

Building Momentum in Human Genetic Diseases™

Amicus Therapeutics is developing a diverse pipeline of novel, small-molecule pharmacological chaperones to treat a range of human genetic diseases. The company is currently conducting Phase II studies of its lead product, Amigal™ (migalastat hydrochloride), for Fabry disease and Phase I clinical trials of AT2101 for Gaucher disease. The company is also developing AT2220 for the treatment of Pompe disease and expects to file an investigational new drug application (IND) with the Food and Drug Administration (FDA) by the end of 2006. In addition, Amicus is leveraging its core pharmacological chaperone platform to actively pursue therapies in other genetic diseases.

Amigal™ (migalastat hydrochloride) for Fabry Disease

Amigal is an experimental, oral therapy for the treatment of Fabry disease, a lysosomal storage disorder resulting from a deficiency in the enzyme, α -galactosidase A (α -Gal). An enzyme is a specific type of protein that helps a chemical or biological reaction to occur, such as breaking down molecules in cells. In Fabry disease, a deficiency in the α -Gal enzyme results in damage to specific areas of the body, including kidneys, heart, nervous system, and skin. In February 2004, the FDA granted Amicus orphan designation for Amigal for the treatment of Fabry disease.

Phase I clinical trials in healthy volunteers taking Amigal have been completed. In these studies, Amigal was well tolerated, with no drug-related adverse events or serious adverse events. In addition, Amigal was shown to have high oral bioavailability and good pharmacokinetics. Finally, studies showed a statistically significant and dose-related increase in the level of activity of the target enzyme (α -Gal) in healthy volunteers.

Amicus is currently conducting open-label, Phase II clinical studies designed to assess Amigal's safety, pharmacokinetics, and pharmacodynamics in patients with Fabry disease caused by a missense mutation.

AT2101 for Gaucher Disease

AT2101 is an experimental, oral therapy for the treatment of Gaucher disease, a lysosomal storage disorder resulting from a deficiency in the enzyme β -glucocerebrosidase (GCCase). When this protein is deficient, Gaucher cells can accumulate in the body and cause damage to areas such as the liver, spleen, and bone marrow. In January 2006, AT2101 was granted orphan drug designation from the FDA for the treatment of Gaucher disease, and has completed Phase I clinical trials.

AT2220 for Pompe Disease

AT2220 is an experimental, oral therapy for the treatment of Pompe disease, a lysosomal storage disorder resulting from a deficiency in the enzyme α -glucosidase (Gaa), leading to lysosomal glycogen accumulation in skeletal, cardiac, and smooth muscle tissues. AT2220 is currently in Phase I clinical trials for the treatment of Pompe disease.

Pipeline

