Amicus Therapeutics Receives European Medicines Agency PRIME Designation for CLN6 Batten Disease Gene Therapy

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Additional Phase 1/2 Data to be Presented at the Child Neurology Society Annual Meeting in October

CRANBURY, N.J., Sept. 24, 2020 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq: FOLD), a global, patient-dedicated biotechnology company focused on discovering, developing and delivering novel medicines for rare diseases, today announced the European Medicines Agency (EMA) has granted Priority Medicines (PRIME) designation to AT-GTX-501, the Company's investigational gene therapy for children living with variant late infantile neuronal ceroid lipofuscinosis 6 (vLINCL6) disease, also known as CLN6 Batten disease.

The PRIME initiative provides enhanced support and increased interaction to developers of promising medicines with the goal of optimizing development plans and speeding regulatory evaluations. The goal of EMA's PRIME is to help patients benefit as early as possible from innovative new therapies that have demonstrated the potential to significantly address an unmet medical need.

The PRIME designation is based on data from the ongoing Phase 1/2 clinical trial evaluating a single dose of AT-GTX-501 for the treatment of children with CLN6 Batten disease. Additional information about the trial is available at ClinicalTrials.gov (NCT02725580).

"We are very pleased that the EMA has recognized the potential of our CLN6 gene therapy. Based on our preliminary clinical data, we believe AT-GTX-501 could potentially be a transformative treatment option for children living with CLN6 Batten disease, an ultra-rare, debilitating condition that presents in early childhood and is often associated with childhood death," said John F. Crowley, Chairman and Chief Executive Officer. "We look forward to continuing to work closely with the EMA to accelerate development of this first potential treatment option for children living with CLN6 Batten disease."

Additional data from the ongoing Phase 1/2 clinical study will be presented at the Child Neurology Society Annual Meeting in October. Regulatory interactions are ongoing and the Company expects to provide feedback on the path forward in early 2021.

In the U.S., AT-GTX-501 previously was granted Rare Pediatric Disease and Orphan Drug designations by the United States Food and Drug Administration. In the EU, the Company now holds PRIME and orphan medicinal product designations.

About AT-GTX-501
AT-GTX-501 is a novel gene therapy in Phase 1/2 development for CLN6 Batten disease, a rare, fatal, inherited lysosomal disorder with no approved treatment that primarily affects the nervous system. AT-GTX-501 is dosed in a one-time intrathecal infusion to deliver a functional copy of the CLN6 gene to cells of the central nervous system. The therapy is designed to address the underlying enzyme deficiency that results in progressive cell damage and neurodevelopmental and physical decline.

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 disorders, a defect in a specific gene triggers a cascade of problems that interferes with a cell's ability to recycle certain molecules. Each gene is called CLN (ceroid lipofuscinosis, neuronal) and given a different number designation as its subtype. There are 13 known forms of Batten disease often referred to as CLN1-8; 10-14. The various types of Batten disease have similar features and symptoms but vary in severity and age of onset.

Most forms of Batten disease/NCLs usually begin during childhood. The clinical course often involves progressive loss of independent adaptive skills such as mobility, feeding and communication. Patients may also experience vision loss, personality changes, behavioral problems, learning impairment and seizures. Patients typically experience progressive loss of motor function and eventually become wheelchair-bound, are then bedridden and die prematurely.

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, and follow on Twitter and LinkedIn.

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, the timing and reporting of results from preclinical studies and clinical trials and the prospects and timing of the potential regulatory approval of our product candidates. In particular, this press release relates to interim data from an ongoing Phase 1/2 study to investigate intrathecal administration of AAV-CLN6 gene therapy. The inclusion of forward-looking statements arising from this interim data, ongoing study and natural history preliminary data 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 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 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; 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. The interim data and Phase 1/2 study discussed herein is inherently preliminary and early in the study, derived from a limited patient set, and later trial results with this patient set or others may not be consistent with these preliminary 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, 2019 and Quarterly Report on Form 10-Q for the quarter ended June 30, 2020. 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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