



Amicus Therapeutics Issued Two U.S. Patents for Pompe Enzyme ATB200

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New Intellectual Property Covers Composition of Matter and Manufacturing Method out to Mid-2030s

CRANBURY, N.J., March 20, 2019 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq: FOLD) today announced that the United States Patent and Trademark Office has issued two new patents directed to the composition of matter and methods of making ATB200, a unique recombinant human acid alpha-glucosidase (rhGAA) enzyme with optimized carbohydrate structures, particularly mannose-6 phosphate (M6P), to enhance uptake. ATB200 is the biologic component of the Company's investigational Pompe treatment paradigm AT-GAA, which consists of ATB200 co-administered with AT2221, a pharmacological chaperone.

The first U.S. patent No. 10,208,299 reflects composition of matter for highly potent rhGAA with enhanced carbohydrates. The second U.S. patent No. 10,227,577 covers the methods for making ATB200. The patents are set to expire in 2035 and 2036, respectively. Amicus is pursuing corresponding patent applications in other regions and countries, including Europe and Japan.

"These newly issued U.S. patents reflect the novelty and uniqueness of our protein engineering and biologics expertise to develop a differentiated, highly potent recombinant GAA enzyme as the key component of our novel Pompe treatment paradigm," said John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics. "We believe these attributes are necessary to optimally target this enzyme to muscles in patients. The issuance of these key patents also embodies the many years of tremendous hard work, perseverance and ingenuity of the Amicus science and technical operations teams as well as the enormous investment of capital required to create and make this new medicine. These patents provide Amicus with broad and long-term intellectual property rights into the mid-2030s and significantly strengthen our position as we advance AT-GAA to become the next potential standard of care for Pompe disease. As we continue to serve patients globally and invest in additional research and development for AT-GAA, we also look forward to further expanding our intellectual property portfolio around the world."

About AT-GAA

[AT-GAA](#) is an investigational therapy that consists of ATB200, a unique recombinant human acid alpha-glucosidase (rhGAA) enzyme with optimized carbohydrate structures, particularly mannose-6 phosphate (M6P), to enhance uptake, co-administered with AT2221, a pharmacological chaperone. In 2019, the U.S. Food and Drug Administration (FDA) granted Breakthrough Therapy Designation to AT-GAA for the treatment of late onset Pompe disease. In preclinical studies, AT-GAA was associated with increased tissue enzyme levels, reduced glycogen levels in muscle, and improvements in muscle strength. A global Phase 1/2 study ([ATB200-02](#)) is ongoing to evaluate the safety, tolerability, pharmacokinetics (PK) and pharmacodynamics of AT-GAA.

Amicus is currently sponsoring PROPEL, a global Phase 3 clinical study (also known as ATB200-03) of [AT-GAA](#) in adult patients with late onset [Pompe disease](#). PROPEL is a 52-week, double-blind randomized study designed to assess the efficacy, safety and tolerability of AT-GAA compared to the current standard of care, alglucosidase alfa, an enzyme replacement therapy (ERT). More information, including a list of participating sites, is available at www.clinicaltrials.gov: NCT03729362.

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 is believed to result 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, 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, including statements relating to patent scope and length of protection, encouraging preliminary data from a global Phase 1/2 study to investigate AT-GAA for the treatment of Pompe and the potential implications on these data for the future advancement and development of AT-GAA. 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 beliefs which are subject to a number of risks, uncertainties and factors, including that biosimilars or others could challenge the validity of the AT-GAA patents, Amicus may not be able to enforce the patents, there could be patent-related litigation, the preliminary data from the Ph 1/2 study is based on a small patient sample and reported before completion of the study will not be predictive of future results, that results of additional preliminary data or data from the completed study or any future study will not yield results that are consistent with the preliminary data presented, that the Company will be not able to demonstrate the safety and efficacy of AT-GAA, that later study results will not support further development, or even if such later results are favorable, that the Company will not be able to successfully complete the development of, obtain regulatory approval for, or successfully commercialize AT-GAA. 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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