Biogen Received European Commission Approval for SKYCLARYS® (omaveloxolone), the First Therapy to Treat Friedreich's Ataxia

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- Friedreich's ataxia is a rare, genetic, life-shortening, debilitating, and neurodegenerative disorder
- Treatment with SKYCLARYS improved patient function compared to placebo
- Biogen is leveraging its expertise and capabilities in rare disease to bring this groundbreaking treatment to patients

CAMBRIDGE, Mass., Feb. 12, 2024 (GLOBE NEWSWIRE) -- Biogen Inc. (Nasdaq: BIIB) announced the European Commission (EC) has authorized SKYCLARYS® (omaveloxolone) for the treatment of Friedreich's ataxia (FA) in adults and adolescents aged 16 years and older. SKYCLARYS is the first treatment approved within the European Union for this rare, genetic, progressive neurodegenerative disease.[1]

"In my clinical practice, I have seen the devastating impact that Friedreich's ataxia has on patients and their families," said Sylvia Boesch, M.D., MSc, Principal Investigator of the MOXIe study and Head of the Center for Rare Movement Disorders Innsbruck, Department of Neurology, Medical University Innsbruck, Austria. "Friedreich's ataxia patients treated with SKYCLARYS in the clinical trial experienced important and clinically meaningful improvements for their daily lives. With this approval, there is optimism within the community that SKYCLARYS has the potential to usher in a new era in the management of Friedreich's ataxia."

Friedreich's ataxia is the most common inherited ataxia. [2,3] Early symptoms typically appear in childhood and include progressive loss of coordination, muscle weakness, and fatigue.[4] As the disease progresses, people living with FA may also experience vision impairment, hearing loss, problems with speech and swallowing, diabetes, scoliosis, and serious heart conditions.[5] Many people with FA use walking aids, and often require a wheelchair within 10-20 years following their diagnosis.[2] Unfortunately, complications from FA contribute to a life expectancy of 37 years on average.[5-7]

"Biogen is proud to add SKYCLARYS to our portfolio of medicines and address a significant unmet need by bringing the first treatment to people living with Friedreich's ataxia in Europe," said Priya Singhal, M.D., M.P.H., Head of Development at Biogen. "Our team is committed to engaging with the medical community and local authorities as we work to urgently secure access for patients. We sincerely thank the Friedreich's ataxia community for their contributions that enabled the development of SKYCLARYS and made today's approval possible."

The EC approval of SKYCLARYS is based on efficacy and safety data from the placebo-controlled MOXIe Part 2 trial. At the end of the 48-week study, patients who received SKYCLARYS had significantly improved modified Friedreich Ataxia Rating Scale (mFARS) scores relative to placebo. All components of the mFARS assessment, including ability to swallow (bulbar), upper limb coordination, lower limb coordination, and upright stability, favored SKYCLARYS over placebo. Additional exploratory data was provided from a post hoc, propensity-matched analysis in which patients treated with SKYCLARYS in MOXIe (Extension) had lower mFARS scores 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 European Commission approval of SKYCLARYS is a significant milestone toward expanding global access, bringing the first approved treatment to the Friedreich's ataxia community in the EU," said Jennifer Farmer, Chief Executive Officer of the Friedreich's Ataxia Research Alliance (FARA). "FARA is grateful to all the researchers, clinical sites, individuals with Friedreich's ataxia and their families, patient organizations, Biogen and the European Medicines Agency for the research, drug development and advocacy efforts that led to this approval. We look forward to continuing our collaboration with the Friedreich's ataxia community with the goal of expanding access where it is needed."

"On behalf of Euro-ataxia and its members, I am delighted to welcome the European Commission approval of SKYCLARYS, a much-needed medicine for adults with Friedreich's ataxia in EU countries," said Andreas Nadke, President of Euro-ataxia. "Our member's patient groups have been working and waiting for this day for many years, and we firmly believe that this will be a successful and gratifying beginning in the treatment of Friedreich's ataxia."

For detailed product information, please see the Summary of Product Characteristics on the European Medicines Agency website at www.ema.europa.eu. Biogen is committed to working closely with all stakeholders to ensure that eligible European patients can have access to this treatment. Early access programs for SKYCLARYS are currently open in Germany and France, with plans to expand to additional countries where possible. SKYCLARYS is also approved for use in the United States, and Biogen is engaging with regulatory authorities in other regions.

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. 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.

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 biotechnology company that pioneers innovative science to deliver new medicines to transform patient's 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 about 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.

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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