



## **Amicus Therapeutics to Present Preclinical Pompe Disease Gene Therapy Data at the American Society of Gene & Cell Therapy (ASGCT) 22nd Annual Meeting**

April 15, 2019

CRANBURY, N.J., April 15, 2019 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq: FOLD) today announced the company will present initial preclinical data from its investigational adeno-associated viral (AAV) gene therapy program for Pompe disease in a poster at the [American Society of Gene & Cell Therapy \(ASGCT\) 22nd Annual Meeting](#) on April 30, 2019 in Washington D.C.

### **Poster Presentation:**

- Title: "Development of a Novel Gene Therapy for Pompe Disease: Engineered Acid Alpha-Glucosidase Transgene for Improved Expression and Muscle Targeting"
- Session Date and Time: Tuesday, Apr 30, 2019, 5:00 PM - 6:00 PM ET
- Session title: Metabolic, Storage, Endocrine, Liver and Gastrointestinal Diseases
- Room: Columbia Hall
- Abstract number: 518

These represent the first set of preclinical results for the Pompe gene therapy which Amicus is currently developing in collaboration with the Gene Therapy Program of the Perelman School of Medicine at the University of Pennsylvania (Penn). This relationship combines Amicus' protein engineering and glycobiology expertise with Penn's adeno associated virus gene transfer technologies to develop AAV gene therapies designed for optimal cellular uptake, targeting, dosing, safety and manufacturability.

### **About Pompe Disease**

[Pompe disease](#) is an inherited lysosomal storage disorder caused by deficiency of the enzyme acid alpha-glucosidase (GAA). Reduced or absent levels of GAA leads to accumulation of glycogen in cells, which results in the clinical manifestations of Pompe disease. The disease can be debilitating, and is characterized by severe muscle weakness that worsens over time. Pompe disease ranges from a rapidly fatal infantile form with significant impacts to heart function to a more slowly progressive, late-onset form primarily affecting skeletal muscle. It is estimated that Pompe disease affects approximately 5,000 to 10,000 people worldwide.

### **About Amicus Therapeutics**

Amicus Therapeutics (Nasdaq: FOLD) is a global, patient-dedicated biotechnology company focused on discovering, developing and delivering novel high-quality medicines for people living with rare metabolic diseases. With extraordinary patient focus, Amicus Therapeutics is committed to advancing and expanding a robust pipeline of cutting-edge, first- or best-in-class medicines for rare metabolic diseases. For more information, please visit the company's website at [www.amicusrx.com](http://www.amicusrx.com), and follow us on [Twitter](#) and [LinkedIn](#).

### **Forward-Looking Statements**

This press release contains "forward- looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. Words such as, but not limited to, "look forward to," "believe," "expect," "anticipate," "estimate," "intend," "confidence," "encouraged," "potential," "plan," "targets," "likely," "may," "will," "would," "should" and "could," and similar expressions or words identify forward-looking statements. The forward looking statements included in this press release are based on management's current expectations and belief's which are subject to a number of risks, uncertainties and factors. In addition, all forward looking statements are subject to the other risks and uncertainties detailed in our Annual Report on Form 10-K for the year ended December 31, 2018. As a consequence, actual results may differ materially from those set forth in this press release. You are cautioned not to place undue reliance on these forward looking statements, which speak only of the date hereof. All forward looking statements are qualified in their entirety by this cautionary statement and we undertake no obligation to revise this press release to reflect events or circumstances after the date hereof.

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