

*"At Achillion, we aim to be as heroic in all of our efforts as the patients and families we are working to help, as they cope with serious diseases."*

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

## COMPANY OVERVIEW

Achillion Pharmaceuticals, Inc. is a science-driven, patient-focused company leveraging 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 specific complement inhibitors. Achillion is rapidly becoming a fully-integrated pharmaceutical company preparing 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.

Achillion is currently focused on small-molecule complement factor D inhibitors. Factor D occupies a critical regulatory position in the complement alternative pathway cascade and plays a key role in the amplification loop. Utilizing its elegant structure-guided design approach, Achillion has assembled a proprietary library of over 1,000 potent and specific small-molecule factor D inhibitors. The Company is building a strong intellectual property estate around its complement factor D program, with nine patent applications published and multiple additional patent applications filed. Achillion expects to initiate clinical studies with its first factor D inhibitor during the first half of 2016.

## PIPELINE

### ACHILLION DISCOVERY ENGINE

INDICATION   PROGRAM	DELIVERY	INHIBITOR	PHASE	PARTNER
<b>FACTOR D INHIBITOR CANDIDATE LIBRARY</b>				
PNH and Ultra-rare Diseases   ACH-4471	Oral	Factor D	1	
Dry AMD	Ophthalmic	Factor D	Preclinical	
COPD	Inhalational	Factor D	Preclinical	
<b>HEPATITIS C VIRUS CANDIDATE LIBRARY</b>				
HCV   Triple Regimen	Oral	odalasvir + simeprevir + ALS-335	2	janssen
HCV   Odalasvir	Oral	NS5A	2	janssen
HCV   ACH-3422	Oral	NS5B Polymerase	1	janssen
HCV   Sovaprevir	Oral	NS3/4A Protease	2	janssen

## REDEFINING BEST-IN-CLASS ANTIVIRAL THERAPY FOR HCV

Achillion discovered and developed a comprehensive portfolio of antivirals for the treatment of HCV, including a second-generation NS5A inhibitor, odalasvir (also known as ACH-3102); a novel nucleotide NS5B polymerase inhibitor, ACH-3422; and a NS3 protease inhibitor, sovalprevir.

To optimize clinical development and commercialization of these lead compounds, and potentially provide a best-in-class triple-combination regimen for the more than 150 million patients worldwide with chronic HCV, Achillion entered into a worldwide license and collaboration agreement with Janssen Pharmaceuticals, Inc. in May 2015. 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.



## BECOMING A FULLY-INTEGRATED COMMERCIAL PHARMACEUTICAL COMPANY

Achillion has the scientific and commercial expertise, as well as the financial resources, to pursue the full potential of its powerful development engine. Achillion plans to make its complement factor D inhibitors available to patients with indicated rare diseases through its own commercialization efforts. Achillion is driven by a corporate culture and working environment that enable breakthrough science, encourage solutions-oriented cross-functional collaboration, and foster innovation focused on the needs of patients and the commercial dynamics of the marketplace.



FOUNDED: 2000

EMPLOYEES: ~75

NASDAQ: ACHN

### LEADERSHIP

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

**David Apelian, M.D.**  
Executive Vice President and  
Chief Medical Officer

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

**Joseph Truitt**  
Executive Vice President of Business  
Development and Chief Commercial Officer

### BOARD OF DIRECTORS

**David I. Scheer, Chairman**  
Scheer & Company, Inc.

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**Michael D. Kishbauch**  
Former CEO of Achillion

**Robert L. Van Nostrand**  
Former CFO of Aureon Laboratories, Inc.

**Nicole Vitullo**  
Domain Associates LLC

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