Phase 2 APPlauDS: Study Design

A Multiple-Dose Study Evaluating Efficacy, Safety, Tolerability, and PD of Mivelsiran in Patients with DS-AD (NCT07636811)[1]

Study Population

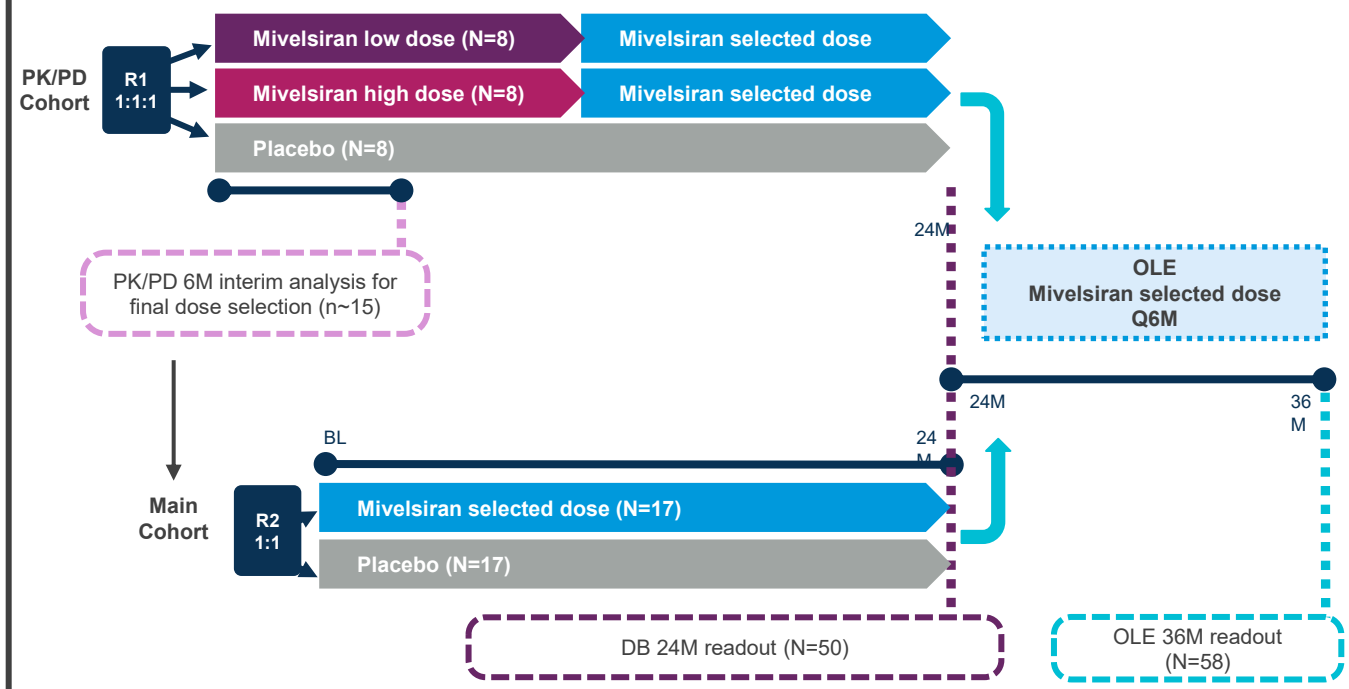
Key Inclusion Criteria

- Age 40 to 55 years
- Clinical DS diagnosis associated with trisomy 21
- Positive amyloid PET scan
- Cognitively stable
- Mild or moderate ID

Key Exclusion Criteria

- MCI or dementia
- Prior ongoing treatment with anti-amyloid antibodies

Other protocol-defined inclusion and exclusion criteria apply



Primary Endpoint

- Change from baseline in brain amyloid burden

Key Secondary Endpoints

- CSF sAPPα and sAPPβ
- CSF Aβ42
- Plasma p-tau217
- mCRT

Safety Endpoint

- Frequency of AEs

AD, Alzheimer's disease; AE, adverse event; APP, amyloid-beta precursor protein; CSF, cerebrospinal fluid; DB, double-blind; DS, Down-Syndrome; DS-AD, Down Syndrome-Associated Alzheimer's Disease; ICH, intracerebral hemorrhage; ID, intellectual disability; MCI, mild cognitive impairment; mCRT, modified Cued Recall Test; OLE, open-label extension; PD, pharmacodynamics; PET, positron emission tomography; PK, pharmacokinetics; R, randomization; ULN, upper limit of normal. 1. ClinicalTrials.gov. 2026. https://clinicaltrials.gov/study/NCT07636811 (Accessed July 30, 2026). 2. Rafii et al. Alzheimer's Association International Conference 2026, July 12-15, 2026, London, UK. Poster.

Alnylam