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Achillion Pharmaceuticals, Inc. (NASDAQ: ACHN)

Hoping for a Buyout is Not an Investment Strategy

Rating: **HOLD**

ACHN RESEARCH REPORT:

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COMPANY OVERVIEW:

Achillion Pharmaceuticals, Inc. (ACHN) is a biopharmaceutical company that is engaged in the discovery, development and commercialization of innovative treatments for infectious diseases. Within the anti-infective market, the company is primarily focused on developing commercially competitive, short duration combination therapies for the treatment of chronic hepatitis C virus (HCV) that are once-daily and ribavirin-free.

The company's leading drug candidates for treating HCV include the following:

- ACH-3422 -- a NS5B nucleotide polymerase inhibitor currently in phase I clinical development and the cornerstone of ACHN's broad genotypic strategy.
- ACH-3102 -- a NS5A inhibitor currently in phase II clinical development and the cornerstone of ACHN's genotype 1b strategy.
- ACH-2684 -- a NS3/4A protease inhibitor currently being prepared for phase II clinical development.
- sovaprevir -- a NS3/4A protease inhibitor that, until June 10, 2014, had been on clinical hold since last June over concerns that the drug may damage the liver. Now that the FDA's clinical hold has been lifted, the company may resume its phase II clinical trial of sovaprevir.

Although the company is not currently devoting significant resources to other drug candidates, ACHN has also established a pipeline of antibacterial product candidates through collaborative partnerships. Additionally, ACHN has also developed and licensed certain development and commercialization rights to elvucitabine for the treatment of both hepatitis B virus (HBV) and human immunodeficiency virus (HIV) to GCA Therapeutics.

ANTI-INFECTIVE DRUGS:

The market for anti-infective drugs can be divided into three main categories: antivirals, antibacterials (or antibiotics) and antifungals. Due to a variety of treatment challenges, such as the increasing prevalence of drug resistance to certain viruses and bacteria; considerable drug-related adverse side effects in some treatment options; complex dosing schedules; and inconvenient methods of administration (i.e. injection or infusion), it is believed that the anti-infective drug market will continue to see strong growth in the future.

By operating within the anti-infective market, ACHN sees several advantages over other therapeutic markets such as oncology, cardiovascular and central nervous system disorders. For one, infectious disease research and development (R&D) programs generally have shorter development cycle times than these other markets. Further, evidence suggests that systemic anti-infectives have a higher clinical success rate than these other areas as well.[i] Lastly, the emergence of drug resistance to certain infectious diseases, including HCV, creates a large and growing business opportunity for those companies operating within the anti-infective market.

HCV MARKET:

Hepatitis C is an infectious disease primarily affecting the liver caused by HCV, and is spread mainly by blood-to-blood contact associated with intravenous drug use, poorly sterilized medical equipment and transfusions. Chronic hepatitis usually occurs in 55-85% of people[ii] and can lead to permanent liver damage, which can result in the development of liver cancer, liver failure or death. The World Health Organization (WHO) estimates that approximately 130-150 million people in the world have chronic HCV, with the most affected regions being Central and East Asia and North Africa.[iii] About 3 to 4 million people are infected each year and more than 350,000 people die yearly from HCV-related diseases.[iv] In a recent report from January 2014, GBI Research estimated that the HCV global market will grow to a value of $18.6 billion by 2019 -- more than tripling its 2012 market value of $5.8 billion.[v]

RECENT PRICE ACTION:

Shares of ACHN have skyrocketed over the past few days on both speculation and positive news from the FDA. Achillion shares initially rose on mergers and acquisitions (M&A) speculation following news that a competitor -- Idenix Pharmaceuticals (IDIX) -- will be bought by Merck for $3.85 billion. The next day, the company extended those gains on news that the FDA removed their clinical hold of ACHN's sovapreir drug candidate, which had been in place since June 2013. The company's nearly 170% rise over two days was also likely buoyed by short covering as ACHN's short interest accounted for roughly 20% of shares outstanding as of the end of May. Considering the company's float -- the number of shares that are freely bought and sold without restrictions by the public -- is considerably less than ACHN's 96.8 million shares outstanding, a short squeeze is more likely to occur.

COMPETITION:

The market for infectious diseases and HCV in particular is fiercely competitive and rapidly changing. Aside from major and specialty pharmaceutical and biotechnology companies, academic institutions, governmental agencies, and public and private research organizations are all currently working on treatment options for HCV.

All of ACHN's current drug candidates compete with approved treatments from various companies, including interferon-alpha-based products from Roche (Pegasys and Roferon-A) or Merck (Intron-A or Peg-Intron); ribavirin-based products from Merck (Rebetrol), Roche (Copegus), and generic versions sold by various companies; as well as recently-approved protease inhibitors telaprevir by Vertex (Incivek), boceprevir by Merck (Victrelis) and simeprevir by Johnson & Johnson (Olysio); and recently approved nucleotide inhibitor sofosbuvir by Gilead Sciences (Sovaldi).

