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New Brunswick, NJ - Amicus Therapeutics, Inc., an emerging drug development company focused on the development of a novel therapeutic approach to the treatment of human genetic disorders, with an initial focus on lysosomal storage diseases, today announced the completion of a \$31 million Series B private equity financing. The Series B Round was led by Canaan Partners, L.P., with participation from other new investors, Frazier Healthcare Ventures, L.P., New Enterprise Associates, L.P., Prospect Venture Partners, L.P., and Radius Venture Partners, L.P. The company's founding investor, CHL Medical Partners, also participated in the round.

"Collectively, these investors have an excellent track record in helping to build significant and successful companies, and they bring considerable experience and knowledge to the table in addition to financial resources," said Norman Hardman, Ph.D., Chief Executive Officer of Amicus Therapeutics, Inc. "With their commitment and support, Amicus is firmly on its way to realizing its vision of becoming the premier company developing treatments for human genetic diseases with small-molecule drugs. During the fundraising we were able to stay focused on ensuring that the pre-clinical development of AT1001, our lead product candidate for treatment of Fabry disease, remained on track. The progress we made has clearly impressed our investors, all of whom are extremely excited about this new product opportunity. With this round of financing secured, our top priorities will be the further advancement of AT1001 --which we plan to have in the clinic by the third quarter of this year -- and the development of our R&D program for Gaucher Disease."

With the close of the financing, Stephen Bloch, M.D., of Canaan Partners, James Topper, M.D., Ph.D., of Frazier Healthcare Ventures, Mike Raab of New Enterprise Associates, and Alex Barkas, Ph.D., of Prospect Venture Partners, have joined the board. They join existing board members, Norman Hardman, Ph.D., and Gregory Weinhoff, M.D., of CHL Medical Partners.

"Successfully concluding this phase of our corporate development brings us a significant step closer to providing potentially effective and convenient oral therapies to those who suffer from Fabry disease, Gaucher disease, and a range of other genetic disorders," concluded Dr. Hardman.

About Fabry Disease

Amicus' lead compound, AT1001, a small molecule drug in pre-clinical development for Fabry disease, received Orphan Drug designation from the U.S. Food and Drug Administration in March of 2004. Fabry disease is a lysosomal storage disease caused by a deficiency of alpha-galactosidase A. Patients with classic Fabry disease, of which there are approximately 5,000 people worldwide, have early-onset symptoms, including neuropathic pain, heart disease and kidney disease, skin problems, and an inability to sweat. Late-onset Fabry disease is characterized by heart and renal involvement in patients who first present to the clinic later in life, typically in early middle-age. Fabrazyme (marketed by Genzyme Corporation) and Replagal (marketed by Transkaryotic Therapies, Inc.) are enzyme replacement therapies that aim to replace the alpha-galactosidase A enzyme that is diminished or absent in Fabry patients. In contrast, AT1001 is designed to provide a small molecule, oral therapy to enhance the patient's own alpha-galactosidase A activity.

About Amicus Therapeutics

Founded in April 2002 by the Mount Sinai School of Medicine (MSSM) and CHL Medical Partners, Amicus is focused on the development of orally-active, small molecule drugs capable of restoring normal function to mutant proteins. Amicus was founded to capitalize on the discovery that many diseases of genetic origin are caused by missense mutations and other rescuable mutations that result in the misfolding of a protein or enzyme. These misfolded mutant proteins become targeted for degradation before reaching their normal site of action, leading to the disease phenotype.

Pharmacological Chaperones are designed to help the mutant protein fold correctly into its normal 3-dimensional conformation, restoring the normal processing and transport of the protein and rescuing its intrinsic biological activity and function.

Amicus technology is based on research conducted by Jian-Qiang Fan, Ph.D., Assistant Professor in MSSM's Department of Human Genetics and a founder of Amicus. Amicus' pharmacological chaperone approach has the potential to be applied to a wide range of genetic disorders. Amicus currently has 10 employees and is headquartered at the New Jersey Technology Center in North Brunswick, New Jersey. Additional information about the Company can be found at

