Research, a pharmaceutical company. Dr. Olek holds an M.P.H. in epidemiology and biostatistics from the Boston University School of Public Health. She also holds a D.O. from Philadelphia College of Osteopathic Medicine and a B.S. in Pharmacy from the Philadelphia College of Pharmacy and Science.

*Gautam Shah, Ph.D., Senior Vice President and Chief Compliance Officer.* Prior to joining Achillion in May 2004, Dr. Shah was Senior Director of Regulatory Affairs with Sepracor, a pharmaceutical company, from February 2003 to May 2004. Prior to Sepracor, Dr. Shah was in the Regulatory Affairs Group of Bayer Health Care, a pharmaceutical company. Before Bayer, he held positions of increasing responsibilities at Pfizer Inc., a pharmaceutical company, in the area of Product and Process Development. Dr. Shah received his Ph.D. in Pharmaceutics from the University of Illinois, as well as a M.S. in Medicinal Chemistry from Wayne State University and a B.A. in Pharmacy from MSU University in India.

*Joseph Truitt, Senior Vice President and Chief Commercial Officer.* Prior to joining Achillion in January 2009, Mr. Truitt was Vice President of Business Development and Product Strategy for Lev Pharmaceuticals, Inc., a biotechnology company, from October 2007 to December 2008. From July 2006 through September 2007, he served as Lev's Vice President of Sales and Marketing and led the build out of the commercial team and infrastructure in preparation for product launch. From February 2002 to July 2006, Mr. Truitt was Vice President of Sales and Operations at Johnson & Johnson, a pharmaceutical company, where he directed commercial operations at the company's OraPharma subsidiary. From 2000 to 2002, Mr. Truitt was Vice President of Sales and Operations of OraPharma, Inc., a pharmaceutical company, prior to its acquisition by Johnson & Johnson. Mr. Truitt holds an M.B.A. from St. Joseph's University, Philadelphia and a B.S. in Marketing from LaSalle University, Philadelphia.

## **ITEM 1A. RISK FACTORS**

### **Risks Related to Our Business**

#### **We depend on the success of our HCV drug candidates, which are still under development.**

We have invested a significant portion of our efforts and financial resources in the development of our candidates for the treatment of HCV, including our protease inhibitors, ACH-1625 and ACH-2684, and our NS5A inhibitors, ACH-2928 and ACH-3102. Our ability to generate revenues will depend heavily on the successful development and commercialization of these drug candidates. The development and commercial success of these drug candidates will depend on several factors, including the following:

- our ability to provide acceptable evidence of the safety and efficacy of these drug candidates in current and future clinical trials;
- our ability to provide acceptable evidence of the ability of our drug candidates to be dosed safely in combination with other drugs and/or drug candidates, both ours and others;
- our ability to develop drug formulations that will deliver the appropriate drug exposures in longer term clinical trials;
- our ability to obtain patent protection for our drug candidates and freedom to operate under third party intellectual property;
- receipt of marketing approvals from the FDA and similar foreign regulatory authorities;
- establishing commercial manufacturing arrangements with third-party manufacturers;
- launching commercial sales of the drugs, whether alone or in collaboration with others;
- acceptance of the drug in the medical community and with third-party payors; and
- our ability to identify, enter into and maintain collaboration agreements with appropriate strategic partners for our compounds.

We are currently conducting a phase IIa clinical trial for ACH-1625, phase I clinical trials for ACH-2684 and ACH-2928 and IND-enabling preclinical testing of ACH-3102. Positive results in preclinical studies of a drug candidate may not be predictive of similar results in human clinical trials, and promising results from early clinical trials of a drug candidate may not be replicated in later clinical trials. A number of companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials even after achieving promising results in early-stage development. Accordingly, the results from the preclinical studies of ACH-1625, ACH-2684, ACH-2928, ACH-3102 or the completed clinical trials for ACH-1625, ACH-2684 or ACH-2928, may not be predictive of the results we may obtain in later stage trials.

We do not expect any of our drug candidates for the treatment of HCV to be commercially available for at least several years, if at all.

#### **We have a limited operating history and have incurred a cumulative loss since inception. If we do not generate significant revenues, we will not be profitable.**

We have incurred significant losses since our inception in August 1998. As of December 31, 2011, our accumulated deficit was approximately \$276 million. We have not generated any revenue from the sale of drug candidates to date. We expect that our annual operating losses will increase over the next several years as we expand our research, development and commercialization efforts.

To become profitable, we must successfully develop and obtain regulatory approval for our drug candidates and effectively manufacture, market and sell any drug candidates we develop. Accordingly, we may never generate significant revenues and, even if we do generate significant revenues, we may never achieve profitability.

**Our market is subject to intense competition. If we are unable to compete effectively, our drug candidates may be rendered noncompetitive or obsolete.**

We are engaged in a segment of the pharmaceutical industry that is highly competitive and rapidly changing. Many large pharmaceutical and biotechnology companies, academic institutions, governmental agencies and other public and private research organizations are pursuing the development of novel drugs that target infectious diseases generally and HCV in particular. We face, and expect to continue to face, intense and increasing competition as new products enter the market and advanced technologies become available. In addition to currently approved drugs, there are a significant number of drugs that are currently under development and may become available in the future for the treatment of HCV. Additionally, there may be competitive drugs currently under development of which we are not aware. We would expect our drug candidates to compete with the following approved drugs and drug candidates currently under development:

If approved, our protease inhibitors, ACH-1625 and ACH-2684, and our NS5A inhibitors, ACH-2928 and ACH-3102, would compete with drugs currently approved for the treatment of HCV, i.e., the interferon-alpha-based products from Roche (Pegasys and Roferon-A) or Merck (Intron-A or Peg-Intron), the ribavirin-based products from Merck (Rebetrol), Roche (Copegus) or generic versions sold by various companies, as well as recently-approved protease inhibitors telaprevir (Incivek) by Vertex and boceprevir (Victrelis) by Merck. In addition, our HCV compounds may compete with the interferon- and ribavirin-based drugs currently in development such as Valeant's ribavirin analog (Viramidine) and Human Genome Sciences' Albuferon, and with other products in development in multiple classes including protease inhibitors, polymerase inhibitors (nucleoside and non-nucleoside), NS5A inhibitors, toll-like receptor inhibitors and cyclophilin inhibitors also under development for the treatment of HCV by companies such as Abbott, Astra-Zeneca, Boehringer Ingelheim, Bristol-Myers Squibb, Celgene, Enanta, Gilead, GlaxoSmithKline, Human Genome Sciences, Idenix, Johnson & Johnson, Presidio, Medivir, Merck, Novartis, Pfizer, Roche, Valeant and Vertex.

Many of our competitors have:

- significantly greater financial, technical and human resources than we have and may be better equipped to discover, develop, manufacture and commercialize drug candidates;
- more extensive experience in preclinical testing and clinical trials, obtaining regulatory approvals and manufacturing and marketing pharmaceutical products;
- drug candidates that have been approved or are in late-stage clinical development; and/or
- collaborative arrangements in our target markets with leading companies and research institutions.

Competitive products, or specific classes of competitive products, may render our products obsolete or noncompetitive before we can recover the expenses of developing and commercializing our drug candidates. Furthermore, the development of new treatment methods and/or the widespread adoption or increased utilization of any vaccine for the diseases we are targeting could render our drug candidates noncompetitive, obsolete or uneconomical. If we successfully develop and obtain approval for our drug candidates, we will face competition based on the safety and effectiveness of our drug candidates, the timing of their entry into the market in relation to competitive products in development, the availability and cost of supply, marketing and sales capabilities, reimbursement coverage, price, patent position and other factors. If we successfully develop drug candidates but those drug candidates do not achieve and maintain market acceptance, our business will not be successful.

**We will need substantial additional capital to fund our operations, including drug candidate development, manufacturing and commercialization. If we do not have or cannot raise additional capital when needed, we will be unable to develop and commercialize our drug candidates successfully, and our ability to operate as a going concern may be adversely affected.**

We believe that our existing cash and cash equivalents will be sufficient to support our current operating plan for at least one year. Our operating plan may change as a result of many factors, including:

- the costs involved in the clinical development, manufacturing and formulation of our protease inhibitors, ACH-1625 and ACH-2684, and our NS5A inhibitors, ACH-2928 and ACH-3102;
- our ability to enter into corporate collaborations for our HCV candidates and the terms and success of these collaborations;
- the costs involved in obtaining regulatory approvals for our drug candidates;
- the scope, prioritization and number of programs we pursue;
- the costs involved in preparing, filing, prosecuting, maintaining, enforcing and defending patent and other intellectual property claims;
- our ability to raise incremental debt or equity capital, including any changes in the credit market that may impact our ability to obtain capital in the future;
- any partnership opportunities that may arise for ACH-702 or ACH-2881 that we determine to pursue;
- our acquisition and development of new technologies and drug candidates; and
- competing technological, regulatory and market developments currently unknown to us.

If our operating plan changes, we may need additional funds sooner than planned. Such additional financing may not be available when we need it or may not be available on terms that are favorable to us. In addition, we may seek additional capital due to favorable market conditions or strategic considerations, even if we believe we have sufficient funds for our current or future operating plans. If adequate funds are not available to us on a timely basis, or at all, we may be required to terminate or delay preclinical studies, clinical trials or other development activities for one or more of our drug candidates.

We may seek additional financing through a combination of private and public equity offerings, debt financings and collaboration, strategic alliance and licensing arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, ownership interest will be diluted, and the terms may include adverse liquidation or other preferences that adversely affect your rights as a stockholder. Since August 2008, we have issued an aggregate of 53,346,006 shares of our common stock in two private placements and two public offerings as well as warrants to purchase an aggregate of 9,599,950 shares of our common stock, the majority of which remain outstanding. These financings substantially diluted our existing stockholders.

Stockholders will be further diluted if, and to the extent, any warrants are exercised. Debt financing, if available, may involve covenants that limit or restrict our ability to take specific actions such as incurring additional debt, making capital expenditures or declaring dividends, or may involve immediate repayment of the debt under certain circumstances. If we raise additional funds through collaborations, strategic alliances and licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies or drug candidates, or grant licenses on terms that are not favorable to us.

**If we are not able to attract and retain key management, scientific personnel and advisors, we may not successfully develop our drug candidates or achieve our other business objectives.**

We depend upon our senior management and scientific staff for our business success. Key members of our senior team include Michael Kishbauch, our president and chief executive officer, and Dr. Milind Deshpande, our president of research and development and chief scientific officer. All of our employment agreements with

our senior management employees are terminable without notice by the employee. The loss of the service of any of the key members of our senior management may significantly delay or prevent the achievement of drug development and other business objectives. Our ability to attract and retain qualified personnel, consultants and advisors is critical to our success. We face intense competition for qualified individuals from numerous pharmaceutical and biotechnology companies, universities, governmental entities and other research institutions. We may be unable to attract and retain these individuals, and our failure to do so would adversely affect our business.

**Our business has a substantial risk of product liability claims. If we are unable to obtain appropriate levels of insurance, a product liability claim could adversely affect our business.**

Our business exposes us to significant potential product liability risks that are inherent in the development, manufacturing and sales and marketing of human therapeutic products. Although we do not currently commercialize any products, claims could be made against us based on the use of our drug candidates in clinical trials. Product liability claims could delay or prevent completion of our clinical development programs. We currently have clinical trial insurance in an amount equal to up to \$10.0 million in the aggregate and will seek to obtain product liability insurance prior to the sales and marketing of any of our drug candidates. However, our insurance may not provide adequate coverage against potential liabilities. Furthermore, clinical trial and product liability insurance is becoming increasingly expensive. As a result, we may be unable to maintain current amounts of insurance coverage or obtain additional or sufficient insurance at a reasonable cost to protect against losses that could have a material adverse effect on us. If a claim is brought against us, we might be required to pay legal and other expenses to defend the claim, as well as uncovered damages awards resulting from a successful claim. Furthermore, whether or not we are ultimately successful in defending any such claims, we might be required to direct significant financial and managerial resources to such defense, and adverse publicity is likely to result.

**If the estimates we make and the assumptions on which we rely in preparing our financial statements prove inaccurate, our actual results may vary significantly.**

Our financial statements have been prepared in accordance with accounting principles generally accepted in the United States. The preparation of these financial statements requires us to make estimates and judgments that affect the reported amounts of our assets, liabilities, revenues and expenses. Such estimates and judgments include revenue recognition, stock-based compensation expense, accrued expenses and deferred tax assets and liabilities. We base our estimates and judgments on historical experience and on various other assumptions that we believe to be reasonable under the circumstances. However, these estimates and judgments, or the assumptions underlying them, may change over time. Accordingly, our actual financial results may vary significantly from the estimates contained in our financial statements.

For a further discussion of the estimates and judgments that we make and the critical accounting policies that affect these estimates and judgments, see “Management’s Discussion and Analysis of Financial Condition and Results of Operations—Critical Accounting Policies and Estimates” elsewhere in this Annual Report on Form 10-K.

**Our business and operations would suffer in the event of system failures or security breaches.**

Despite the implementation of security measures, our internal computer systems are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. Any system failure, accident or security breach that causes interruptions in our operations could result in a material disruption of our product development programs. For example, the loss of clinical trial data from completed clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach results in a loss or damage to our data or applications, or inappropriate disclosure of confidential or proprietary information, we may incur liabilities and the further development of our product candidates may be delayed.

## **Risks Related to the Development of Our Drug Candidates**

**All of our drug candidates are still in the early stages of development and remain subject to clinical testing and regulatory approval. If we are unable to successfully develop, test and commercialize our drug candidates, we will not be successful.**

To date, we have not commercially marketed, distributed or sold any drug candidates. The success of our business depends primarily upon our ability to develop and commercialize our drug candidates successfully. Our drug candidates must satisfy rigorous standards of safety and efficacy before they can be approved for sale. To satisfy these standards, we must engage in expensive and lengthy testing and obtain regulatory approval of our drug candidates. Despite our efforts, our drug candidates may not:

- offer therapeutic or other improvement over existing, comparable drugs;
- be proven safe and effective in clinical trials;
- have the desired effects, or may include undesirable effects or may have other unexpected characteristics;
- meet applicable regulatory standards;
- be capable of being produced in commercial quantities at acceptable costs; or
- be successfully commercialized.

In addition, we may experience numerous unforeseen events during, or as a result of, preclinical testing and the clinical trial process that could delay or prevent our ability to receive regulatory approval or commercialize our drug candidates, including:

- regulators or Institutional Review Boards, or IRBs, may not authorize us to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- our preclinical tests or clinical trials for our drug candidates may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional preclinical testing or clinical trials, or we may abandon projects that we expect to be promising;
- enrollment in our clinical trials may be slower than we currently anticipate as potential participants have access to recently commercially launched direct acting antivirals, or DAAs, telaprevir (Incivek) or boceprevir (Victrelis), as well as other experimental therapies under development, or participants may drop out of our clinical trials at a higher rate than we currently anticipate, resulting in significant delays;
- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner;
- we might have to suspend or terminate our clinical trials if the participants are exposed to unacceptable health risks;
- IRBs or regulators, including the FDA, may require that we hold, suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements;
- the FDA may require us to carry out more extensive studies, evaluate different treatment combinations or complete comparative effectiveness studies, resulting in significant delays and/or increased costs; and
- the supply or quality of our drug candidates or other materials necessary to conduct our clinical trials may be insufficient or inadequate.

In addition, in the phase IIa clinical study currently on-going, ACH-1625 is being studied in combination with the current standard of care. Recently approved therapies, including telaprevir (Incivek) and boceprevir

(Vitreolis) could result in a change to the standard of care which may require us to carry out more extensive studies, evaluate different treatment combinations or complete comparative effectiveness studies, resulting in significant delays and/or increased costs.

We, and a number of other companies in the pharmaceutical and biotechnology industries, have suffered significant setbacks in later stage clinical trials even after achieving promising results in early-stage development.

**If we are unable to obtain U.S. and/or foreign regulatory approval, we will be unable to commercialize our drug candidates.**

Our drug candidates are subject to extensive governmental regulations relating to, among other things, research, testing, development, manufacturing, safety, efficacy, record keeping, labeling, marketing and distribution of drugs. Rigorous preclinical testing and clinical trials and an extensive regulatory approval process are required in the United States and in many foreign jurisdictions prior to the commercial sale of our drug candidates. Satisfaction of these and other regulatory requirements is costly, time consuming, uncertain and subject to unanticipated delays. It is possible that none of the drug candidates we are developing will obtain marketing approval. In connection with the clinical trials for ACH-1625, ACH-2684, ACH-2928, ACH-3102 and any other drug candidate we may seek to develop in the future, we face risks that:

- the drug candidate may not prove to be efficacious;
- the drug may not prove to be safe;
- the results may not confirm the positive results from earlier preclinical studies or clinical trials;
- the results may not meet the level of statistical significance required by the FDA or other regulatory agencies; and
- the FDA or other regulatory agencies may require us to carry out additional studies.

We have limited experience in conducting and managing the clinical trials necessary to obtain regulatory approvals, including approval by the FDA. The time required to complete clinical trials and for the FDA and other countries' regulatory review processes is uncertain and typically takes many years. Our analysis of data obtained from preclinical and clinical activities is subject to confirmation and interpretation by regulatory authorities, which could delay, limit or prevent regulatory approval. We may also encounter unanticipated delays or increased costs due to government regulation from future legislation or administrative action or changes in FDA policy during the period of product development, clinical trials and FDA regulatory review.

Any delay in obtaining or failure to obtain required approvals could materially adversely affect our ability to progress the development of a drug candidate and to generate revenues from that drug candidate. Any regulatory approval to market a product may be subject to limitations on the indicated uses for which we may market the product and affect reimbursement by third-party payors. These limitations may limit the size of the market for the product. We are also subject to numerous foreign regulatory requirements governing the conduct of clinical trials, manufacturing and marketing authorization, pricing and third-party reimbursement. The foreign regulatory approval process includes all of the risks associated with FDA approval described above as well as risks attributable to the satisfaction of foreign regulations. Approval by the FDA does not ensure approval by regulatory authorities outside the United States. Foreign jurisdictions may have different approval procedures than those required by the FDA and may impose additional testing requirements for our drug candidates.



**If clinical trials for our drug candidates are prolonged or delayed, we may be unable to commercialize our drug candidates on a timely basis, which would require us to incur additional costs and delay our receipt of any product revenue.**

We cannot predict whether we will encounter problems with any of our completed, ongoing or planned clinical trials that will cause us or regulatory authorities to delay, suspend or terminate clinical trials, or delay the analysis of data from our completed or ongoing clinical trials. Any of the following could delay the clinical development of our drug candidates:

- ongoing discussions with the FDA or comparable foreign authorities regarding the scope or design of our clinical trials;
- delays in receiving, or the inability to obtain, required approvals from institutional review boards or other reviewing entities at clinical sites selected for participation in our clinical trials;
- delays in enrolling volunteers and patients into clinical trials;
- a lower than anticipated retention rate of volunteers and patients in clinical trials;
- delays in gathering and interpreting clinical data;
- the need to repeat clinical trials as a result of inconclusive or negative results or unforeseen complications in testing;
- the requirement by the FDA, in connection with future HCV development guidelines recently circulated for comment, to carry out additional studies;
- delays in completing formulation development of our drug candidates, or delays in planning and executing the bridging studies required to use the new formulations in subsequent clinical trials;
- inadequate supply or deficient quality of drug candidate materials or other materials necessary to conduct our clinical trials;
- unfavorable FDA inspection and review of a clinical trial site or records of any clinical or preclinical investigation;
- serious and unexpected drug-related side effects experienced by participants in our clinical trials; or
- the placement by the FDA of a clinical hold on a trial.

Our ability to enroll patients in our clinical trials in sufficient numbers and on a timely basis will be subject to a number of factors, including the size of the patient population, the nature of the protocol, the existence of clinical trials for competing drugs also in clinical development, the proximity of patients to clinical sites, the availability of effective treatments for the relevant disease and the eligibility criteria for the clinical trial. Delays in patient enrollment may result in increased costs and longer development times. We currently face competition for subjects to enroll in our clinical trials and may have to expand the number of sites at which the trials are conducted. If we are not successful in doing so, the planned timing for release of data from these trials may not be achieved. In addition, subjects may drop out of our clinical trials, and thereby impair the validity or statistical significance of the trials.

We, the FDA or other applicable regulatory authorities may suspend clinical trials of a drug candidate at any time if we or they believe the subjects or patients participating in such clinical trials are being exposed to unacceptable health risks or for other reasons. For example, as we advance ACH-1625 into longer term clinical trials in phase IIa, we have established predetermined stopping rules, as well as a Data Safety Monitoring Board (DSMB) in order to monitor and ensure patient safety. The FDA has also required us to perform data analysis between patient cohorts in our phase I clinical trials of ACH-2684 and ACH-2928. Any interruption of these clinical trials, whether as a result of one of our drug candidates, or of co-administration of a concomitant anti-HCV agent, or of administrative review delays on the part of the FDA, could cause delays in our drug development.

We cannot predict whether any of our drug candidates will encounter problems during clinical trials which will cause us or regulatory authorities to delay or suspend these trials, or which will delay the analysis of data from these trials. In addition, it is impossible to predict whether legislative changes will be enacted, or whether FDA regulations, guidance or interpretations will be changed, or what the impact of such changes, if any, may be. If we experience any such problems, we may not have the financial resources to continue development of the drug candidate that is affected or the development of any of our other drug candidates.

In addition, we, along with our collaborators or subcontractors, may not employ, in any capacity, persons who have been debarred under the FDA's Application Integrity Policy. Employment of such a debarred person (even if inadvertently) may result in delays in the FDA's review or approval of our products, or the rejection of data developed with the involvement of such persons.

**Fast Track designation does not guarantee approval, or expedited approval, of ACH-1625 and there is no guarantee that ACH-1625 will maintain Fast Track designation.**

In December 2011, we announced that the FDA granted Fast Track designation to ACH-1625 for the treatment of HCV. Under the FDA Modernization Act of 1997, Fast Track designation is designed to facilitate the development and expedite the review of new drugs that are intended to treat serious or life-threatening conditions. Compounds selected must demonstrate the potential to address an unmet medical need for such a condition. Mechanisms intended to facilitate development include opportunities for frequent dialogue with FDA reviewers and for timely review of submitted protocols. However, the designation does not guarantee approval or expedited approval of any application for the product. Furthermore, the FDA may revoke Fast Track designation from a product candidate at any time if it determines that the criteria are no longer met.

**Even if we obtain regulatory approvals, our drug candidates will be subject to ongoing regulatory review. If we fail to comply with continuing U.S. and applicable foreign regulations, we could lose those approvals, and our business would be seriously harmed.**

Even if we receive regulatory approval of any drugs we are developing or may develop, we will be subject to continuing regulatory review, including the review of clinical results which are reported after our drug candidates become commercially available approved drugs. As greater numbers of patients use a drug following its approval, side effects and other problems may be observed after approval that were not seen or anticipated during pre-approval clinical trials. In addition, the manufacturer, and the manufacturing facilities we use to make any approved drugs, will also be subject to periodic review and inspection by the FDA. The subsequent discovery of previously unknown problems with the drug, manufacturer or facility may result in restrictions on the drug, manufacturer or facility, including withdrawal of the drug from the market. If we fail to comply with applicable continuing regulatory requirements, we may be subject to fines, suspension or withdrawal of regulatory approval, product recalls and seizures, operating restrictions and criminal prosecutions.

