

AMICUS AT-A-GLANCE

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

AT2101 – Amicus filed an IND for the treatment of Gaucher disease in April 2006; clinical trials expected second half of year

AT2220 – currently in preclinical development for Pompe disease; company expects to file an IND by end of 2006

Founded in 2002

Headquartered in Cranbury, NJ

Recently completed \$55 million Series C financing

Filed for an initial public offering in May 2006

PATIENT ADVOCACY

At Amicus, we are committed to helping patients and their families 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.

LEADERSHIP STRENGTH

John F. Crowley
President and CEO

Donald J. Hayden, Jr.
Executive Chairman

Matthew R. Patterson
Chief Business Officer

Pedro Huertas, M.D., Ph.D.
Chief Strategic 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
Vice President, General Counsel and
Secretary

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

Building Momentum in Human Genetic Diseases

Amicus Therapeutics is a clinical-stage biopharmaceutical company developing small molecule, orally-delivered drugs to treat a range of human genetic diseases. Amicus develops pharmacological chaperones - oral therapies that bind to the affected proteins, increase stability, and restore shape, proper trafficking, and biological activity.

Amicus' innovative therapies apply to conditions in which crucial proteins are defective as a result of improper folding. Instead of trying to replace these complex proteins, Amicus' paradigm-shifting approach uses pharmacological chaperones to selectively bind and "rescue" the misfolded target protein to increase its stability and restore its proper conformation, trafficking, and biological activity, which in turn restores the function of the affected cells. This unique technology represents the next-generation approach to the management of human genetic diseases and offers the potential to improve treatment options for patients.



Amicus has assembled an outstanding leadership team that includes scientific, medical, regulatory, and business professionals with successful track records of developing and bringing to market drugs for rare genetic diseases. Amicus continues to develop its pharmacological chaperone technology and has built a significant intellectual property portfolio, as well as a robust clinical and preclinical pipeline of small molecule, orally-delivered agents.

Our Technology

At Amicus, we have developed a novel approach to address human genetic diseases resulting from misfolded proteins. We use small-molecule drugs, which are called pharmacological chaperones, to selectively bind to a target protein and increase its stability. The binding of the chaperone molecule helps the protein fold into its correct three-dimensional shape. This allows the protein to be trafficked from the ER to the appropriate location in the cell, thereby increasing protein activity and cellular function and reducing stress on cells.

We believe that our pharmacological chaperone compounds will:

Restore Protein Function

Because of the interaction with the pharmacological chaperones, the target proteins are able to pass through the quality control system of the ER and be trafficked to their appropriate location within the cell, where they are biologically active. In this way, proteins are delivered *where it matters*™.

Eliminate Aggregation and/or Accumulation of Misfolded Protein

Restoring trafficking of misfolded proteins by reducing their retention in the ER has the added potential benefit of alleviating the proteotoxic effects associated with mutant protein accumulation and/or aggregation. Thus, our pharmacological chaperones restore protein function and trafficking *how it matters*™.

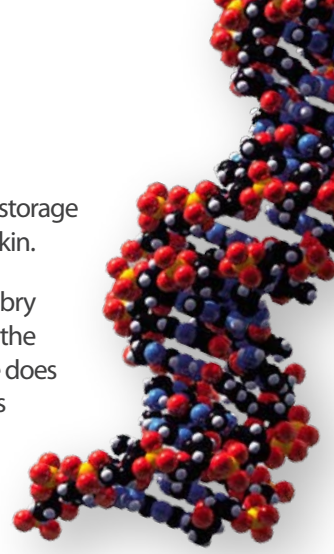
Product Pipeline

Amigal™ for Fabry Disease

Amigal (migalastat hydrochloride) is an experimental, oral therapy for the treatment of Fabry disease, a lysosomal storage disorder, which can cause damage to specific areas of the body, including the kidneys, heart, nervous system, and skin.

Amigal, a pharmacological chaperone, acts by selectively binding to the misfolded enzyme responsible for Fabry disease, α -GAL. After binding to the enzyme, Amigal promotes the proper folding, processing, and trafficking of the enzyme from the endoplasmic reticulum to its final destination, the lysosome, the area of the cell where the enzyme does its work. Once it reaches the lysosome, the pharmacological chaperone is displaced, and the enzyme can perform its normal, biological function, which is the breakdown of its natural substrate, GL-3.

In February 2004, the U.S. FDA's Office of Orphan Products Development granted Amicus orphan drug designation for the treatment of Fabry disease. In May 2006, the European Commission granted orphan medicinal product designation for Amigal. Orphan designation is granted by many regulatory agencies in order to promote the development of therapies for rare diseases.



Phase I clinical studies of Amigal (AT1001)

Phase I clinical trials of Amigal have been completed. Administration of Amigal to healthy volunteers resulted in the following:

- No drug-related adverse events
- High oral bioavailability
- Good pharmacokinetics

Notably, administration of Amigal also resulted in a statistically significant and dose-related increase in the activity of the target enzyme in each of the healthy volunteers who took part in the study.

Phase II clinical studies of Amigal in the USA

Amicus is currently sponsoring a Phase II clinical study of Amigal: "A Phase 2, Open-Label, Multicenter, Ascending-Dose, 12-Week Study to Evaluate the Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of Amigal in Patients With Fabry Disease." This study is being conducted through three investigational sites. The purpose of the study is to collect information about the safety and preliminary effectiveness of Amigal to treat certain patients with Fabry Disease. Males with Fabry Disease who are 18 years of age and older may be eligible to participate if they meet certain study-criteria, including having a genetic mutation associated with increased α -Gal activity by the investigational drug. The study will involve multiple visits to one of three investigational sites over an eighteen-week period (four weeks of screening, twelve weeks of treatment, and two weeks of follow-up). Patients may be eligible to continue in an extension phase of the protocol for additional 36 weeks if they have a positive response to treatment. Additional information about the clinical trial may be found at <http://clinicaltrials.gov/show/NCT00214500>. Amicus is committed to paying for travel costs associated with participation. Furthermore, some patients may be eligible to receive cash reimbursements for time lost from employment due to study participation.

If you are interested in learning more, contact one of the following investigators responsible for conducting the clinical studies in the US:

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If you are a physician or health care provider and would like to contact Amicus Therapeutics, please contact **Tendai Merriweather, CRA (609-662-2052)** or **Elizabeth Moffat, CRA (609-662-2049)**