



Amicus Therapeutics Enters Research and Development Collaboration with University of Pennsylvania to Develop AAV Gene Therapies

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Collaboration Focuses on Four Genetic Disorders: Pompe, Fabry, CDKL5 and One Additional Undisclosed Rare Metabolic Disorder

Combines Amicus Expertise in Protein Engineering with Penn's AAV Vector Technology, Manufacturing and Immunology Capabilities

Leverages The Wilson Lab's Extensive Translational Science Capabilities in Gene Therapy Development

Collaboration to be Featured During Amicus Analyst Day in New York City on October 11th

CRANBURY, N.J., Oct. 08, 2018 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq: FOLD) today announced a major collaboration with the Gene Therapy Program in the Perelman School of Medicine at the University of Pennsylvania (Penn) to pursue research and development of novel gene therapies for Pompe disease, Fabry disease, CDKL5 deficiency and one additional undisclosed rare metabolic disorder. This relationship will combine Amicus' protein engineering and glycobiology expertise with Penn's adeno associated virus (AAV) gene transfer technologies to develop AAV gene therapies designed for optimal cellular uptake, targeting, dosing, safety and manufacturability.

"This groundbreaking collaboration with Penn offers a new opportunity to potentially transform the lives of people living with these severe genetic disorders," stated John F. Crowley, Chairman and Chief Executive Officer of Amicus. "For people living with Fabry, it is a fulfillment of our pledge to advance science toward a cure for Fabry disease. We are partners now with the Fabry community for life. For Pompe, this is another important step on a lifelong journey toward the ultimate answer to finally cure Pompe. If we are to do so, we must have a gene therapy that addresses the many technical challenges in Pompe disease and that employs state of the art science in both protein and gene therapy engineering. For CDKL5 and other rare metabolic disorders, this collaboration is the foundation for advancing new therapies for these devastating genetic disorders. Dr. Wilson's laboratory and The Gene Therapy Program at Penn has strong capabilities in its AAV vector technology, as well as manufacturing and immunology strategies to develop novel gene therapy candidates, and to successfully advance them through preclinical development. This is another important step in Amicus becoming the leader in gene therapy for rare metabolic disorders."

Penn's AAV vector technology is designed to improve targeting, tropism, safety, immunogenicity, and gene delivery, while Amicus' protein engineering capabilities may optimize protein expression, secretion, targeting and uptake of the target protein. The agreement between Amicus and Penn is a Research, Collaboration and License Agreement, with funding provided to Penn to advance the preclinical research programs in the Wilson Lab and to license certain technologies invented under the funded Research Collaboration. The collaboration program will focus on developing innovative new AAV gene therapies for Pompe disease, Fabry disease, CDKL5 deficiency and one additional undisclosed rare metabolic disorder.

"Amicus has developed unique abilities in drug development in the lysosomal storage disorders, particularly in Pompe and Fabry diseases," said James M. Wilson, MD PhD, Professor of Medicine and Pediatrics at the Perelman School of Medicine. "There are multiple and unique challenges in developing optimal gene therapy products for patients living with Pompe and Fabry diseases. I believe that we can combine the technologies and capabilities from my research laboratory at Penn with the Amicus expertise in protein engineering, glycobiology and disease biology understanding to rapidly advance novel gene therapies to the clinic. Because of their unique capabilities and their commitment to great science and to patients, we are excited about our new partnership with Amicus to develop AAV gene therapies for patients with urgent unmet needs."

Additional details and an overview of the collaboration will be provided during the Amicus Analyst Day on October 11, 2018.

About Amicus Therapeutics

Amicus Therapeutics (Nasdaq: FOLD) is a global, patient-centric 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.

Forward Looking Statement

This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to the collaboration with the University of Pennsylvania, and the development of potential gene therapy product candidates. The inclusion of forward-looking statements should not be regarded as a representation by us that any of our plans will be achieved. Any or all of the forward-looking statements in this press release may turn out to be wrong and can be affected by inaccurate assumptions we might make or by known or unknown risks and uncertainties. For example, the benefits of this collaboration may never be realized, the potential that results of clinical or preclinical studies indicate that the product candidates are unsafe or ineffective; the potential that it may be difficult to enroll patients in our clinical trials; the potential that regulatory authorities, including the FDA, EMA, and PMDA, may not grant or may delay approval for our product candidates; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; the potential that we may not be able to manufacture or supply sufficient clinical or commercial products; the potential that we will need additional funding to complete all of our studies and manufacturing and the potential that certain individuals may not continue to support the development of product candidates. In addition, all forward-looking statements are subject to other risks detailed in our Annual Report on Form 10-K for the year ended December 31, 2017 as well as our Quarterly Report on Form 10-Q for the quarter ended June 30, 2018 filed August 7, 2018 with the Securities and Exchange Commission. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement, and we undertake no obligation to revise or update this presentation to reflect events or circumstances after the date hereof.

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