Our product promotion and advertising is also subject to regulatory requirements and continuing regulatory review. In particular, the marketing claims we will be permitted to make in labeling or advertising regarding our marketed products will be limited by the terms and conditions of the FDA-approved labeling. We must submit copies of our advertisements and promotional labeling to the FDA at the time of initial publication or dissemination. If the FDA believes these materials or statements promote our products for unapproved indications, or with unsubstantiated claims, or if we fail to provide appropriate safety-related information, the FDA could allege that our promotional activities misbrand our products. Specifically, the FDA could issue a warning letter, which may demand, among other things, that we cease such promotional activities and issue corrective advertisements and labeling. The FDA also could take enforcement action including seizure of allegedly misbranded product, injunction or criminal prosecution against us and our officers or employees. If we repeatedly or deliberately fail to submit such advertisements and labeling to the agency, the FDA could withdraw our approvals. Moreover, the Department of Justice can bring civil or criminal actions against companies that promote drugs or biologics for unapproved uses, based on the False Claims Act and other federal laws governing

reimbursement for such products under the Medicare, Medicaid and other federally supported healthcare programs. Monetary penalties in such cases have often been substantial, and civil penalties can include costly mandatory compliance programs and exclusion from federal healthcare programs.

**If we do not comply with laws regulating the protection of the environment and health and human safety, our business could be adversely affected.**

Our research and development efforts involve the controlled use of hazardous materials, chemicals and various radioactive compounds. Although we believe that our safety procedures for the use, manufacture, storage, handling and disposing of these materials comply with the standards prescribed by federal, state and local laws and regulations, the risk of accidental contamination or injury from these materials cannot be eliminated. If an accident occurs, we could be held liable for resulting damages, which could be substantial. We are also subject to numerous environmental, health and workplace safety laws and regulations, including those governing laboratory procedures, exposure to blood-borne pathogens and the handling of biohazardous materials.

Additional federal, state and local laws and regulations affecting our operations may be adopted in the future. Although we maintain workers' compensation insurance to cover us for costs we may incur due to injuries to our employees resulting from the use of these materials, this insurance may not provide adequate coverage against potential liabilities. In addition, though we have environmental liability insurance, such coverage may not provide for all related losses. We may incur substantial costs to comply with, and substantial fines or penalties, if we violate any of these laws or regulations.

**Risks Related to Our Dependence on Third Parties**

**We may not be able to execute our business strategy if we are unable to enter into alliances with other companies that can provide capabilities and funds for the development and commercialization of our drug candidates. If we are unsuccessful in forming or maintaining these alliances on favorable terms, our business may not succeed.**

We have an existing arrangement with GCAT for the development and commercialization of our HIV drug candidate, elvucitabine, in mainland China, Hong Kong, and Taiwan. We may enter into additional license arrangements in the future.

We also may enter into alliances with major biotechnology or pharmaceutical companies to jointly develop, and commercialize if approved, our protease inhibitor candidates and/or our NS5A inhibitor candidates. In such alliances, we would expect our biotechnology or pharmaceutical collaborators to provide substantial funding, as well as significant capabilities in clinical development, regulatory affairs, marketing and sales. We may not be successful in entering into any such alliances on favorable terms or in a timely manner, if at all. There are a limited number of collaboration partners whose pipeline of HCV clinical candidates are suitable for co-development with ours. There are also a limited number of potential collaboration partners without a robust HCV drug candidate pipeline, but demonstrated commercial interest in HCV therapeutics who may have interest in gaining rights to our HCV drug candidates. Recent consolidation may have reduced the number of potential partners further, making achieving a suitable partnership more difficult, potentially limiting our ability to command a significant premium in any such transaction. Further, if potential collaboration partners enter alliances with other competing HCV companies, our future business prospects may be harmed, as these alliances could reduce the pool of potential partners for our compounds and/or limit the value of such alliance.

Even if we do succeed in securing such alliances, we may not be able to maintain them if development or approval of a drug candidate is delayed or sales of an approved drug are disappointing. For example, a 2004 license and collaboration agreement between us and Gilead for the advancement of certain HCV compounds operating by the mechanism of action known as NS4A antagonism was terminated as neither party was devoting significant time to advancing the compounds under the agreement. Furthermore, any delay in entering into collaboration agreements could delay the development and commercialization of our drug candidates and reduce

their competitiveness even if they reach the market. Any such delay related to our collaborations could adversely affect our business. At this time, we do not plan to clinically advance our antibacterial drug candidates, ACH-702 and ACH-2881, independently.

**If biopharmaceutical companies involved in HCV drug development continue to consolidate, competition may increase and our business may be harmed.**

In late 2011 and early 2012, several acquisitions of smaller biopharmaceutical companies by larger biopharmaceutical companies took place at substantial premiums over the market capitalizations of the target companies, including the acquisitions of Anadys Pharmaceuticals, Pharmasset, Inc. and Inhibitex Pharmaceuticals, by Roche, Gilead and Bristol Myers Squibb, respectively. If such consolidation continues to take place, we may face competitive pressures to a far greater degree than had those consolidations not occurred, resulting from the greater resources the larger pharmaceutical companies can put toward their development pipelines. Further, if investors who provide capital to our industry continue to seek and advocate for similar acquisitions at similar premiums, we may not be able to satisfy their higher expectations for market value appreciation and our stock price may decline.

**We rely on third parties to conduct our clinical trials, and those third parties may not perform satisfactorily, including failing to meet established deadlines for the completion of such trials.**

We do not have the ability to independently conduct clinical trials for our drug candidates, and we rely on third parties such as contract research organizations, medical institutions and clinical investigators to enroll qualified patients and conduct our clinical trials. Our reliance on these third parties for clinical development activities reduces our control over these activities. These third-party contractors may not complete activities on schedule, or may not conduct our clinical trials in accordance with regulatory requirements or our trial design. To date, we believe our contract research organizations and other similar entities with which we are working have performed well. However, if these third parties do not successfully carry out their contractual duties or meet expected deadlines, we may be required to replace them. Although we believe that there are a number of other third-party contractors we could engage to continue these activities, it may result in a delay of the affected trial. Accordingly, our efforts to obtain regulatory approvals for and commercialize our drug candidates may be delayed.

**We currently depend on third-party manufacturers to produce our preclinical and clinical drug supplies and intend to rely upon third-party manufacturers to produce commercial supplies of any approved drug candidates. We also depend on third parties to assist us in developing appropriate formulations of our drug candidates. If, in the future, we manufacture any of our drug candidates, we will be required to incur significant costs and devote significant efforts to establish and maintain these capabilities.**

We rely upon third parties to produce material for preclinical and clinical testing purposes and intend to continue to do so in the future. We also depend on third parties to assist us in developing appropriate formulations of our drug candidates. We also expect to rely upon third parties to produce materials required for the commercial production of our drug candidates if we succeed in obtaining necessary regulatory approvals. If we are unable to arrange for third-party manufacturing, or to do so on commercially reasonable terms, we may not be able to complete development of our drug candidates or market them. Further, if third parties are not successful in formulation development of our drug candidates, our development timelines may be delayed. Reliance on third-party manufacturers entails risks to which we would not be subject if we manufactured drug candidates ourselves, including reliance on the third party for regulatory compliance and quality assurance, the possibility of breach of the manufacturing agreement by the third party because of factors beyond our control and the possibility of termination or nonrenewal of the agreement by the third party, based on its own business priorities, at a time that is costly or damaging to us. In addition, the FDA and other regulatory authorities require that our drug candidates be manufactured according to current good manufacturing practice regulations. Any failure by us or our third-party manufacturers to comply with current good manufacturing practices and/or our

failure to scale up our manufacturing processes could lead to a delay in, or failure to obtain, regulatory approval of any of our drug candidates. In addition, such failure could be the basis for action by the FDA to withdraw approvals for drug candidates previously granted to us and for other regulatory action.

To date, our third-party formulators and manufacturers have met our formulation and manufacturing requirements, but we cannot be assured that they will continue to do so. Any performance failure on the part of our existing or future formulators or manufacturers could delay clinical development or regulatory approval of our drug candidates or commercialization of any approved products. If for some reason our current contractors cannot perform as agreed, we may be required to replace them. Although we believe that there are a number of potential replacements given our formulation and manufacturing processes are not contractor specific, we may incur added costs and delays in identifying and qualifying any such replacements. Furthermore, although we generally do not begin a clinical trial unless we believe we have a sufficient supply of a drug candidate to complete the trial, any significant delay in the supply of a drug candidate for an ongoing trial due to the need to replace a third-party manufacturer could delay completion of the trial.

We may in the future elect to manufacture certain of our drug candidates in our own manufacturing facilities. If we do so, we will require substantial additional funds and need to recruit qualified personnel in order to build or lease and operate any manufacturing facilities.

### **Risks Related to Commercialization of Our Drug Candidates**

#### **If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell our drug candidates, we may not generate product revenue.**

We have no commercial products, and we do not currently have an organization for the sales and marketing of pharmaceutical products. In order to successfully commercialize any drugs that may be approved in the future by the FDA or comparable foreign regulatory authorities, we must build our sales and marketing capabilities or make arrangements with third parties to perform these services. For certain drug candidates in selected indications where we believe that an approved product could be commercialized by a specialty North American sales force that calls on a limited but focused group of physicians, we may commercialize these products ourselves. However, in therapeutic indications that require a large sales force selling to a large and diverse prescribing population and for markets outside of North America, we may enter into arrangements with other companies for commercialization. If we are unable to establish adequate sales, marketing and distribution capabilities, whether independently or with third parties, we may not be able to generate product revenue and may not become profitable.

#### **The development of directly acting antivirals (DAAs) to treat HCV, and the potential changes in market dynamics that may result from their introduction for HCV therapy, may present additional risks beyond those inherent in drug development.**

We are developing multiple DAA compounds, in two distinct classes, for treatment of HCV. Other companies are also developing DAAs in these classes, as well as other classes. Until the recent introduction of DAA therapy, the standard of care for HCV infection included therapy with pegylated interferon and ribavirin. Two DAAs developed by our competitors, telaprevir (Incivek) by Vertex and boceprevir (Victrelis) by Merck, were recently approved by the FDA. We cannot currently predict with any certainty the impact of the commercial launch of these compounds or any other compounds on the HCV market, although marketed DAAs may now be added to that standard regimen.

The development plans for our compounds include treatment regimens with our inhibitors in combination with another DAA, or our inhibitors with one or more DAAs with or without concomitant ribavirin therapy. These development programs carry all the risks inherent in drug development activities, including the risk that they will fail to show efficacy or acceptable safety. In addition, these development programs may also be subject to additional regulatory, commercial and manufacturing risks that may be additional to the risks inherent in drug development activities.

Regulatory guidelines for approval of DAA drugs for the treatment of HCV are evolving in the United States, Europe, and other countries. We anticipate that regulatory guidelines and regulatory agency responses to our and our competitors' development programs will continue to change, resulting in the risk that our activities may not meet unanticipated new standards or requirements, which could lead to delay, additional expense, or potential failure of development activities.

Furthermore, even if we or our competitors successfully develop DAAs whose use improves the current standard of care, current HCV-treating physicians, HCV patients, healthcare payers, and others may not readily accept or pay for such improvements or new treatments. In addition, because development of DAAs for HCV infection is an emerging field, the delay or failure of a competitor attempting to develop therapeutics that could have been combined with our product candidates or that are perceived to be similar to our product candidates could have a significant adverse effect on the commercial or regulatory environment for our product candidates or on the price of our stock. Other companies developing DAAs have more advanced development programs than we do. Their success or failure to successfully conclude clinical development and obtain marketing approval could have a material adverse effect on our development and commercialization plans and activities.

**If physicians and patients do not accept our future drugs, we may be unable to generate significant revenue, if any.**

Even if ACH-1625, ACH-2684, ACH-2928, ACH-3102 or any other drug candidates we may develop or acquire in the future obtain regulatory approval, they may not gain market acceptance among physicians, health care payors, patients and the medical community. Factors that we believe could materially affect market acceptance of our product candidates include:

- the timing of market introduction of competitive drugs, and the impact of the recent commercial launch of telaprevir (Incivek) by Vertex and boceprevir (Victrelis) by Merck;
- the demonstrated clinical safety and efficacy of our product candidates compared to other drugs and other drug candidates;
- the suitability of our drug candidates to be co-administered or combined with other drugs or drug candidates;
- the durability of our drug candidates in their ability to prevent the emergence of drug-resistant viral mutants;
- the convenience and ease of administration of our product candidates;
- the existence, prevalence and severity of adverse side effects;
- other potential advantages of alternative treatment methods;
- the effectiveness of marketing and distribution support;
- the cost-effectiveness of our product candidates; and
- the availability of reimbursement from managed care plans, the government and other third-party payors.

If our approved drugs fail to achieve market acceptance, we would not be able to generate significant revenue.

**If third-party payors do not adequately reimburse patients for any of our drug candidates that are approved for marketing, they might not be purchased or used, and our revenues and profits will not develop or increase.**

Our revenues and profits will depend significantly upon the availability of adequate reimbursement for the use of any approved drug candidates from governmental and other third-party payors, both in the United States and in foreign markets. Reimbursement by a third party may depend upon a number of factors, including the third-party payor's determination that use of a product is:

- a covered benefit under its health plan;
- safe, effective and medically necessary;
- appropriate for the specific patient;
- cost effective; and
- neither experimental nor investigational.

Obtaining reimbursement approval for a product from each third-party and government payor is a time-consuming and costly process that could require us to provide supporting scientific, clinical and cost-effectiveness data for the use of any approved drugs to each payor. We may not be able to provide data sufficient to gain acceptance with respect to reimbursement. There also exists substantial uncertainty concerning third-party reimbursement for the use of any drug candidate incorporating new technology, and even if determined eligible, coverage may be more limited than the purposes for which the drug is approved by the FDA. Moreover, eligibility for coverage does not imply that any drug will be reimbursed in all cases or at a rate that allows us to make a profit or even cover our costs. Interim payments for new products, if applicable, may also be insufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the drug and the clinical setting in which it is used, may be based on payments allowed for lower-cost products or combinations of products that are already reimbursed, may be incorporated into existing payments for other products or services, and may reflect budgetary constraints and/or imperfections in Medicare or Medicaid data used to calculate these rates. Net prices for products may be reduced by mandatory discounts or rebates required by government health care programs or by any future relaxation of laws that restrict imports of certain medical products from countries where they may be sold at lower prices than in the United States.

In the United States, at both the federal and state levels, the government regularly proposes legislation to reform health care and its cost, and such proposals have received increasing political attention. Congress recently passed legislation to reform the U.S. health care system by expanding health insurance coverage, reducing health care costs and making other changes. While health care reform may increase the number of patients who have insurance coverage for the use of any approved drug candidate, it may also include changes that adversely affect reimbursement for approved drug candidates. In addition, there has been, and we expect that there will continue to be, federal and state proposals to constrain expenditures for medical products and services, which may affect payments for any of our approved products. The Centers for Medicare and Medicaid Services frequently change product descriptors, coverage policies, product and service codes, payment methodologies and reimbursement values. Third-party payors often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates and may have sufficient market power to demand significant price reductions. As a result of actions by these third-party payors, the health care industry is experiencing a trend toward containing or reducing costs through various means, including lowering reimbursement rates, limiting therapeutic class coverage and negotiating reduced payment schedules with service providers for drug products.

Our inability to promptly obtain coverage and profitable reimbursement rates from government-funded and private payors for any approved products could have a material adverse effect on our operating results and our overall financial condition.

### **Healthcare reform measures, if implemented, could hinder or prevent our commercial success.**

There have been, and likely will continue to be, legislative and regulatory proposals at the federal and state levels directed at broadening the availability of healthcare and containing or lowering the cost of healthcare. We cannot predict the initiatives that may be adopted in the future. The continuing efforts of the government, insurance companies, managed care organizations and other payors of healthcare services to contain or reduce costs of healthcare may adversely affect:

- the demand for any drug products for which we may obtain regulatory approval;
- our ability to set a price that we believe is fair for our products;
- our ability to generate revenues and achieve or maintain profitability;
- the ability of government agencies to continue to pay for such care;
- the level of taxes that we are required to pay; and
- the availability of capital.

### **Risks Related to Patents and Licenses**

**If our patent position does not adequately protect our drug candidates, others could compete against us more directly, which would harm our business.**

We own or hold exclusive licenses to several issued patents U.S. and pending U.S. provisional and non-provisional patent applications, as well as pending PCT applications and associated non-US patents and patent applications. Our success depends in large part on our ability to obtain and maintain patent protection both in the United States and in other countries for our drug candidates. Our ability to protect our drug candidates from unauthorized or infringing use by third parties depends in substantial part on our ability to obtain and maintain valid and enforceable patents. Due to evolving legal standards relating to the patentability, validity and enforceability of patents covering pharmaceutical inventions and the scope of claims made under these patents, our ability to maintain, obtain and enforce patents is uncertain and involves complex legal and factual questions. Accordingly, rights under any issued patents may not provide us with sufficient protection for our drug candidates or provide sufficient protection to afford us a commercial advantage against competitive products or processes. We cannot guarantee that any patents will issue from any pending or future patent applications owned by or licensed to us.

Even if patents have issued or will issue, we cannot guarantee that the claims of these patents are or will be valid or enforceable or will provide us with any significant protection against competitive products or otherwise be commercially valuable to us. Patent applications in the United States are maintained in confidence for up to 18 months after their filing. In some cases, however, patent applications remain confidential in the U.S. Patent and Trademark Office, which we refer to as the U.S. Patent Office, for the entire time prior to issuance as a U.S. patent. Similarly, publication of discoveries in the scientific or patent literature often lag behind actual discoveries. Consequently, we cannot be certain that we or our licensors or co-owners were the first to invent, or the first to file patent applications on, our drug candidates or their use as anti-infective drugs. In the event that a third party has also filed a U.S. patent application relating to our drug candidates or a similar invention, we may have to participate in interference proceedings declared by the U.S. Patent Office to determine priority of invention in the United States. The costs of these proceedings could be substantial and it is possible that our efforts would be unsuccessful, resulting in a loss of our U.S. patent position. Furthermore, we may not have identified all U.S. and foreign patents or published applications that affect our business either by blocking our ability to commercialize our drugs or by covering similar technologies that affect our drug market.

The HCV inhibitor space is particularly crowded in terms of intellectual property, and we are aware that certain competitors such as Merck, Vertex, AstraZeneca, Bayer, Gilead Sciences and Bristol-Myers Squibb, have disclosed compounds that may be prior art to our patent applications and prevent issuance or alter the scope of any claims that we may pursue related to our drug candidates. For example, with regard to ACH-2928, we are



aware that this compound and closely related inhibitors have been disclosed in third party published patent applications and ultimately could be deemed to constitute prior art. These competitive activities may substantially impact our ability to obtain patent protection on our lead drug candidates and/or to commercialize such drug candidates in the absence of patent rights from one or more third parties.

The claims of the issued patents that are licensed to us, and the claims of any patents which may issue in the future and be owned by or licensed to us, may not confer on us significant commercial protection against competing products. Additionally, our patents may be challenged by third parties, resulting in the patent being deemed invalid, unenforceable or narrowed in scope, or the third party may circumvent any such issued patents. Also, our pending patent applications may not issue, and we may not receive any additional patents. Our patents might not contain claims that are sufficiently broad to prevent others from utilizing our technologies. For instance, the issued patents relating to our drug candidates may be limited to a particular molecule. Consequently, our competitors may independently develop competing products that do not infringe our patents or other intellectual property. To the extent a competitor can develop similar products using a different molecule, our patents may not prevent others from directly competing with us.

The laws of some foreign jurisdictions do not protect intellectual property rights to the same extent as in the United States and many companies have encountered significant difficulties in protecting and defending such rights in foreign jurisdictions. If we encounter such difficulties in protecting or are otherwise precluded from effectively protecting our intellectual property rights in foreign jurisdictions, our business prospects could be substantially harmed.

Because of the extensive time required for development, testing and regulatory review of a potential product, it is possible that, before any of our drug candidates can be commercialized, any related patent may expire or remain in force for only a short period following commercialization of our drug candidates, thereby reducing any advantages of the patent. To the extent our drug candidates based on that technology are not commercialized significantly ahead of the date of any applicable patent, or to the extent we have no other patent protection on such product candidates, those drug candidates would not be protected by patents, and we would then rely solely on other forms of exclusivity, such as regulatory exclusivity provided by the Federal Food, Drug and Cosmetic Act or trade secret protection.

The U.S. Congress recently passed the Leahy-Smith America Invents Act, or the America Invents Act, which was signed into law in September 2011, with many of the substantive changes becoming effective in one year or 18 months. The America Invents Act reforms United States patent law in part by changing the standard for patent approval from a “first to invent” standard to a “first to file” standard and developing a post-grant review system. This new legislation changes United States patent law in a way that may weaken our ability to obtain patent protection in the United States.

**We license patent rights from third-party owners. If such owners do not properly maintain or enforce the patents underlying such licenses, our competitive position and business prospects will be harmed.**

We are party to a number of licenses that give us rights to third-party intellectual property that is necessary or useful for our business. In particular, we have obtained licenses from Yale University and Emory University with respect to elvucitabine. We may enter into additional licenses for third-party intellectual property in the future. Our success will depend in part on the ability of our licensors to obtain, maintain and enforce patent protection for their intellectual property, in particular, those patents to which we have secured exclusive rights. Our licensors may not successfully prosecute the patent applications to which we are licensed. Even if patents issue in respect of these patent applications, our licensors may fail to maintain these patents, may determine not to pursue litigation against other companies that are infringing these patents, or may pursue such litigation less aggressively than we would. In addition, our licensors may terminate their agreements with us in the event we breach the applicable license agreement and fail to cure the breach within a specified period of time. Without protection for the intellectual property we license, other companies might be able to offer substantially identical products for sale, which could adversely affect our competitive business position and harm our business prospects.

Because our research and development of drug candidates incorporates compounds and other information that is the intellectual property of third parties, we depend on continued access to such intellectual property to conduct and complete our preclinical and clinical research and commercialize the drug candidates that result from this research. Some of our existing licenses impose, and we expect that future licenses would impose, numerous obligations on us. For example, under our existing and future license agreements, we may be required to pay minimum annual royalty amounts and/or payments upon the achievement of specified milestones. We may also be required to reimburse patent costs incurred by the licensor, or we may be obligated to pay additional royalties, at specified rates, based on net sales of our product candidates that incorporate the licensed intellectual property rights. We may also be obligated under some of these agreements to pay a percentage of any future sublicensing revenues that we may receive. Future license agreements may also include payment obligations such as milestone payments or minimum expenditures for research and development. In addition to our payment obligations under our current licenses, we are required to comply with reporting, insurance and indemnification requirements under the agreements. We expect that any future licenses would contain similar requirements.

If we fail to comply with these obligations or otherwise breach a license agreement, the licensor may have the right to terminate the license in whole, terminate the exclusive nature of the license or bring a claim against us for damages. Any such termination or claim could prevent or impede our ability to market any drug that is covered by the licensed intellectual property. Even if we contest any such termination or claim and are ultimately successful, our financial results and stock price could suffer. In addition, upon any termination of a license agreement, we may be required to grant to the licensor a license to any related intellectual property that we developed. For example, the Licensors have the right to terminate our license of the intellectual property covered by its licenses to us under certain circumstances, including our failure to make payments to the Licensor when due and our uncured breach of any other terms of the licenses. If access to such intellectual property is terminated, or becomes more expensive as a result of renegotiation of any of our existing license agreements, our ability to continue development of our product candidates or the successful commercialization of our drug candidates could be severely compromised and our business could be adversely affected.

**If we infringe or are alleged to infringe intellectual property rights of third parties, our business could be harmed.**

Our research, development and commercialization activities, including any drug candidates resulting from these activities, may infringe or be claimed to infringe patents or other proprietary rights owned by third parties and to which we do not hold licenses or other rights. There may be applications that have been filed but not published that, if issued, could be asserted against us. We are aware that certain third parties, including Bristol-Myers Squibb, Gilead, GlaxoSmithKline plc and Enanta Pharmaceuticals, Inc., have applications that are broadly directed to HCV inhibitors. Certain of these third parties, in particular Gilead and Enanta, have patent applications with pending claims that, if issued, could be construed to encompass our drug candidate, ACH-2928. These third parties could bring claims against us that would cause us to incur substantial expenses and, if successful against us, could cause us to pay substantial damages. Further, if a patent infringement suit were brought against us, we could be forced to stop or delay research, development, manufacturing or sales of the drug or drug candidate that is the subject of the suit.

