Azafaros Completes Enrollment of Phase 3 (NAVIGATE) Study Evaluating Nizubaglustat for the Treatment of Patients with GM1/GM2 Gangliosidoses

- **NAVIGATE comprises two pivotal Phase 3 studies investigating nizubaglustat**
- **Recruitment completed in Phase 3 registrational study in patients with late-infantile and juvenile GM1/GM2 gangliosidoses**
- **Enrolment in the Niemann-Pick type C disease (NPC) Phase 3 study is ongoing**
- **Topline data from the GM1/GM2 gangliosidoses Phase 3 is anticipated in early 2028**

Leiden, Netherlands, July 23, 2026 – Azafaros, a private company building a portfolio of disease-modifying therapeutics to become a leader in lysosomal storage disorders (LSDs) focused on addressing neurological symptoms, today announced the completion of patient recruitment in its Phase 3 study evaluating nizubaglustat for the treatment of GM1/GM2 gangliosidoses.

“Completing enrollment in our Phase 3 GM1/GM2 study is a significant step toward potentially bringing nizubaglustat to patients and families living with these devastating neurodegenerative diseases," **said Stefano Portolano, Chief Executive Officer at Azafaros**. "We are deeply grateful to the patients, caregivers, investigators and advocacy groups who have made this milestone possible. As we advance the GM1/GM2 study toward data readout and regulatory submission, we continue to enroll in our Phase 3 NPC study and remain focused on our goal of addressing the significant unmet medical needs faced by these rare disease communities."

Professor Roberto Giugliani, Principal Investigator, said: "With enrollment now complete, we look forward to generating the clinical evidence needed to evaluate the potential of nizubaglustat for patients living with GM1/GM2 gangliosidoses. These are devastating neurodegenerative disorders with no treatments approved for use in these patients today. Robust Phase 3 data from a long-term randomized study are essential to advancing potential the first treatment option for patients and their families, and we hope that the data from this study are supportive of approval."

Azafaros’ NAVIGATE program consists of two global Phase 3 multi-centre, randomized, placebo-controlled studies designed to evaluate the potential of oral nizubaglustat to alter disease progression and improve functional outcomes in patients with late-infantile and juvenile-onset forms of NPC or GM1/GM2 gangliosidoses. The Phase 3 GM1/GM2 study enrolled a minimum of 75 patients across 25 clinical sites in 13 countries. The study is evaluating oral administration of nizubaglustat compared to treatment with placebo over an 18-month treatment period, with efficacy assessments focused on neurological manifestations and disease progression. All patients treated in the study are eligible to continue nizubaglustat post the 18-month treatment period in an open label extension study. Enrolment in the NAVIGATE Phase 3 study in patients with NPC, recruiting 72 patients, is ongoing.

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About Nizubaglustat

Nizubaglustat is a small molecule, orally available and brain penetrant azasugar with a unique dual mode of action, developed as a potential treatment for rare lysosomal storage disorders with

neurological involvement, including GM1 and GM2 gangliosidoses and Niemann-Pick type C disease (NPC).

Nizubaglustat has received Rare Pediatric Disease Designations (RPDD) for the treatment of GM1 and GM2 gangliosidoses and NPC, Orphan Drug Designations (ODD) for GM1 and GM2 gangliosidosis (Sandhoff and Tay-Sachs Diseases) and NPC, as well as Fast Track Designation and IND clearance for GM1/GM2 gangliosidoses and NPC from the US Food and Drug Administration (FDA). Additionally, nizubaglustat has been awarded Orphan Medicinal Product Designation (OMPD) for the treatment of GM1 and GM2 gangliosidoses by the European Medicines Agency (EMA) and Innovation Passport for the treatment of GM1 and GM2 gangliosidoses from the UK Medicines and Healthcare Products Regulatory Agency (MHRA).

About GM1 and GM2 gangliosidoses

GM1 gangliosidosis and GM2 gangliosidosis (Tay-Sachs and Sandhoff diseases) are lysosomal storage disorders caused by the accumulation of GM1 or GM2 gangliosides respectively, in the central nervous system (CNS). This results in progressive and severe neurological impairment and premature death. These diseases mostly affect infants and children, and no disease-modifying treatments are currently available.

About Niemann-Pick type C disease (NPC)

Niemann-Pick type C disease is a progressive, life-limiting, neurological, lysosomal storage disorder, caused by mutations in the NPC1 or NPC2 gene and aberrant endosomal-lysosomal trafficking, leading to the accumulation of various lipids, including gangliosides in the CNS. The onset of the disease can happen throughout the lifespan of an affected individual, from prenatal life through adulthood.

About Nizubaglustat NAVIGATE Phase 3 Program

The Azafaros NAVIGATE Phase 3 program comprises two global studies evaluating oral nizubaglustat in patients with late-infantile and juvenile-onset forms of NPC or GM1/GM2 gangliosidoses. Both studies are randomized, double-blind, placebo-controlled, multicenter trials designed to assess the efficacy, safety and tolerability of nizubaglustat over an 18-month treatment period. The studies are being conducted across major regions including North America, Europe, Latin America, India, Pakistan and other selected countries.

About Azafaros

Azafaros is a clinical-stage company founded in 2018 with a deep understanding of rare genetic disease mechanisms using compound discoveries made by scientists at Leiden University and Amsterdam UMC and is led by a team of highly experienced industry experts. Azafaros aims to build a pipeline of disease-modifying therapeutics to offer new treatment options to patients and their families. By applying its knowledge, network and courage, the Azafaros team challenges traditional development pathways to rapidly bring new drugs to the rare disease patients who need them. Azafaros is supported by leading healthcare investors including Forbion, Jeito Capital, Seroba, Pictet Group, BioGeneration Ventures (BGV), BioMedPartners, Asahi Kasei Pharma Ventures, and Schroders Capital.

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