# OUR COMMITMENT TO ADVOCACY

Amicus prides itself on ensuring that the rare and orphan disease community has a voice in the larger community while providing a support structure for daily challenges. We partner with the patient community to ensure that we continue to gain a wide range of insights that help us better understand the community's experience. By building alliances and facilitating collaboration within the rare disease communities, we seek to ease the difficulties in accessing support and services. We hope that this approach will help us to better meet the ongoing need for improved patient care.

We are committed to providing valuable information about genetic disorders for which we are actively developing therapies, as well as resources and services that support the rare and orphan disease communities.

*we support the disease communities*

***AND THEIR FAMILIES***

- AMICUS BELIEF STATEMENT

## PLEASE FEEL FREE TO CONTACT US FOR ANY ADDITIONAL INFORMATION


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# Insight Into Amicus Therapeutics

# The history, purpose, and vision behind our mission

# ABOUT AMICUS

### *WHO WE ARE*

We began as a small but devoted research-focused company. In a few short years, and with a specialized team of experts, we've come a long way towards achieving our goal of becoming an integrated, clinically advanced biotechnology company. Here at Amicus, we are striving to develop and deliver the highest quality therapies to persons afflicted with rare diseases.

Amicus has a development pipeline of first-in-class treatments and is invested in creating medicines and therapies for a range of human genetic disorders.

*we encourage and embrace*

***CONSTANT INNOVATION***

**- AMICUS BELIEF STATEMENT**

# OUR FOCUS AND COLLECTIVE VISION AT AMICUS

Amicus is driven to develop novel treatments and therapies for rare human genetic disorders, such as Fabry disease, Pompe disease, and Epidermolysis Bullosa (EB). We take pride in our innovative scientific capabilities and concentrate our efforts on improving the lives of the rare disease community. Our passion for patient advocacy and innovation pushes us to look into the future of rare and orphan disease treatments. We believe every step towards bettering patients' lives is a step in the right direction.

*we seek to deliver the highest quality therapies for*

***PERSONS LIVING WITH THESE DISEASES***

**- AMICUS BELIEF STATEMENT**

# AMICUS' PROGRAMS

### *LYSOSOMAL STORAGE DISORDERS*

Many genetic disorders can be caused by mutations that make proteins less stable or disrupt the normal three-dimensional folding of proteins. These genetic disorders include Lysosomal Storage Disorders (LSDs). LSDs are caused by mutations that alter a patient's endogenous enzymes, which may lead to a decrease in protein stability and even misfolding of enzymes. This may cause the deficient enzyme's substrate to accumulate in the body.

### *FABRY DISEASE*

Fabry disease is an inherited LSD caused by deficiency of an enzyme called α-galactosidase A (α-Gal A). The function of α-Gal A is to degrade glycosphingolipids, or sugary-fat material, in lysosomes, including globotriaosylceramide (GL-3, also known as Gb3). Progressive accumulation of GL-3 is believed to lead to the morbidity and mortality of Fabry disease, including pain, kidney failure, heart disease, and stroke.

### *POMPE DISEASE*

Pompe disease is an inherited LSD caused by deficiency of an enzyme called acid α-glucosidase (GAA). Reduced or absent levels of GAA lead to the accumulation of the substrate glycogen in the lysosomes of various cells within the body. Progressive accumulation of glycogen is believed to lead to the morbidity and mortality associated with Pompe disease, including muscle weakness and respiratory insufficiency.

### *EPIDERMOLYSIS BULLOSA*

EB is a rare genetic disorder that manifests as blistering or erosion of the skin and, in some cases, the epithelial lining of other organs. EB is chronic, debilitating, and can potentially be disfiguring and fatal. There is currently no approved treatment for EB; however, Amicus is committed to addressing this significant unmet need.