As a result of intellectual property infringement claims, or in order to avoid potential claims, we may choose or be required to seek a license from the third party. These licenses may not be available on acceptable terms, or at all. Even if we are able to obtain a license, the license would likely obligate us to pay license fees or royalties or both, and the rights granted to us might be nonexclusive, which could result in our competitors gaining access to the same intellectual property. Ultimately, we could be prevented from commercializing a product, or be forced to cease some aspect of our business operations, if, as a result of actual or threatened patent infringement claims, we are unable to enter into licenses on acceptable terms. All of the issues described above could also affect our potential collaborators to the extent we have any collaborations then in place, which would also affect the success of the collaboration and therefore us.

There has been substantial litigation and other proceedings regarding patent and other intellectual property rights in the pharmaceutical and biotechnology industries. In addition to infringement claims against us, we may become a party to other patent litigation and other proceedings, including interference proceedings declared by the U. S. Patent and Trademark Office and opposition proceedings in the European Patent Office, regarding intellectual property rights with respect to our product candidates and technology. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could have a material adverse effect on our ability to compete in the marketplace.

**Litigation regarding patents, patent applications and other proprietary rights may be expensive and time consuming. If we are involved in such litigation, it could cause delays in bringing drug candidates to market and harm our ability to operate.**

Our success will depend in part on our ability to operate without infringing the proprietary rights of third parties. Although we are not currently aware of any litigation or other proceedings or third-party claims of intellectual property infringement related to our drug candidates, the pharmaceutical industry is characterized by extensive litigation regarding patents and other intellectual property rights. Other parties may obtain patents in the future and allege that the use of our technologies infringes these patent claims or that we are employing their proprietary technology without authorization. Likewise, third parties may challenge or infringe upon our existing or future patents. Under our license agreements with Yale University we have the right, but not an obligation, to bring actions against an infringing third party. If we do not bring an action within a specified number of days, the licensor may bring an action against the infringing party. Proceedings involving our patents or patent applications or those of others could result in adverse decisions regarding:

- the patentability of our inventions relating to our drug candidates; and/or
- the enforceability, validity or scope of protection offered by our patents relating to our drug candidates.

Even if we are successful in these proceedings, we may incur substantial costs and divert management time and attention in pursuing these proceedings, which could have a material adverse effect on us. If we are unable to avoid infringing the patent rights of others, we may be required to seek a license, defend an infringement action or challenge the validity of the patents in court. Patent litigation is costly and time consuming. We may not have sufficient resources to bring these actions to a successful conclusion. In addition, if we do not obtain a license, develop or obtain non-infringing technology, fail to defend an infringement action successfully or have infringed patents declared invalid, we may:

- incur substantial monetary damages;
- encounter significant delays in bringing our drug candidates to market; and/or
- be precluded from participating in the manufacture, use or sale of our drug candidates or methods of treatment requiring licenses.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. In addition, during the course of this kind of litigation, there could be public announcements of the results of hearings, motions or other interim proceedings or developments. If investors perceive these results to be negative, the market price for our common stock could be significantly harmed.

**Because of the relative weakness of the Chinese legal system in general, and the intellectual property rights in particular, we may not be able to enforce intellectual property rights in China.**

The legal regime protecting intellectual property rights in China is weak. Because the Chinese legal system in general, and the intellectual property regime in particular, are relatively weak, it is often difficult to create and enforce intellectual property rights in China. Accordingly, we may not be able to effectively protect our intellectual property rights in China under the GCAT agreement.

**We rely on our ability to stop others from competing by enforcing our patents, however some jurisdictions may require us to grant licenses to third parties. Such compulsory licenses could be extended to include some of our product candidates, which may limit our potential revenue opportunities.**

Many foreign countries, including certain countries in Europe, have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, most countries limit the enforceability of patents against government agencies or government contractors. In these countries, the patent owner may be limited to monetary relief and may be unable to enjoin infringement, which could materially diminish the value of the patent. Compulsory licensing of life-saving products is also becoming increasingly popular in developing countries, either through direct legislation or international initiatives. Such compulsory licenses could be extended to include some of our product candidates, which may limit our potential revenue opportunities.

**The rights we rely upon to protect our unpatented trade secrets may be inadequate.**

We rely on unpatented trade secrets, know-how and technology, which are difficult to protect, especially in the pharmaceutical industry, where much of the information about a product must be made public during the regulatory approval process. We seek to protect trade secrets, in part, by entering into confidentiality agreements with employees, consultants and others. These parties may breach or terminate these agreements, or may refuse to enter into such agreements with us, and we may not have adequate remedies for such breaches. Furthermore, these agreements may not provide meaningful protection for our trade secrets or other proprietary information or result in the effective assignment to us of intellectual property, and may not provide an adequate remedy in the event of unauthorized use or disclosure of confidential information or other breaches of the agreements. Despite our efforts to protect our trade secrets, we or our collaboration partners, board members, employees, consultants, contractors or scientific and other advisors may unintentionally or willfully disclose our proprietary information to competitors.

If we fail to maintain trade secret protection, our competitive position may be adversely affected. Competitors may also independently discover our trade secrets. Enforcement of claims that a third party has illegally obtained and is using trade secrets is expensive, time consuming and uncertain. If our competitors independently develop equivalent knowledge, methods and know-how, we would not be able to assert our trade secrets against them and our business could be harmed.

**Confidentiality agreements with employees and others may not adequately prevent disclosure of trade secrets and other proprietary information and may not adequately protect our intellectual property.**

We rely on trade secrets to protect our technology, especially where we do not believe patent protection is appropriate or obtainable. However, trade secrets are difficult to protect. In order to protect our proprietary technology and processes, we also rely in part on confidentiality and intellectual property assignment agreements with our corporate partners, employees, consultants, outside scientific collaborators and sponsored researchers and other advisors. These agreements may not effectively prevent disclosure of confidential information nor result in the effective assignment to us of intellectual property, and may not provide an adequate remedy in the event of unauthorized disclosure of confidential information or other breaches of the agreements. In addition, others may independently discover our trade secrets and proprietary information, and in such case we could not assert any trade secret rights against such party. Enforcing a claim that a party illegally obtained and is using our trade secrets is difficult, expensive and time consuming, and the outcome is unpredictable. In addition, courts outside the United States may be less willing to protect trade secrets. Costly and time-consuming litigation could be necessary to seek to enforce and determine the scope of our proprietary rights, and failure to obtain or maintain trade secret protection could adversely affect our competitive business position.

## **Risks Relating to Our Securities**

**We may be required to dilute our existing stockholders further in connection with capital raising activities. Additionally, the market price of our common stock may fall due to the increased number of shares available in the public market.**

In connection with capital raising activities, we may be required to dilute our existing stockholders substantially. For example, in June 2011 we issued an aggregate of 11,040,000 shares of our common stock in a public offering. In August 2010, we issued an aggregate of 19,775,101 shares of our common stock, plus common stock warrants to purchase a total of 6,921,286 additional shares of common stock in a private placement. In January and February 2010, we issued an aggregate of 11,816,250 shares of our common stock in an underwritten offering. Additionally, in August 2008, we issued 10,714,655 shares of our common stock, plus common stock warrants to purchase a total of 2,678,664 additional shares of common stock in a private placement. Stockholders will be further diluted if, and to the extent, any investors exercise their warrants. The issuance of these shares and warrants resulted in substantial dilution to stockholders who held our common stock prior to the issuance. All of the shares of common stock we issued, as well as those shares issuable upon exercise of the warrants, are freely tradable pursuant to registration statements filed with the SEC that were declared effective by the SEC on April 25, 2011, September 30, 2010, October 16, 2009 and October 30, 2008, making such shares available for immediate resale in the public market.

In addition, amounts remain available for the future issuance of common stock, preferred stock and/or warrants that we may issue from time to time under the shelf registration statement on Form S-3 that we filed in March 2011. If we issue additional securities pursuant to this shelf registration statement, these securities would be available for immediate resale in the public market.

The market price of our common stock could fall due to an increase in the number of shares available for sale in the public market.

**Our executive officers, directors and principal stockholders own a large percentage of our voting common stock and could limit our stockholders' influence on corporate decisions or could delay or prevent a change in corporate control.**

As of March 1, 2012, our directors, executive officers and current holders of more than 5% of our outstanding common stock, together with their affiliates and related persons, beneficially own, in the aggregate, approximately 48% of our outstanding common stock. As a result, these stockholders, if acting together, have the ability to determine the outcome of all matters submitted to our stockholders for approval, including the election and removal of directors and any merger, consolidation or sale of all or substantially all of our assets and other extraordinary transactions. The interests of this group of stockholders may not always coincide with our corporate interests or the interest of other stockholders, and they may act in a manner with which you may not agree or that may not be in the best interests of other stockholders. This concentration of ownership may have the effect of:

- delaying, deferring or preventing a change in control of our company;
- entrenching our management and/or board;
- impeding a merger, consolidation, takeover or other business combination involving our company; or
- discouraging a potential acquirer from making a tender offer or otherwise attempting to obtain control of our company.

**Our stock price is likely to be volatile, and the market price of our common stock may decline in value in the future.**

The market price of our common stock has fluctuated in the past and is likely to fluctuate in the future. During the period from January 1, 2007 to March 1, 2012, our stock price has ranged from a low of \$0.68 to a high of \$19.61. Market prices for securities of early stage pharmaceutical, biotechnology and other life sciences companies have historically been particularly volatile. Some of the factors that may cause the market price of our common stock to fluctuate include:

- the results of our clinical trials of our protease inhibitors, ACH-1625 and ACH-2684 and our NS5A inhibitors, ACH-2928 and ACH-3102;
- the ability of our drug candidates to be dosed safely in combination with other drugs and/or drug candidates, both ours and others;
- the entry into, modification of, or termination of key agreements, or any new collaboration agreement we may enter;
- market expectations about the timeliness of our entry into, or failure to enter, collaboration arrangements with third parties;
- the entry by a potential third-party collaborator into an alliance with a competitor, or the entry by any other HCV drug developer into an alliance that may be perceived as competitive to us;
- the continued industry consolidation of pharmaceutical companies developing HCV drug therapies, or the acquisition of any one of our HCV drug development competitors;
- the premiums on other transactions and any significant increases or decreases of those premiums;
- the results of regulatory reviews relating to the approval of our drug candidates;
- our failure to obtain patent protection for any of our drug candidates or the issuance of third party patents that cover our drug candidates;
- the initiation of, material developments in, or conclusion of litigation to enforce or defend any of our intellectual property rights;
- failure of any of our drug candidates, if approved, to achieve commercial success;
- general and industry-specific economic conditions that may affect our research and development expenditures;
- the results of clinical trials conducted by others on drugs that would compete with our drug candidates;
- the launch of drugs by others that would compete with our drug candidates;
- the failure or discontinuation of any of our research programs;
- issues in manufacturing our drug candidates or any approved products;
- the introduction of technological innovations or new commercial products by us or our competitors;
- changes in estimates or recommendations by securities analysts, if any, who cover our common stock;
- future sales of our common stock;
- changes in the structure of health care payment systems;
- period-to-period fluctuations in our financial results; and
- low trading volume of our common stock.

In addition, if we fail to reach an important research, development or commercialization milestone or result by a publicly expected deadline, even if by only a small margin, there could be significant impact on the market price of our common stock. Additionally, as we approach the announcement of important clinical data or other

significant information and as we announce such results and information, we expect the price of our common stock to be particularly volatile, and negative results would have a substantial negative impact on the price of our common stock.

The stock markets in general have experienced substantial volatility that has often been unrelated to the operating performance of individual companies. These broad market fluctuations may adversely affect the trading price of our common stock.

In the past, following periods of volatility in the market price of a company's securities, stockholders have often instituted class action securities litigation against those companies. Such litigation, if instituted, could result in substantial costs and diversion of management attention and resources, which could significantly harm our business operations and reputation.

**Unstable market and economic conditions may have serious adverse consequences on our business.**

Our general business strategy may be adversely affected by the recent economic downturn and volatile business environment and continued unpredictable and unstable market conditions. If the current equity and credit markets deteriorate further, or do not improve, it may make any necessary debt or equity financing more difficult, more costly, and more dilutive. Failure to secure any necessary financing in a timely manner and on favorable terms could have a material adverse effect on our growth strategy, financial performance and stock price and could require us to delay or abandon clinical development plans. In addition, there is a risk that one or more of our current service providers, manufacturers and other partners may not survive these difficult economic times, which would directly affect our ability to attain our operating goals on schedule and on budget.

**Our management is required to devote substantial time and incur additional expense to comply with public company regulations. Our failure to comply with such regulations could subject us to public investigations, fines, enforcement actions and other sanctions by regulatory agencies and authorities and, as a result, our stock price could decline in value.**

As a public company, the Sarbanes-Oxley Act of 2002 and the related rules and regulations of the SEC, as well as the rules of the NASDAQ Global Market, have required us to implement additional corporate governance practices and adhere to a variety of reporting requirements and complex accounting rules. Compliance with these public company obligations places significant additional demands on our limited number of finance and accounting staff and on our financial, accounting and information systems.

In particular, as a public company, our management is required to conduct an annual evaluation of our internal controls over financial reporting and include a report of management on our internal controls in our annual reports on Form 10-K. If we are unable to continue to conclude that we have effective internal controls over financial reporting or, if our independent auditors are unable to provide us with an attestation and an unqualified report as to the effectiveness of our internal controls over financial reporting, investors could lose confidence in the reliability of our financial statements, which could result in a decrease in the value of our common stock.

**We do not anticipate paying cash dividends, and accordingly stockholders must rely on stock appreciation for any return on their investment in us.**

We anticipate that we will retain our earnings, if any, for future growth and therefore do not anticipate paying cash dividends in the future. As a result, only appreciation of the price of our common stock will provide a return to stockholders.

**ITEM 1B. UNRESOLVED STAFF COMMENTS**

None.

**ITEM 2. PROPERTIES**

We currently lease approximately 32,000 square feet of laboratory and office space in New Haven, Connecticut, which we occupy under a seven-year lease expiring in 2017. We believe our existing facilities are adequate for our current needs and that additional space will be available in the future on commercially reasonable terms as needed.

**ITEM 3. LEGAL PROCEEDINGS**

None.

**ITEM 4. MINE SAFETY DISCLOSURES**

None.



## PART II

### ITEM 5. MARKET FOR REGISTRANT'S COMMON EQUITY, RELATED STOCKHOLDER MATTERS AND ISSUER PURCHASES OF EQUITY SECURITIES

#### *Market Information*

Our common stock began trading on the NASDAQ Global Market on October 26, 2006 under the symbol "ACHN". Prior to that time, there was no established public trading market for our common stock. The following table sets forth the high and low sale prices per share for our common stock on the NASDAQ Global Market for the period indicated:

	<u>High</u>	<u>Low</u>
<b>2011</b>		
First Quarter .....	\$7.50	\$3.57
Second Quarter .....	\$8.95	\$4.55
Third Quarter .....	\$8.60	\$4.50
Fourth Quarter .....	\$8.22	\$3.81
<b>2010</b>		
First Quarter .....	\$3.80	\$2.12
Second Quarter .....	\$3.30	\$1.99
Third Quarter .....	\$3.10	\$2.00
Fourth Quarter .....	\$4.20	\$2.60

Information regarding our equity compensation plans and the securities authorized for issuance thereunder is set forth in Item 12 below.

#### *Holdings of record*

As of February 29, 2012, there were approximately 100 holders of record of our common stock.

#### *Dividends*

We have never paid or declared any cash dividends on our common stock. We currently intend to retain any earnings for future growth and, therefore, do not expect to pay cash dividends in the foreseeable future.

#### *Issuer Purchases of Equity Securities*

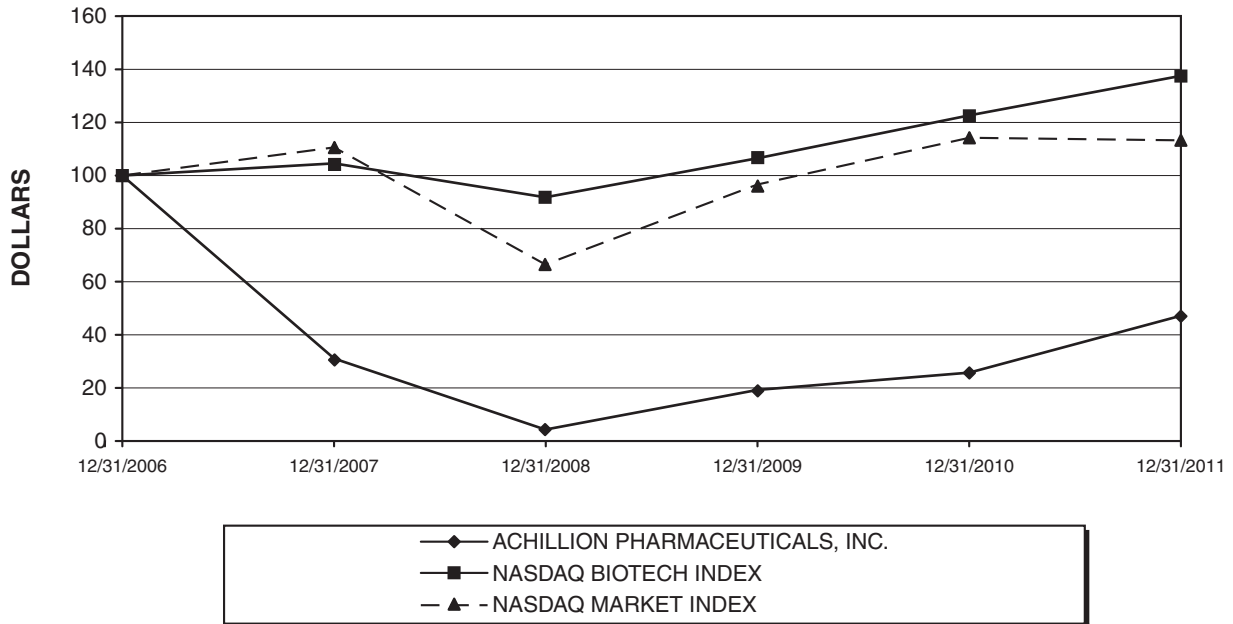
Neither we nor any affiliated purchaser or anyone acting on behalf of us or an affiliated purchaser made any purchases of shares of our common stock in the fourth quarter of 2011.

#### *Comparative Stock Performance*

The following graph and related information should not be deemed "soliciting material" or to be "filed" with the Securities and Exchange Commission, nor shall such information be incorporated by reference into any future filing under the Securities Act of 1933 or Securities Exchange Act of 1934, each as amended, except to the extent that we specifically incorporate it by reference into such filing.

The following graph compares the cumulative total stockholder return on our common stock from January 1, 2007 to December 31, 2011 with the cumulative total return of (i) the NASDAQ Market Index and (ii) the NASDAQ Biotechnology Index. This graph assumes the investment of \$100.00 on January 31, 2007 in our common stock, the NASDAQ Market Index and the NASDAQ Biotechnology Index, and assumes any dividends are reinvested.

**COMPARISON OF CUMULATIVE TOTAL RETURN  
AMONG ACHILLION PHARMACEUTICALS, INC.,  
NASDAQ MARKET INDEX AND NASDAQ BIOTECH INDEX**



ASSUMES \$100 INVESTED ON JAN. 01, 2007  
ASSUMES DIVIDEND REINVESTED  
FISCAL YEAR ENDING DEC. 31, 2011

## ITEM 6. SELECTED FINANCIAL DATA

The following selected financial data should be read together with the information under “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and our financial statements and the notes to those financial statements included elsewhere in this Annual Report on Form 10-K. The selected statements of operations data for the years ended December 31, 2011, 2010 and 2009 and balance sheet data as of December 31, 2011 and 2010 set forth below have been derived from our audited financial statements included elsewhere in this Annual Report. The selected statement of operations data for the years ended December 31, 2008 and 2007 and balance sheet data as of December 31, 2009, 2008 and 2007 set forth below have been derived from the audited financial statements for such years not included in this Annual Report. The historical results presented here are not necessarily indicative of future results.

	Years Ended December 31,				
	2011	2010	2009	2008	2007
	(in thousands, except per share amounts)				
<b>Statement of Operations Data:</b>					
Total revenue	\$ 247	\$ 2,436	\$ (294)	\$ (234)	\$ 4,038
Research and development	35,441	20,529	18,419	21,018	27,160
General and administrative	9,153	7,205	6,553	6,546	6,476
Restructuring charges	—	—	274	—	—
Total operating expenses	44,594	27,734	25,246	27,564	33,636
Loss from operations	(44,347)	(25,298)	(25,540)	(27,798)	(29,598)
Interest income (expense), net	141	(183)	(392)	(353)	1,496
Net loss	(44,206)	(25,481)	(25,932)	(28,151)	(28,102)
Net loss per share—basic and diluted	\$ (0.69)	\$ (0.57)	\$ (0.98)	\$ (1.42)	\$ (1.80)
Weighted average number of shares outstanding—basic and diluted	64,248	45,079	26,537	19,812	15,583
	<b>2011</b>	<b>2010</b>	<b>2009</b>	<b>2008</b>	<b>2007</b>
<b>Balance Sheet Data:</b>					
Cash and cash equivalents	\$ 16,110	\$ 25,373	\$ 9,712	\$ 11,060	\$ 8,971
Short-term marketable securities	37,456	29,827	—	24,297	22,138
Long-term marketable securities	26,377	—	—	—	—
Working capital	46,148	52,296	2,803	24,359	20,224
Total assets	82,630	58,235	11,670	38,561	35,632
Long-term liabilities	2,718	2,489	2,906	1,361	1,402
Total liabilities	11,662	7,691	10,648	13,540	14,094
Total stockholders’ equity	70,968	50,544	1,022	25,021	21,538

## **ITEM 7. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS**

### **Overview**

We are a biopharmaceutical company focused on the discovery, development and commercialization of innovative treatments for infectious diseases. Within the anti-infective market, we are currently concentrating on the development of antivirals for the treatment of chronic hepatitis C infection, or HCV, and the development of antibacterials for the treatment of resistant bacterial infections. We are currently focusing our efforts on developing the following drug candidates for the treatment of HCV:

- ACH-1625, a NS3 protease inhibitor for the treatment of HCV, currently being tested in a phase IIa clinical trial;
- ACH-2684, a NS3 protease inhibitor for the treatment of HCV, currently being tested in a phase I clinical trial;
- ACH-2928, a NS5A inhibitor for the treatment of HCV, currently being tested in a phase I clinical trial; and
- ACH-3102, a NS5A inhibitor for the treatment of HCV, currently being prepared for IND-filing and initiation of a phase I clinical trial during the first half of 2012.

In addition, we have established a pipeline of certain antibacterial product candidates for which we are currently seeking appropriate collaborative partners, but to which we are not devoting significant resources at this time. These product candidates include ACH-702 for the treatment of dermatologic and ophthalmic infections, and ACH-2881 for the treatment of serious resistant bacterial infections, including methicillin resistant staphylococcus aureus.

We have devoted and are continuing to devote substantially all of our efforts toward product research and development. We have incurred losses of \$262 million from inception through December 31, 2011 and had an accumulated deficit of \$276 million at December 31, 2011, which includes preferred stock dividends recognized until our initial public offering in 2006. Our net losses were \$44.2 million, \$25.5 million and \$25.9 million for the years ended December 31, 2011, 2010, and 2009, respectively. We have funded our operations primarily through:

- proceeds from the sale of equity securities, including our initial public offering in October 2006, private placements of our common stock in August 2008 and August 2010 and public offerings of our common stock in January 2010 and June 2011;
- borrowings from debt facilities; and
- receipts from up-front and milestone payments, as well as cost-sharing receipts, from our former collaboration partner, Gilead.

