Biogen Initiates Phase 3 Pediatric Study of Omaveloxolone for the Treatment of Friedreich Ataxia

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- Global Phase 3 BRAVE study will evaluate the efficacy and safety of omaveloxolone in children 2 to <16 years old with Friedreich ataxia, a rare neurodegenerative disorder
- BRAVE study will explore the potential of omaveloxolone to address the critical unmet need of the pediatric FA population
- Omaveloxolone is currently marketed under the brand name SKYCLARYS® for the treatment of adults and adolescents aged 16 years and older affected by Friedreich ataxia

CAMBRIDGE, Mass., June 18, 2025 (GLOBE NEWSWIRE) -- Biogen Inc. (Nasdaq: BIIB) announced the initiation of dosing in the BRAVE study, a global Phase 3 clinical trial. The BRAVE study will evaluate the efficacy and safety of omaveloxolone in children with Friedreich ataxia (FA) between the ages of 2 to <16. Both non-ambulatory and ambulatory participants may qualify for the study. Participants will be randomized 2:1 to receive omaveloxolone or placebo once a day for 52 weeks before having the opportunity to move into the open-label extension (OLE). Currently, omaveloxolone is commercialized under the brand name SKYCLARYS® in over 40 countries, including in the U.S. and the European Union, and is the only approved product for FA in adults and adolescents aged 16 years and older.

“Recognizing the symptoms of Friedreich ataxia typically begin in childhood, and earlier onset of symptoms is associated with faster disease progression, there is tremendous unmet need in the pediatric community. Building on the work of Reata we have been urgently advancing the pediatric development plan for omaveloxolone and are thrilled that the Phase 3 BRAVE study has now begun,” said Stephanie Fradette, Pharm.D., Head of the Neuromuscular Development Unit at Biogen. “We are immensely grateful for the input from the entire FA community that has helped shape the design of this important study.”

The BRAVE study will evaluate the efficacy, safety, pharmacokinetics and pharmacodynamics of omaveloxolone in approximately 255 children living with FA. Part 1 is a 52 week, randomized, double-blind, placebo-controlled study, designed to evaluate efficacy of omaveloxolone compared to placebo. The primary outcome measure for Part 1 is change from baseline in Upright Stability Score (USS), a subscale that is recognized by the FA community as the most sensitive means of measuring disease progression in children living with FA. USS is part of the validated modified FA rating scale (mFARS). Part 2 will be an open-label extension (up to week 104) where all participants will receive omaveloxolone to further our understanding of the long-term effects of the drug.

“Early onset patients often have the most aggressive and fast progressive form of Friedreich ataxia and through the BRAVE study we aim to determine the potential safety and efficacy of omaveloxolone for children living with the disease. This vulnerable population faces significant unmet need, with no approved treatments currently available,” said Susan Perlman, M.D., Professor of Neurology and Director of the Ataxia Center, David Geffen School of Medicine at UCLA.

The design of this Phase 3 study has been informed by previous studies and input from investigators, global medical experts and the FA community. Enrollment has initiated in the United States and we plan to open BRAVE study sites around the world pending final alignment with local regulators and ethics committees. Individuals interested in participating in this study should speak with their healthcare provider. They can also email Biogen at clinicaltrials@biogen.com. Individuals located in the U.S. can also call the Biogen Clinical Trials Center at 866-633-4636. Both clinicaltrials.gov (NCT06953583) and Biogen Trial Link (Biogen Trial Link) will be updated as more information about the BRAVE study becomes available.

About SKYCLARYS® (omaveloxolone)
SKYCLARYS® (omaveloxolone) is an oral, once-daily medication indicated for the treatment of Friedreich’s ataxia (FA) in adults and adolescents aged 16 years and older in over 40 countries, including the U.S. and European Union. SKYCLARYS received Orphan Drug, Fast Track, and Rare Pediatric Disease Designations from the U.S. Food and Drug Administration. The European Commission granted Orphan Drug designation in Europe to SKYCLARYS for the treatment of FA.

Please click here for Important Safety Information and full Prescribing Information for SKYCLARYS® (omaveloxolone) in the U.S. or visit your respective country’s product website.

