Amicus Therapeutics Announces Positive Initial Clinical Data for CLN3 Batten Disease Gene Therapy at the 17th Annual WORLDSymposium™ 2021

February 8, 2021

Initial Data Suggest Early Signs of Disease Stabilization in Children with Fatal Neurologic Disease

Plan to Submit IND for Next Clinical Study in 2H2021

PHILADELPHIA, Feb. 08, 2021 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq: FOLD) today announced positive initial results from its first in-human study of its CLN3 Batten disease gene therapy program, AT-GTX-502. The results are featured in a virtual poster presentation at the 17th Annual WORLD*Symposium* ™ 2021, being held February 8-12, 2021. The poster is also available in the Events and Presentationssection of the Amicus Therapeutics corporate website.

The Abigail Wexner Research Institute (AWRI) at Nationwide Children's Hospital is conducting the ongoing Phase 1/2 clinical study of a single one-time administration of AT-GTX-502 gene therapy for classic juvenile neuronal ceroid lipofuscinosis (JNCL), also known as CLN3 Batten disease. With no approved treatments, CLN3 Batten disease is a fatal neurologic disease that leads to blindness, motor impairment, learning difficulties, epilepsy and, ultimately, premature death.

Primary outcome measures are determined using the physical impairment subscale of the Unified Batten Disease Rating Scale (UBDRS), a clinical rating instrument developed specifically to assess disease progression in children with verified JNCL and includes evaluations of motor, behavioral, seizure and functional capabilities. UBDRS separately scores measures of vision, motor, speech, tone and abnormal movement over time. Higher scores indicate greater physical impairment.

Clinical Data Highlights:

Initial safety data are available for the first four children up to 15 months post-administration of the AAV-CLN3 gene therapy. Preliminary efficacy data are available for the first three children with CLN3 Batten disease in the low-dose cohort for up 15 months post-administration of the AAV-CLN3 gene therapy, as well as one participant with CLN3 Batten disease in the high-dose cohort for up to 3 months post-administration of the AAV-CLN3 gene therapy. Initial results of the study suggest that AT-GTX-502 was well tolerated and demonstrated potential early signs of disease stabilization compared to a natural history dataset.

- **Safety (n=4)**: Treatment with AT-GTX-502 was generally well tolerated. The majority of adverse events (AEs) were mild or moderate and unrelated to treatment. No pattern of AEs related to AAV or CLN3 immunogenicity were observed. Additional details are provided in the presentation.
- **Unified Batten Disease Rating Scale**: For the three subjects treated in the low-dose cohort (n=3), the mean yearly rate of change in UBDRS Physical Impairment scores was +0.07 over 12 months vs +2.86 in untreated subjects from published natural history (n=82).

Jeff Castelli, Ph.D., Chief Development Officer of Amicus Therapeutics, stated, "We are pleased to share this first set of clinical data for our intrathecal AAV gene therapy for CLN3 Batten disease and the second clinical program in our Batten portfolio. Preliminary results from this analysis suggest early signs of disease stabilization and has the potential to slow the neurological disease progression in children with CLN3 Batten disease. We are encouraged by the data and hope to make a meaningful impact for individuals living with CLN3 Batten disease, an ultra-rare, devastating neurodegenerative disease with no approved treatments."

Emily de los Reyes, M.D., Ph.D., Principal Investigator at Nationwide Children's and Professor of Clinical Pediatrics and Neurology at The Ohio State University College of Medicine is leading the CLN3 clinical trial at AWRI.

Regulatory interactions for AT-GTX-502 are ongoing and the Company expects to provide feedback on the clinical path forward later this year.

Amicus has exclusive rights under a license to the CLN3 gene therapy program developed at the Abigail Wexner Research Institute at Nationwide Children's Hospital.

About AT-GTX-502

AT-GTX-502 is a novel gene therapy in Phase 1/2 development for CLN3 Batten disease, a rare, fatal, inherited lysosomal disorder with no approved treatment that primarily affects the nervous system. AT-GTX-502 is dosed in a one-time infusion to deliver a functional copy of the CLN3 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. In the U.S., AT-GTX-502 was granted Fast Track Designation and Rare Pediatric Designation by the United States Food and Drug Administration. AT-GTX-502 also holds Orphan Drug designations in both the U.S. and in the EU.

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. Affected children may also experience vision loss, personality changes, behavioral problems, learning impairment and seizures. Children living with Batten disease 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-CLN3 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 September 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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