

Arctic Therapeutics Receives EMA Approval for Phase IIa Study of AT-001 in Alzheimer's Disease

- *The European Medicines Agency (EMA) authorises initiation of a Phase IIa study evaluating AT-001 in patients diagnosed with Alzheimer's disease*
- *EMA grants Orphan Drug Designation (ODD) to AT-001 for hereditary cystatin C amyloid angiopathy (HCCAA), a rare familial dementia*

REYKJAVIK, ICELAND – November 3, 2025 – Arctic Therapeutics (ATx), an Iceland-based clinical-stage biopharmaceutical company, today announced key regulatory milestones for its lead investigational therapy, AT-001, which is in development for both rare and common forms of dementia.

ATx has initiated a Phase IIa clinical trial to evaluate the safety and biomarker-based efficacy of AT-001 in individuals with mild cognitive impairment (MCI) or mild dementia due to Alzheimer's disease (AD). The multicentre, randomized, double-blind, placebo-controlled, dose-escalation study will assess the safety profile of AT-001, as well as its impact on novel blood-based biomarkers and brain amyloid deposition.

*"Alzheimer's disease is one of the world's most urgent healthcare challenges. Advances in **early diagnosis**, combined with treatments that enable **earlier intervention**, hold the **potential to transform** how we approach Alzheimer's, as well as other forms of dementia,"* ATx founder Dr. Hakon Hakonarson said. *"Through our clinical trials targeting both rare and common forms of dementia, we are pushing the science forward with the goal of not only delaying progression, but **ultimately preventing** these diseases altogether,"* Dr. Hakonarson added.

The study authorisation in AD follows the EMA's authorisation last year for ATx to initiate a Phase IIb/III study of AT-001 in HCCAA, an ultra-rare condition marked by amyloid buildup in the brain's blood vessels that can cause cerebral haemorrhage, stroke and progressive neurological decline, including dementia. In addition, the EMA has granted AT-001 ODD for the treatment of HCCAA, a form of hereditary cerebral amyloid angiopathy (CAA).

*"The ODD status **confirms the ground-breaking nature** of AT-001 as a potentially **disease-modifying treatment** for HCCAA and **underpins our expansion** into other forms of dementia, including Alzheimer's,"* CEO and co-founder Ivar Hakonarson said. *"This is a significant milestone and underscores the urgent unmet medical need in HCCAA and provides ATx with an important regulatory milestone to advance AT-001 in this rare and devastating condition."*

About the Phase IIa Study

The study will evaluate the safety, tolerability, and biomarker-based efficacy of AT-001, a small molecule oral therapy, in patients aged 50–85 with mild cognitive impairment (MCI) or mild Alzheimer's disease. The study will be a multicentre, randomized, double-blind, placebo-controlled trial conducted at four sites across Denmark and Iceland in collaboration with Sanos Group, a global multi-niche CRO.

The trial will enrol patients for 12 months of treatment with escalating oral doses of AT-001. Safety labs will be monitored monthly, together with biomarker assessments every three months, MRI at baseline, mid- and end of study, and PET scans at baseline and study completion.

Endpoints and Biomarkers

The study's primary focus is on safety and biomarker efficacy. Selected biomarkers include:

- **Plasma pTau217 and total Tau** — measured with Quanterix Simoa® technology to track early tau pathology and neuronal damage.
- **Neurofilament Light Chain (NfL)** — a marker of axonal injury and neurodegeneration.
- **Toxic amyloid-β oligomers** — measured with the novel SOBA assay to detect synaptotoxic species driving memory loss.

Imaging

- **MRI** — to evaluate brain atrophy and white matter integrity.
- **PET scans** — to visualize amyloid plaque burden and assess potential reductions with AT-001.

Upon completion of the trial the biomarker/brain amyloid results will be analysed in the context of clinical improvement observed with recent monoclonal antibody-based amyloid reduction therapies.

About Arctic Therapeutics

Arctic Therapeutics is an Icelandic drug discovery and development company established in 2015 as a spin-off from the US-based Center for Applied Genomics (CAG), a research centre at the Children's Hospital of Philadelphia. ATx leverages the transformative power of applied genomics, steering the course of drug development towards safer, more effective treatments for some of the world's most challenging diseases. The company has operations in Iceland, the US and multiple collaborations across Europe. For more information, please visit: www.arctictherapeutics.com and follow us on [LinkedIn](#).

For media inquires

Gulli Arnason, Chief Strategy Officer

gulli@arctictherapeutics.com

+354 660 0053

###