About Friedreich Ataxia
Friedreich ataxia (FA) is a rare, genetic, life-shortening, debilitating, and degenerative neuromuscular disorder. It is the most common inherited ataxia.[1,2,3] Early symptoms of FA, such as progressive loss of coordination, muscle weakness and fatigue, typically appear in childhood and can overlap with other diseases.[4] Most people living with FA will need to use a wheelchair within 10-20 years of their first symptoms.[2] The reported average age of death for FA patients is just 37 years old, although with appropriate and targeted care, individuals may live many years after confinement to a wheelchair.[5-7]

About Biogen
Founded in 1978, Biogen is a leading biotechnology company that pioneers innovative science to deliver new medicines to transform patients’ lives and to create value for shareholders and our communities. We apply deep understanding of human biology and leverage different modalities to advance first-in-class treatments or therapies that deliver superior outcomes. Our approach is to take bold risks, balanced with return on investment to deliver long-term growth.

We routinely post information that may be important to investors on our website at www.biogen.com. Follow us on social media - Facebook, LinkedIn, X, YouTube.

Biogen Safe Harbor
This news release contains forward-looking statements including relating to the potential benefits, safety and efficacy of SKYCLARYS; potential regulatory discussions, submissions and approvals and the timing thereof; the treatment of Friedreich’s ataxia; the potential of Biogen’s commercial

business and pipeline programs, including Friedreich's ataxia; and risks and uncertainties associated with drug development and commercialization. These statements may be identified by words such as "aim," "anticipate," "believe," "could," "estimate," "expect," "forecast," "intend," "may," "plan," "possible," "potential," "will," "would" and other words and terms of similar meaning. Drug development and commercialization involve a high degree of risk, and only a small number of research and development programs result in commercialization of a product. Results in early-stage clinical studies may not be indicative of full results or results from later stage or larger scale clinical studies and do not ensure regulatory approval. You should not place undue reliance on these statements.

Given their forward-looking nature, these statements involve substantial risks and uncertainties that may be based on inaccurate assumptions and could cause actual results to differ materially from those reflected in such statements. These forward-looking statements are based on management's current beliefs and assumptions and on information currently available to management. Given their nature, we cannot assure that any outcome expressed in these forward looking statements will be realized in whole or in part. We caution that these statements are subject to risks and uncertainties, many of which are outside of our control and could cause future events or results to be materially different from those stated or implied in this document, including, among others, factors relating to: our substantial dependence on revenue from our products and other payments under licensing, collaboration, acquisition or divestiture agreements; uncertainty of long-term success in developing, licensing, or acquiring other product candidates or additional indications for existing products; expectations, plans and prospects relating to product approvals, approvals of additional indications for our existing products, sales, pricing, growth, reimbursement and launch of our marketed and pipeline products; the potential impact of increased product competition in the biopharmaceutical and healthcare industry, as well as any other markets in which we compete, including increased competition from new originator therapies, generics, prodrugs and biosimilars of existing products and products approved under abbreviated regulatory pathways; our ability to effectively implement our corporate strategy; the successful execution of our strategic and growth initiatives, including acquisitions; the drivers for growing our business; difficulties in obtaining and maintaining adequate coverage, pricing, and reimbursement for our products; the drivers for growing our business, including our dependence on collaborators and other third parties for the development, regulatory approval, and commercialization of products and other aspects of our business, which are outside of our full control; risks associated with current and potential future healthcare reforms; risks related to commercialization of biosimilars, which is subject to such risks related to our reliance on third parties, intellectual property, competitive and market challenges and regulatory compliance; failure to obtain, protect, and enforce our data, intellectual property, and other proprietary rights and the risks and uncertainties relating to intellectual property claims and challenges; the risk that positive results in a clinical trial may not be replicated in subsequent or confirmatory trials or success in early stage clinical trials may not be predictive of results in later stage or large scale clinical trials or trials in other potential indications; risks associated with clinical trials, including our ability to adequately manage clinical activities, unexpected concerns that may arise from additional data or analysis obtained during clinical trials, regulatory authorities may require additional information or further studies, or may fail to approve or may delay approval of our drug candidates; the occurrence of adverse safety events, restrictions on use with our products, or product liability claims; risks relating to technology, including our incorporation of new technologies such as artificial intelligence into some of our processes; risks related to use of information technology systems and potential impacts of any breakdowns, interruptions, invasions, corruptions, data breaches, destructions and/or other cybersecurity incidents of our systems or those of connected and/or third-party systems; problems with our manufacturing capacity, including our ability to manufacture products efficiently or adequately address global bulk supply risks; risks relating to management, personnel and other organizational changes, including our ability to attracting, retaining and motivating qualified individuals; risks related to the failure to comply with current and new legal and regulatory requirements, including judicial decisions, accounting standards, and tariff or trade restrictions; the risks of doing business internationally, including geopolitical tensions, acts of war and largescale crises; risks relating to investment in our manufacturing capacity; risks relating to the distribution and sale by third parties of counterfeit or unfit versions of our products; risks relating to the use of social media for our business, results of operations and financial condition; fluctuations in our operating results; risks related to investment in properties; risks relating to access to capital and credit markets to finance our present and future operations and business initiatives and obtain funding for such activities on favorable terms; risks related to indebtedness; the market, interest, and credit risks associated with our investment portfolio; risks relating to share repurchase programs; change in control provisions in certain of our collaboration agreements; fluctuations in our effective tax rate and obligations in various jurisdictions in which we are subject to taxation; environmental risks; and any other risks and and uncertainties that are described in other reports we have filed with the U.S. Securities and Exchange Commission. These statements speak only as of the date of this press release and are based on information and estimates available to us at this time. Should known or unknown risks or uncertainties materialize or should underlying assumptions prove inaccurate, actual results could vary materially from past results and those anticipated, estimated or projected. Investors are cautioned not to put undue reliance on forward-looking statements. A further list and description of risks, uncertainties and other matters can be found in our Annual Report on Form 10-K for the fiscal year ended December 31, 2024 and in our subsequent reports on Form 10-Q and Form 10-K, in each case including in the sections thereof captioned "Note Regarding Forward-Looking Statements" and "Item 1A. Risk Factors," and in our subsequent reports on Form 8-K. Except as required by law, we do not undertake any obligation to publicly update any forward-looking statements whether as a result of any new information, future events, changed circumstances or otherwise.

