

## Building Momentum in Human Genetic Diseases™

### AMICUS AT-A-GLANCE

Amigal™ – currently in Phase II clinical trials for Fabry disease

AT2101 – has completed Phase I clinical trials for Gaucher disease

AT2220 – currently in Phase I clinical trials for Pompe disease.

Founded in 2002

Headquartered in Cranbury, NJ

### LEADERSHIP TEAM

**John F. Crowley**  
President and CEO

**Donald J. Hayden, Jr.**  
Executive Chairman

**Matthew R. Patterson**  
Chief Operating Officer

**James E. Dentzer**  
Chief Financial Officer

**David J. Lockhart, Ph.D.**  
Chief Scientific Officer

**David Palling, Ph.D.**  
Senior VP, Drug Development

**Karin Ludwig, M.D.**  
Senior VP, Clinical Research

**Gregory P. Licholai, M.D., M.B.A.**  
VP, Medical Affairs and  
Corporate Development

**Douglas A. Branch**  
VP, General Counsel and Secretary

**S. Nicole Schaeffer, M.B.A.**  
VP, Human Resources & Leadership  
Development

Amicus Therapeutics is a biopharmaceutical company developing novel, oral therapeutics known as pharmacological chaperones for the treatment of a range of human genetic diseases. Pharmacological chaperone technology involves the use of small molecules to restore or improve biological activity in cells by selectively binding to a misfolded protein caused by a genetic mutation. Amicus is initially targeting lysosomal storage disorders, which are severe, chronic genetic diseases.



### Our Technology

Human genetic diseases result from mutations in specific genes that, in many cases, lead to the production of proteins with reduced stability and which may not achieve their correct three-dimensional shape (generally referred to as “misfolded proteins”). Misfolded proteins are often eliminated by the cell, resulting in reduced biological activity that can lead to impaired cellular function and ultimately to disease. In certain instances, misfolded proteins can accumulate in the ER instead of being eliminated. This accumulation of misfolded proteins may lead to various types of stress on cells, which may also contribute significantly to cellular dysfunction and disease. Our novel approach to the treatment of human genetic diseases consists of using a pharmacological chaperone that selectively binds to the target protein, which increases the stability of the protein and helps it fold into its correct three-dimensional shape. This restores appropriate trafficking of the protein, thereby increasing protein activity, improving cellular function and reducing stress on cells.

### Patient Advocacy

Amicus is committed to helping individuals and families with genetic diseases by searching for new and better therapies, initiating clinical trials for those therapies as our research advances, and providing useful information and resources to help manage the daily challenges of living with a rare genetic disease.