In addition, ACHN's drug candidates may also compete with all-oral treatments currently in development including protease inhibitors, polymerase inhibitors (nucleoside, nucleotide and nonnucleoside), NS5A inhibitors and cyclophilin inhibitors. These competing drug candidates are being developed by AbbVie, Astra-Zeneca, Boehringer Ingelheim, Bristol-Myers Squibb, Enanta, Gilead, GlaxoSmithKline, Idenix, Johnson & Johnson, Presidio, Medivir, Merck, Novartis, Pfizer, Roche, Valeant and Vertex.

Furthermore, M&A within the industry -- such as the recent buyout of IDIX -- is helping transform the market as these companies work to develop new treatments as well as combine existing treatments

with one another. For example, Merck's decision to purchase IDIX will help strengthen and increase their existing portfolio of HCV drug therapy, specifically in the nucleotide inhibitor market.

ADVANTAGES OF ACHILLION'S PIPELINE:

Many drug developers, including ACHN, have focused on three of the HCV proteins: protease or NS3, polymerase or NS5B, and 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 viral replication.[vi] It is the belief of many experts that drugs targeting these proteins will need to be used in combination with other drugs in order to improve upon the efficacy obtained in current HCV treatment options.

Achillion believes that their NS3/4A protease inhibitors can be used in combination with their NS5A inhibitor for the treatment of HCV, and that this combination therapy has the potential to improve efficacy against the most common US strain known as genotype 1 (and even the difficult to treat genotype 1a), and also develop all oral treatments for patients with genotypes 2 through 6. In addition, among other things, ACHN hopes to shorten treatment durations, reduce adverse side effects, and offer a more convenient, orally available, treatment option.

Overall, the results thus far for ACHN's drug candidates have been promising. For example, their NS5A inhibitor ACH-3102 has demonstrated robust antiviral activity as a single agent, and in combination with ribavirin, even in the presence of pre-existing resistance mutations. Also, compared to Bristol-Myers Squibb's daclatasvir, ACH-3102 demonstrated higher efficacy against the common resistance mutations.[vii]

Meanwhile, the company's NS5B polymerase inhibitor ACH-3422 also demonstrated highly potent activity against all HCV genotypes, inhibiting genotypes 1b, 1a, 2, 3, and 4 at lower concentrations than Gilead's sofosbuvir drug. Further, ACH-3422 demonstrated low risk of showing mitochondrial toxicity, which has been known to make other drug candidates unsafe for further clinical development.[viii] Both ACHN and investors alike are also excited about this drug candidate given its classification as a uridineanalog nucleotide polymerase inhibitor. It is the belief that these nucleotide inhibitors, also called "nucs" or "nukes," are at the cornerstone of HCV combination therapy for their high response rates and short duration of therapy. For example, Gilead's Sovaldi drug is a "nuke" while Idenix is developing one of their own and was supposedly the main reason for Merck's recent purchase.

In addition, the company's HCV protease inhibitors -- ACH-2684 and sovaprevir -- have demonstrated strong *in vitro* potency and good safety profiles in animals. Moreover, in clinical trials completed to date, the compounds have demonstrated efficacy and safety in human subjects as well.[ix] While elevations in liver enzymes were noted in a phase I healthy subject drug-drug interaction study evaluating the effects of sovaprevir with ritonavir-boosted atazanavir, which led to the FDA's clinical hold in June 2013, ACHN found that sovaprevir "has several times greater potency *in vitro* than either the Victrelis (boceprevir) or Incivek (telaprevir) recently approved HCV protease inhibitors."[x] Furthermore, sovaprevir was found to not give rise to certain viral mutations commonly seen with treatment from other protese inhibitors, which has led ACHN to believe that their drug might be a more durable treatment option for HCV than others.[xi]

PAST TROUBLES NOT UNIQUE TO ACHN:

Initially, the FDA's clinical hold on sovaprevir last June sent shares of ACHN plummeting 25%. However, shares fell another 58% in September 2013 when the agency maintained its clinical hold after ACHN provided the FDA with the necessary data they had requested. While this news kept shares depressed until just this week, it is not a unique story to ACHN. In fact, other companies developing HCV treatments have experienced similar obstacles as ACHN has over the past few years.
For example, both Idenix (IDIX) -- the company recently bought by Merck -- and Vertex Pharmaceuticals (VRTX) both had drug candidates that were placed on clinical hold by the FDA. While VRTX's VX-135 drug was placed on partial hold due to similar problems as ACHN regarding elevated liver enzymes, IDIX had two compounds placed on clinical hold. Further, IDIX had to eventually scrap those compounds as well due to their similarity with a drug from Bristol-Myers Squibb that had issues with adverse side effects.

Ultimately, while a clinical hold is not a good sign for a developing drug company, it is certainly not the end of the world for that company. Both IDIX and VRTX were able to overcome the pitfalls associated with the FDA's judgments, and ACHN took a major step forward this week as the FDA lifted the hold on their drug candidate sovaprevir.

