



New Data Presented at AD/PD™ 2023 Show Biogen's BIIB080 (MAPT ASO) Substantially Reduced Tau Protein Levels in Patients with Early-stage Alzheimer's Disease

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- The BIIB080 Phase 1b clinical study is the first to show a reduction of this magnitude in tau PET across brain regions
- Phase 1b Study of BIIB080 showed dose-dependent and sustained reduction of tau protein (CSF t-tau and p-tau181) in the cerebral spinal fluid (CSF) throughout the open-label long-term extension (LTE)
- Neurofibrillary tangles in the brain are composed of tau proteins and are a key pathology associated with Alzheimer's disease¹

CAMBRIDGE, Mass., March 29, 2023 (GLOBE NEWSWIRE) -- [Biogen](#) Inc. (Nasdaq: BIIB) reported new promising Phase 1b clinical data showing that the investigational antisense oligonucleotide (ASO) therapy, BIIB080, reduced soluble tau protein in cerebrospinal fluid (CSF) in a dose-dependent and sustained manner in patients with early-stage Alzheimer's disease (AD). BIIB080 also reduced aggregated tau pathology, as measured by positron emission tomography (PET) in all brain composites assessed. The primary endpoint of the Phase 1b trial (week 25) and open-label long-term extension study (through week 100) was safety and tolerability, with biomarker data as an exploratory endpoint. The results were presented at the International Conference on Alzheimer's and Parkinson's Diseases (AD/PD™ 2023) held in Gothenburg, Sweden from March 28 – April 1.

"The BIIB080 Phase 1b clinical study is the first to demonstrate this magnitude of a reduction of tau PET across brain regions," said Priya Singhal, M.D., M.P.H., Executive Vice President, Head of Development at Biogen. "Given the complexity and urgent unmet need in Alzheimer's disease, Biogen continues to evaluate multiple modalities and targets including tau, which is believed to play a critical role in cognitive decline."

In patients with Alzheimer's disease, tau protein can form "tangles" which progressively accumulate in brain regions involved in cognition. ² The accumulation of pathological tau tangles has been shown to promote brain cell damage and death. BIIB080 is designed to target microtubule-associated protein tau (MAPT) mRNA and prevent production of tau protein.

The Phase 1b trial and its open-label long-term extension study was designed to evaluate the safety and tolerability of multiple dose levels of BIIB080 in patients with mild AD (n=46). In this study, the majority of adverse events were mild or moderate in severity, of which the most common were headache, back pain, and post-lumbar puncture syndrome (PLPS). The results showed that BIIB080 reduced biomarkers of soluble tau in CSF (t-tau and p-tau181) in a dose-dependent and sustained manner, with all dose groups showing approximately a 60% reduction from baseline CSF tau levels by the end of the long-term extension (LTE). BIIB080 impacted aggregated tau pathology as measured by PET as early as week 25 and up to the end of the LTE at week 100, including in patients who began on placebo and received BIIB080 treatment starting at week 25 in the long-term extension. By the end of the LTE, BIIB080 reduced tau pathology in all dose groups across all brain composites assessed.

The Phase 2 CELIA study of BIIB080 (NCT05399888) is in progress and currently recruiting participants in the United States.

In December 2019, Biogen exercised a license option with Ionis and obtained a worldwide, exclusive, royalty-bearing license to develop and commercialize BIIB080 (tau ASO).

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, and two co-developed treatments to address a defining pathology of Alzheimer's disease. 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 - [Twitter](#), [LinkedIn](#), [Facebook](#), [YouTube](#).

Biogen Safe Harbor

This news release contains forward-looking statements, about the potential clinical effects of BIIB080; the potential benefits, safety and efficacy of BIIB080; potential regulatory discussions, submissions and approvals and the timing thereof; the treatment of Alzheimer's disease; the potential of Biogen's commercial business and pipeline programs, including BIIB080; 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 lecanemab; actual timing and content of submissions to and decisions made by the regulatory authorities regarding lecanemab; uncertainty of success in the development and potential commercialization of lecanemab; 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.

MEDIA CONTACT:

Biogen
Jack Cox
+ 1 210 544 7920
public.affairs@biogen.com

INVESTOR CONTACT:

Biogen
Mike Hencke
+1 781 464 2442
IR@biogen.com

¹ BrightFocus. Foundation. Tau Protein and Alzheimer's Disease: What's the Connection? <https://www.brightfocus.org/alzheimers/article/tau-protein-and-alzheimers-disease-whats-connection>. Accessed March 2023.

² Alzheimer's Association. Tau Topic Sheet. <https://www.alz.org/media/Documents/alzheimers-dementia-tau-ts.pdf>. Accessed February 2023.