In January 2010, we issued 10,275,000 shares of our common stock in an underwritten public offering. In February 2010, we issued an additional 1,541,250 shares of common stock in connection with the underwriters' exercise of an over-allotment option. We received net proceeds of \$22.6 million.

In August 2010, we issued 19,775,101 shares of our common stock and warrants to purchase 6,921,286 shares of common stock in a private placement to institutional and other accredited investors. We received net proceeds of \$49.9 million.

In June 2011, we issued 11,040,000 shares of our common stock in an underwritten public offering, including the underwriters' exercise of an over-allotment option. We received net proceeds of \$60.9 million.

We expect to incur substantial and increasing losses for at least the next several years as we seek to:

- continue clinical testing of ACH-1625, ACH-2684 and ACH-2928;

- initiate clinical testing of ACH-3102; and
- identify and progress additional drug candidates.

In June 2011, at our annual meeting of stockholders, our stockholders approved an amendment to our Amended and Restated Certificate of Incorporation to increase the Company's authorized shares of common stock from 100,000,000 to 200,000,000 in order to give the Company greater flexibility in considering and planning for potential business needs.

We will need substantial additional financing to obtain regulatory approvals, fund operating losses, and, if deemed appropriate, establish manufacturing and sales and marketing capabilities, which we will seek to raise through public or private equity or debt financings, collaborative or other arrangements with third parties or through other sources of financing. There can be no assurance that such funds will be available on terms favorable to us, if at all.

In addition to the risks associated with early-stage companies, there can be no assurance that we will successfully complete our research and development, obtain adequate patent protection for our technology, obtain necessary government regulatory approval for drug candidates we develop, find and maintain appropriate collaboration partners or that any approved drug candidates will be commercially viable. In addition, we may not be profitable even if we succeed in commercializing any of our drug candidates.

## **Financial Operations Overview**

### ***Revenue***

To date, we have not generated revenue from the sale of any drugs. The majority of our revenue recognized to date has been derived from our collaboration with Gilead to develop compounds for use in treating chronic hepatitis C. During the years ended December 31, 2011, 2010 and 2009 we recognized \$247,000, \$180,000 and \$(294,000), respectively, under this collaboration agreement.

Upon initiating the collaboration with Gilead in 2004, we received a payment of \$10.0 million, which included an equity investment by Gilead determined to be worth approximately \$2.0 million. The remaining \$8.0 million is accounted for as a nonrefundable up-front fee recognized under the proportionate performance model. Revenue under the proportionate performance model is recognized as effort under the collaboration is incurred. Payments made by us to Gilead in connection with this collaboration were recognized as a reduction of revenue.

We did not recognize any revenue related to the amortization of deferred revenue during the years ended December 31, 2011, 2010 and 2009 as we were unable to accurately estimate our total performance obligations under the Gilead collaboration. Effective with the February 2012 termination of the collaboration, we will recognize the remaining \$2.5 million of deferred revenue.

We have also recognized revenue under a Small Business Innovation Research, or SBIR, grant by the National Institutes of Health, or NIH, for the further study of a back-up series of compounds related to ACH-702 for the treatment of tuberculosis infection. During the year ended December 31, 2010, we recognized revenue of \$300,000 under this grant.

Additionally, we recognized revenue related to the Qualifying Therapeutic Discovery Project program, or QTDP. The QTDP program was created by the United States Congress as part of the Patient Protection and Affordable Care Act and provided for reimbursement of certain costs paid or incurred during 2009 and 2010 directly related to the conduct of a Qualifying Therapeutic Discovery Project, as defined. The Department of Health and Human Services designated such projects based on the potential for them to result in new therapies to

treat areas of unmet medical need, the potential to create and sustain jobs in the U.S. and to advance U.S. competitiveness. During the year ended December 31, 2010, we recognized revenue of \$2.0 million related to this program.

**Research and Development**

Our research and development expenses reflect costs incurred for our proprietary research and development projects as well as costs for research and development projects conducted as part of collaborative arrangements. These costs consist primarily of salaries and benefits for our research and development personnel, costs of services by clinical research organizations, other outsourced research, materials used during research and development activities, facility-related costs such as rent and utilities associated with our laboratory and clinical development space and operating supplies. We expect a slight increase in research and development expenses over the next year as we continue clinical testing of ACH-1625, ACH-2684 and ACH-2928 and initiate clinical testing for ACH-3102.

All costs associated with internal research and development, and research and development services for which we have externally contracted, are expensed as incurred. The costs of obtaining patents for our candidates are expensed as incurred as indirect costs. Our research and development expenses for the years ended December 31, 2011, 2010 and 2009 were as follows:

	<u>For the Years Ended December 31,</u>		
	<u>2011</u>	<u>2010</u>	<u>2009</u>
	(in thousands)		
Direct external costs:			
ACH-1625 (and related compounds) . . . . .	\$12,210	\$ 5,679	\$ 6,233
ACH-2684 (and related compounds) . . . . .	5,764	2,115	—
ACH-2928 (and related compounds) . . . . .	3,265	1,378	—
ACH-3102 (and related compounds) . . . . .	2,795	—	—
Other . . . . .	657	861	1,333
	<u>24,691</u>	<u>10,033</u>	<u>7,566</u>
Direct internal personnel costs . . . . .	7,664	6,755	6,657
Sub-total direct costs . . . . .	32,355	16,788	14,223
Indirect costs and overhead . . . . .	3,504	3,871	4,345
Research and development tax credit . . . . .	(418)	(130)	(149)
Total research and development . . . . .	<u>\$35,441</u>	<u>\$20,529</u>	<u>\$18,419</u>

We are currently conducting a phase IIa clinical trial of ACH-1625, phase I clinical trials of ACH-2684 and ACH-2928 and completing IND-enabling preclinical studies for ACH-3102.

The successful development of our drug candidates is highly uncertain. At this time, we cannot reasonably estimate or know the nature, timing and estimated costs of the efforts that will be necessary to complete the remainder of the development of our drug candidates. We are also unable to predict when, if ever, material net cash inflows will commence from any of our compounds. This is due to the numerous risks and uncertainties associated with developing drugs, including the uncertainty of:

- the scope, rate of progress and expense of our clinical trials and other research and development activities;
- the potential benefits of our drug candidates over other therapies;
- our ability to market, commercialize and achieve market acceptance for any of our drug candidates that we are developing or may develop in the future;
- future clinical trial results;

- the terms and timing of any collaborative, licensing and other arrangements that we may establish;
- the expense and timing of regulatory approvals; and
- the expense of filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights.

A change in the outcome of any of these variables with respect to the development of any of our drug candidates would significantly change the costs and timing associated with the development of that drug candidate. For example, if the FDA or another regulatory authority were to require us to conduct clinical trials beyond those which we currently anticipate will be required to complete clinical development of a drug candidate, or if we experience significant delays in enrollment in any of our clinical trials, we would be required to expend significant additional financial resources and time on the completion of clinical development.

We expect expenses associated with the completion of these programs to be substantial and to increase over time. We do not believe, however, that it is possible at this time to accurately project total program-specific expenses through commercialization. There exist numerous factors associated with the successful commercialization of any of our drug candidates, including future trial design and various regulatory requirements, many of which cannot be determined with accuracy at this time based on our stage of development. Additionally, future commercial and regulatory factors beyond our control will evolve and therefore impact our clinical development programs and plans over time.

### ***General and Administrative***

Our general and administrative expenses consist primarily of salaries and benefits for management and administrative personnel, professional fees for legal, accounting and other services, travel costs and facility-related costs such as rent, utilities and other general office expenses.

### **Critical Accounting Policies and Estimates**

The discussion and analysis of our financial condition and results of operations set forth below are based on our financial statements, which have been prepared in accordance with accounting principles generally accepted in the United States, or U.S. GAAP. The preparation of these financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent liabilities at the date of the financial statements and the reported amounts of revenues and expenses during the reporting period. On an ongoing basis, we evaluate our estimates and assumptions, including those described below. We base our estimates on historical experience and on various other assumptions that we believe to be reasonable under the circumstances. These estimates and assumptions form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Management makes estimates and exercises judgment in revenue recognition, research and development costs, stock-based compensation and accrued expenses. Actual results may differ from these estimates under different assumptions or conditions.

We believe the following critical accounting policies affect management's more significant judgments and estimates used in the preparation of our financial statements:

#### ***Revenue Recognition***

We recognize revenue from contract research and development and research progress payments in accordance with Accounting Standards Codification 605, or ASC 605, *Revenue Recognition*. Revenue-generating research and development collaborations are often multiple element arrangements, providing for a license as well as research and development services. Such arrangements are analyzed to determine whether the deliverables can be separated or whether they must be accounted for as a single unit of accounting. We recognize upfront license payments as revenue upon delivery of the license only if the license has standalone value and the fair value of the

undelivered performance obligations can be determined. If the fair value of the undelivered performance obligations can be determined, such obligations would then be accounted for separately as performed. If the license is considered to either (i) not have standalone value or (ii) have standalone value but the fair value of any of the undelivered performance obligations cannot be determined, the arrangement would then be accounted for as a single unit of accounting and the upfront license payments are recognized as revenue over the estimated period of when our performance obligations are performed.

When we determine that an arrangement should be accounted for as a single unit of accounting, we must determine the period over which the performance obligations will be performed and revenue related to upfront license payments will be recognized. Revenue will be recognized using either a proportionate performance or straight-line method. We recognize revenue using the proportionate performance method provided that we can reasonably estimate the level of effort required to complete our performance obligations under an arrangement and such performance obligations are provided on a best-efforts basis. Under the proportionate performance method, periodic revenue related to up-front license payments is recognized as the percentage of actual effort expended in that period to total effort expected for all of our performance obligations under the arrangement. Actual effort is generally determined based upon actual direct labor hours or FTEs incurred and include research and development activities performed by internal scientists. Total expected effort is generally based upon the total projected direct labor hours. Significant management judgment is required in determining the level of effort required under an arrangement and the period over which we expect to complete the related performance obligations. In the event that a change in estimate occurs, the change will be accounted for using the cumulative catch-up method which provides for an adjustment to revenue in the current period. Estimates of our level of effort may change in the future, resulting in a material change in the amount of revenue recognized in future periods, including negative revenue in some periods. Generally under collaboration arrangements, payments received during the period of performance may include up-front payments, time-or performance-based milestones and reimbursement of internal and external costs. The proportion of actual performance to total expected performance is applied to these payments in determining periodic revenue, but will be limited by the aggregate cash received or receivable to date.

Substantive milestone payments are recognized upon achievement of the milestone. Determining whether a milestone is substantive requires judgment that should be made at the inception of the arrangement. To meet the definition of a substantive milestone, the consideration earned by achieving the milestone (1) would have to be commensurate with either the level of effort required to achieve the milestone or the enhancement in the value of the item delivered, (2) would have to relate solely to past performance, and (3) should be reasonable relative to all deliverables and payment terms in the arrangement. No bifurcation of an individual milestone is allowed and there can be more than one milestone in an arrangement.

#### ***Stock-Based Compensation—Employee Stock-Based Awards***

We apply ASC 718, *Stock Compensation*, which requires measurement and recognition of compensation expense for all stock-based awards made to employees and directors, including employee stock options and employee stock purchases under our 2006 ESPP Plan, based on estimated fair values.

We primarily grant qualified stock options for a fixed number of shares to employees with an exercise price equal to the market value of the shares at the date of grant. To the extent that the amount of the aggregate fair market value of qualified stock options that become exercisable for an individual exceeds \$100,000 during any tax year, those stock options are treated as non qualified stock options. Under the fair value recognition provisions, stock-based compensation cost is based on the value of the portion of stock-based awards that is ultimately expected to vest.

We utilize the Black-Scholes option pricing model for determining the estimated fair value for stock-based awards. The Black-Scholes model requires the use of assumptions which determine the fair value of the stock-based awards. Determining the fair value of stock-based awards at the grant date requires judgment, including estimating the expected term of stock options, the expected volatility of our stock and expected dividends.



Due to our limited exercise history and the period of time that our shares have been publicly traded, we utilize the simplified method in developing an estimate of the expected term of “plain vanilla” share options. Additionally, we use a weighted average rate of historical and peer group volatility. Actual volatility from the end of our initial public offering lock-up period to the end of the current period is weighted as a percentage of actual time to the 6.1 year term determined under the simplified method. We are also required to estimate forfeitures at the grant date and recognize compensation costs for only those awards that are expected to vest.

If factors change and we employ different assumptions in future periods, or if we experience significant fluctuations in our stock price, the compensation expense that we record may differ significantly from what we have recorded in the current period. Therefore, we believe it is important for investors to be aware of the degree of subjectivity involved when using option pricing models to estimate share-based compensation. There is risk that our estimates of the fair values of our share-based compensation awards on the grant dates may differ from the actual values realized upon the exercise, expiration, early termination or forfeiture of those share-based payments in the future. Certain share-based payments, such as employee stock options, may expire worthless or otherwise result in zero intrinsic value as compared to the fair values originally estimated on the grant date and reported in our financial statements. Alternatively, value may be realized from these instruments that is significantly in excess of the fair values originally estimated on the grant date and reported in our financial statements. Although the fair value of employee share-based awards is determined using an option pricing model, that value may not be indicative of the fair value observed in a willing buyer/willing seller market transaction.

### ***Accrued Expenses***

As part of the process of preparing financial statements, we are required to estimate accrued expenses. This process involves identifying services which have been performed on our behalf and estimating the level of service performed and the associated cost incurred for such service as of each balance sheet date in our financial statements.

In accruing service fees, we estimate the time period over which services will be provided and the level of effort in each period. If the actual timing of the provision of services or the level of effort varies from the estimate, we will adjust the accrual accordingly. The majority of our service providers invoice us monthly in arrears for services performed. Some of our service providers require upfront or milestone payments. If our estimate of services performed is less than the upfront or milestone payments, the difference is accounted for as a prepaid expense. In the event that we do not identify costs that have been incurred or we underestimate or overestimate the level of services performed or the costs of such services, our actual expenses could differ from such estimates. The date on which some services commence, the level of services performed on or before a given date and the cost of such services are often subjective determinations. We make judgments based upon facts and circumstances known to us in accordance with U.S. GAAP.

### ***Income Taxes***

We use an asset and liability approach for financial accounting and reporting of income taxes. Deferred tax assets and liabilities are determined based on temporary differences between financial reporting and tax basis assets and liabilities and are measured by applying enacted rates and laws to taxable years in which differences are expected to be recovered or settled. Further, the effect on deferred tax assets and liabilities of a change in tax rates is recognized in income in the period that the rate changes. A valuation allowance is required when it is “more likely than not” that all or a portion of deferred tax assets will not be realized.

We apply the provisions of ASC 740, *Income Taxes*, which prescribes a comprehensive model for how a company should recognize, measure, present and disclose in its financial statements uncertain tax positions that the company has taken or expects to take on a tax return, including a decision whether to file or not file a return in a particular jurisdiction. Our financial statements reflect expected future tax consequences of such positions presuming the taxing authorities’ full knowledge of the position and all relevant facts.

We do not have any unrecognized tax benefits as of December 31, 2011. We review all tax positions to ensure the tax treatment selected is sustainable based on its technical merits and that the position would be sustained if challenged.

## Results of Operations

Results of operations may vary from period to period depending on numerous factors, including the progress of our research and development projects, technological advances and determinations as to the commercial potential of proposed products, the timing of payments received under existing or future strategic alliances, joint ventures or financings, if any.

### Revenues:

Our sources of revenue during the years ended December 31, 2011, 2010 and 2009 are shown below. During the year ended December 31, 2010, in addition to revenue under our Gilead collaboration, we recognized revenue under a SBIR grant and the QTDP program.

	For the Years Ended			Change	
	2011	2010	2009	2011 vs. 2010	2010 vs. 2009
	(in thousands)				
Gilead collaboration revenue	\$247	\$ 180	\$(294)	\$ 67	\$ 474
QTDP revenue	—	1,956	—	(1,956)	1,956
SBIR revenue	—	300	—	(300)	300
Total revenue	<u>\$247</u>	<u>\$2,436</u>	<u>\$(294)</u>	<u>\$(2,189)</u>	<u>\$2,730</u>

We did not recognize any revenue related to the amortization of deferred revenue during the years ended December 31, 2011, 2010 and 2009 as we were unable to accurately estimate our total performance obligations under the Gilead collaboration. Effective with the February 2012 termination, we will recognize the remaining \$2.5 million of deferred revenue.

### *Comparison of the Years Ended December 31, 2011 and 2010*

The increase in collaboration revenue in 2011 is due to increased intellectual property costs related to our NS4A antagonist. These costs were incurred by us and are shared with Gilead. Reimbursement of costs under our collaboration with Gilead is recorded as revenue.

During 2010, we recognized \$300,000 in grant revenue under a SBIR grant for the further study of a back-up series of compounds related to ACH-702 for the treatment of tuberculosis infection. We also recognized \$2.0 million in grant revenue related to the QTDP program which provided for reimbursement of certain costs paid or incurred during 2009 and 2010 directly related to the conduct of a Qualifying Therapeutic Discovery Project. No revenue related to these grants was recognized in 2009 or 2011.

### *Comparison of the Years Ended December 31, 2010 and 2009*

The increase in collaboration revenue in 2010 is due to lower external costs incurred by Gilead under our collaboration, which are shared by us and recorded as a reduction in revenue. Additionally, because we were unable to estimate our future performance obligations under our collaboration with Gilead, we did not recognize revenue related to upfront, milestone and FTE payments previously received.

During 2010, we recognized \$300,000 in grant revenue under a SBIR, grant for the further study of a back-up series of compounds related to ACH-702 for the treatment of tuberculosis infection. We also recognized \$2.0 million in grant revenue related to the QTDP program.

### Research and Development Expenses:

Our research and development expenses consist primarily of salaries and benefits for our research and development personnel, costs of services by clinical research organizations, other outsourced research, materials used during research and development activities, facility-related costs such as rent and utilities associated with our laboratory and clinical development space, operating supplies and other costs associated with our research and development activities. Research and development expenses consisted of the following:

	For the Years Ended			Change	
	2011	2010	2009	2011 vs. 2010	2010 vs. 2009
	(in thousands)				
Personnel costs	\$ 6,511	\$ 5,970	\$ 5,904	\$ 541	\$ 66
Stock based compensation	1,153	785	754	368	31
Outsourced research and supplies	24,039	10,033	7,767	14,006	2,266
Professional and consulting fees	2,164	1,588	1,370	576	218
Facilities costs	1,856	2,075	2,624	(219)	(549)
Travel and other costs	136	208	149	(72)	59
Research and development tax credit	(418)	(130)	(149)	(288)	19
Total	<u>\$35,441</u>	<u>\$20,529</u>	<u>\$18,419</u>	<u>\$14,912</u>	<u>\$2,110</u>

### Comparison of the Years Ended December 31, 2011 and 2010

The increase in research and development expenses from 2010 to 2011 was primarily the result of increased expenses related to clinical testing and manufacturing of ACH-1625, ACH-2684 and ACH-2928, combined with increased preclinical costs primarily related to ACH-3102.

We expect a slight increase in research and development expenses over the next year as we continue clinical testing of ACH-1625, ACH-2684 and ACH-2928 and initiate clinical testing for ACH-3102.

### Comparison of the Years Ended December 31, 2010 and 2009

The increase in research and development expenses from 2009 to 2010 was primarily the result of increased expenses related to clinical testing of ACH-1625, combined with increased preclinical costs for ACH-2684 and ACH-2928. These increases were partially offset by decreased preclinical costs related to ACH-1625 and ACH-1095 and decreased facilities costs related to our reduction of leased laboratory and office space.

### General and Administrative Expenses:

General and administrative expenses consist primarily of salaries and benefits for management and administrative personnel, professional and consulting fees for legal, business development, accounting and other services, travel costs and facility-related costs such as rent, utilities and other general office expenses. General and administrative expenses consisted of the following:

	For the Years Ended			Change	
	2011	2010	2009	2011 vs. 2010	2010 vs. 2009
	(in thousands)				
Personnel costs	\$3,110	\$2,650	\$2,345	\$ 460	\$ 305
Stock based compensation	1,836	1,478	1,186	358	292
Professional and consulting fees	2,219	1,429	1,247	790	182
Facilities costs	975	988	1,269	(13)	(281)
Travel and other costs	1,013	660	506	353	154
Total	<u>\$9,153</u>	<u>\$7,205</u>	<u>\$6,553</u>	<u>\$1,948</u>	<u>\$ 652</u>

### ***Comparison of the Years Ended December 31, 2011 and 2010***

The increase in general and administrative expenses from 2010 to 2011 was primarily due to an increase in professional and consulting fees including business development consulting fees and directors' compensation. Additionally, corporate legal fees, salaries, non-cash charges related to stock based compensation, corporate taxes and general corporate fees also increased.

### ***Comparison of the Years Ended December 31, 2010 and 2009***

The increase in general and administrative expenses from 2009 to 2010 was primarily due to increased personnel costs primarily related to the addition of business development personnel, combined with increased business development consulting fees and public relations costs. These increases were partially offset by decreased facilities costs related to our reduction of leased laboratory and office space.

### **Restructuring Charges:**

During the year ended December 31, 2009, we incurred restructuring charges of \$274,000. These charges consisted primarily of employee severance payments and outplacement services resulting from the implementation of our restructuring plan in July 2009 which reduced employee headcount by approximately 25%. There were no restructuring related costs incurred during the years ended December 31, 2010 and 2011.

### **Other Income and Expense:**

#### ***Comparison of the Years Ended December 31, 2011 and 2010***

Interest income was \$186,000 and \$101,000 for the years ended December 31, 2011 and 2010, respectively. The \$85,000 increase from 2010 to 2011 was primarily due to increased average cash balances.

Interest expense was \$45,000 and \$284,000 for the years ended December 31, 2011 and 2010, respectively. The decrease of \$239,000 was primarily due to lower average debt balances outstanding in 2011.

#### ***Comparison of the Years Ended December 31, 2010 and 2009***

Interest income was \$101,000 and \$172,000 for the years ended December 31, 2010 and 2009, respectively. The \$71,000 decrease from 2009 to 2010 was primarily due to decreased average cash balances.

Interest expense was \$284,000 and \$564,000 for the years ended December 31, 2010 and 2009, respectively. The decrease of \$280,000 was primarily due to lower average debt facility balances outstanding in 2010.

### **Liquidity and Capital Resources**

Since our inception in August 1998, we have financed our operations primarily through the issuance of stock, borrowings under debt facilities, as well as through receipts from our collaboration with Gilead. Through December 31, 2011, we have received approximately \$332.2 million in aggregate gross proceeds from stock issuances, including convertible preferred stock, our initial public offering, our 2008 and 2010 private placements and our 2010 and 2011 public offerings, \$19.5 million from our collaboration with Gilead and approximately \$22.6 million under debt facilities. As of December 31, 2011, amounts remain outstanding under the following debt facility:

<u>Lender</u>	<u>Date</u>	<u>Interest Rate (per annum)</u>	<u>Principal Amount</u>	<u>Outstanding Balance</u>	<u>Maturity Date</u>
Webster Bank .....	June 2011	6.97%	\$437,959	\$370,022	June 2014

We had \$79.9 million, \$55.2 million and \$9.7 million in aggregate cash, cash equivalents and marketable securities as of December 31, 2011, 2010 and 2009, respectively.

In June 2011, we issued 11,040,000 shares of our common stock at a price of \$5.90 per share in an underwritten public offering, including the exercise of the underwriter's exercise of an over-allotment option. We received net proceeds of \$60.9 million, after deducting offering related expenses and underwriting discounts from this offering.