References:

1. Friedreich's Ataxia Research Alliance. "What is FA?" Available at: https://www.curefa.org/what-is-friedreichs-ataxia#. Accessed February 2024.
2. National Institute of Neurological Disorders and Stroke. Friedreich Ataxia. Available at: https://www.ninds.nih.gov/health-information/disorders/friedreich-ataxia. Accessed February 2024.
3. Schulz JB, Boesch S, Bürk K, Dürr A, Giunti P, Mariotti C, Pousset F, Schöls L, Vankan P, Pandolfo M. Diagnosis and treatment of Friedreich ataxia: a European perspective. Nat Rev Neurol. 2009 Apr;5(4):222-34. doi: 10.1038/nrneurol.2009.26. PMID: 19347027.
4. Fogel BL, Perlman S. Clinical features and molecular genetics of autosomal recessive cerebellar ataxias. Lancet Neurol. 2007 Mar;6(3):245-57. doi: 10.1016/S1474-4422(07)70054-6. PMID: 17303531.
5. Parkinson MH, Boesch S, Nachbauer W, Mariotti C, Giunti P. Clinical features of Friedreich's ataxia: classical and atypical phenotypes. J Neurochem. 2013 Aug;126 Suppl 1:103-17. doi: 10.1111/jnc.12317. PMID: 23859346.
6. Tsou AY, Paulsen EK, Lagedrost SJ, Perlman SL, Mathews KD, Wilmot GR, Ravina B, Koeppen AH, Lynch DR. Mortality in Friedreich ataxia. J Neurol Sci. 2011 Aug 15;307(1-2):46-9. doi: 10.1016/j.jns.2011.05.023. Epub 2011 Jun 8. PMID: 21652007.
7. Corben LA, Collins V, Milne S, Farmer J, Musheno A, Lynch D, Subramony S, Pandolfo M, Schulz JB, Lin K, Delatycki MB; Clinical Management Guidelines Writing Group. Clinical management guidelines for Friedreich ataxia: best practice in rare diseases. Orphanet J Rare Dis. 2022 Nov 12;17(1):415. doi: 10.1186/s13023-022-02568-3. PMID: 36371255; PMCID: PMC9652828.

MEDIA CONTACT:
Biogen

INVESTOR CONTACT:
Biogen

Jack Cox
+ 1 781 464 3260
public.affairs@biogen.com

Tim Power
+1 781 464 2442
IR@biogen.com