Topline Results from Phase 2 CELIA Study of Diranersen (BIIB080): First Study to Show Reduction in Tau Pathology and Cognitive Benefit in Patients with Early Alzheimer's Disease

May 14, 2026

CELIA did not meet its primary endpoint assessing dose response; based on the strength of the biomarker and efficacy data, Biogen plans to advance diranersen to registrational development

- Robust reductions in tau pathology were observed across all studied doses, with results generally consistent with those observed in the Phase 1b study[1]
- Pre-specified analyses of cognitive endpoints demonstrated slowing of clinical decline across all studied doses, particularly at the lowest dose
- The safety and tolerability profile of diranersen was generally consistent with the Phase 1b study[1]
- CELIA is an 18-month Phase 2 randomized, placebo-controlled, dose-ranging study evaluating diranersen, a tau-targeting antisense oligonucleotide (ASO)
- Data will be presented at the Alzheimer's Association International Conference (AAIC) 2026 and other upcoming scientific congresses

CAMBRIDGE, Mass., May 14, 2026 (GLOBE NEWSWIRE) -- Biogen Inc. (Nasdaq: BIIB) today announced compelling topline results from the Phase 2 CELIA study evaluating diranersen (BIIB080), an investigational antisense oligonucleotide (ASO) therapy targeting tau, in individuals with early Alzheimer's disease. The CELIA results provide the first evidence from a randomized Phase 2 study of a tau-directed therapy demonstrating both robust biomarker impact and cognitive benefit in early Alzheimer's disease.

"In CELIA, we believe we have seen an unprecedented and compelling confluence of efficacy and biomarkers results from a tau-directed agent in a randomized early Alzheimer's disease study," said Priya Singhal, M.D., M.P.H., Executive Vice President and Head of Development at Biogen. "We are excited by these Phase 2 data, which give us the confidence to advance diranersen to registrational development. We look forward to engaging with regulators and the broader Alzheimer's disease community on next steps. I would like to thank the patients, families, investigators, and study teams who participated in this pioneering study."

Pre-specified analyses of cognitive endpoints demonstrated slowing of clinical decline across all studied doses, particularly in participants receiving the lowest dose of diranersen, 60 mg administered every 24 weeks. Diranersen also demonstrated robust reductions in both cerebrospinal fluid (CSF) tau and tau pathology, as measured by positron emission tomography (PET), across all studied doses, with reductions maintained throughout the dosing period. CELIA did not meet its primary endpoint assessing dose response for change from baseline on the Clinical Dementia Rating–Sum of Boxes (CDR-SB) at Week 76.

"The CELIA topline results represent an important advance for the field, providing the first evidence that reducing tau, a hallmark of Alzheimer's disease closely associated with neurodegeneration and cognitive decline, may meaningfully impact disease progression," said Dr. Jeff Cummings, Professor of Brain Sciences at the University of Nevada, Las Vegas. "I am encouraged by these promising data, which represent meaningful progress toward advancing a new mechanism of action and shaping the next generation of Alzheimer's disease treatments."

The safety and tolerability profile of diranersen across all studied doses was generally consistent with the Phase 1b study and the known profile of diranersen to date.[1] The incidence of adverse events (AEs) was comparable across dose groups, with a higher incidence of serious adverse events (SAEs) observed at the highest dose studied.

CELIA is a pioneering study evaluating diranersen, a first-in-class investigational ASO designed to reduce the production of tau protein at its source in early Alzheimer's disease. While tau plays an important role in the normal function of brain cells, in Alzheimer's disease abnormal tau can accumulate and form intracellular tangles that contribute to neurodegeneration and cognitive decline.[2] Unlike many investigational approaches that have focused on targeting extracellular tau, diranersen is designed to reduce both extracellular and intracellular tau.

About diranersen (BIIB080)
Diranersen (BIIB080) is an investigational antisense oligonucleotide (ASO) therapy designed to target microtubule-associated protein tau (MAPT) mRNA to reduce the production of tau protein. Abnormal accumulation of tau in the brain is a hallmark of Alzheimer's disease associated with neurodegeneration and cognitive decline.[2]

Diranersen is being investigated as a potential treatment for early Alzheimer's disease. In 2025, the U.S. Food and Drug Administration (FDA) granted Fast Track designation to diranersen for the treatment of Alzheimer's disease.