FINANCIALS:

As is the case with most development-stage companies, especially in the biopharmaceutical industry, it is difficult to perform a more traditional financial analysis. However, there are some aspects of ACHN's financials that should be highlighted.
First, ACHN has not yet generated revenue from the sale of any of its drugs. The majority of the company's revenue has been derived from a former collaboration with Gilead to develop compounds for use in treating HCV, and was terminated in February 2012. Under the collaboration agreement, ACHN recognized no revenue in 2013, $2.5 million in 2012 and $247,000 in 2011.

Separately, in October 2012, ACHN entered into a license and development agreement with Ora, Inc. for the worldwide development and commercialization of ACH-702 (a topical antibacterial drug candidate). During that year, ACHN recognized $100,000 in revenue upon the initiation of their agreement with Ora and an additional $18,000 upon the sublicensing of the drug to Taejoon Pharmaceutical Co. later that year.

Due to their lack of steady and sufficient revenues, the company has experienced net losses since their inception and has posted growing net losses over the last four years.

	2013	2012	2011	2010	2009
Net Loss (in thousands)	(58,947)	(47,127)	(44,206)	(25,481)	(25,932)
YoY Change	*-25.08%*	*-6.61%*	*-73.49%*	*1.74%*	

Source: ACHN Form 10-K, 31 December 2013

Since inception through March 31, 2014, the company has incurred cumulative losses of nearly $384 million and had an accumulated deficit of approximately -$398 million at March 31, 2014. By comparison, despite both companies being incorporated in 1998, IDIX's accumulated deficit stood at $865 million as of March 31, 2014 -- more than double ACHN's accumulated deficit.

Considering ACHN's lack of revenues and earnings, it is not surprising that the company has primarily financed their operations through the proceeds of stock issuances -- mostly via common stock. In fact, the company's most recent issuance of almost 17 million shares of common stock was in February 2013 and netted the company $133.2 million. As of March 31, 2014, ACHN had approximately $140 million in cash, cash equivalents, and marketable securities -- with about $34 million of that amount in straight cash and cash equivalents -- which the company believes will be sufficient to meet its projected operating requirements through at least March 31, 2015.

However, further dilution to existing stockholders is a significant risk going forward. As shown below, ACHN has grown their share count by over 38% each year on average, and over 254% cumulatively since 2009. Although the company appears to have ample cash on hand to fund their operations for the time being, shareholders should be cognizant of the risks of future dilution, especially as shares climb and near their 52-week high.

	2013	2012	2011	2010	2009
Shares Outstanding	93,983	73,965	64,248	45,079	26,537
YoY Growth	*27.06%*	*15.12%*	*42.52%*	*69.87%*	

Source: ACHN Form 10-K, 31 December 2013

Separately, although the company never had a large long-term debt position on their balance sheet, ACHN recently extinguished their remaining long-term debt in the first quarter of 2014. Moreover, the company's recent debt to equity (D/E) ratio stood at only 0.07 as of March 31, 2014. Essentially, for all intents and purposes, ACHN's balance sheet is relatively strong, albeit boosted mostly by their recent stock issuances.

BOTTOM LINE:

Following multiple examples of consolidation within the HCV space -- including Inhibitex, Pharmasset, and now Idenix -- there is certainly evidence to suggest further acquisitions are likely. While ACHN may or may not be the next company purchased by big pharma, there are several possible suitors who may still be interested in purchasing another biopharmaceutical company involved in HCV treatment. While investing in a company in the hopes of it being acquired is not an investment strategy, ACHN has several things working in its favor.

Most importantly, their leading drug candidates have yielded favorable results, upon which the company expects to build. The recent removal of the FDA's clinical hold on their drug candidate sovaprevir will likely help shares build on their recent rise. Lastly, following the stock's violent move up earlier this week, I would not be shocked to see shares retreat more than their -12.3% retreat over the last two days. Further, it would not be unheard of if the company decided to raise extra cash through a secondary offering if shares remained at current levels despite their already large cash position. As such, I would be a buyer of shares on a pullback and suggest current shareholders hold their shares at these levels for the time being.

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Sources:

[i] ACHN Form 10-K. 31 December 2013. Page 5. [ii] World Health Organization (WHO). Hepatitis C Fact Sheet. April 2014. http://www.who.int/mediacentre/factsheets/fs164/en/
[iii] Ibid.

[iv] Wikipedia. "Hepatitis C." http://en.wikipedia.org/wiki/Hepatitis_C [v] GBI Research. "Hepatitis C Therapeutics in Major Developed Markets to 2019." January 2014. [vi] ACHN Form 10-K. 31 December 2013. Page 7. [vii] Ibid. Page 8. [viii] Ibid. Page 10. [ix] Ibid. Page 11. [x] Ibid. Page 12. [xi] Ibid.

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