CHMP Issues Positive Opinion for Biogen's SKYCLARYS® (omaveloxolone), the First Therapy to Treat Friedreich's Ataxia, a Rare Neurodegenerative Disease

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- Friedreich's ataxia is a genetic, debilitating and life-shortening neuromuscular disease [1]
- Milestone highlights Biogen's growing portfolio in rare diseases and focus on addressing unmet needs of patients living with neuromuscular diseases

CAMBRIDGE, Mass., Dec. 15, 2023 (GLOBE NEWSWIRE) -- Biogen Inc. (Nasdaq: BIIB) announced the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) recommended marketing authorization for SKYCLARYS® (omaveloxolone) for the treatment of Friedreich's ataxia (FA) in people aged 16 years and older. If approved by the European Commission (EC), SKYCLARYS will be the first treatment authorized within the European Union for this rare, genetic, progressive neuromuscular disease.[1] If approved, omaveloxolone will be marketed as SKYCLARYS.

"The CHMP's recommendation for SKYCLARYS is a significant milestone toward our goal of bringing a treatment that slows the progression of FA to the patient community in the region," said Priya Singhal, M.D., M.P.H., Head of Development at Biogen. "Upon approval of SKYCLARYS, we look forward to leveraging Biogen's rare disease expertise and capabilities to bring this groundbreaking treatment to patients in the European Union living with this debilitating disease."

The CHMP's positive opinion for SKYCLARYS is based on efficacy and safety data from the placebo-controlled MOXIe Part 2 trial. At the end of the 48-week MOXIe Part 2 study, patients who received SKYCLARYS had less physical impairment compared to patients who received placebo, as measured by the modified Friedreich Ataxia Rating Scale (mFARS). Improvements across subscales of mFARS, including upright stability, lower limb coordination, ability to swallow and upper limb coordination, were also observed in patients treated with SKYCLARYS compared to placebo. Additional data was provided from a post hoc, propensity-matched analysis in which patients treated with SKYCLARYS in MOXIe (Parts 1 and 2) had lower mFARS score at 3 years, as compared to a matched natural history group. The most common side effects are increased liver enzymes, decreased weight and appetite, nausea, vomiting, diarrhea, headache, fatigue, oropharyngeal and back pain, muscle spasms, and influenza.

The CHMP's recommendation for SKYCLARYS will now be reviewed by the EC for marketing authorization in the European Union with a final decision expected in the first quarter of 2024. The U.S. Food and Drug Administration (FDA) approved omaveloxolone, marketed as SKYCLARYS®, in February 2023 for the treatment of Friedreich's ataxia in adults and adolescents aged 16 years and older.

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 the U.S. SKYCLARYS has received Orphan Drug, Fast Track, and Rare Pediatric Disease Designations from the U.S. Food and Drug Administration. The European Commission has granted Orphan Drug designation in Europe to omaveloxolone for the treatment of FA.

About Friedreich's Ataxia
Friedreich's ataxia (FA) is a rare, genetic, life-shortening, debilitating, and degenerative neuromuscular disorder. It is the most common inherited ataxia.[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 global biotechnology company that has pioneered multiple breakthrough innovations including a broad portfolio of medicines to treat multiple sclerosis, the first approved treatment for spinal muscular atrophy, two co-developed treatments to address a defining pathology of Alzheimer's disease, the first treatment to target a genetic form of ALS, the first oral treatment approved for postpartum depression, and the first approved treatment for Friedreich's ataxia. Biogen is advancing a pipeline of potential novel therapies across neurology, neuropsychiatry, specialized immunology and rare diseases and remains acutely focused on its purpose of serving humanity through science while advancing a healthier, more sustainable and equitable world.

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 about the potential benefits, safety and efficacy of omaveloxolone; 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.

These statements involve risks and uncertainties that could cause actual results to differ materially from those reflected in such statements, including without limitation unexpected concerns that may arise from additional data, analysis or results obtained during clinical studies; the occurrence of adverse safety events; risks of unexpected costs or delays; the risk of other unexpected hurdles; regulatory submissions may take longer or be more difficult to complete than expected; regulatory authorities may require additional information or further studies, or may fail or refuse to approve or may

delay approval of Biogen's drug candidates, including omaveloxolone; actual timing and content of submissions to and decisions made by the regulatory authorities regarding omaveloxolone; uncertainty of success in the development and potential commercialization of omaveloxolone; failure to protect and enforce Biogen's data, intellectual property and other proprietary rights and uncertainties relating to intellectual property claims and challenges; product liability claims; third party collaboration risks; and the direct and indirect impacts of the ongoing COVID-19 pandemic on Biogen's business, results of operations and financial condition. The foregoing sets forth many, but not all, of the factors that could cause actual results to differ from Biogen's expectations in any forward-looking statement. Investors should consider this cautionary statement as well as the risk factors identified in Biogen's most recent annual or quarterly report and in other reports Biogen has filed with the U.S. Securities and Exchange Commission. These statements speak only as of the date of this news release. Biogen does not undertake any obligation to publicly update any forward-looking statements.

References:

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