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## U.S. FDA Grants Orphan Drug Designation for ATB200/AT2221 for Pompe Disease

### New Phase 1/2 Clinical Data to be Presented at World Muscle Society

CRANBURY, N.J., Sept. 21, 2017 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq:[FOLD](#)) today announced the U.S. FDA has granted [orphan drug designation](#) to [ATB200/AT2221](#) for the treatment of [Pompe disease](#), an inherited lysosomal storage disorder caused by deficiency of an enzyme called acid alpha-glucosidase (GAA). This novel treatment paradigm 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.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, stated, "Today there are significant unmet needs among people living with Pompe, and this orphan drug designation recognizes the potential for ATB200/AT2221 to become a differentiated treatment paradigm for this devastating neuromuscular disease. The initial positive clinical data we have seen to date from our ongoing Phase 1/2 study, including significant improvements in key biomarkers as well as functional outcomes following six months of treatment, are very encouraging. We look forward to announcing new clinical data in all patients from this study at World Muscle Society in early October."

The FDA, through its Office of Orphan Products Development (OOPD), grants orphan status to drugs and biologic products that are intended for the safe and effective treatment, diagnosis, or prevention of rare diseases or disorders that affect fewer than 200,000 people in the United States. Orphan drug designation provides a drug developer with certain benefits and incentives, including a period of marketing exclusivity if regulatory approval is ultimately received for the designated indication.

### Poster at 22<sup>nd</sup> International Congress of the World Muscle Society - October 3-7, 2017

Amicus Therapeutics is currently investigating ATB200/AT2221 in a global Phase 1/2 study ([ATB200-02](#)) that enrolled 20 patients with Pompe disease. Positive [results](#), including six-month functional outcomes on motor and pulmonary function, were previously reported in the 10 initial patients. Additional data from all patients, including six-month functional outcomes, will be presented at the [22<sup>nd</sup> International Congress of the World Muscle Society](#) in a late breaker poster (LB.P.3: First-in-Human Study of ATB200/AT2221 in Patients with Pompe Disease: Interim Results from the ATB200-02 Trial (Mark Roberts, Department of Neurology, Salford Royal NHS Foundation Trust)). World Muscle Society will take place October 3-7, 2017 in St. Malo, France. For more information please visit [www.wms2017.com](http://www.wms2017.com).

### About ATB200-02 Clinical Study

The objectives of the open-label ATB200-02 clinical study are to evaluate the safety, tolerability, pharmacokinetics (PK), pharmacodynamics (PD), and efficacy of ATB200/AT2221 over an 18-week primary treatment period followed by a long-term extension. The study enrolled a total of 20 patients across three patient cohorts: ambulatory ERT-switch (Cohort 1, n=11), non-ambulatory ERT-switch (Cohort 2, n=4) and ERT-naïve (Cohort 3, n=5). Patients in Cohort 1 received escalating doses of ATB200 (5, 10, 20 mg/kg), followed by 3 doses of 20 mg/kg ATB200 plus low dose AT2221, followed by ongoing doses of 20 mg/kg ATB200 plus high dose AT2221. Patients in Cohort 2 and 3 patients have all received 20 mg/kg ATB200 plus high dose AT2221.

### **About ATB200/AT2221**

[ATB200/AT2221](#) is a novel treatment paradigm 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 preclinical studies, ATB200 was associated with increased tissue enzyme levels and reduced glycogen levels in muscle, which was further improved when AT2221 was co-administered with ATB200. Amicus Therapeutics is currently conducting a global Phase 1/2 study ([ATB200-02](#)) to evaluate the safety, tolerability, pharmacokinetics (PK) and pharmacodynamics of ATB200/AT2221.

### **About Pompe Disease**

[Pompe disease](#) is an inherited lysosomal storage disorder caused by deficiency of an enzyme called acid alpha-glucosidase (GAA). Reduced or absent levels of GAA lead to the accumulation of the substrate glycogen in the lysosomes of muscles and other tissues. Signs and symptoms of Pompe disease can be severe and debilitating and include progressive muscle weakness throughout the body, particularly the heart and skeletal muscles. This leads to accumulation of glycogen in cells, which is believed to result in the clinical manifestations of Pompe disease. Pompe disease ranges from a rapidly fatal infantile form with severe cardiac involvement to a more slowly progressive, late-onset form primarily affecting skeletal muscle. All forms are characterized by severe muscle weakness that worsens over time. It is estimated that Pompe disease affects approximately 5,000 to 10,000 people worldwide.

For more information, download our [Pompe disease infographic](#).

### **About Amicus Therapeutics**

[Amicus Therapeutics](#) (Nasdaq:[FOLD](#)) is a global biotechnology company at the forefront of therapies for rare and orphan diseases. The Company has a robust pipeline of advanced therapies for a broad range of human genetic diseases. Amicus' lead programs in development include the small molecule pharmacological chaperone [migalastat](#) as a monotherapy for Fabry disease, as well as novel enzyme replacement therapy (ERT) and biologic products for [Fabry disease](#), [Pompe disease](#), and other rare and devastating diseases.

### **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 encouraging preliminary data from a global Phase 1/2 study to investigate ATB200/AT2221 for the treatment of Pompe and the potential implications on these data for the future advancement and development of ATB200/AT2221. 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 the preliminary data 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 ATB200/AT2221, 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 ATB200/AT2221. 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, 2016 and Quarterly Report on 10-Q for the Quarter ended June 30, 2017. 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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