



Amicus Therapeutics and the University of Pennsylvania Announce Major Expansion of Gene Therapy Collaboration

May 29, 2019

Exclusive Disease-Specific Worldwide Rights to Penn's Next Generation Gene Therapy Technologies from the Wilson Lab for the Majority of Lysosomal Disorders



Current Collaboration Extended to Include Three New Indications: Niemann-Pick Type C (NPC), Mucopolysaccharidosis Type IIIA (MPS IIIA) and Mucopolysaccharidosis Type IIIB (MPS IIIB)

New Research Programs also Encompass 12 Additional Rare Diseases, including Rett Syndrome, Angelman Syndrome, Myotonic Dystrophy and Select Other Muscular Dystrophies

Robust Amicus R&D Engine Created through Combination of Amicus Expertise in Protein Engineering with Penn's Vector Technology, Translational Science, Manufacturing and Immunology Capabilities

Amicus Now Positioned with Industry Leading Gene Therapy Pipeline in Rare Diseases

Conference Call Today at 8:30am

CRANBURY, N.J. and PHILADELPHIA, May 29, 2019 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq: FOLD) and the Perelman School of Medicine at the University of Pennsylvania (Penn) today announced a major expansion to their collaboration with rights to pursue collaborative research and development of novel gene therapies for lysosomal disorders (LDs) and 12 additional rare diseases. The collaboration has been expanded from three to six programs for rare genetic diseases and now includes: Pompe disease, Fabry disease, CDKL5 deficiency disorder (CDD), Niemann-Pick Type C (NPC), Mucopolysaccharidosis Type IIIB (MPS IIIB), as well as a next generation program in Mucopolysaccharidosis Type IIIA (MPS IIIA), both also known as part of Sanfillipo Syndrome. In addition to these three new programs, a discovery research agreement provides Amicus with exclusive disease-specific access to rights to collaborate with Penn's Gene Therapy Program (GTP) to develop potentially disruptive new gene therapy platform technologies and programs for the majority of lysosomal disorders and 12 additional rare diseases.

John F. Crowley, Chairman and Chief Executive Officer of Amicus stated, "The extension of our collaboration with Penn is a bold step forward in our commitment to create potential cures that may alleviate an enormous amount of suffering for countless numbers of people in the world living with rare diseases, many of them children. Together with Penn we are now able to focus on additional lysosomal disorders, as well as several more prevalent rare diseases for which we can apply our understanding of underlying disease biology in rare metabolic disease, Amicus' protein-engineering and development expertise and the world renowned capabilities of Dr. Jim Wilson's laboratory to develop novel gene therapy candidates. With a globally approved precision medicine product for Fabry, a late-stage biologic product with breakthrough therapy designation for Pompe, and now the industry's largest rare disease gene therapy pipeline, Amicus is well-positioned to become a leading global biotechnology company at the forefront of human genomic medicine."

Building off the initial success of the ongoing Amicus-Penn collaboration, including compelling initial preclinical proof-of-concept data in Pompe disease, this expanded relationship will continue to combine Amicus' protein engineering and glycobiology expertise with Penn's gene transfer technologies to develop novel gene therapies designed for optimal cellular uptake, targeting, dosing, safety and manufacturability.

"This agreement is a significant step forward in creating a world class industry-academia gene therapy partnership in rare diseases," said James M. Wilson, MD PhD, Professor of Medicine and Pediatrics at the Perelman School of Medicine. "We have already seen highly encouraging preclinical results and proof-of-concept in Pompe disease through our existing collaboration and are excited by what we can further achieve together. We are looking forward to expanding the relationship further for additional preclinical programs and committing to the research required to further advance the technology platforms at Penn. We have seen the first results of our combined capabilities and platforms and I believe that we can further expand and accelerate our efforts to rapidly develop gene therapies for many more patients with unmet needs."

"Penn Medicine has put Philadelphia on the map as the global epicenter of gene therapy research and development, and under the leadership and vision of Jim Wilson, our expanded agreement with Amicus is an exciting milestone for a field which is in the midst of transformative breakthroughs," said J. Larry Jameson, MD, PhD, Executive Vice President of the University of Pennsylvania for the Health System and Dean of the Perelman School

of Medicine. "We are thrilled to be part of this collaboration, which will help to bolster our city's growing reputation as a magnet for talent and an engine for gene therapy innovation."

