Biogen resurrects Alzheimer's program

Biogen and Eisai have reversed course and will seek US Food and Drug Administration (FDA) approval for aducanumab, an Alzheimer's disease drug they seemed ready to discard in March following a futility analysis that led the companies to discontinue two phase 3 studies. The latest decision, announced October 22, came after a new analysis of their EMERGE study in patients with mild cognitive impairment and dementia due to Alzheimer's disease was corroborated by data from a subset of patients in their ENGAGE study receiving a different dosing regimen.

In an investor presentation, Biogen said the earlier futility analysis was "incorrect" because it was based on a smaller dataset of patients as it included only those who, by 26 December, had completed 18 months of treatment. When the companies analyzed a larger dataset gathered after the aducanumab studies were halted, they observed "dose-dependent effects in reducing brain amyloid and in reducing clinical decline," measuring cognition and function using the Clinical Dementia Rating–Sum of Boxes (CDR-SB) score as the primary endpoint. The company concluded that the different outcomes could be explained by a subset of patients who had been exposed to sufficient high-dose aducanumab and had met the primary endpoint.

Biogen had meetings with Food and Drug Administration this June and October to discuss the new analysis and, on the basis of those discussions, decided to submit a Biologics License Application in early 2020.

If approved, aducanumab would be the first successful therapy targeting β-amyloid for removal in Alzheimer's disease. In recent years, a number of high-profile failures for therapies targeting β-amyloid and related pathways—including BACE inhibitors, designed to prevent the BACE1 enzyme from cutting up the amyloid precursor protein that forms β-amyloid plaques—have cast doubt on the amyloid hypothesis and a pall over drug development in neurodegeneration. Earlier this year, Biogen and Eisai also announced they would discontinue a phase 3 trial for their BACE inhibitor elenbecestat, following partners Amgen and Novartis with their BACE1 inhibitor umibecestat. In October, Amgen announced it would shrink its neurology R&D footprint.

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Patients with porphyria bask in sunlight of FDA approval

The US regulator's go-ahead is a boon for people with a rare form of porphyria that results in painful photosensitivity.

The US Food and Drug Administration (FDA) has approved Scenesse (afamelanotide), a drug that stimulates melanin production, to protect people with erythropoietic protoporphyria (EPP) from the damage and inflammation they experience when their skin is exposed to bright light. Scenesse, which is manufactured by Clinuvel Pharmaceuticals, a Melbourne, Australia-based biotech, can now be used in the United States for treating EPP. Meanwhile, at the 2019 International Congress on Porphyrins and Porphyrias in September, Alnylam presented follow-up data on an small interfering RNA (siRNA) drug to treat a hepatic form of

Children with erythropoietic protoporphyria have sunlight sensitivity that limits the time they can spend outdoors. Credit: PhotoAlto sas / Alamy Stock Photo