In August 2010, we issued 19,775,101 shares of our common stock at a price of \$2.49 per share, as well as common stock warrants which represent the right to acquire an aggregate of 6,921,286 shares of common stock in a private placement to institutional and other accredited investors. The warrants have a seven-year term and are exercisable at a price of \$3.1125 per share. The warrants allow for a net share settlement. We received net proceeds of \$49.9 million from this private placement.

In January 2010, we issued 10,275,000 shares of our common stock at a price of \$2.08 per share in an underwritten public offering. In February 2010, we issued an additional 1,541,250 shares of common stock in connection with the underwriter's exercise of an over-allotment option. We received net proceeds of \$22.6 million from these share issuances.

Cash used in operating activities was \$35.6 million for the year ended December 31, 2011 and was primarily attributable to our \$44.2 million net loss, primarily offset by \$3.8 million in non-cash charges related to depreciation, amortization and non-cash interest and stock based compensation, a \$2.1 million increase in accounts payable and a \$1.9 million increase in accrued expenses. Cash used in operating activities was \$23.9 million for the year ended December 31, 2010 and was primarily attributable to our \$25.5 million net loss, a \$1.3 million increase in prepaid expenses and a \$500,000 decrease in accrued expenses, partially offset by \$2.9 million in non-cash charges related to depreciation, amortization and non-cash interest and stock based compensation. Cash used in operating activities was \$22.3 million for the year ended December 31, 2009 and was primarily attributable to our \$25.9 million net loss, offset by \$3.0 million in non-cash charges related to depreciation, amortization and non-cash interest and stock based compensation.

Cash used in investing activities was \$35.2 million for the year ended December 31, 2011 and was primarily attributable to purchases of marketable securities partially offset by maturities of marketable securities. Cash used in investing activities was \$30.3 million for the year ended December 31, 2010 and was primarily attributable to purchases of marketable securities partially offset by maturities of marketable securities. Cash provided by investing activities was \$24.1 million for the year ended December 31, 2009 and was primarily attributable to maturities of marketable securities partially offset by purchases of marketable securities.

Cash provided by financing activities was \$61.5 million for the year ended December 31, 2011 and was primarily attributable to \$60.9 million in net proceeds from our public offering in June 2011, partially offset by \$0.5 million used for repayments of debt. Cash provided by financing activities was \$69.9 million for the year ended December 31, 2010 and was primarily attributable to \$72.6 million in net proceeds from our public and private offerings, partially offset by \$2.9 million used for repayments of debt. Cash used in financing activities was \$3.1 million for the year ended December 31, 2009 and was primarily attributable to \$3.0 million used for repayments of debt.

We expect to incur continuing and increasing losses from operations for at least the next several years as we seek to:

- continue clinical testing of ACH-1625, ACH-2684 and ACH-2928;
- initiate clinical testing of ACH-3102; and
- identify and progress additional drug candidates.

We do not expect our existing capital resources, together with the milestone payments and research and development funding we expect to receive, to be sufficient to fund the completion of the development of any of our drug candidates. As a result, we will need to raise additional funds prior to, among other things, being able to

market any drug candidates, to obtain regulatory approvals, fund operating losses, and, if deemed appropriate, establish manufacturing and sales and marketing capabilities. We will seek to raise such additional financing through (i) public or private equity or debt financings, (ii) collaborative or other arrangements with third parties or (iii) other sources of financing.

We believe that our existing cash and cash equivalents will be sufficient to meet our projected operating requirements through at least December 31, 2012. However, our funding resources and requirements may change and will depend upon numerous factors, including but not limited to:

- the costs involved in the clinical development, manufacturing and formulation of ACH-1625, ACH-2684, ACH-2928 and ACH-3102;
- our ability to, and our choice whether to, enter into corporate collaborations for our HCV candidates and the terms and success of these collaborations;
- the costs involved in obtaining regulatory approvals for our drug candidates;
- the scope, prioritization and number of programs we pursue;
- the costs involved in preparing, filing, prosecuting, maintaining, enforcing and defending patent and other intellectual property claims;
- our ability to raise incremental debt or equity capital, including any changes in the credit market that may impact our ability to obtain capital in the future;
- any partnership opportunities that may arise for elvucitabine, ACH-702 or ACH-2881 that we determine to pursue;
- our acquisition and development of new technologies and drug candidates; and
- competing technological and market developments currently unknown to us.

We intend to augment our cash balance through financing transactions, including the issuance of debt or equity securities, and/or further corporate alliances. There can be no assurance that we will be able to obtain adequate levels of additional funding or favorable terms, if at all. If adequate funds are not available, we will be required to:

- delay, reduce the scope of or eliminate research and development programs;
- obtain funds through arrangements with collaborators or others on terms unfavorable to us or that may require us to relinquish rights to certain drug candidates that we might otherwise seek to develop or commercialize independently; and/or
- pursue merger or acquisition strategies.

Any future equity funding may dilute the ownership of our equity investors.

## Off-Balance Sheet Arrangements, Contractual Obligations and Commitments

The following table sets forth a summary of our commitments as of December 31, 2011:

	Payment Due by Period				
	Total	Less Than 1 Year	1-3 Years	3-5 Years	More than 5 Years
	(in thousands)				
Debt, including interest	\$ 370	\$ 141	\$ 229	\$ —	\$ —
Operating lease obligations	3,302	598	1,236	1,300	168
Clinical research obligations	14,559	13,942	577	40	—
Research obligations and licenses	575	115	230	230	—
Other professional obligations	584	584	—	—	—
Other license and research development agreements	1,250	—	100	—	1,150
Total	<u>\$20,640</u>	<u>\$15,380</u>	<u>\$2,372</u>	<u>\$1,570</u>	<u>\$1,318</u>

Other professional obligations consist mainly of general and administrative consulting obligations. Other license and research development agreements consists of potential payments due to Yale University and Emory University upon the achievement of specified development milestones for elvucitabine.

## Related Party Transactions

Our board of directors is committed to upholding the highest legal and ethical conduct in fulfilling its responsibilities and recognizes that related party transactions can present a heightened risk of potential or actual conflicts of interest.

In accordance with our audit committee charter, members of the audit committee, all of whom are independent directors, review and approve all related party transactions for which approval is required under applicable laws or regulations, including SEC and the NASDAQ Stock Market rules. Current SEC rules define a related party transaction to include any transaction, arrangement or relationship in which we are a participant and the amount involved exceeds \$120,000, and in which any of the following persons has or will have a direct or indirect interest:

- our executive officers, directors or director nominees;
- any person who is known to be the beneficial owner of more than 5% of our common stock;
- any person who is an immediate family member, as defined under Item 404 of Regulation S-K, of any of our executive officers, directors or director nominees or beneficial owner of more than 5% of our common stock; or
- any firm, corporation or other entity in which any of the foregoing persons is employed or is a partner or principal or in a similar position or in which such person, together with any other of the foregoing persons, has a 5% or greater beneficial ownership interest.

In addition, the audit committee reviews and investigates any matters pertaining to the integrity of management, including conflicts of interest and adherence to our Code of Business Conduct and Ethics. Under our Code of Business Conduct and Ethics, our directors, officers and employees are expected to avoid any relationship, influence or activity that would cause or even appear to cause a conflict of interest. Under our Code of Business Conduct and Ethics, a director is required to promptly disclose to our board of directors any potential or actual conflict of interest involving him or her. In accordance with our Code of Business Conduct and Ethics, the board of directors will determine an appropriate resolution on a case-by-case basis. All directors must recuse themselves from any discussion or decision affecting their personal, business or professional interests.

We have entered into or engaged in the following transactions with the following directors, officers and stockholders who beneficially owned more than 5% of our outstanding common stock at the time of these transactions, as well as affiliates or immediate family members of those directors, officers stockholders. We believe that the terms of the transactions described below were no less favorable than those that we could have obtained from unaffiliated third parties.

#### *Nicholas Simon*

In connection with Clarus Ventures, LLC's ("Clarus") agreement to invest in Achillion, the Board of Directors of the Company elected Nicholas Simon as a Class I member of the Board of Directors to serve until his successor is duly elected and qualified. Mr. Simon is a managing director of Clarus.

In August 2008, Clarus purchased units consisting of 5,163,689 shares of common stock and common stock warrants to purchase 1,290,922 shares of common stock for an aggregate purchase price of \$15 million. Additionally, in August 2010, Clarus purchased 4,875,502 shares of common stock and warrants to purchase 1,706,426 shares of common stock for an aggregate purchase price of \$12.4 million.

As of December 31, 2011, Clarus is the beneficial owner of approximately 17% of our total issued and outstanding shares.

#### *Nicole Vitullo*

In connection with Domain Associates, LLC's ("Domain") agreement to invest in Achillion, the Board of Directors of the Company elected Nicole Vitullo of Domain as a Class II member of the Board of Directors on September 30, 2010 to serve until her successor is duly elected and qualified. Ms. Vitullo is a partner at Domain. In August 2010, Domain purchased 8,032,129 shares of common stock and warrants to purchase 2,811,245 shares of common stock for an aggregate purchase price of \$20.4 million.

As of December 31, 2011, Domain is the beneficial owner of approximately 15% of our total issued and outstanding shares.

### **Recently Issued Accounting Standards**

In October 2009, an update was made to ASC 605, *Revenue Recognition*, which provides accounting principles and application guidance on how revenue arrangements with multiple deliverables should be separated and the consideration allocated. Assuming other criteria are met, this guidance eliminates the requirement to establish the fair value of undelivered products and services and instead provides for separate revenue recognition. Allocation of consideration is now based on management's estimate of the selling price for an undelivered item where there is no other means to determine the fair value of that undelivered item. This update was effective prospectively for revenue arrangements entered into or materially modified in fiscal years beginning on or after June 15, 2010. We adopted this standard as of January 1, 2011. There was no impact to our financial statements upon adoption of this standard, as there were no new or materially modified agreements.

In June 2011, the FASB issued ASU No. 2011-05 "Comprehensive Income: Presentation of Comprehensive Income." Under the amendment, an entity will have the option to present the total of comprehensive income, the components of net income, and the components of other comprehensive income either in a single continuous statement of comprehensive income or in two separate but consecutive statements. This amendment, therefore, eliminates the currently available option to present the components of other comprehensive income as part of the statement of changes in stockholders' equity. The amendment does not change the items that must be reported in other comprehensive income or when an item of other comprehensive income must be reclassified to net income. In December 2011, the FASB issued ASU No. 2011-12, "Comprehensive Income: Deferral of the Effective Date for Amendments to the Presentation of Reclassifications of Items Out of Accumulated Other Comprehensive



Income in Accounting Standards Update No. 2011-05” (“ASU 2011-12”). ASU 2011-12 defers changes in Update 2011-05 that relate to the presentation of reclassification adjustments. ASU 2011-12 is effective for fiscal years, and interim periods within those years, beginning after December 15, 2011. As this guidance relates to presentation only, the adoption of this guidance will not have any other effect on our financial statements.

#### **ITEM 7A. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK**

*Interest Rate Risk.* Our exposure to market risk is confined to our cash, cash equivalents and marketable securities. We regularly review our investments and monitor the financial markets. We do not believe the recent distress in the financial markets has had a significant impact on our financial position. We invest in high-quality financial instruments, primarily money market funds, government sponsored bond obligations and certain other corporate debt securities, with the effective duration of the portfolio less than twelve months and no security with an effective duration in excess of twenty four months, which we believe are subject to limited credit risk. We currently do not hedge interest rate exposure. Due to the short-term nature of our investments, we do not believe that we have any material exposure to interest rate risk or changes in credit ratings arising from our investments.

*Capital Market Risk.* We currently have no product revenues and depend on funds raised through other sources. One source of funding is through future equity offerings. Our ability to raise funds in this manner depends upon capital market forces affecting our stock price.

#### **ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA**

The information required by this Item is included in our Financial Statements and Supplementary Data listed in Item 15 of Part IV of this annual report on Form 10-K.

#### **ITEM 9. CHANGES IN AND DISAGREEMENTS WITH ACCOUNTANTS ON ACCOUNTING AND FINANCIAL DISCLOSURE**

None.

#### **ITEM 9A. CONTROLS AND PROCEDURES**

##### *Evaluation of Disclosure Controls and Procedures*

Our management, with the participation of our chief executive officer and chief financial officer, evaluated the effectiveness of our disclosure controls and procedures as of December 31, 2011. The term “disclosure controls and procedures,” as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the SEC’s rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company’s management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of December 31, 2011, our chief executive officer and chief financial officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

### *Management's Annual Report on Internal Control Over Financial Reporting*

Our management is responsible for establishing and maintaining adequate internal control over financial reporting. Internal control over financial reporting is defined in Rule 13a-15(f) and 15d-15(f) promulgated under the Securities Exchange Act of 1934 as a process designed by, or under the supervision of, the Company's principal executive and principal financial officers and effected by our board of directors, management and other personnel, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles and includes those policies and procedures that:

- Pertain to the maintenance of records that in reasonable detail accurately and fairly reflect the transactions and dispositions of the assets of the company;
- Provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the company are being made only in accordance with authorizations of management and directors of the company; and
- Provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use or disposition of the company's assets that could have a material effect on the financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

Our management assessed the effectiveness of our internal control over financial reporting as of December 31, 2011. In making this assessment, our management used the criteria set forth by the Committee of Sponsoring Organizations of the Treadway Commission (COSO) in *Internal Control-Integrated Framework*.

Based on this assessment, management concluded that, as of December 31, 2011, our internal control over financial reporting is effective based on the criteria set forth in *Internal Control—Integrated Framework* issued by the COSO.

The effectiveness of our internal control over financial reporting as of December 31, 2011 has been audited by PricewaterhouseCoopers LLP, an independent registered public accounting firm, as stated in their report which is included herein.

### *Changes in Internal Control over Financial Reporting*

No change in our internal control over financial reporting occurred during the fiscal quarter ended December 31, 2011 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

### **ITEM 9B. OTHER INFORMATION**

None.

## **PART III**

### **ITEM 10. DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE**

We intend to file with the Securities and Exchange Commission a definitive Proxy Statement, which we refer to herein as the Proxy Statement, not later than 120 days after the close of the fiscal year ended December 31, 2011. The information required by this item is incorporated herein by reference to the information contained under the sections captioned “Election of Class II Directors,” “Section 16(a) Beneficial Ownership Reporting Compliance” and “Corporate Governance” of the Proxy Statement. The information required by this item relating to executive officers is included in “Part I, Item 1—Business—Executive Officers of the Registrant” of this Annual Report on Form 10-K on page 26 and is incorporated by reference.

We have adopted a written code of business conduct and ethics, which applies to our principal executive officer, principal financial or accounting officer or person serving similar functions and all of our other employees and members of our board of directors. The text of our amended code of ethics is available on our website at [www.achillion.com](http://www.achillion.com). We did not waive any provisions of the code of business ethics during the year ended December 31, 2011. If we amend, or grant a waiver under, our code of business ethics that applies to our principal executive officer, principal financial or accounting officer, or persons performing similar functions, we intend to post information about such amendment or waiver on our website at [www.achillion.com](http://www.achillion.com).

### **ITEM 11. EXECUTIVE COMPENSATION**

The information required by this item is incorporated herein by reference to the information contained under the sections captioned “Executive Compensation,” “Compensation of Directors,” “Compensation Committee Interlocks and Insider Participation” and “Employment Arrangements” of the Proxy Statement.

### **ITEM 12. SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT AND RELATED STOCKHOLDER MATTERS**

The information required by this item is incorporated herein by reference to the information contained under the sections captioned “Security Ownership of Certain Beneficial Owners and Management” and “Equity Compensation Plan Information” of the Proxy Statement.

### **ITEM 13. CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS, AND DIRECTOR INDEPENDENCE**

The information required by this item is incorporated herein by reference to the information contained under the sections captioned “Employment Arrangements” and “Certain Relationships and Related Transactions” of the Proxy Statement.

### **ITEM 14. PRINCIPAL ACCOUNTANT FEES AND SERVICES**

The information required by this item is incorporated herein by reference to the information contained under the sections captioned “Auditor’s Fees” and “Pre-Approval Policies and Procedures” of the Proxy Statement.

**PART IV**

**ITEM 15. EXHIBITS AND FINANCIAL STATEMENT SCHEDULES**

(a)(1) Financial Statements

The following documents are included on pages F-1 through F-29 attached hereto and are filed as part of this annual report on Form 10-K.

Report of Independent Registered Public Accounting Firm .....	F-2
Balance Sheets as of December 31, 2011 and 2010 .....	F-3
Statements of Operations for the Years Ended December 31, 2011, 2010 and 2009 .....	F-4
Statements of Stockholders' Equity and Comprehensive Loss for the Years Ended December 31, 2009, 2010 and 2011 .....	F-5
Statements of Cash Flows for the Years Ended December 31, 2011, 2010 and 2009 .....	F-6
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(a)(2) Financial Statement Schedules

Not applicable

(a)(3) List of Exhibits

The exhibits which are filed with this report or which are incorporated herein by reference are set forth in the Exhibit Index hereto.

## SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the Registrant has duly caused this Report to be signed on its behalf by the undersigned, thereunto duly authorized, on March 8, 2012.

### ACHILLION PHARMACEUTICALS, INC.

By:           /s/ MICHAEL D. KISHBAUCH            
**Michael D. Kishbauch**  
**President and Chief Executive Officer**

Pursuant to the requirements of the Securities Exchange Act of 1934, the Report has been signed below by the following persons on behalf of the Registrant and in the capacities indicated as of March 8, 2012.

<u>Signature</u>	<u>Title</u>	<u>Date</u>
/s/ MICHAEL D. KISHBAUCH <b>Michael D. Kishbauch</b>	President and Chief Executive Officer and Director (Principal executive officer)	March 8, 2012
/s/ MARY KAY FENTON <b>Mary Kay Fenton</b>	Senior Vice President and Chief Financial Officer (Principal financial and accounting officer)	March 8, 2012
/s/ JASON FISHERMAN, M.D. <b>Jason Fisherman, M.D.</b>	Director	March 8, 2012
/s/ GARY E. FRASHIER <b>Gary E. Frashier</b>	Director	March 8, 2012
/s/ DENNIS LIOTTA <b>Dennis Liotta</b>	Director	March 8, 2012
/s/ DAVID SCHEER <b>David Scheer</b>	Chairman of the Board	March 8, 2012
/s/ NICHOLAS SIMON <b>Nicholas Simon</b>	Director	March 8, 2012
/s/ ROBERT VAN NOSTRAND <b>Robert Van Nostrand</b>	Director	March 8, 2012
/s/ NICOLE VITULLO <b>Nicole Vitullo</b>	Director	March 8, 2012
/s/ DAVID WRIGHT <b>David Wright</b>	Director	March 8, 2012

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## INDEX TO FINANCIAL STATEMENTS

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## Report of Independent Registered Public Accounting Firm

To the Board of Directors and Stockholders of  
Achillion Pharmaceuticals, Inc.

In our opinion, the accompanying balance sheets and the related statements of operations, of stockholders' equity and comprehensive loss and of cash flows present fairly, in all material respects, the financial position of Achillion Pharmaceuticals, Inc. at December 31, 2011 and December 31, 2010, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2011 in conformity with accounting principles generally accepted in the United States of America. Also in our opinion, the Company maintained, in all material respects, effective internal control over financial reporting as of December 31, 2011, based on criteria established in *Internal Control—Integrated Framework* issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO). The Company's management is responsible for these financial statements, for maintaining effective internal control over financial reporting and for its assessment of the effectiveness of internal control over financial reporting, included in Management's Annual Report on Internal Control over Financial Reporting appearing under Item 9A. Our responsibility is to express opinions on these financial statements and on the Company's internal control over financial reporting based on our audits (which were integrated audits in 2011 and 2009). We conducted our audits in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audits to obtain reasonable assurance about whether the financial statements are free of material misstatement and whether effective internal control over financial reporting was maintained in all material respects. Our audits of the financial statements included examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements, assessing the accounting principles used and significant estimates made by management, and evaluating the overall financial statement presentation. Our audit of internal control over financial reporting included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, and testing and evaluating the design and operating effectiveness of internal control based on the assessed risk. Our audits also included performing such other procedures as we considered necessary in the circumstances. We believe that our audits provide a reasonable basis for our opinions.

