JAMA Neurology Publishes Data on Biogen’s Investigational Antisense Oligonucleotide Targeting Tau

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JAMA Neurology published biomarker data from the placebo-controlled period (PCP) and long-term extension phase of the BIIB080 Phase 1b study of the antisense oligonucleotide (ASO) which targets tau pre-mRNA in early-stage Alzheimer's disease (AD). The publication follows the presentation of late-breaking study results at the 2023 Clinical Trials on Alzheimer's Disease (CTAD) meeting which included data from multiple exploratory endpoints of cognition and activities of daily living.

This publication includes preliminary data in 46 patients which showedthat the investigational therapy substantially reduced soluble and aggregated pathologic tau in patients with mild AD. This was the first study to show a reduction of this magnitude with a tau-targeting therapy in tau PET across brain regions. Tau tangles in the brain are a key hallmark of AD pathology and their accumulation has been shown to lead to neuronal loss.
The randomized, double-blind, placebo-controlled study assessed the safety, tolerability, and efficacy of the ASO targeting tau. Primary safety outcomes from the PCP were previously published in Nature Medicine. Efficacy results presented at CTAD were based on exploratory endpoints.

Recruitment for the Phase 2 CELIA study (NCT05399888), which is evaluating the potential for an ASO targeting tau to slow the worsening of mild cognitive impairment due to AD or mild AD dementia, is ongoing in the North America, Europe and Asia Pacific.
In December 2019, Biogen exercised a license option with Ionis Pharmaceuticals, Inc.to obtain a worldwide, exclusive, royalty-bearing license to develop and commercialize BIIB080.