Extended Research, Collaboration and License Agreement for Six Rare Metabolic Diseases

Penn's 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 initial collaboration program focused on developing innovative new gene therapies for Pompe disease, Fabry disease, CDD and one additional undisclosed rare metabolic disorder. With the extension of the agreement, NPC and MPS IIIB, as well as a next-generation program for MPS IIIA, have now been added as three additional collaboration programs.

New Next Generation Research Program: R&D Engine for LDs and Additional Rare Diseases

Under a new five-year next-generation research agreement, Penn will conduct discovery research to develop potentially disruptive new gene therapy technologies. Amicus will continue to advance its own research and technology platforms to combine with Penn's technologies, which can be used in the collaborative research programs for the disease indications. Terms of the agreement include a \$10 million annual investment from Amicus, each year for five years, into GTP's discovery research program with the ability to extend. Amicus will receive exclusive disease-specific rights to collaborate with GTP to research and develop products for many lysosomal disorders. The Amicus rights for additional collaborative research programs also include additional rare diseases, including Rett Syndrome, Angelman Syndrome, Myotonic Dystrophy and select other muscular dystrophies. For many of these indications, there is potential to apply protein-engineering and targeting motifs to enable cross-correction with next-generation gene therapy technology.

Conference Call and Webcast on May 29, 2019 at 8:30 a.m. ET

Amicus Therapeutics will host a conference call and audio webcast today, May 29, 2019 at 8:30 a.m. ET to discuss the expanded collaboration. Interested participants and investors may access the conference call by dialing 877-303-5859 (U.S./Canada) or 678-224-7784 (international), conference ID: 1695255.

A live audio webcast and accompanying [slide deck](#) can also be accessed via the Investors section of the Amicus Therapeutics corporate website at <http://ir.amicusrx.com/>, and will be archived for 30 days. Web participants are encouraged to register on the website 15 minutes prior to the start of the call. A replay of the call will be available for seven days beginning at 11:30 a.m. ET on May 29, 2019. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); conference ID: 1695255.

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.

About the Perelman School of Medicine at the University of Pennsylvania

Penn Medicine is one of the world's leading academic medical centers, dedicated to the related missions of medical education, biomedical research, and excellence in patient care. Penn Medicine consists of the [Raymond and Ruth Perelman School of Medicine at the University of Pennsylvania](#) (founded in 1765 as the nation's first medical school) and the [University of Pennsylvania Health System](#), which together form a \$7.8 billion enterprise. The Perelman School of Medicine has been ranked among the top medical schools in the United States for more than 20 years, according to U.S. News & World Report's survey of research-oriented medical schools. The School is consistently among the nation's top recipients of funding from the National Institutes of Health, with \$425 million awarded in the 2018 fiscal year. The University of Pennsylvania Health System's patient care facilities include: the Hospital of the University of Pennsylvania and Penn Presbyterian Medical Center—which are recognized as one of the nation's top "Honor Roll" hospitals by U.S. News & World Report—Chester County Hospital; Lancaster General Health; Penn Medicine Princeton Health; and Pennsylvania Hospital, the nation's first hospital, founded in 1751. Additional facilities and enterprises include Good Shepherd Penn Partners, Penn Home Care and Hospice Services, Lancaster Behavioral Health Hospital, and Princeton House Behavioral Health, among others. Penn Medicine is powered by a talented and dedicated workforce of more than 40,000 people. The organization also has alliances with top community health systems across both Southeastern Pennsylvania and Southern New Jersey, creating more options for patients no matter where they live. Penn Medicine is committed to improving lives and health through a variety of community-based programs and activities. In fiscal year 2018, Penn Medicine provided more than \$525 million to benefit our community.

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, 2018 as well as our Quarterly Report on Form 10-Q for the quarter ended March 31, 2019. 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.

Penn's Financial Disclaimer - As an inventor of technology licensed or optioned to Amicus pursuant to this Gene Therapy Collaboration, Dr. Wilson and Penn may receive additional financial benefits under the license in the future.

CONTACTS:

Investors/Media:

Amicus Therapeutics
Sara Pellegrino, IRC
Vice President, Investor Relations & Corporate Communications
spellegrino@amicusrx.com
(609) 662-5044

Media:

Amicus Therapeutics
Marco Winkler
Director, Corporate Communications
mwinkler@amicusrx.com
(609) 662-2798

Penn Medicine:

Karen Kreeger
Senior Science Communications Manager
Karen.kreeger@pennmedicine.upenn.edu
(215) 459-0544

FOLD-G



Source: Amicus Therapeutics, Inc.