A company's internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles. A company's internal control over financial reporting includes those policies and procedures that (i) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (ii) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the company are being made only in accordance with authorizations of management and directors of the company; and (iii) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of the company's assets that could have a material effect on the financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

/s/ PricewaterhouseCoopers LLP

Hartford, Connecticut  
March 8<sup>th</sup> 2012



**Achillion Pharmaceuticals, Inc.**  
**Balance Sheets**  
(in thousands, except per share amounts)

	As of December 31,	
	2011	2010
<b>Assets</b>		
Current assets:		
Cash and cash equivalents .....	\$ 16,110	\$ 25,373
Marketable securities .....	37,456	29,827
Accounts and other receivables .....	103	246
Prepaid expenses and other current assets .....	1,423	2,052
Total current assets .....	55,092	57,498
Marketable securities .....	26,377	—
Fixed assets, net .....	994	468
Deferred financing costs .....	15	117
Restricted cash .....	152	152
Total assets .....	\$ 82,630	\$ 58,235
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities:		
Accounts payable .....	\$ 4,795	\$ 2,672
Accrued expenses .....	4,008	2,061
Current portion of long-term debt .....	141	469
Total current liabilities .....	8,944	5,202
Deferred revenue .....	2,489	2,489
Long-term debt .....	229	—
Total liabilities .....	11,662	7,691
Commitments (Notes 14 and 15)		
Stockholders' Equity:		
Preferred Stock, undesignated, \$.01 par value; 5,000 shares authorized at December 31, 2011 and 2010; no shares issued or outstanding .....	—	—
Common Stock, \$.001 par value; 200,000 and 100,000, respectively, shares authorized at December 31, 2011 and 2010; 69,788 and 58,376 shares issued and outstanding at December 31, 2011 and 2010, respectively .....	70	58
Additional paid-in capital .....	346,518	281,878
Accumulated deficit .....	(275,600)	(231,394)
Accumulated other comprehensive (loss) income .....	(20)	2
Total stockholders' equity .....	70,968	50,544
Total liabilities and stockholders' equity .....	\$ 82,630	\$ 58,235

*The accompanying notes are an integral part of these financial statements.*

**Achillion Pharmaceuticals, Inc.**  
**Statements of Operations**  
**(in thousands, except per share amounts)**

	<u>Years Ended December 31,</u>		
	<u>2011</u>	<u>2010</u>	<u>2009</u>
Revenue .....	\$ 247	\$ 2,436	\$ (294)
Operating expenses			
Research and development .....	35,441	20,529	18,419
General and administrative .....	9,153	7,205	6,553
Restructuring charges (Note 13) .....	—	—	274
Total operating expenses .....	<u>44,594</u>	<u>27,734</u>	<u>25,246</u>
Loss from operations .....	(44,347)	(25,298)	(25,540)
Other income (expense)			
Interest income .....	186	101	172
Interest expense .....	(45)	(284)	(564)
Net loss .....	<u>\$(44,206)</u>	<u>\$(25,481)</u>	<u>\$(25,932)</u>
Basic and diluted net loss per share attributable to common stockholders (Note 4) .....	<u>\$ (0.69)</u>	<u>\$ (0.57)</u>	<u>\$ (0.98)</u>
Weighted average shares used in computing basic and diluted net loss per share attributable to common stockholders .....	<u>64,248</u>	<u>45,079</u>	<u>26,537</u>

*The accompanying notes are an integral part of these financial statements.*

**Achillion Pharmaceuticals, Inc.**

**Statements of Stockholders' Equity and Comprehensive Loss for the Years Ended December 31, 2009, 2010 and 2011**  
(in thousands)

	Common Stock Shares	Amount	Additional Paid-In Capital	Accumulated Deficit	Accumulated Other Comprehensive Income (Loss)	Total Stockholders' Equity
Balances at December 31, 2008	26,399	\$ 26	\$204,861	\$(179,981)	\$ 115	\$ 25,021
Net loss	—	—	—	(25,932)	—	(25,932)
Unrealized income (loss) on marketable securities	—	—	—	—	(115)	(115)
Comprehensive loss	—	—	—	—	—	(26,047)
Stock compensation	—	—	1,940	—	—	1,940
Issuance of common stock upon exercise stock options	3	—	5	—	—	5
Issuance of common stock under the Employee Stock Purchase Plan	113	—	102	—	—	102
Issuance of common stock in connection with Standby Equity Distribution Agreement	191	1	—	—	—	1
Balances at December 31, 2009	26,706	\$ 27	\$206,908	\$(205,913)	\$ —	\$ 1,022
Net loss	—	—	—	(25,481)	—	(25,481)
Unrealized income (loss) on marketable securities	—	—	—	—	2	2
Comprehensive loss	—	—	—	—	—	(25,479)
Stock compensation	—	—	2,263	—	—	2,263
Issuance of common stock upon exercise stock options	26	—	77	—	—	77
Issuance of common stock under the Employee Stock Purchase Plan	53	—	99	—	—	99
Issuance of common stock and warrants in connection with the public offering and private placement, net of issuance costs	31,591	31	72,531	—	—	72,562
Balances at December 31, 2010	58,376	\$ 58	\$281,878	\$(231,394)	\$ 2	\$ 50,544
Net loss	—	—	—	(44,206)	—	(44,206)
Unrealized income (loss) on marketable securities	—	—	—	—	(22)	(22)
Comprehensive loss	—	—	—	—	—	(44,228)
Stock compensation	—	—	2,989	—	—	2,989
Issuance of common stock upon exercise of warrants	8	—	—	—	—	—
Issuance of common stock upon exercise of stock options	320	1	569	—	—	570
Issuance of common stock under the Employee Stock Purchase Plan	44	—	146	—	—	146
Issuance of common stock in connection with the public offering, net of issuance costs	11,040	11	60,936	—	—	60,947
Balances at December 31, 2011	69,788	\$ 70	\$346,518	\$(275,600)	\$ (20)	\$ 70,968

*The accompanying notes are an integral part of these financial statements.*

**Achillion Pharmaceuticals, Inc.**

**Statements of Cash Flows**  
(in thousands)

	Years Ended December 31,		
	2011	2010	2009
<b>Cash flows from operating activities</b>			
Net loss .....	\$(44,206)	\$(25,481)	\$(25,932)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation and amortization .....	327	615	987
Noncash stock-based compensation .....	2,989	2,263	1,940
Noncash interest expense .....	9	52	70
(Gain) loss on disposal/trade-in of equipment .....	(111)	6	—
Amortization of premium (discount) on securities .....	478	325	125
Changes in operating assets and liabilities:			
Accounts and other receivables .....	143	(181)	(65)
Prepaid expenses and other current assets .....	739	(1,348)	490
Accounts payable .....	2,123	395	(267)
Accrued expenses .....	1,947	(537)	338
Net cash used in operating activities .....	(35,562)	(23,891)	(22,314)
<b>Cash flows from investing activities</b>			
Purchase of fixed assets .....	(732)	(169)	(42)
Release of restriction on cash .....	—	—	53
Purchase of available for sale marketable securities .....	(80,280)	(39,700)	(7,339)
Maturities of marketable securities .....	45,774	9,550	31,396
Net cash (used in) provided by investing activities .....	(35,238)	(30,319)	24,068
<b>Cash flows from financing activities</b>			
Proceeds from issuance of common stock and warrants in connection with the public offering and private placement, net of issuance costs .....	60,947	72,562	—
Proceeds from exercise of stock options .....	570	77	6
Proceeds from sale of stock under the Employee Stock Purchase Plan .....	146	99	102
Borrowings of debt .....	438	—	—
Repayments of debt .....	(546)	(2,867)	(3,035)
Payment of deferred financing costs .....	(18)	—	(175)
Net cash provided by (used in) financing activities .....	61,537	69,871	(3,102)
Net increase (decrease) in cash and cash equivalents .....	(9,263)	15,661	(1,348)
Cash and cash equivalents, beginning of period .....	25,373	9,712	11,060
Cash and cash equivalents, end of period .....	\$ 16,110	\$ 25,373	\$ 9,712
<b>Supplemental disclosure of cash flow information</b>			
Cash paid for interest .....	\$ 33	\$ 207	\$ 468
<b>Supplemental disclosure of noncash financing activities</b>			
Cashless exercise of warrants .....	\$ 43	\$ —	\$ —
Issuance of common stock for Standby Equity Distribution Agreement commitment fee .....	\$ —	\$ —	\$ 300

*The accompanying notes are an integral part of these financial statements.*

**Achillion Pharmaceuticals, Inc.**  
**Notes to Financial Statements**  
**(in thousands, except per share amounts)**

**1. Nature of the Business**

Achillion Pharmaceuticals, Inc. (the “Company”) was incorporated on August 17, 1998 in Delaware. The Company was established to discover, develop and commercialize innovative anti-infective drug therapies. The Company is devoting substantially all of its efforts towards product research and development.

The Company incurred losses of \$261,738 from inception through December 31, 2011 and had an accumulated deficit of \$275,600 at December 31, 2011, which includes preferred stock dividends recognized until the Company’s initial public offering in 2006. The Company has funded its operations primarily through the sale of equity securities, borrowings from debt facilities and the receipt of milestone and cost-sharing receipts from a collaboration partner, Gilead Sciences, Inc. (“Gilead”).

The Company believes that its existing cash, cash equivalents and marketable securities will be sufficient to support its current operating plan through at least December 31, 2012. However, the Company’s operating plan may change as a result of many factors, including but not limited to:

- the costs involved in the clinical development, manufacturing and formulation of ACH-1625, ACH-2684, ACH-2928 and ACH-3102;
- the Company’s ability to, and choice whether to, enter into corporate collaborations for its HCV candidates and the terms and success of these collaborations, if any;
- any partnership opportunities that may arise for ACH-702 or ACH-2881 that the Company determines to pursue; and
- the Company’s ability to raise incremental debt or equity capital, including any changes in the credit market that may impact its ability to obtain capital in the future.

**2. Summary of Significant Accounting Policies**

***Use of Estimates***

The preparation of financial statements in conformity with accounting principles generally accepted in the United States (“GAAP”) requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of revenues and expenses during the reporting period. Actual results could differ from those estimates.

***Revenue Recognition***

The Company recognizes revenue from contract research and development and research progress payments in accordance with ASC 605, *Revenue Recognition*. Revenue-generating research and development collaborations are often multiple element arrangements, providing for a license as well as research and development services. Such arrangements are analyzed to determine whether the deliverables, including research and development services, can be separated or whether they must be accounted for as a single unit of accounting. The Company recognizes upfront license payments as revenue upon delivery of the license only if the license has standalone value and the fair value of the undelivered performance obligations can be determined. If the fair value of the undelivered performance obligations can be determined, such obligations would then be accounted for separately as performed. If the license is considered to either (i) not have standalone value or (ii) have standalone value but the fair value of any of the undelivered performance obligations cannot be determined, the arrangement would then be accounted for as a single unit of accounting and the upfront license payments are recognized as revenue over the estimated period of when our performance obligations are performed.

When the Company determines that an arrangement should be accounted for as a single unit of accounting, it must determine the period over which the performance obligations will be performed and revenue related to upfront license payments will be recognized. Revenue will be recognized using either a proportionate performance or straight-line method. The Company recognizes revenue using the proportionate performance method provided that it can reasonably estimate the level of effort required to complete its performance obligations under an arrangement and such performance obligations are provided on a best-efforts basis. Under the proportionate performance method, periodic revenue related to up-front license payments is recognized as the percentage of actual effort expended in that period to total effort expected for all of its performance obligations under the arrangement. Actual effort is generally determined based upon actual direct labor hours or full-time equivalents (“FTE”) incurred and include research and development activities performed by internal scientists. Total expected effort is generally based upon the total direct labor hours of FTEs incorporated into the detailed budget and project plan that is agreed to by both parties to the collaboration. Significant management judgment is required in determining the level of effort required under an arrangement and the period over which the Company expects to complete the related performance obligations. In the event that a change in estimate occurs, the change will be accounted for using the cumulative catch-up method which provides for an adjustment to revenue in the current period. Estimates of the Company’s level of effort may change in the future, resulting in a material change in the amount of revenue recognized in future periods, including negative revenue in some periods. Generally under collaboration arrangements, payments received during the period of performance may include up-front payments, time-or performance-based milestones and reimbursement of internal and external costs. The proportion of actual performance to total expected performance is applied to these payments in determining periodic revenue, but will be limited by the aggregate cash received or receivable to date.

Substantive milestone payments are recognized upon achievement of the milestone. Determining whether a milestone is substantive requires judgment that should be made at the inception of the arrangement. To meet the definition of a substantive milestone, the consideration earned by achieving the milestone (1) would have to be commensurate with either the level of effort required to achieve the milestone or the enhancement in the value of the item delivered, (2) would have to relate solely to past performance, and (3) should be reasonable relative to all deliverables and payment terms in the arrangement. No bifurcation of an individual milestone is allowed and there can be more than one milestone in an arrangement.

Reimbursement of costs is recognized as revenue provided the amounts are determinable and collection of the related receivable is reasonably assured. Under the Company’s collaboration arrangement with Gilead, amounts owed to Gilead for external costs were treated as contra revenue as the Company concluded that it does not receive a separate identifiable benefit.

During the years ended December 31, 2011, 2010 and 2009, the Company did not recognize any revenue from upfront, milestone and FTE fees previously received under the Gilead collaboration as the Company could not accurately estimate its future obligations under the collaboration. In February 2012, the Company will recognize the remaining deferred revenue balance relating to upfront, milestone and FTE payments received under the collaboration coincident with the termination of the agreement.

The Company recognizes grant revenue when the following criteria are met: persuasive evidence of an arrangement exists, services have been rendered, the price is fixed or determinable, and collectability is reasonably assured. For the year ended December 31, 2010, the Company’s grant revenue consisted of amounts related to a Small Business Innovation Research, or SBIR, grant by the National Institutes of Health and revenue related to the Qualifying Therapeutic Discovery Project program, or QTDP. The SBIR grant was for the further study of a back-up series of compounds related to ACH-702 for the treatment of tuberculosis infection. The QTDP program was created by the United States Congress as part of the Patient Protection and Affordable Care Act and provided for reimbursement of certain costs paid or incurred during 2009 and 2010 directly related to the conduct of a Qualifying Therapeutic Discovery Project, as defined. The Department of Health and Human Services designated such projects based on the potential for them to result in new therapies to treat areas of unmet medical need, the potential to create and sustain jobs in the U.S. and to advance U.S. competitiveness. No grant revenue was recognized during the years ended December 31, 2011 or 2009.

### ***Stock-Based Compensation—Employee Stock-Based Awards***

The Company applies the provisions of ASC 718, *Stock Compensation*, which requires measurement and recognition of compensation expense for all stock-based awards made to employees and directors, including employee stock options and employee stock purchases under the Company's 2006 ESPP Plan based on estimated fair values.

The Company primarily grants qualified stock options for a fixed number of shares to employees with an exercise price equal to the market value of the shares at the date of grant. To the extent that the amount of the aggregate fair market value of qualified stock options that become exercisable for an individual exceeds \$100,000 during any tax year, those stock options are treated as non-qualified stock options. Under the fair value recognition provisions, stock-based compensation cost is based on the fair value of the portion of stock-based awards that is ultimately expected to vest. Stock-based compensation expense recognized during the year ended December 31, 2009 includes compensation expense for stock-based awards granted prior to, but not yet vested as of December 31, 2005, as well as amounts related to the stock-based awards granted subsequent to December 31, 2005, based on the fair value on the grant date.

The Company utilizes the Black-Scholes option pricing model for determining the estimated fair value for stock-based awards. The Black-Scholes model requires the use of assumptions which determine the fair value of the stock-based awards. Determining the fair value of stock-based awards at the grant date requires judgment, including estimating the expected term of stock options, the expected volatility of our stock and expected dividends.

Due to the Company's limited exercise history, the Company utilizes the simplified method in developing an estimate of the expected term of "plain vanilla" share options. Additionally, the Company uses a weighted average rate of historical and peer group volatility. The Company's actual volatility from the end of its initial public offering lock-up period to the end of the current period is weighted as a percentage of actual time to the 6.1 year term determined under the simplified method. The Company is also required to estimate forfeitures at the grant date and recognize compensation costs for only those awards that are expected to vest.

If factors change and the Company employs different assumptions in future periods, or if the Company experiences significant fluctuations in its stock price, the compensation expense that it records may differ significantly from what the Company has recorded in the current period. Therefore, the Company believes it is important for investors to be aware of the degree of subjectivity involved when using option pricing models to estimate share-based compensation. There is risk that the Company's estimates of the fair values of its share-based compensation awards on the grant dates may differ from the actual values realized upon the exercise, expiration, early termination or forfeiture of those share-based payments in the future. Certain share-based payments, such as employee stock options, may expire worthless or otherwise result in zero intrinsic value as compared to the fair values originally estimated on the grant date and reported in the Company's financial statements. Alternatively, value may be realized from these instruments that is significantly in excess of the fair values originally estimated on the grant date and reported in our financial statements. Although the fair value of employee share-based awards is determined using an option pricing model, that value may not be indicative of the fair value observed in a willing buyer/willing seller market transaction.

### ***Accrued Expenses***

As part of the process of preparing financial statements, the Company is required to estimate accrued expenses. This process involves identifying services which have been performed on its behalf and estimating the level of service performed and the associated cost incurred for such service as of each balance sheet date in its financial statements.

In accruing service fees, the Company estimates the time period over which services will be provided and the level of effort in each period. If the actual timing of the provision of services or the level of effort varies from the estimate, the Company will adjust the accrual accordingly. The majority of service providers invoice the

Company monthly in arrears for services performed. Some service providers require upfront or milestone payments. If the estimate of services performed is less than the upfront or milestone payments, the difference is accounted for as a prepaid expense. In the event that the Company does not identify costs that have begun to be incurred or the Company underestimates or overestimates the level of services performed or the costs of such services, actual expenses could differ from such estimates. The date on which some services commence, the level of services performed on or before a given date and the cost of such services are often subjective determinations. The Company makes judgments based upon facts and circumstances known to it in accordance with GAAP.

### ***Cash, Cash Equivalents and Restricted Cash***

Cash and cash equivalents are stated at cost, which approximates fair value, and include short-term, highly-liquid investments with original maturities of less than three months. The Company also holds certificates of deposit, which collateralize the Company's facility lease which are classified as restricted cash in the accompanying balance sheets. The restricted cash will be released from restriction in 2017. At December 31, 2011, the Company had \$5,460 of cash and \$10,650 of cash equivalents.

### ***Marketable Securities and Equity Investments***

The Company applies the provisions of ASC 820, *Fair Value Measurements and Disclosures*, for financial assets and liabilities measured on a recurring basis which requires disclosure that establishes a framework for measuring fair value and expands disclosures in the financial statements. The statement requires that fair value measurements be classified and disclosed in one of the three categories:

Level 1: Quoted prices in active markets for identical assets and liabilities that the reporting entity has the ability to access at the measurement date;

Level 2: Inputs other than quoted prices included within Level 1 that are observable for the asset or liability, either directly or indirectly; or

Level 3: Unobservable inputs.

The fair value of the Company's securities of \$63,833 as of December 31, 2011 was valued based on level 2 inputs. The Company's investments consist mainly of U.S government and agency securities, government sponsored bond obligations and certain other corporate debt securities. Fair value is determined based upon quoted market prices; however, due to lack of sufficiency of transactions and trading volume, the Company has assessed these as level 2 within the fair value hierarchy of ASC 820. The Company classifies its entire investment portfolio as available for sale in accordance with ASC 320, *Debt and Equity Securities*.

### ***Fair Value of Financial Instruments***

The Company's financial instruments, including cash, cash equivalents, accounts receivable, and accounts payable are carried at cost, which approximates their fair value because of the short-term maturity of these instruments.

The Company believes that the carrying value of its debt balance outstanding approximates fair value due to the short term nature.

### ***Concentration of Risk***

Concentration of credit risk exists with respect to cash and cash equivalents, accounts receivable, and investments. The Company maintains its cash and cash equivalents and investments with high quality financial institutions. At times, amounts may exceed federally insured deposit limits.

For the years ended December 31, 2011, 2010 and 2009, 100%, 7% and 100%, respectively, of the Company's revenue was generated from an agreement with one collaboration partner. At December 31, 2011, 2010 and 2009, 60%, 7% and 100%, respectively, of accounts receivable was due from the same collaboration partner.



### ***Fixed Assets***

Property and equipment are recorded at cost and are depreciated and amortized over the shorter of their remaining lease term or their estimated useful lives on a straight-line basis as follows:

Laboratory equipment . . . . .	4-7 years
Office equipment . . . . .	3-5 years
Leasehold improvements . . . . .	Lesser of life of improvement or lease term

Expenditures for maintenance and repairs, which do not improve or extend the useful lives of the respective assets, are expensed as incurred. When assets are sold or retired, the related cost and accumulated depreciation are removed from their respective accounts and any resulting gain or loss is included in income (loss) from operations.

### ***Long-lived Assets***

ASC 360, *Property, Plant and Equipment*, addresses the financial accounting and reporting for impairment or disposal of long-lived assets. The Company reviews the recorded values of long-lived assets for impairment whenever events or changes in business circumstance indicate that the carrying amount of an asset or group of assets may not be fully recoverable.

### ***Research and Development Expenses***

All costs associated with internal research and development, research and development services for which the Company has externally contracted and licensed technology are expensed as incurred. Research and development expense includes direct costs for salaries, employee benefits, subcontractors, including clinical research organizations (“CROs”), operating supplies, facility-related expenses and depreciation.

### ***Patent Costs***

The Company expenses the costs of obtaining and maintaining patents.

### ***Comprehensive Loss***

The Company reports and presents comprehensive loss in accordance with ASC 220, *Comprehensive Income*, which establishes standards for reporting and display of comprehensive loss and its components in a full set of general purpose financial statements. The objective of the statement is to report a measure of all changes in equity of an enterprise that result from transactions and other economic events of the period other than transactions with owners (comprehensive loss). The Company’s other comprehensive income (loss) arises from net unrealized gains or losses on marketable securities.

### ***Income Taxes***

The Company uses an asset and liability approach for financial accounting and reporting of income taxes. Deferred tax assets and liabilities are determined based on temporary differences between financial reporting and tax basis of assets and liabilities and are measured by applying enacted rates and laws to taxable years in which differences are expected to be recovered or settled. Further, the effect on deferred tax assets and liabilities of a change in tax rates is recognized in income in the period that the rate change is enacted. A valuation allowance is required when it is “more likely than not” that all or a portion of deferred tax assets will not be realized.

The Company applies the provisions of ASC 740, *Income Taxes*, which prescribes a comprehensive model for how a company should recognize, measure, present, and disclose in its financial statements uncertain tax positions that the company has taken or expects to take on a tax return (including a decision whether to file or not

file a return in a particular jurisdiction). The financial statements reflect expected future tax consequences of such positions presuming the taxing authorities' full knowledge of the position and all relevant facts.

The Company did not have any unrecognized tax benefits as of December 31, 2011. The Company reviews all tax positions to ensure the tax treatment selected is sustainable based on its technical merits and that the position would be sustained if challenged.

### ***Segment Information***

The Company is engaged solely in the discovery and development of innovative anti-infective drug therapies. Accordingly, the Company has determined that it operates in one operating segment.

### ***Accounting Standards Updates***

In October 2009, an update was made to the Accounting Standards Codification ("ASC") 605, *Revenue Recognition*, which provides accounting principles and application guidance on how revenue arrangements with multiple deliverables should be separated and the consideration allocated. Assuming other criteria are met, this guidance eliminates the requirement to establish the fair value of undelivered products and services and instead provides for separate revenue recognition. Allocation of consideration is now based on management's estimate of the selling price for an undelivered item where there is no other means to determine the fair value of that undelivered item. This update was effective prospectively for revenue arrangements entered into or materially modified in fiscal years beginning on or after June 15, 2010. The Company adopted this guidance as of January 1, 2011. There was no impact to the Company's financial statements upon adoption of this standard as there were no new or materially modified agreements.

In June 2011, the FASB issued ASU No. 2011-05 "Comprehensive Income: Presentation of Comprehensive Income." Under the amendment, an entity will have the option to present the total of comprehensive income, the components of net income, and the components of other comprehensive income either in a single continuous statement of comprehensive income or in two separate but consecutive statements. This amendment, therefore, eliminates the currently available option to present the components of other comprehensive income as part of the statement of changes in stockholders' equity. The amendment does not change the items that must be reported in other comprehensive income or when an item of other comprehensive income must be reclassified to net income. In December 2011, the FASB issued ASU No. 2011-12, "Comprehensive Income: Deferral of the Effective Date for Amendments to the Presentation of Reclassifications of Items Out of Accumulated Other Comprehensive Income in Accounting Standards Update No. 2011-05" ("ASU 2011-12"). ASU 2011-12 defers changes in Update 2011-05 that relate to the presentation of reclassification adjustments. ASU 2011-12 is effective for fiscal years, and interim periods within those years, beginning after December 15, 2011. As this guidance relates to presentation only, the adoption of this guidance will not have any other effect on the Company's financial statements.

## **3. Financing Activities**

### ***Public Offerings***

In June 2011, the Company entered into an underwriting agreement (the "Underwriting Agreement") with Merrill Lynch, Pierce, Fenner & Smith Incorporated and Leerink Swann LLC, as underwriters (the "Underwriters") related to a public offering of shares of the Company's common stock, par value \$.001 per share, at a price of \$5.90 per share less underwriting discounts and commissions (the "Offering"). The Company issued and sold an aggregate of 11,040 shares of common stock in connection with the Offering and the exercise of the over-allotment option that was granted to the underwriters in the Underwriting Agreement. The Offering resulted in net proceeds to the Company of \$60,947, after deducting offering related expenses and underwriting discounts. The Company intends to use the net proceeds to continue clinical testing of ACH-1625, ACH-2684 and ACH-2928, to initiate clinical testing of ACH-3102 and for general corporate expenses.

In January 2010, the Company entered into an underwriting agreement (the “Underwriting Agreement”) with Roth Capital Partners, LLC, Noble Financial Capital Markets and National Securities Corporation, as underwriters (the “Underwriters”), related to a public offering of shares of the Company’s common stock, par value \$.001 per share, at a price of \$2.08 per share less underwriting discounts and commissions (the “Offering”). The Company issued and sold 10,275 shares of common stock in connection with the Offering in January 2010. In February 2010, the Company issued and sold an additional 1,541 shares of common stock in connection with the exercise of the over-allotment option that was granted to the underwriters in the Underwriting Agreement. The Offering resulted in net proceeds to the Company of \$22,628.

**Private Placements**

In August 2010, the Company issued 19,775 shares of the Company’s common stock at a price of \$2.49 per share, as well as warrants to purchase 0.35 shares of common stock for each share issued (the “Common Warrants”) of common stock underlying each Common Warrant in a private placement to institutional and other accredited investors (the “Private Placement”). The Common Warrants, which represent the right to acquire an aggregate of 6,921 shares of common stock, expire on August 20, 2017, and are exercisable at a price of \$3.1125 per share. The warrants allow for a net share settlement. The Private Placement resulted in net proceeds to the Company of \$49,934. The fees associated with issuing the shares in the private placement were \$171 and were recorded as a reduction of additional paid-in capital.

