Amicus Therapeutics Acquires Gene Therapy Portfolio of Ten Clinical and Pre-Clinical Stage AAV Programs in Neurologic Lysosomal Storage Disorders

September 20, 2018

License with Nationwide Children's Hospital (NCH) through the Acquisition of Celenex (NCH Spinout)
Establishes Amicus as Leading Gene Therapy Company in Neurologic Lysosomal Storage Disorders (LSD) with Potential to Transform the Lives of 10,000+ Children with Fatal Genetic Diseases

Includes Clinical Stage Programs in CLN6 and CLN3 Batten Disease, a Preclinical Program in CLN8 Batten Disease and Additional LSD Programs in Niemann Pick C, Wolman Disease, Tay Sachs and Other Disorders

Leverages Amicus Therapeutics Expertise in Global Development and Delivery of Novel Therapies for Rare Metabolic Disorders with Nationwide Children's AAV Gene Therapy Technologies

Balance Sheet Strengthened with $150 Million in Non-Dilutive Debt Financing

Conference Call and Webcast Scheduled for Today, September 20, 2018 at 8:00am ET

CRANBURY, N.J., Sept. 20, 2018 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq: FOLD) today announced the signing of a definitive agreement in which Amicus Therapeutics will receive worldwide development and commercial rights for ten gene therapy programs developed at The Center for Gene Therapy at The Research Institute at Nationwide Children's Hospital and The Ohio State University. The ten programs are licensed to Amicus from Nationwide Children's Hospital through the acquisition of Celenex, a private, clinical stage gene therapy company. The lead programs in CLN6, CLN3, and CLN8 Batten disease are potential first-to-market curative therapies for these rare, devastating diseases. Batten disease, also known as Neuronal Ceroid Lipofuscinosis (NCL), is a family of rare disorders that can be life-threatening and debilitating, with high unmet medical need. More detail about these programs will be presented on this morning's conference call. The slides for this conference call are available on our website.

"The in-licensing and acquisition of these gene therapy programs provides an extraordinary opportunity to transform the lives of thousands of children living with some of the most devastating forms of lysosomal storage disorders, for which there are virtually no treatment options today," said John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics. "The groundbreaking work of Drs. Brian Kaspar and Kathrin Meyer at Nationwide Children's Hospital, along with collaborator, Arthur Burghes, Ph.D., professor at The Ohio State University, on these programs has led to remarkably strong and consistent pre-clinical results and now, in CLN6 Batten disease, encouraging early results in children. This is science and biotechnology at its best. And it has at its core the love, drive and passion of two remarkable parents, Gordon and Kristen Gray, who moved heaven and earth to partner with these researchers to advance these potentially life-saving medicines for their daughters and now for many thousands more. I am honored that they and their research team have chosen to entrust these ten programs to the passionate team of scientists and entrepreneurs at Amicus. I cannot think of a better foundation for Amicus' entry into gene therapies."

All acquired programs leverage intrathecal delivery, using the same AAV vector approach utilized successfully in clinical trials across other rare CNS indications, such as SMA. This approach and technology are considered to be a clinically validated gene delivery platform for diseases of the central nervous system (CNS). Brian Kaspar, Ph.D., co-founder of Celenex, and Kathrin Meyer, Ph.D., a Principal Investigator at Nationwide Children's Hospital Center for Gene Therapy, will continue to support these programs as scientific advisors to Amicus Therapeutics.

"The preclinical proof-of-concept we have seen to date in CLN6, CLN3, and CLN8 further support the applicability of the AAV vector we developed at Nationwide Children's in genetic disease of the CNS," said Kathrin Meyer, Ph.D. "I firmly believe that Amicus is the optimal scientific and clinical partner to advance these programs and look forward to actively collaborating with the Amicus team on the development of these important potential therapies and getting them to as many children as quickly as possible. They truly have the potential to transform lives."

Deal Terms
Under the terms of the agreement, Amicus will pay $100 million in an upfront cash payment to acquire all of these assets.

Celenex shareholders are also eligible for up to $15 million in development milestones and $262 million in BLA/MAA submission and approval milestones across multiple programs. Amicus expects to pay no more than $75 million over the next 4 years in these milestones. No royalties are owed to Celenex for any of these programs. Celenex shareholders may also be eligible for up to $75 million in tiered sales ($500 million/$750 million) milestone payments. The acquisition and several years of related development costs for all of these programs will be financed through a new $150 million debt facility provided by BioPharma Credit PLC, an investment fund managed by Pharmakon Advisors, L.P.

Skadden, Arps, Slate, Meagher & Flom LLP acted as legal counsel to Amicus Therapeutics on the transaction. RBC Capital Markets acted as exclusive financial advisor and Fenwick & West LLP acted as exclusive legal counsel to Celenex on the transaction. The transaction was approved by the Board of Directors of both companies and closed immediately.

Debt Facility
Today, Amicus Therapeutics also announced it has closed a five-year, senior credit facility with BioPharma Credit. The new credit facility consists of a $150 million non-dilutive term loan, which requires interest-only payments through 2022 and matures in 2023. Interest will accrue at a floating rate of LIBOR plus 7.5%, subject to a floor and ceiling on the rate. There are no warrants or any equity conversion features associated with the loan. The proceeds from this financing will be used to support the cost of the acquisition and several years of related development costs.

Conference Call and Webcast
Amicus Therapeutics will host a conference call and audio webcast today, September 20, 2018, at 8:00 a.m. ET to discuss the new gene therapy pipeline. Interested participants and investors may access the conference call by dialing 877-303-5859 (U.S./Canada) or 678-224-7784 (international), conference ID: 7084639.

An audio webcast 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 go to the website 15 minutes prior to the start of the call to register, download, and install any necessary software. A telephonic replay of the call will be available for seven days beginning at 11:30 a.m. ET on September 20, 2018. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); conference ID: 7084639.

About Batten Disease
Batten disease is the common name for a broad class of rare, fatal, inherited disorders of the nervous system also known as neuronal ceroid lipofuscinoses, or NCLs. In these diseases, a defect in a specific gene triggers a cascade of problems that interferes with a cell's ability to recycle certain molecules. The disease has several forms with similar features and symptoms but vary in severity and age of onset. Each form is caused by a mutation in a different gene. Most forms of Batten disease/NCLs usually begin during childhood. The first symptom is usually progressive vision loss in previously healthy children followed by personality changes, behavioral problems and slow learning. Seizures commonly appear within 2-4 years of vision loss. Seizures and psychosis can appear at any time during the course of disease. Patients typically experience progressive loss of motor functions and eventually, those affected become wheelchair-bound, are bedridden, and die prematurely.

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 acquisition of Celenex, preclinical and clinical development of our acquired product candidates, the timing and reporting of results from these preclinical studies and clinical trials, and financing plans for the Company. 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 acquisition 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 product candidates as advisors. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. 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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