At Achillion, we are committed to innovative science, novel approaches to treat rare diseases and most of all, bringing life-changing and live-saving medicines to patients.”

Milind S. Deshpande, Ph.D., President and Chief Executive Officer

COMPANY OVERVIEW

Headquartered in New Haven, CT., Achillion Pharmaceuticals, Inc. is a science-driven, patient-focused company seeking to leverage its strengths across the continuum from discovery to commercialization to provide better treatments for people with serious diseases. The Company employs a highly-disciplined discovery and development approach that has allowed it to pursue best-in-class therapy for chronic hepatitis C (HCV) and build a platform of potent and selective complement factor D inhibitor compounds. Achillion aims to become a fully-integrated pharmaceutical company prepared to bring life-saving medicines to patients with rare diseases.

POTENTIAL TO DELIVER THE FULL PROMISE OF COMPLEMENT-BASED THERAPEUTICS FOR A WIDE VARIETY OF DISEASES

The complement system is part of the body’s first line of defense against infection and trauma, playing a role in both innate and adaptive immunity. However, excessive activation of the complement system can induce inflammation and tissue damage and is associated with a variety of autoimmune and inflammatory diseases. A number of serious rare disorders are thought to be complement-mediated.

Factor D occupies a critical regulatory position in the complement alternative pathway cascade and plays a key role in the amplification loop. Therefore, small-molecule factor D inhibition may provide important therapeutic advantages, including a more profound impact on certain diseases where the alternative pathway is the driver.

Utilizing its elegant structure-guided design approach, Achillion has assembled a proprietary library of over 1,300 potent and specific small-molecule factor D inhibitor compounds that were identified and developed in the company’s own labs. The Company continues to build strong intellectual property around its complement program and is committed to extensive development of this portfolio.

Achillion is developing ACH-4471, its first orally-administered, small-molecule complement factor D inhibitor drug candidate, for the treatment of two serious and rare diseases, paroxysmal nocturnal hemoglobinuria (PNH) and C3 Glomerulopathy (C3G). Achillion is also progressing additional factor D inhibitor compounds for other potential indications, including dry age-related macular degeneration (dry AMD) and chronic obstructive pulmonary disease (COPD).

PIPELINE

<table>
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<tr>
<th>COMPOUND</th>
<th>PROGRAM</th>
<th>DELIVERY</th>
<th>INHIBITOR</th>
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<td>ACH-4471</td>
<td>PNH C3 Glomerulopathy (C3G)</td>
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<td>Factor D</td>
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<td>Dry AMD</td>
<td>Highly water soluble inhibitors Potential &gt;3 month delivery</td>
<td>Ophthalmic</td>
<td>Factor D</td>
<td>Preclinical</td>
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<td>COPD</td>
<td>Crystalline inhibitors Favorable lung PK No systemic exposure</td>
<td>Oral</td>
<td>odalasvir + ALS-335 ± simeprevir</td>
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HEPATITIS C VIRUS CANDIDATE PORTFOLIO

Achillion discovered and developed a comprehensive portfolio of antivirals for the treatment of HCV, including odalasvir, also known as ACH-3102, a second-generation NS5A protease inhibitor.

In May, 2015 Achillion announced an exclusive worldwide collaboration with Janssen Pharmaceuticals, Inc. (Janssen) for the treatment of HCV. This collaboration created a combined pipeline that has the potential to provide more effective and shorter duration regimens for the treatment of HCV, a disease that affects more than 150 million patients worldwide. Janssen is currently advancing the combined pipelines in ongoing phase 2 clinical trials evaluating regimens consisting of AL-335, a NS5B nucleotide polymerase inhibitor, and odalasvir, with or without simeprevir, a marketed NS3/4A protease inhibitor. Results from the ongoing studies evaluating these doublet and triplet regimens are expected to be reported during 2016.

Under the collaboration, Achillion is eligible to receive up to $905 million in development, regulatory and commercial milestones. Furthermore, Achillion has the potential to receive royalties from mid-teens to low-twenties percentages on worldwide sales of any regimen containing at least one Achillion HCV candidate.
REDEFINING BEST-IN-CLASS ANTIVIRAL THERAPY FOR HCV

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ACHILLION IS FOCUSED ON HELPING PATIENTS WITH RARE OR UNDERSERVED DISEASES

Achillion’s overarching goal is to deliver improved clinical outcomes for patients who suffer from underserved diseases, particularly orphan or rare conditions. Achillion has successfully expanded its focus from its HCV portfolio – now under clinical development in the Janssen collaboration – with its small-molecule complement inhibitor platform, and is well funded to pursue the full potential of its powerful development engine. Armed with a disruptive approach to treating multiple complement diseases, Achillion is growing rapidly and becoming a fully integrated commercial pharmaceutical company as it extends its commitment to advancing science and improving human health.