The Common Warrants issued in the Private Placement meet the conditions necessary for equity classification pursuant to ASC 815, *Derivatives and Hedging*.

Pursuant to the Company’s obligations, in September 2010, the Company filed a registration statement with the Securities and Exchange Commission covering the resale of the 19,775 shares of common stock issued in the Private Placement and the 6,921 shares of common stock issuable upon the exercise of the Common Warrants. This registration statement was declared effective by the Securities and Exchange Commission on September 30, 2010.

**4. Earnings (Loss) Per Share (“EPS”)**

Basic EPS is calculated in accordance with ASC 260, *Earnings Per Share*, by dividing net income or loss attributable to common stockholders by the weighted average common stock outstanding. Diluted EPS is calculated by adjusting weighted average common shares outstanding for the dilutive effect of common stock options and warrants. In periods in which a net loss is recorded, no effect is given to potentially dilutive securities, since the effect would be antidilutive. Securities that could potentially dilute basic EPS in the future were not included in the computation of diluted EPS because to do so would have been antidilutive. Potentially dilutive securities were as follows for the years ended December 31, 2011, 2010 and 2009 (prior to consideration of the treasury stock method):

	<u>Years Ended December 31,</u>		
	<u>2011</u>	<u>2010</u>	<u>2009</u>
Options .....	6,610	5,860	3,320
Warrants .....	9,650	9,677	2,785
Total potentially dilutive securities outstanding .....	<u>16,260</u>	<u>15,537</u>	<u>6,105</u>

**5. Collaboration Arrangements**

***Gilead Sciences, Inc.***

In November 2004, the Company entered into a research collaboration and license agreement with Gilead Sciences, Inc. pursuant to which the Company agreed to collaborate exclusively with Gilead throughout the world to develop and commercialize compounds for the treatment of chronic hepatitis C, or HCV, that inhibit HCV replication through a novel mechanism of action targeting the HCV NS4A protein.

The Company received \$10,000 from Gilead upon the execution of the license agreement, of which \$2,000 was allocated to the fair value of the preferred stock purchased. The remaining \$8,000 of the non-refundable up-front license fee, as well as a \$2,000 milestone achieved during the period prior to achievement of proof-of-concept, are being accounted for under the proportionate performance model.

Due to certain provisions contained within the Gilead Arrangement relating to services to be performed on both the primary and back-up compounds, as defined, the non-refundable up-front license fee of \$8,000, as well as a \$2,000 milestone achieved during the period prior to achievement of proof-of-concept (the "Research Period"), were accounted for under the proportionate performance model.

During the years ended December 31, 2011, 2010 and 2009, the Company did not recognize revenue from upfront, milestone and FTE fees previously received under the collaboration. In the February 2012, the Company will recognize the remaining deferred revenue balance relating to upfront, milestone and FTE payments received under the collaboration coincident with the termination of the agreement.

During the years ended December 31, 2011, 2010 and 2009, the Company recognized cost-sharing revenue of \$247, \$180 and \$(294), respectively, of external costs billed by the Company to Gilead, net of Gilead billings to the Company of \$0, \$0 and \$523 for the years ended December 31, 2011, 2010 and 2009, respectively. Payments to Gilead under this collaboration are recognized as a reduction in revenue. External costs incurred by Gilead exceeded amounts incurred by the Company for the year ended December 31, 2009, resulting in the recognition of negative revenue for the year ended December 31, 2009.

Included in the accompanying balance sheets as of December 31, 2011 and 2010 are \$62 and \$18 respectively, of accounts receivable resulting from this collaboration agreement and \$2,489 and \$2,489, respectively, of deferred revenue resulting from the up-front fee, a milestone payment and FTE costs.

### ***GCA Therapeutics, Ltd.***

In February 2010, the Company entered into a license agreement (the "Agreement") with GCA Therapeutics, Ltd. ("GCAT") for elvucitabine, the Company's nucleoside reverse transcriptase inhibitor for the treatment of both hepatitis B virus ("HBV") infection and human immunodeficiency virus ("HIV") infection. The Agreement was amended and restated in March 2010. The exclusive license grants GCAT the right, through a Chinese joint venture with Tianjing Institute of Pharmaceutical Research, to clinically develop and commercialize elvucitabine in mainland China, Hong Kong and Taiwan.

Under the terms of the Agreement, GCAT, through a sublicense agreement with a Chinese joint venture, T&T Pharma Co., Ltd., will assume all development and regulatory responsibility and associated costs for elvucitabine. There was no financial impact upon the signing of the agreement. The Company will be eligible to receive development milestones and royalties on net sales in those territories.

The Agreement may be terminated by either party based upon material breaches by the other party, effective 90 days after providing written notice to the breaching party, if the breaching party fails to cure its material breach.

The Company may terminate the Agreement upon 30 days written notice in the event GCAT fails to meet any of the development or commercialization diligence milestones by the deadlines specified in the Agreement, or may terminate upon 90 days written notice in the event of a change of corporate control. In the event of a change of control, as defined, the Company shall pay GCAT termination fees, in an amount determined based upon specified progress milestones.

## 6. Marketable Securities

The fair value of the Company's marketable securities of \$63,833 and \$29,827 as of December 31, 2011 and 2010, respectively, is valued based on level 2 inputs. The Company's investments consist mainly of U.S. government and agency securities, government sponsored bond obligations and certain other corporate debt securities. Fair value is determined based upon quoted market prices; however, due to lack of sufficiency of transactions and trading volume, the Company has assessed these as level 2 within the fair value hierarchy of ASC 820. There were no transfers between levels within the hierarchy during the year ended December 31, 2011. The Company classifies its entire investment portfolio as available for sale as defined in ASC 320, *Debt and Equity Securities*. Securities are carried at fair value with the unrealized gains (losses) reported as a separate component of stockholders' equity.

The unrealized (loss) gain from marketable securities was \$(20) and \$2 at December 31, 2011 and 2010, respectively.

As of December 31, 2011, none of the Company's investments were determined to be other than temporarily impaired.

The following table summarizes the Company's investments:

	<u>As of December 31,</u>					
	<u>2011</u>			<u>2010</u>		
	<u>Amortized Cost</u>	<u>Unrealized Gain (Loss)</u>	<u>Estimated Fair Value</u>	<u>Amortized Cost</u>	<u>Unrealized Gain (Loss)</u>	<u>Estimated Fair Value</u>
Commercial Paper . . . . .	\$19,488	\$ 11	\$19,499	\$17,482	\$ 9	\$17,491
Corporate Debt Securities . . . . .	12,866	(18)	12,848	12,343	(7)	12,336
Government and Agency Securities . . .	28,499	(13)	28,486	—	—	—
Certificate of Deposit . . . . .	3,000	—	3,000	—	—	—
Total . . . . .	<u>\$63,853</u>	<u>\$ (20)</u>	<u>\$63,833</u>	<u>\$29,825</u>	<u>\$ 2</u>	<u>\$29,827</u>

The following additional table summarizes, by industry, the fair value of investments:

	<u>As of December 31,</u>	
	<u>2011</u>	<u>2010</u>
Government . . . . .	\$34,989	\$ 1,249
Banking . . . . .	21,070	12,323
Non-financial . . . . .	7,774	16,255
Total . . . . .	<u>\$63,833</u>	<u>\$29,827</u>

## 7. Prepaid Expenses and Other Current Assets

A summary of prepaid expenses and other current assets is as follows:

	<u>As of December 31,</u>	
	<u>2011</u>	<u>2010</u>
Prepaid research and development costs . . . . .	\$ 343	\$1,250
Tax credit receivable . . . . .	420	130
Maintenance agreements . . . . .	159	218
Interest receivable . . . . .	260	288
Prepaid insurance . . . . .	31	60
Prepayment of equipment . . . . .	159	—
Other prepaid expenses . . . . .	51	106
Total . . . . .	<u>\$1,423</u>	<u>\$2,052</u>

## 8. Fixed Assets, net

A summary of property and equipment is as follows:

	As of December 31,	
	2011	2010
Laboratory equipment . . . . .	\$ 2,907	\$ 2,883
Office equipment . . . . .	658	588
Leasehold improvements . . . . .	2,919	2,898
	6,484	6,369
Less—accumulated depreciation and amortization . . . . .	(5,490)	(5,901)
Total . . . . .	<u>\$ 994</u>	<u>\$ 468</u>

Depreciation expense was \$317, \$571 and \$936 for the years ended December 31, 2011, 2010 and 2009, respectively.

## 9. Accrued Expenses

Accrued expenses consist of the following:

	As of December 31,	
	2011	2010
Accrued compensation . . . . .	\$1,169	\$ 978
Accrued research and development expenses . . . . .	2,341	676
Accrued professional expenses . . . . .	281	317
Other accrued expenses . . . . .	217	90
Total . . . . .	<u>\$4,008</u>	<u>\$2,061</u>

Accrued research and development expenses are comprised of amounts owed to third-party contract research organizations or CROs, clinical investigators, laboratories and data managers for research and development work performed on behalf of the Company.

## 10. Debt

Debt consists of the following:

	As of December 31,	
	2011	2010
2011 Credit Facility, payable in equal monthly installments through June 2014, with fixed interest of 6.79% . . . . .	\$ 370	\$ —
2008 Credit Facility, payable in monthly installments as notes mature through March 2011, with interest of 9.97% to 11.58% per annum . . . . .	—	469
Total long-term debt . . . . .	370	469
Less: current portion . . . . .	(141)	(469)
Total long-term debt, net of current portion . . . . .	<u>\$ 229</u>	<u>\$ —</u>

In March 2011, the Company entered into a Master Security Agreement for a \$2,000 Capital Expenditure Line of Credit, (“the 2011 Credit Facility”) with Webster Bank. Under the 2011 Credit Facility, the Company may draw down equipment loan advances for the purchase of new laboratory equipment through March 2012. The purchased equipment serves as collateral for the 2011 Credit Facility. Through December 31, 2011, the Company had drawn down \$438 under the 2011 Credit Facility.

In February 2008, the Company entered into a credit facility with GE Capital Corporation and Oxford Finance Corporation, (“the 2008 Credit Facility”). The 2008 Credit Facility provided an incremental \$5,000 to fund the Company’s working capital needs and was collateralized by substantially all of the Company’s tangible assets. In connection with the 2008 Credit Facility, the Company issued warrants to purchase 43 shares of common stock at an exercise price of \$4.68 per share. The fair value of the warrants at the date of issuance was estimated to be \$155, utilizing the Black Scholes method, and was recorded as a debt discount. This amount was amortized as interest expense over the term of the loan.

The Company believes that the carrying value of its debt balance outstanding approximates fair value due to the short term nature.

## **11. Capital Structure**

### *Preferred Stock*

At December 31, 2011, the Company had 5,000 authorized shares of undesignated preferred stock of which no shares were issued and outstanding.

### *Common Stock*

At December 31, 2011, the Company had 200,000 authorized shares of \$0.001 par value common stock. As of December 31, 2011 there were 16,582 shares reserved for future exercise of outstanding stock options, warrants and shares available for issuance under the Company’s 2006 Stock Incentive Plan and 2006 Employee Stock Purchase Plan.

In June 2011, the stockholders of the Company approved an amendment to the Company’s Amended and Restated Certificate of Incorporation to increase the Company’s authorized shares of common stock from 100,000,000 to 200,000,000.

### *Warrants*

At December 31, 2011, there were 9,650 warrants outstanding with a weighted average exercise price of \$3.25.

## **12. Stock-Based Compensation**

### *1998 Stock Option Plan*

The Company’s 1998 Stock Option Plan, or the 1998 Plan, as amended and restated, was adopted by the Company’s board of directors in January 2000 and approved by its stockholders in March 2000. A maximum of 1,094 shares of common stock were authorized for issuance under the 1998 Plan.

The 1998 Plan, as amended, provided for the grant of options intended to qualify as incentive stock options under Section 422 of the Internal Revenue Code of 1986, as amended, and nonqualified stock options. The Company’s employees, officers, directors, consultants and advisors were eligible to receive options under the 1998 plan. Under present law, however, incentive stock options may only be granted to the Company’s employees. The Plan was administered by the Company’s board of directors.

Following the adoption of the 2006 Stock Incentive Plan described below, the Company no longer grants stock options or other awards under the 1998 Plan.

### *2006 Stock Incentive Plan*

The Company's 2006 Stock Incentive Plan, or the 2006 Plan, was adopted by the Company's board of directors in May 2006, amended by its board of directors in September 2006, approved by its stockholders in September 2006 and became effective in October 2006, upon the closing of the Company's initial public offering. The Company originally reserved for issuance 750 shares of common stock under the 2006 Plan. In addition, the Plan contained an "evergreen" provision, which allowed for an annual increase in the number of shares available for issuance under the Plan on the first day of each fiscal year during the period beginning on the first day of fiscal year 2007 and ending on the second day of fiscal year 2010. Under the evergreen provision, the Company registered an additional 2,673 shares of common stock to be issued under the 2006 Plan.

On June 10, 2010, stockholders of the Company approved an amendment to the 2006 Plan to increase by 3,000 shares the number of shares of common stock reserved for issuance under the 2006 Plan from 3,423 shares to 6,423 shares.

The 2006 Plan provides for the grant of incentive stock options, nonstatutory stock options, restricted stock, restricted stock units, stock appreciation rights and other stock-based awards. The Company's officers, employees, consultants, advisors and directors, and those of any subsidiaries, are eligible to receive awards under the 2006 Plan; however, incentive stock options may only be granted to employees.

The Company's board of directors administers the 2006 Plan, although it may delegate its authority to a committee. The board, or a committee to which it has delegated its authority, will select the recipients of awards and determine, subject to any limitations in the 2006 Plan:

- the number of shares of common stock covered by options and the dates upon which those options become exercisable;
- the exercise prices of options;
- the duration of options;
- the methods of payment of the exercise price; and
- the number of shares of common stock subject to any restricted stock or other stock-based awards and the terms and conditions of those awards, including the conditions for repurchase, issue price and repurchase price.

Options granted under the Company's 1998 Stock Option Plan and 2006 Stock Incentive Plan (the "Plans"), are exercisable for a period determined by the Company, but in no event longer than ten years from the date of the grant. Options generally vest ratably over four years.

As of December 31, 2011, there were 100 shares available to be granted under the 2006 Plan.



A summary of the status of the Company's stock option activity for the year ended December 31, 2011 is presented in the table and narrative below:

	<u>2011</u>	
	<u>Options</u>	<u>Weighted Average Exercise Price</u>
Outstanding at January 1, 2011 .....	5,860	\$3.67
Granted .....	1,084	7.55
Exercised .....	(320)	1.78
Forfeited/Cancelled .....	(14)	2.41
Outstanding at December 31, 2011 .....	<u>6,610</u>	<u>\$4.40</u>
Options exercisable at December 31, 2011 .....	<u>3,230</u>	<u>\$4.45</u>
Options vested and expected to vest at December 31, 2011 .....	<u>6,403</u>	<u>\$4.44</u>

The following table summarizes information about stock options outstanding at December 31, 2011:

<u>Range of Exercise Prices</u>	<u>Options Outstanding</u>			<u>Options Vested</u>	
	<u>Number Outstanding</u>	<u>Weighted Average Remaining Contractual Life (Years)</u>	<u>Weighted Average Exercise Price</u>	<u>Number Vested</u>	<u>Weighted Average Exercise Price</u>
\$0.00 – \$2.00 .....	1,023	5.8	\$ 1.17	807	\$ 1.21
\$2.01 – \$4.00 .....	3,461	8.4	3.17	1,337	3.24
\$4.01 – \$6.00 .....	686	6.0	5.04	661	5.01
\$6.01 – \$8.00 .....	1,090	9.8	7.59	75	7.50
\$12.01 – \$14.00 .....	2	4.8	14.00	2	14.00
\$14.01 – \$16.00 .....	344	4.9	14.75	344	14.75
\$18.01 – \$20.00 .....	4	5.1	19.00	4	19.00
	<u>6,610</u>	<u>7.8</u>	<u>\$ 4.40</u>	<u>3,230</u>	<u>\$ 4.45</u>

As of December 31, 2011, the intrinsic value of the options outstanding and options vested was \$23,818 and \$12,758, respectively. The intrinsic value for stock options is calculated based on the difference between the exercise prices of the underlying awards and the quoted stock price of the Company's common stock as of the reporting date.

The total intrinsic value of stock options exercised for the years ended December 31, 2011, 2010 and 2009 was \$1,721, \$2 and \$1, respectively.

The weighted-average grant-date fair value of options granted during the years ended December 31, 2011, 2010 and 2009 was \$5.40, \$2.25 and \$2.08, respectively. The weighted-average grant-date fair value of options vested at December 31, 2011 and 2010 was \$3.35 and \$3.66, respectively.

The weighted average remaining contractual life is 6.6 years for options exercisable and 7.8 years for options vested and expected to vest.

#### *Stock Based Compensation*

Under the provisions of ASC 718, stock-based compensation cost is based on the fair value of the portion of stock-based awards that is ultimately expected to vest during the period. Stock-based compensation expense recognized during the year ended December 31, 2009 includes compensation expense for stock-based awards

granted prior to, but not yet vested as of December 31, 2005, based on the fair value on the grant date and compensation expense for the stock-based awards granted subsequent to December 31, 2005.

The Company utilizes the Black-Scholes option pricing model for determining the estimated fair value for stock-based awards. The Black-Scholes model requires the use of assumptions which determine the fair value of the stock-based awards. Determining the fair value of stock-based awards at the grant date requires judgment, including estimating the expected term of stock options, the expected volatility of our stock and expected dividends. The Company is also required to estimate forfeitures at the grant date and recognize compensation costs for only those awards that are expected to vest. Judgment is required in estimating the amount of stock-based awards that are expected to be forfeited. In addition, due to the Company's limited exercise history, the Company utilizes the simplified method in developing an estimate of expected term of "plain vanilla" options.

The assumptions used to value options granted are as follows:

	For the Years Ended December 31,		
	2011	2010	2009
Expected term of option . . . . .	5.0 - 6.1 years	5.0 - 6.1 years	6.1 years
Expected volatility . . . . .	87% - 88%	86% - 87%	79% - 82%
Risk free interest rate . . . . .	1.09 - 2.57%	1.59 - 2.92%	1.97 - 3.04%
Expected dividend yield . . . . .	0%	0%	0%

Total compensation expense recorded in the accompanying statements of operations associated with option grants made to employees for the years ended December 31, 2011, 2010 and 2009 was \$2,747, \$2,163 and \$1,892, respectively. The Company recorded no tax benefit related to these options as the Company is currently in a net operating loss position and maintains a full valuation allowance.

As of December 31, 2011, the total compensation cost related to options not yet recognized in the financial statements is approximately \$9,853, net of estimated forfeitures, and the weighted average period over which it is expected to be recognized is 1.8 years.

#### *2006 Employee Stock Purchase Plan*

The Company established an Employee Stock Purchase Plan effective December 1, 2006 (the "2006 ESPP Plan"). Eligible employees can purchase common stock pursuant to payroll deductions at a price equal to 85% of the lower of the fair market value of the common stock at the beginning or end of each six-month offering period. The Company originally reserved for issuance 250 shares of common stock under the 2006 ESPP Plan. On June 10, 2010, stockholders of the Company approved an amendment to the 2006 ESPP Plan to increase by 250 shares the number of shares of common stock reserved for issuance under the 2006 ESPP Plan from 250 shares to 500 shares.

The Company measures the fair value of issuances under the 2006 ESPP Plan using the Black-Scholes option pricing model at the end of each reporting period. The compensation cost for the Plan consists of the 15% of the grant date stock price discount and the fair value of the option features.

The Company recorded compensation cost related to 2006 ESPP Plan of \$67, \$39 and \$48 for the years ended December 31, 2011, 2010 and 2009, respectively. As of December 31, 2011, 222 shares remained available for future issuance under the 2006 ESPP Plan.

### **13. Restructuring**

In July 2009, the Company implemented a restructuring plan that reduced employee headcount by approximately 25% to approximately 40. The restructuring plan was implemented following a strategic assessment of the Company's portfolio of therapeutic compounds. During the assessment, the Company's management and board of directors determined that the Company would focus its discovery and development

efforts on its HCV candidates and therefore prioritized certain projects. The Company assessed the staffing levels required to accomplish its revised goals, resulting in a reduction of staff across all functional areas. In connection with this reduction, the Company offered individuals whose employment was terminated a severance package that included severance pay, continuation of benefits and outplacement services. The Company paid \$274 of severance benefits.

#### **14. Other License and Research and Development Agreements**

The Company has entered into certain HCV license and collaborative research agreements with third parties relating to the Company's drug discovery and development initiatives. Under these agreements, the Company has been granted certain worldwide non-exclusive licenses to use the licensed compounds or technologies. Included in the accompanying 2011, 2010 and 2009 statements of operations is \$145, \$140 and \$145, respectively, of research and development expense resulting from these arrangements. In order to maintain its rights under these agreements, provided that the Company does not terminate such agreements, the Company will also be required to pay an additional \$475 of aggregate minimum payments over the next five years.

In February 2000, the Company entered into a license agreement with Vion Pharmaceuticals, ("Vion"), pursuant to which it obtained a worldwide exclusive sublicense from Vion on the composition of matter and use of elvucitabine. Vion's license rights were granted to it by Yale University, ("Yale"). Upon the dissolution of Vion in a 2011 bankruptcy, the Company became a direct licensee of Yale. This license covers the use of elvucitabine alone, as a pharmaceutical composition containing elvucitabine alone, or its use as monotherapy to treat HIV. Yale has retained rights to utilize the intellectual property licensed by this agreement for its own noncommercial purposes. Through December 31, 2011, the Company has made aggregate payments of \$35 to Yale under this agreement, including a \$10 initial license fee and a \$25 development milestone payment. Under the terms of the agreement, the Company may be required to make additional milestone payments to Yale of up to an aggregate of \$850 for each licensed product based on the achievement of specified development and regulatory approval milestones. The Company is also required to pay Yale specified royalties on net product sales and a specified share of sublicensing fees that it receives under any sublicenses that it grants. No other payments are included in the Company's financial statements as these payments are contingent on the achievement of certain milestones that have not yet been reached.

In July 2002, the Company entered into a license agreement with Emory University ("Emory"), pursuant to which it obtained a worldwide exclusive license under specified licensed patents to use elvucitabine in combination with other antivirals. Under the license, Emory retains a right to use the intellectual property for educational and research purposes only and also retains the right to approve sublicenses under specified circumstances. Through December 31, 2011, the Company has made aggregate payments of \$150 to Emory under this agreement, including an initial license fee of \$100 and a development milestone payment of \$50. The Company may also be required to make additional payments of up to an aggregate of \$400 based on the achievement of specified development and regulatory approval milestones. Under this agreement, the Company is also required to pay Emory specified royalties on net product sales and a specified share of sublicensing fees that it receives under any sublicenses that it grants. As these payments are contingent on the achievement of certain milestones that have not yet been reached, the related amounts are not recognized as expense in the accompanying financial statements.

#### **15. Commitments**

##### ***401(k) Retirement Plan***

The Company has a 401(k) defined contribution retirement plan covering substantially all full-time employees. The Company currently matches employee contributions at a rate of \$0.50 cents for each dollar contribution, up to 6% of salary deferrals. However, the decision to match any employee contributions is at the sole discretion of the Company. During the year ended December 31, 2009, the Company only made matching contributions through the first half of 2009. The Company made matching contributions of \$177, \$165 and \$89 for the years ended December 31, 2011, 2010 and 2009.

### *Operating Leases*

The Company leases its operating facility located in New Haven, Connecticut. The lease agreements require monthly lease payments through March 2017. The Company is recording the expense associated with the lease on a straight-line basis over the expected seven-year term of the lease and, as a result, has accrued \$72 and \$31 at December 31, 2011 and 2010, respectively.

