



## Amicus Therapeutics Announces Phase 1/2 Study of Gene Therapy for CLN3 Batten Disease

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***Second Gene Therapy to Enter Clinic at Nationwide Children's Hospital for Neurologic Lysosomal Storage Disorders***

***Initial Safety is Encouraging with No Serious Adverse Events at One Month***

CRANBURY, N.J., Jan. 03, 2019 (GLOBE NEWSWIRE) -- [Amicus Therapeutics](#) (Nasdaq: FOLD), a global biotechnology company focused on discovering, developing and delivering novel medicines for rare metabolic diseases, today announced the initiation of a Phase 1/2 clinical [study](#) to evaluate the safety and efficacy of a single intrathecal administration of adeno-associated virus serotype 9 AAV9-CLN3 (AAV9-CLN3) gene therapy in children with CLN3 [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. The initial patient completed a one-month observation period following dosing with no serious adverse events reported to date.

This first in human study of an investigational gene therapy in CLN3 Batten disease, a life-threatening genetic neurologic disorder that typically begins in early childhood and results in premature death, is currently being conducted at Nationwide Children's Hospital (Columbus, Ohio). CLN3 is the most prevalent of the Batten's disorders affecting an estimated 5,000+ patients.

"We are pleased to announce that the first child has been dosed in the Phase 1/2 study for CLN3 Batten disease," said Jay Barth, MD, Chief Medical Officer at Amicus Therapeutics, Inc. "With this pioneering study in CLN3 Batten disease, a severe and devastating neurodegenerative disorder with no approved treatments, we hope that a single intrathecal administration of our gene therapy has the potential to treat the underlying cause of disease and provide a durable and meaningful treatment benefit. This initial participant in our CLN3 Batten disease study, together with the participants in our ongoing clinical study in CLN6 Batten disease, further supports the encouraging safety profile for the intrathecal AAV delivery platform. Advancing this program is another important step forward to potentially improve the lives of thousands of children living with Batten disease and other neurologic lysosomal storage disorders."

Emily C. De Los Reyes, MD, Principal Investigator at Nationwide Children's stated, "The initiation of this clinical study in CLN3 Batten disease is an important initial milestone. The proof-of-concept demonstrated in preclinical studies and validation of the intrathecal AAV platform across multiple indications here at Nationwide Children's Hospital provides promise for the delivery approach in this study. We look forward to enrolling additional participants in the study and comparing the findings to the existing natural history data in CLN3 Batten disease."

The Phase 1/2 study is enrolling children aged 3 to 10 years with a confirmed diagnosis of CLN3 Batten disease in two sequential intrathecal dose groups: a one-time low-dose of AAV9-CLN3 (Group 1) and a one-time high-dose of AAV9-CLN3 (Group 2) after evaluation of Group 1 participants. Both groups will participate in the current study for a period of three years. Amicus plans to present clinical data from this study, including interim data, at future scientific congresses and other relevant venues. The primary outcome measures are safety and efficacy as determined using the physical disability subscale of the Unified Batten Disease Rating Scale (UBDRS) in CLN3 Batten disease. More information is available at [www.clinicaltrials.gov](http://www.clinicaltrials.gov): NCT03770572.

CLN3 Batten disease, the most common form of NCL results from a mutation in the CLN3 gene which primarily affects the nervous system. Children with this condition develop vision impairment, intellectual disability, progressive loss of motor function, speech difficulties, and seizures which worsen over time.

### **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 those affected 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](http://www.amicusrx.com), and follow us 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, including statements relating to preliminary safety information from a Phase 1/2 study to investigate intrathecal administration of AAV9-CLN3 for the treatment of CLN3 Batten disease. Words such as, but not limited to, "look forward to," "believe," "expect," "anticipate," "estimate," "intend," "confidence," "encouraged," "potential," "plan," "targets," "likely," "may," "will," "would," "should" and "could," and similar expressions or words identify forward-looking statements. The forward looking statements included in this press release are based on management's current expectations and beliefs which are

subject to a number of risks, uncertainties and factors, including that the preliminary information based on a single patient and reported before completion of the study will not be predictive of future results, that results of additional preliminary data or data from the completed study or any future study will not yield results that are consistent with the preliminary data presented from this study or the CLN6 study, that the Company will be not able to demonstrate the safety and efficacy of intrathecal administration of AAV9-CLN3, that later study results will not support further development, or even if such later results are favorable, that the Company will not be able to successfully complete the development of, obtain regulatory approval for, obtain any desirable third party licenses or successfully commercialize AAV9-CLN3. In addition, all forward looking statements are subject to the other risks and uncertainties detailed in our Annual Report on Form 10-K for the year ended December 31, 2017 and Quarterly Report on 10-Q for the Quarter ended September 30, 2018. As a consequence, actual results may differ materially from those set forth in this press release. You are cautioned not to place undue reliance on these forward looking statements, which speak only of the date hereof. All forward looking statements are qualified in their entirety by this cautionary statement and we undertake no obligation to revise this press release to reflect events or circumstances after the date hereof.

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