Amicus Announces Additional Positive Interim Clinical Data for CLN6 Batten Disease Gene Therapy at 48th Annual Meeting of the Child Neurology Society

October 24, 2019

New Data Support Meaningful Impact on Motor and Language Function in Children with Fatal Neurologic Disease

Evidence of Disease Stabilization in 7 of 8 Children with Data for up to 2 Years Post-Treatment

CRANBURY, N.J., and CHARLOTTE, N.C., Oct. 24, 2019 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq: FOLD) today announced additional positive interim results from its CLN6 Batten disease gene therapy program licensed from the Abigail Wexner Research Institute (AWRI) at Nationwide Children's Hospital. The results are featured in a poster presentation at the 48th Annual Meeting of the Child Neurology Society to be held October 23-26, 2019 in Charlotte, NC. The poster is available in the Events and Presentations section of the Amicus Therapeutics corporate website at http://ir.amicusrx.com/events-and-presentations.

AWRI is conducting the ongoing Phase 1/2 clinical study of a single one-time intrathecal administration of AAV-CLN6 gene therapy for CLN6 Batten disease. With no approved treatments, CLN6 Batten disease is a fatal neurologic disease that rapidly robs children of their ability to walk, speak, think, and see.

Clinical Data Highlights:
Interim safety data are available for 12 patients. Interim efficacy data are available for the first eight children with CLN6 Batten disease for up to 24 months post-administration of the AAV-CLN6 gene therapy.

- **Safety (n=12)**: as previously announced, treatment with AAV-CLN6 gene therapy was generally well tolerated. The majority of adverse events (AEs) were mild and unrelated to treatment. Additional details are provided in the poster.
- **Hamburg Motor & Language Aggregate Score (n=8)**: as previously announced, as of the interim analysis from this ongoing study, the Hamburg Motor & Language Score, an assessment of ambulation and speech, shows a positive impact on motor and language function for 7 of 8 patients treated (16-25 months post-administration of the gene therapy as of this data cutoff). Additional details are provided in the poster.
- **Hamburg Motor, Language, Seizure and Vision Subscores (n=8)**: new data on motor, language, seizure and vision subscores suggest stabilization of these individual components in most patients from baseline to months 12 and 24, in particular those patients treated at a younger age, further supporting the positive impact of one-time intrathecal AAV gene therapy in children with CLN6 Batten disease. Additional details are provided in the poster.
- **Natural History and Sibling Pair Comparisons**: as previously announced, the AAV-CLN6 gene therapy demonstrated a positive impact on motor and language function compared to a natural history data set, including matched comparisons, as well as in comparisons within sibling pairs. Additional details are provided in the poster.

Jay Barth, Chief Medical Officer of Amicus Therapeutics, stated, "We are pleased to share these positive interim clinical data for our intrathecal AAV gene therapy with the CLN6 physician community for the first time at Child Neurology Society. The aggregate and individual Hamburg components, in addition to the natural history data and sibling comparisons, continue to suggest that our gene therapy in CLN6 Batten disease has the potential to halt the progression of this devastating disease that causes loss of brain function and is fatal in childhood."

Emily de los Reyes, MD, PhD, Principal Investigator of the CLN6 clinical trial at AWRI at Nationwide Children's and Professor of Clinical Pediatrics and Neurology at The Ohio State University College of Medicine stated, "I continue to be pleased with the progress of this trial as well as our collection of matched natural history data to further inform the results for the AAV-CLN6 gene therapy. I look forward to following these initial study participants as well as collecting new data for additional participants as we continue to advance the program in CLN6 and move additional Batten disease programs through the clinic in collaboration with Amicus."

Upcoming Amicus Milestones in Next 12 Months:

- Collection and presentation of additional natural history data in CLN6 Batten disease
- Dosing of additional patients
- Advance regulatory discussions
- Manufacturing of additional AAV-CLN6 gene therapy underway at Thermo Fisher (Brammer Bio)
- Continued advancement of AAV gene therapy programs in CLN3, CLN8 and CLN1 Batten disease.

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. 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, 2018 and Quarterly Report on Form 10-Q for the quarter ended June 30, 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 news release to reflect events or circumstances after the date hereof.

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