The future minimum annual lease payments under these operating leases at December 31, 2011 are as follows:

<u>Year Ended December 31,</u>	
2012 .....	\$ 598
2013 .....	\$ 606
2014 .....	\$ 630
2015 .....	\$ 638
2016 .....	\$ 662
2017 .....	\$ 168
Total .....	<u>\$3,302</u>

Rent expense under operating leases was approximately \$616, \$693 and \$983 for the years ended December 31, 2011, 2010 and 2009, respectively.

### **16. Income Taxes**

The Company uses an asset and liability approach for financial accounting and reporting of income taxes. Deferred tax assets and liabilities are determined based on temporary differences between financial reporting and tax basis of assets and liabilities and are measured by applying enacted rates and laws to taxable years in which differences are expected to be recovered or settled. Further, the effect on deferred tax assets and liabilities of a change in tax rates is recognized in income in the period that the rate changes.

The Company applies the provisions of ASC 740, *Income Taxes*, which prescribes a comprehensive model for how a company should recognize, measure, present, and disclose in its financial statements uncertain tax positions that the company has taken or expects to take on a tax return (including a decision whether to file or not file a return in a particular jurisdiction). The Company's financial statements reflect expected future tax consequences of such positions presuming the taxing authorities' full knowledge of the position and all relevant facts.

The Company does not have any interest or penalties accrued related to uncertain tax positions as it does not have any unrecognized tax benefits. In the event the Company determines that accrual of interest or penalties is necessary in the future, the amount will be presented as a component of income taxes.

The income tax provision (benefit) consists of the following:

	<u>As of December 31,</u>		
	<u>2011</u>	<u>2010</u>	<u>2009</u>
Deferred:			
Federal and state .....	\$(19,855)	\$(10,882)	\$(11,326)
Valuation allowance .....	19,855	10,882	11,326
Total deferred .....	<u>\$ —</u>	<u>\$ —</u>	<u>\$ —</u>

A reconciliation of the provision for income taxes at statutory rates to the provision in the financial statements is as follows:

	<b>Years Ended December 31,</b>		
	<b>2011</b>	<b>2010</b>	<b>2009</b>
Federal statutory rate .....	(34.0)%	(34.0)%	(34.0)%
State tax, net of federal benefit .....	(5.0)	(5.0)	(5.0)
Other .....	0.1	0.1	0.1
Share-based compensation .....	1.3	4.2	3.9
Valuation allowance .....	37.6	34.7	35.0
	<u>0%</u>	<u>0%</u>	<u>0%</u>

Future tax benefits (deferred tax assets) related to temporary differences are as follows:

	<b>As of December 31,</b>	
	<b>2011</b>	<b>2010</b>
<b>Gross deferred tax assets:</b>		
Net operating losses .....	\$ 102,022	\$ 84,206
Tax credits (federal and state) .....	10,132	8,474
Deferred revenue .....	1,033	1,033
Share-based compensation .....	2,781	2,109
Other .....	902	1,192
	<u>\$ 116,870</u>	<u>\$ 97,014</u>
Less—valuation allowance .....	(116,870)	(97,014)
Net deferred tax asset .....	<u>\$ —</u>	<u>\$ —</u>

At December 31, 2011 and 2010, the Company had gross deferred income tax assets of approximately \$116,870 and \$97,014, respectively, which result primarily from net operating loss and tax credit carryforwards. ASC 740 requires that a valuation allowance be established when it is “more likely than not” that all or a portion of deferred tax assets will not be realized. A review of all positive and negative evidence is required when measuring the need for a valuation allowance. The Company’s cumulative loss from inception represents sufficient negative evidence to require a valuation allowance. The Company concluded that it is appropriate to maintain a full valuation allowance for its net deferred tax assets. Additionally, the Company intends to maintain a valuation allowance until sufficient positive evidence exists to support its reversal.

At December 31, 2011 and 2010, the Company had available the following net operating loss and credit carryforwards:

	<b>As of December 31,</b>	
	<b>2011</b>	<b>2010</b>
Federal net operating loss carryforwards .....	\$245,266	\$202,343
State net operating loss carryforwards .....	248,420	205,165
Federal research and development credit carryforwards .....	6,695	5,309
State research and development credit carryforwards .....	3,437	3,165

The Company’s federal net operating loss carryforwards expire commencing in 2018 through 2031 and state net operating loss carryforwards which expire commencing in 2020 through 2031. The Company’s federal research and development credit carryforwards expire commencing in 2015 through 2031. The Connecticut research and development carryforwards have no expiration period.

Deferred tax assets relating to tax benefits of employee stock options have been reduced to reflect exercises. Some exercises resulted in tax deductions in excess of previously recorded benefits based on the option value at the time of grant (“windfalls”). Although these windfalls are reflected in net operating loss carryforwards, the additional tax benefit associated with the windfall is not recognized until the deduction reduces taxes payable. Accordingly, approximately \$794 of the net operating loss carryforwards available, if realized, would be credited to additional paid-in capital.

Utilization of the net operating losses and research and development credit carryforwards may be subject to a substantial annual limitation under Section 382 of the Internal Revenue Code of 1986, or Section 382, due to changes in ownership of the Company that have occurred previously or that could occur in the future. These ownership changes may limit the amount of net operating losses and research and development credit carryforwards that can be utilized annually to offset future taxable income and tax. In general, an ownership change, as defined by Section 382, results from transactions increasing the ownership of certain shareholders or public groups in the stock of a corporation by more than 50 percentage points over a three-year period. Since the Company’s formation, the Company has raised capital through the issuance of capital stock on several occasions which, combined with the purchasing shareholders’ subsequent disposition of those shares, may have resulted in a change of control, as defined by Section 382. Due to the significant complexity and cost associated with a change in control study, and because there could be additional changes in control in the future, the Company has not assessed whether there has been one or more changes in control since the Company’s formation. If the Company has experienced a change of control at any time since Company formation, utilization of its net operating losses or research and development credit carryforwards would be subject to an annual limitation under Section 382. Any limitation may result in expiration of a portion of the net operating loss or research and development credit carryforwards before utilization which would reduce the Company’s gross deferred tax assets.

The federal and state tax authorities could challenge tax positions taken by the Company for the periods for which there are open tax years. Years subject to audit are years in which unused net operating losses were generated that remain open by the statute of limitations. The Company is open to challenge for the periods of 2000 through 2011 in federal and the State of Connecticut jurisdictions.

The Company did not have any unrecognized tax benefits as of December 31, 2011.

The State of Connecticut provides companies with the opportunity to exchange certain research and development credit carryforwards for cash in exchange for foregoing the carryforward of the research and development credit. The program provides for such exchange of the research and development credits at a rate of 65% of the annual research and development credit, as defined. During the years ended December 31, 2011, 2010 and 2009, the Company had recorded a benefit of approximately \$418, \$130 and \$149, respectively, for the estimated proceeds from this exchange. This benefit is recorded as a reduction of research and development expenditures.

## **17. Related Party Transactions**

### *Nicholas Simon*

In connection with Clarus Ventures, LLC’s (“Clarus”) agreement to invest in Achillion, the Board of Directors of the Company elected Nicholas Simon as a Class I member of the Board of Directors to serve until his successor is duly elected and qualified. Mr. Simon is a managing director of Clarus.

In August 2008, Clarus purchased units consisting of 5,164 shares of common stock and common stock warrants to purchase 1,291 shares of common stock for an aggregate purchase price of \$15 million. Additionally, in August 2010, Clarus purchased 4,875 shares of common stock and warrants to purchase 1,706 shares of common stock for an aggregate purchase price of \$12.4 million.

As of December 31, 2011, Clarus is the beneficial owner of approximately 17% of the Company's total issued and outstanding shares.

*Nicole Vitullo*

In connection with Domain Associates, LLC's ("Domain") agreement to invest in Achillion, the Board of Directors of the Company elected Nicole Vitullo of Domain as a Class II member of the Board of Directors on September 30, 2010 to serve until her successor is duly elected and qualified. Ms. Vitullo is a partner at Domain.

In August 2010, Domain purchased 8,032 shares of common stock and warrants to purchase 2,811 shares of common stock for an aggregate purchase price of \$20.4 million.

As of December 31, 2011, Domain is the beneficial owner of approximately 15% of the Company's total issued and outstanding shares.

**18. Unaudited Quarterly Results**

The following tables summarize unaudited quarterly financial data for the years ended December 31, 2011 and 2010. This data has been derived from unaudited financial statements that, in the Company's opinion, include all adjustments necessary for a fair presentation of such information. The operating results for any quarter are not necessarily indicative of results for any future period.

	<b>2011 Quarters</b>			
	<b>First</b>	<b>Second</b>	<b>Third</b>	<b>Fourth</b>
Total operating revenue	\$ 65	\$ 56	\$ 64	\$ 62
Total operating expenses	10,216	11,332	10,537	12,509
Net loss	(10,133)	(11,250)	(10,438)	(12,385)
Net loss per share—basic and diluted	\$ (0.17)	\$ (0.19)	\$ (0.15)	\$ (0.18)
Weighted average number of shares outstanding—basic and diluted	58,389	58,938	69,725	69,755

	<b>2010 Quarters</b>			
	<b>First</b>	<b>Second</b>	<b>Third</b>	<b>Fourth</b>
Total operating revenue	\$ 74	\$ 187	\$ 170	\$ 2,005
Total operating expenses	5,627	6,504	7,347	8,256
Net loss	(5,637)	(6,384)	(7,217)	(6,243)
Net loss per share—basic and diluted	\$ (0.16)	\$ (0.17)	\$ (0.15)	\$ (0.11)
Weighted average number of shares outstanding—basic and diluted	35,576	38,540	47,576	58,356

**18. Subsequent Event**

In February 2012, following on-going discussions between the Company and Gilead, Gilead provided a notice of termination of the collaboration as neither party was devoting significant time to advancing the compounds under the agreement. The Company retains the right to develop ACH-1095, although it does not have current plans to do so.

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## EXHIBIT INDEX

<u>Exhibit No.</u>	<u>Description</u>	<u>Incorporated by Reference</u>			
		<u>Form</u>	<u>SEC Filing date</u>	<u>Exhibit Number</u>	<u>Filed with this 10-K</u>
3.1	Amended and Restated Certificate of Incorporation of the Registrant, as amended to date.			3.1	X
3.2	Amended and Restated Bylaws.	10-K	03/29/07	3.2	
4.1	Specimen Certificate evidencing shares of common stock.	S-1/A	09/22/06	4.1	
† 10.1	Amended and Restated License Agreement, dated March 5, 2010 by and between the Registrant and GCA Therapeutics, Ltd.	10-K	03/11/10	10.5	
† 10.2	License Agreement, dated February 3, 2000, by and between Vion Pharmaceuticals, Inc. and the Registrant, as amended on January 28, 2002.	S-1	03/31/06	10.2	
10.3	Letter Agreement, dated September 22, 2006, by and between the Registrant and Yale University.	S-1	10/10/06	10.2.1	
† 10.4	License Agreement, dated July 19, 2002 by and between the Registrant and Emory University.	S-1	03/31/06	10.3	
10.5	Third Amended and Restated Investor Rights Agreement, dated as of August 11, 2008, by and among the Registrant and the Holders named therein.	S-3	10/06/08	10.5	
10.6	Amendment No. 1 to the Third Amended and Restated Investor Rights Agreement, dated as of August 20, 2010.	S-3	09/17/10	10.4	
10.7	Securities Purchase Agreement, dated as of August 5, 2008, by and among the Registrant and the Purchasers named therein.	S-3	10/06/08	10.1	
10.8	Form of Common Warrant pursuant to the Securities Purchase Agreement dated as of August 5, 2008.	S-3	10/06/08	10.3	
10.9	Registration Rights Agreement, dated as of August 11, 2008, by and among the Registrant and the Purchasers named therein.	S-3	10/06/08	10.4	
10.10	Securities Purchase Agreement, dated as of August 18, 2010, by and among the Registrant and the Holders named therein.	S-3	09/17/10	10.1	
10.11	Form of Common Warrant pursuant to the Securities Purchase Agreement dated as of August 18, 2010.	S-3	09/17/10	10.2	
10.12	Registration Rights Agreement, dated as of August 18, 2010, by and among the Registrant and the Purchasers named therein.	S-3	09/17/10	10.3	
10.13	Lease Agreement by and between the Registrant and WE George Street LLC for Suite 202, dated as of March 6, 2002.	S-1	03/31/06	10.14	
10.14	Lease Agreement by and between the Registrant and WE George Street LLC, dated as of May, 2000.	S-1	03/31/06	10.15	

<u>Exhibit No.</u>	<u>Description</u>	<u>Incorporated by Reference</u>		
		<u>Form</u>	<u>SEC Filing date</u>	<u>Exhibit Number</u>
10.15	Lease Agreements and subsequent Assignment and Assumption of Lease Agreements by and between the Registrant, Yale University and WE George Street LLC for Suites 802, 803, 804.	S-1	03/31/06	10.16
10.16	Surrender and Termination Agreement by and between the Registrant and WE George Street LLC for Suite 803, dated as of December 18, 2009.	8-K	12/22/09	10.1
10.17	Amendment No. 2 to Lease, dated as of March 31, 2010, by and between Achillion Pharmaceuticals, Inc. and WE George Street, LLC.	8-K	04/06/10	10.1
# 10.18	1998 Stock Option Plan, as amended, dated March 30, 2001.	S-1	03/31/06	10.17
# 10.19	Form of Incentive Stock Option Agreement under the 1998 Stock Option Plan.	S-1	03/31/06	10.19
# 10.20	Form of Incentive Stock Option Agreement for Non-Executives under the 1998 Stock Option Plan.	S-1	03/31/06	10.2
# 10.21	Form of Nonstatutory Stock Option Agreement under the 1998 Stock Option Plan.	S-1/A	03/31/06	10.21
# 10.22	2006 Stock Incentive Plan as amended September 18, 2006 and March 9, 2010.	10-K	03/11/10	10.26
# 10.23	Form of Nonstatutory Stock Option Agreement under the 2006 Stock Incentive Plan.	8-K	12/22/10	99.1
# 10.24	Form of Incentive Stock Option Agreement under the 2006 Stock Incentive Plan.	8-K	12/22/10	99.2
# 10.25	2006 Employee Stock Purchase Plan as amended September 18, 2006 and March 9, 2010.	10-K	03/11/10	10.32
# 10.26	Employment Agreement entered into by the Company and Michael D. Kishbauch, dated April 5, 2011.	8-K	04/08/11	10.3
# 10.27	Second Amended and Restated Employment Agreement and Supplemental Severance Agreement, dated as of March 9, 2010, and Supplemental Terms of Compensation, dated as of April 5, 2011, entered into by the Company and Milind S. Deshpande, Ph.D.	8-K	04/08/11	10.1
# 10.28	Employment Agreement entered into by the Company and Gautam Shah, Ph.D., dated April 5, 2011.	8-K	04/08/11	10.5
# 10.29	Second Amended and restated Employment Agreement and Supplemental Severance Agreement, dated as of March 9, 2010, and Supplemental Terms of Compensation, dated as of April 5, 2011, entered into by the Company and Mary Kay Fenton.	8-K	04/08/11	10.2
# 10.30	Employment Agreement entered into by the Company and Elizabeth A. Olek, B.S. Pharm., D.O., M.P.H., dated April 5, 2011.	8-K	04/08/11	10.4
# 10.31	Employment Agreement entered into by the Company and Joseph Truitt, dated April 5, 2011.	8-K	04/08/11	10.6

<u>Exhibit No.</u>	<u>Description</u>	<u>Incorporated by Reference</u>			<u>Filed with this 10-K</u>
		<u>Form</u>	<u>SEC Filing date</u>	<u>Exhibit Number</u>	
10.32	Master Security Agreement and Promissory Notes by and between the Registrant and GE Capital Corporation and Oxford Finance Corporation, dated as of February 26, 2008.	10-K	03/05/08	10.13	
10.33	Form of Common Stock Warrant under Loan and Security Agreement of GE Capital Corporation and Oxford Finance Corporation.	10-K	03/05/08	10.14	
10.34	Master Security Agreement between the Registrant and Webster Bank, National Association, dated as of March 21, 2011.	8-K	03/25/11	10.1	
23.1	Consent of PricewaterhouseCoopers LLP, Independent Registered Public Accounting Firm.				X
31.1	Certification of Chief Executive Officer pursuant to Rule 13a-14(a)/Rule 15d-14(a) of the Securities Exchange Act of 1934				X
31.2	Certification of Chief Financial Officer pursuant to Rule 13a-14(a)/Rule 15d-14(a) of the Securities Exchange Act of 1934				X
32.1	Certification of Chief Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.				X
32.2	Certification of Chief Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.				X
101.CAL	XBRL Taxonomy Calculation Linkbase Document				*
101.INS	XBRL Instance Document				*
101.SCH	XBRL Taxonomy Extension Schema Document				
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document				
101.LAB	XBRL Taxonomy Label Linkbase Document				*
101.PRE	XBRL Taxonomy Presentation Linkbase Document				*

# Management contracts or compensatory plans or arrangement

\* Submitted electronically herewith

† Indicates confidential treatment requested as to certain portions, which portions were omitted and filed separately with the Securities and Exchange Commission pursuant to a Confidential Treatment Request.

Attached as Exhibit 101 to this report are the following formatted in XBRL (Extensible Business Reporting Language): (i) Balance Sheets at December 31, 2011 and December 31, 2010, (ii) Statements of Operations for the years ended December 31, 2011, 2010 and 2009, (iii) Statements of Stockholders' Equity and Comprehensive Loss for the years ended December 31, 2009, 2010 and 2011, (iv) Statements of Cash Flows for the years ended December 31, 2011, 2010 and 2009 and (v) Notes to Financial Statements.

In accordance with Rule 406T of Regulation S-T, the XBRL-related information in Exhibit 101 to this Annual Report on Form 10-K is deemed not filed or part of a registration statement or prospectus for purposes of sections 11 or 12 of the Securities Act, is deemed not filed for purposes of section 18 of the Exchange Act, and otherwise is not subject to liability under these sections.

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**Certification of Chief Executive Officer pursuant to Exchange Act Rules 13a-14 and 15d-14, as adopted pursuant to Section 302 of Sarbanes-Oxley Act of 2002**

I, Michael D. Kishbauch, certify that:

1. I have reviewed this Annual Report on Form 10-K of Achillion Pharmaceuticals, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15(d)-15(f)) for the registrant and have:
  - a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this annual report is being prepared;
  - b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of registrant's board of directors (or persons performing the equivalent functions):
  - a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control and financial reporting.

/s/ MICHAEL D. KISHBAUCH

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**Michael D. Kishbauch**  
**Chief Executive Officer**

Dated: March 8, 2012

**Certification of Chief Financial Officer pursuant to Exchange Act Rules 13a-14  
and 15d-14, as adopted pursuant to Section 302 of Sarbanes-Oxley Act of 2002**

I, Mary Kay Fenton certify that:

1. I have reviewed this Annual Report on Form 10-K of Achillion Pharmaceuticals, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15(d)-15(f)) for the registrant and have:
  - a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this annual report is being prepared;
  - b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of registrant's board of directors (or persons performing the equivalent functions):
  - a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control and financial reporting.

/s/ MARY KAY FENTON

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**Mary Kay Fenton**  
**Chief Financial Officer**

Date: March 8, 2012

**CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO  
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

In connection with the annual report on Form 10-K of Achillion Pharmaceuticals, Inc. (the “Company”) for the period ended December 31, 2011 as filed with the Securities and Exchange Commission on the date hereof (the “Report”), the undersigned, Michael D. Kishbauch, Chief Executive Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350 as adopted by Section 906 of the Sarbanes-Oxley Act of 2002, that:

(1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: March 8, 2012

/s/ MICHAEL D. KISHBAUCH

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**Michael D. Kishbauch**  
**President and Chief Executive Officer**

A signed original of this written statement required by Section 906 has been provided to Achillion Pharmaceuticals, Inc. and will be retained by Achillion Pharmaceuticals, Inc. and furnished to the Securities and Exchange Commission or its staff upon request.

**CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO  
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

In connection with the annual report on Form 10-K of Achillion Pharmaceuticals, Inc. (the "Company") for the period ended December 31, 2011 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Mary Kay Fenton, Chief Financial Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350 as adopted by Section 906 of the Sarbanes-Oxley Act of 2002, that:

(1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: March 8, 2012

/s/ MARY KAY FENTON

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**Mary Kay Fenton**  
**Chief Financial Officer**

A signed original of this written statement required by Section 906 has been provided to Achillion Pharmaceuticals, Inc. and will be retained by Achillion Pharmaceuticals, Inc. and furnished to the Securities and Exchange Commission or its staff upon request.



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# ACHILLION

## CORPORATE INFORMATION

### EXECUTIVE MANAGEMENT AND CORPORATE OFFICERS

**Michael D. Kishbauch**  
President and  
Chief Executive Officer

**Milind S. Deshpande, Ph.D.**  
President, Research and  
Development and  
Chief Scientific Officer

**Mary Kay Fenton**  
Senior Vice President and  
Chief Financial Officer

**Gautam Shah, Ph.D.**  
Senior Vice President and  
Chief Compliance Officer

**Elizabeth Olek, D.O., MPH**  
Senior Vice President and  
Chief Medical Officer

**Joseph Truitt**  
Senior Vice President and  
Chief Commercial Officer



MICHAEL KISHBAUCH



MILIND DESHPANDE



MARY KAY FENTON



GAUTAM SHAH



ELIZABETH OLEK



JOSEPH TRUITT

### BOARD OF DIRECTORS

**Jason Fisherman, M.D.**  
Managing Director  
Advent Healthcare Ventures

**Gary E. Frashier**  
Principal  
Management Associates

**Michael D. Kishbauch**  
President and  
Chief Executive Officer  
Achillion Pharmaceuticals, Inc.

**Dennis Liotta, Ph.D.**  
Professor of Chemistry  
Emory University

**David I. Scheer**  
President  
Scheer & Co., Inc.

**Nicholas Simon**  
Managing Director  
Clarus Ventures

**Robert L. Van Nostrand**  
Former Chief Financial Officer  
of Aureon Laboratories,  
AGI Dermatics and  
OSI Pharmaceuticals

**Nicole Vitullo**  
Partner  
Domain Associates

**David P. Wright**  
President  
CWD Enterprises

### CORPORATE COUNSEL

Wilmer Cutler Pickering Hale and Dorr LLP  
New York, NY

### INDEPENDENT AUDITORS

PricewaterhouseCoopers LLP  
Hartford, CT

### TRANSFER AGENT & REGISTRAR

Computershare Shareholder  
Services, Inc.  
(781) 575-2879  
250 Royall Street  
Canton, MA 02021

### CORPORATE HEADQUARTERS

300 George Street  
New Haven, CT 06511  
(203) 624-7000

### INVESTOR RELATIONS

ir@achillion.com

### COMMON STOCK

Achillion Pharmaceuticals, Inc.  
common stock trades on The  
NASDAQ Global Select Market  
under the symbol ACHN

### ANNUAL MEETING

Tuesday, June 5, 2012  
9:00 a.m. Eastern Daylight Time  
300 George Street  
New Haven, CT 06511

### Important Note About Forward-Looking Statements

This Annual Report contains forward looking statements as to future outcomes, such as plans for our research and development programs, including the expected timing of future IND filings, initiation of clinical trials and reporting of clinical data. Forward-looking statements are based on the Company's current beliefs and expectations. A number of risks and uncertainties could cause actual results to differ materially. For more detailed information on the risks and uncertainties associated with these forward-looking statements and the Company's other activities, see the "Risk Factors" section in the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2011 that accompanies the Annual Report. Achillion does not undertake any obligation to update any forward-looking statements contained in this document as a result of new information, future events or otherwise.



ACHILLION

Achillion Pharmaceuticals, Inc.  
300 George Street • New Haven, CT 06511  
[www.achillion.com](http://www.achillion.com)