Amicus Announces Positive Interim Clinical Data for AAV Gene Therapy in Children with CLN6 Batten Disease

August 1, 2019

Study Shows 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

Conference Call at 8:30 a.m. ET Today

CRANBURY, N.J., Aug. 01, 2019 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq: FOLD) today announced positive interim results from its CLN6 Batten disease gene therapy program licensed from the Abigail Wexner Research Institute (AWRI) at Nationwide Children's Hospital. 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.

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. The Hamburg Motor & Language Score, an assessment of ambulation and speech, showed that the AAV-CLN6 gene therapy demonstrated a positive impact on motor and language function compared to a natural history dataset, as well as in comparisons within sibling pairs. Treatment with AAV-CLN6 gene therapy was generally well tolerated. This intrathecal AAV-CLN6 gene therapy uses the same capsid as the recently FDA approved systemic gene therapy, also initially developed at AWRI, for the fatal neurologic disease Spinal Muscular Atrophy Type 1.

More detailed information on the initial clinical results can be found in the Events and Presentations section of the Amicus Therapeutics corporate website at http://ir.amicusrx.com/events-and-presentations.

Clinical Data Highlights:

- **Safety (n=12)**: The majority of adverse events (AEs) were mild and unrelated to treatment in a total of 12 patients in the clinical study (exposure duration 6 to 39 months). No pattern of AEs related to anti-AAV capsid or anti-CLN6 immunogenicity were observed.
- **Hamburg Motor & Language (n=8)**: As of the interim analysis from this ongoing study, efficacy data show a positive impact on motor and language function for 7 of 8 patients treated. These eight patients are from 16-25 months post-administration of the gene therapy as of this data cutoff. Seven patients (treated at 19 to 66 months of age) maintained their Hamburg score or had an initial change (ranging from +1 to -1 points) followed by stabilization. The oldest patient in the study (treated at 79 months of age) had a 2 point decline.
- **Hamburg Motor & Language in Sibling Pairs**: Three treated patients demonstrated stabilization relative to their untreated siblings in the natural history data set who experienced substantial declines in motor and language ability or died over the same time period. For the two pairs of in-study siblings, the younger siblings demonstrated an increase or stabilization in their score compared to their older siblings who had an initial change followed by stabilization.
- **Hamburg Motor & Language Natural History (n=14)**: An initial natural history cohort from Nationwide Children's shows disease progression in all untreated patients, with at least a 2- to 3-point decline in Hamburg Motor & Language score from the initial point of decline over 24 months.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, stated, "This program and these initial results represent the heart and soul of who we are and why we do what we do at Amicus. These interim clinical data suggest that our gene therapy in CLN6 Batten disease has the potential to halt the progression of this devastating fatal disease that untreated destroys brain function and kills children. It is remarkable that most children in this study appear to show stabilization, particularly the younger children who were able to maintain high baseline motor and language scores for up to two years. We know that brain damage here is irreversible, and early intervention will be critical to preserve the ability to speak and walk. We look forward to presenting additional data throughout this year and continuing to advance our CLN6 and other Batten disease gene therapy programs that all apply the same AAV technology platform developed by Dr. Brian Kaspar and his former colleagues at Nationwide Children's. Early intervention is crucial, so we move forward with a great sense of urgency here for these children and their families."

Tauna Batiste, Executive Director of the Batten Disease Support and Research Association (BDSRA) commented, "We are hopeful the clinical results presented today represent a notable step forward towards finding a treatment for children suffering from CLN6 disease. This data also provides encouragement for many families within the broader Batten disease community who are following the progress of the Amicus gene therapy programs for CLN3, CLN8, and CLN1 disease. We look forward to seeing additional data in CLN6 disease and to the continued advancement of all the Batten disease programs."

The Hamburg Motor & Language Score (0-6) separately measures performance of mobility (0-3) and speech (0-3). For each domain, a 3 represents the child's normal function and a 0 represents no ability to walk or speak, with each point decline representing significant impairment. Children living with CLN6 Batten disease often experience typical development for the first few years of life. Following symptom onset, natural history indicates that there often is a rapid progression from a 6 to a 0 score within 3-4 years (or a 1-2 point annual decline).

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, "Comparing the data collected during the CLN6 Batten disease clinical trial with the natural history data collected for Batten's disease patients, I am pleased with the progress of this trial. It is powerful to have pairs of siblings as clinical trial participants since siblings are expected to have similar progression of the disease."

Dr. Brian Kaspar, PhD, former Professor of Pediatrics, Faculty at AWRI, Dr. Kathrin Meyer, Principal Investigator at AWRI, and their team at AWRI, in collaboration with Dr. Jill Weimer's laboratory at Sanford Research, developed the AAV gene replacement strategy that is the basis for the AAV-CLN6 gene therapy.

Dr. Kaspar added, "I am thrilled to see these clinical results for AAV-CLN6 gene therapy. It is gratifying to see the teamwork between Nationwide Children's and Sanford researchers and clinicians to translate gene therapy from preclinical animal studies to humans, leading to the current data which demonstrate evidence for safety and initial efficacy in the first children treated. I believe this AAV-CLN6 gene therapy has the potential to make a very meaningful impact for children with CLN6 Batten disease, and provides great promise to address many types of Batten disease and other neurologic disorders."

Upcoming Amicus Milestones in Next 12 Months:

- Presentation of additional data measures in the eight initial patients at the Amicus Analyst Day this Fall and in a poster presentation by Dr. de los Reyes at the Child Neurology Society Annual Meeting, October 23-26, 2019 (Charlotte, NC).
- 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.

Conference Call and Webcast on August 1, 2019 at 8:30 a.m. ET

Amicus Therapeutics will host a conference call and audio webcast today, August 1, 2019 at 8:30 a.m. ET. Members of the Amicus leadership team to discuss the positive interim Phase 1/2 CLN6 Batten disease data. Interested participants and investors may access the conference call by dialing (877) 303-5859 (U.S./Canada) or (678) 224-7784 (international), conference ID: 1186207.

A live 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 register on the website 15 minutes prior to the start of the call. A replay of the call will be available for seven days beginning at 11:30 a.m. ET on August 1, 2019. Access numbers for this replay are (855) 859-2056 (U.S./Canada) and (404) 537-3406 (international); conference ID: 1186207.

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 March 31, 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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