In December 2019, Biogen exercised a license option with Ionis Pharmaceuticals and obtained a worldwide, exclusive, royalty-bearing license to develop and commercialize diranersen. Diranersen was discovered by Ionis.

About the CELIA Study
CELIA is a global Phase 2 randomized, double-blind, placebo-controlled, dose-ranging study evaluating the efficacy, safety and tolerability of diranersen in individuals with early Alzheimer's disease. The study enrolled 416 participants with mild cognitive impairment due to Alzheimer's disease or mild Alzheimer's disease dementia. All participants enrolled in CELIA had not previously received anti-amyloid therapy.

The study evaluated three doses of diranersen administered intrathecally over a 76-week placebo-controlled treatment period: 60 mg every 24 weeks, 115 mg every 24 weeks, and 115 mg every 12 weeks.

The primary endpoint of CELIA was assessment of dose response for change from baseline on the Clinical Dementia Rating–Sum of Boxes (CDR-SB) at Week 76. Secondary and exploratory endpoints included additional clinical, biomarker and imaging measures, including cerebrospinal fluid tau biomarkers and tau positron emission tomography (PET). Additional information on the CELIA study design is available in the ClinicalTrials.gov listing for the CELIA study.

An ongoing long-term extension (LTE) study is continuing to evaluate the long-term safety, tolerability and durability of diranersen in early Alzheimer's disease.

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, relating to, among others, the potential, benefits, safety and efficacy of diranersen; the potential clinical effects of diranersen; the clinical development program, clinical trials, data readouts and presentations related to diranersen; the treatment of Alzheimer's disease and the relation between tau protein and Alzheimer's disease; the potential of Biogen's commercial business and pipeline programs, including diranersen; and risks and uncertainties associated with drug development and commercialization. These forward-looking statements may be accompanied by such words as "aim," "anticipate," "assume," "believe," "contemplate," "continue," "could," "estimate," "expect," "forecast," "goal," "guidance," "hope," "intend," "may," "objective," "outlook," "plan," "possible," "potential," "predict," "project," "prospect," "should," "target," "will," "would" or the negative of these words or 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 trials may not be indicative of full results or results from later stage or larger scale clinical trials 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 differ materially from those stated or implied in this document, including, among others, uncertainty of success in the development and potential commercialization of diranersen; unexpected concerns may arise from additional data, analysis or results obtained during the CELIA Study; uncertainty of our long-term success in developing, licensing, or acquiring other product candidates or additional indications for existing products; expectations, plans, prospects and timing of actions 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; 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 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; 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; and the occurrence of adverse safety events, restrictions on use with our products, or product liability claims; and any other risks and uncertainties that are described in other reports we have filed with the U.S. Securities and Exchange Commission, which are available on the SEC's website at www.sec.gov.

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, 2025 and in our subsequent reports on Form 10-Q. 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.

Digital Media Disclosure
From time to time, we have used, or expect in the future to use, our investor relations website (investors.biogen.com), the Biogen LinkedIn account (linkedin.com/company/biogen-) and the Biogen X account (https://x.com/biogen) as a means of disclosing information to the public in a broad, non-exclusionary manner, including for purposes of the SEC's Regulation Fair Disclosure (Reg FD). Accordingly, investors should monitor our investor relations website and these social media channels in addition to our press releases, SEC filings, public conference calls and websites, as the information posted on them could be material to investors.

References:

1. Shulman M, Wu S, Ziogas N, et al. Exploratory analyses of clinical outcomes from the BIIB080 phase 1b study in mild Alzheimer's disease. *Nature Aging*. 2026;6:445-453. https://doi.org/10.1038/s43587-025-01031-9. Accessed May 2026.
2. Alzheimer's Association. Tau. 2021. https://www.alz.org/getmedia/c612ad59-8a8d-495d-982a-14b046a273d0/alzheimers-dementia-tau-ts.pdf. Accessed May 2026.

MEDIA CONTACT:
Madeleine Shin
+ 1 781 464 3260
public.affairs@biogen.com

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