Phase 2 cAPPricorn-1: Study Design

A Multiple-Dose Study Evaluating Efficacy, Safety, and PD of Mivelsiran in Patients with CAA (NCT06393712)[1,2]

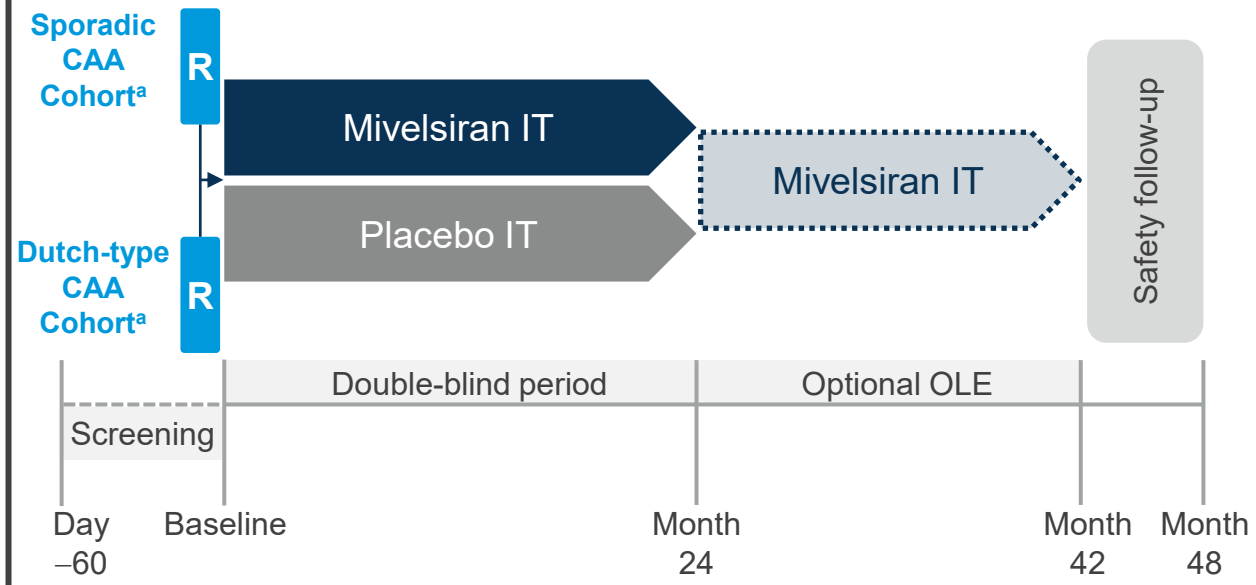
Study Population (N~200)

Sporadic CAA Cohort
- Age ≥50 years
- Probable CAA (Boston v2.0 with adaptations)

Dutch-type CAA Cohort
- Age ≥30 years
- Documented E693Q *APP* variant

Key Exclusion Criteria (Both Cohorts)
- Moderate or severe stage AD or severe cognitive impairment
- Previous ICH with onset <90 days before randomization

Other protocol-defined inclusion and exclusion criteria apply



Primary Endpoint
- Annualized rate of new lobar cerebral microbleeds

Key Secondary Endpoints
- Global rank endpoint
- Hemorrhagic disease progression
- Vascular reactivity
- Nonhemorrhagic disease progression
- PD: CSF sAPPα and sAPPβ

Safety Endpoint
- Frequency of AEs

[a]Sporadic and Dutch-type CAA cohorts will be analyzed separately. AD, Alzheimer's disease; AE, adverse event; APP, amyloid-beta precursor protein; CAA, cerebral amyloid angiopathy; CSF, cerebrospinal fluid; ICH, intracerebral hemorrhage; IT, intrathecal; OLE, open-label extension; PD, pharmacodynamics; R, randomization; sAPP, soluble APP. 1. ClinicalTrials.gov. 2024. https://clinicaltrials.gov/study/NCT06393712 (Accessed February 17, 2026). 2. Lee JM et al. Clinical Trials on Alzheimer's Disease (CTAD) Conference, October 29–November 1, 2024. Madrid, Spain. Oral presentation.

Alnylam