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Amicus Therapeutics Expands Biologics Pipeline with New Preclinical Program for Cyclin-Dependent Kinase-Like 5 (CDKL5) Deficiency

Potential First-in-Class Protein Replacement Therapy for Devastating Rare Genetic Neurological Disorder with No Approved Treatments

CRANBURY, N.J., July 06, 2016 (GLOBE NEWSWIRE) -- Amicus Therapeutics, Inc. (Nasdaq:FOLD), a biotechnology company at the forefront of rare and orphan diseases, has expanded its biologics pipeline with a new preclinical program for cyclin-dependent kinase-like 5 (CDKL5) deficiency, a rare and devastating genetic neurological disease for which there is no currently approved treatment. Signs and symptoms typically begin with persistent, spontaneous seizures in infancy followed by severe delays in neurological development.

"The CDKL5 program fits perfectly with our vision to build a leading global biotechnology company focused on rare and devastating diseases," stated John F. Crowley, Chairman and Chief Executive Officer of Amicus. "While we remain sharply focused on the Galafold™ launch and advancing our core clinical programs in Pompe and Epidermolysis Bullosa, this CDKL5 program is an important investment in our stated strategy to expand our biologics pipeline by integrating new, innovative technologies to develop first- and best-in-class therapies for patients who are in desperate need of new treatments. CDKL5 is a rare and devastating disease with no approved treatment. Most children with CDKL5 have frequent seizures that begin shortly after birth. They experience severe impairment in neurological development, and many of them are unable to walk, talk or care for themselves. We are pleased to partner with the CDKL5 patient and medical community to elevate disease awareness as we advance towards a treatment."

Amicus has obtained the rights and related intellectual property to a preclinical CDKL5 program through the acquisition of MiaMed, Inc. Under the terms of the acquisition agreement with MiaMed, at closing, Amicus paid approximately $1.8 million in cash and approximately $4.7 million in Amicus common stock to the former shareholders of MiaMed. In addition, the former shareholders of MiaMed are eligible to receive up to $18 million upon the achievement of clinical and regulatory milestones and up to $65 million upon achievement of commercial milestones. The acquisition of MiaMed does not impact previous full-year 2016 net cash spend guidance of $135 million to $155 million.

"As the parent of a child living with CDKL5 deficiency, I strongly believe that restoration of missing CDKL5 protein holds the most promise for a future therapy," stated Michael Jasulavic, President and Chief Executive Officer of MiaMed. "It is this belief that led to the formation of MiaMed, a company dedicated to a protein replacement therapy for CDKL5. With this announcement, I am very pleased that Amicus will continue the work that was started amongst the patient and academic communities. Amicus is a truly patient-centric company with a global reach and proven R&D expertise necessary for the development of such important treatments. I am confident Amicus' advancement of this program will raise CDKL5 awareness and, most importantly, increase the potential for success in developing a CDKL5 protein replacement therapy."

"Today there is no approved treatment option for patients with CDKL5. The number of patients diagnosed has been increasing rapidly as more people learn about the disease, and physicians become more familiar with testing and diagnostic protocols. We welcome all efforts that accelerate the ongoing research and development of CDKL5 protein replacement therapy and help to raise awareness of CDKL5 deficiency," said Ashley R. Winslow, PhD, Director of Neurogenetics of the Orphan Disease Center at the University of Pennsylvania.[1]

"While there has been a significant collaboration between the patient organizations and academia in research and developing new treatments for CDKL5 deficiency, Amicus is among the first in the industry to add a CDKL5 development program and we look forward to learning more as the preclinical studies progress," added Dr. Winslow, who is also Chief Scientific Officer of the LouLou Foundation, a non-profit funder of CDKL5 research globally which held a beneficial interest in MiaMed.

About CDKL5 Deficiency

CDKL5 (cyclin-dependent kinase-like 5) is a gene on the X-chromosome encoding the CDKL5 protein that regulates the expression of several essential proteins for normal brain development. Genetic mutations in the CDKL5 gene result in CDKL5 protein deficiency. The disorder manifests clinically as persistent seizures starting in infancy, followed by severe impairment in neurological development. Most children affected by CDKL5 deficiency cannot walk or care for themselves

and may also suffer from scoliosis, visual impairment, sensory issues, and gastrointestinal complications. CDKL5 mutations have been found in children diagnosed with cerebral palsy, infantile epilepsies, and autism, among other conditions, and the disorder was previously classified as atypical Rett Syndrome, an early seizure variant of Rett Syndrome. There are more than 1,200 documented cases of CDKL5 deficiency worldwide, with the number of identified patients increasing as genetic testing for the disorder becomes more common. For more information, and for a list of CDKL5 deficiency resources and foundations, please visit the Patient Advocacy section of the Amicus Therapeutics corporate website at http://www.amicusrx.com/patient_advocacy.php.

About Amicus Therapeutics

Amicus Therapeutics, Inc. (Nasdaq:FOLD) is a 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, SD-101 for Epidermolysis Bullosa (EB), as well as novel enzyme replacement therapy (ERT) and biologic products for Fabry disease, Pompe disease, and other rare and devastating diseases.

[1]Ashley Winslow, PhD, is an employee of the University of Pennsylvania and has no financial conflict of interest with MiaMed or Amicus. She does not receive any financial compensation from the LouLou Foundation although part of her salary is covered from a grant from LouLou Foundation to Penn.

Forward-Looking Statements

This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to preclinical and clinical development of our product candidates and related programs, including the preclinical CDKL5 program, the timing and reporting of results from preclinical studies and clinical trials, the prospects and timing of the potential regulatory approval of our product candidates, commercialization plans, financing plans, and the projected cash position for the Company. Words such as, but not limited to, "believe," "expect," "anticipate," "estimate," "intend," "potential," "plan," "likely," "may," "will," "would," "should" and "could," and similar expressions or words identify forward-looking statements. Such forward-looking statements are based upon current expectations that involve risks, changes in circumstances, assumptions and uncertainties. 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, with respect to statements regarding the expected benefits of the MiaMed acquisition and the anticipated impact on our business, the future prospects of the CDKL5 program, the goals, progress, timing, and outcomes of discussions with regulatory authorities, and in particular the potential goals, progress, timing, and results of preclinical studies and clinical trials, actual results may differ materially from those set forth in this release due to the risks and uncertainties inherent in our business, including, without limitation: the possibility that the expected benefits of the MiaMed acquisition may not be fully realized or may take longer to realize than expected; the possibility that we may not be able to timely and successfully develop, gain regulatory approval for or commercialize a CDKL5 protein replacement therapy; 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 and EMA, may not grant or may delay approval for our product candidates; the potential that we may not be successful in commercializing our product candidates if and when approved; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; and the potential that we will need additional funding to complete all of our studies. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. With respect to statements regarding projections of the Company's cash position, including the impact of the completion of the MiaMed acquisition on our business, actual results may differ based on market factors and the Company's ability to execute its operational and budget plans. 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, 2015, our Quarterly Report on Form 10-Q for the quarter ended March 31, 2016, and our other periodic reports filed 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 news release to reflect events or circumstances after the date hereof.

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CONTACTS:

Investors/Media:

Amicus Therapeutics

Sara Pellegrino

Senior Director, Investor Relations

spellegrino@amicusrx.com

(609) 662-5044

Media:

MWW PR

Sid Dinsay

sdinsay@mww.com

(646) 381-9017


Primary Logo

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