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Azafaros Announces Initiation of two Global Phase 3 studies with Nizubaglustat in Niemann-Pick disease Type C (NPC) and GM1/GM2 gangliosidoses, respectively

- *First patient dosed in global pivotal clinical trial program for late infantile/juvenile onset NPC and GM1/GM2 gangliosidoses.*
- *Both studies are part of Azafaros' mission to develop treatments for the unmet need of patients with rare lysosomal storage disorders*
- *The news follows the company's recent successful €132M Series B financing, aimed at supporting the rapid development of nizubaglustat and the expansion of Azafaros' pipeline to other indications*

LEIDEN, Netherlands - (BUSINESS WIRE) - **Azafaros, a company focused on developing treatments for the unmet needs of patients with rare lysosomal storage disorders, today announced that the first patient has been dosed in the company's pivotal, multicenter Phase 3 clinical program to evaluate the safety and efficacy of the company's lead asset, nizubaglustat, in patients with Niemann-Pick disease Type C (NPC) and GM1/GM2 gangliosidoses.**

The initiation of the two Phase 3 studies **(NCT07054515)** represents a major milestone in Azafaros' commitment to addressing the urgent unmet medical needs of children affected by these devastating neurodegenerative disorders.

The Phase 3 program consists of two studies targeting the late-infantile and juvenile-onset forms of NPC, and GM1/GM2 gangliosidoses. The studies aim to assess the potential of nizubaglustat to alter disease progression and improve functional outcomes in these patient populations.

Today's news follows the recent, successful completion of an oversubscribed series B financing, raising €132M to support the acceleration of nizubaglustat and the expansion of the company's pipeline to other indications.

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"The dosing of the first patient in our Phase 3 program with nizubaglustat is a significant achievement for Azafaros and a huge step forward in our efforts to bring new, disease modifying treatments to patients with these seriously debilitating diseases," **said Stefano Portolano, Chief Executive Officer at Azafaros.** "We are deeply grateful to the patients, families, clinicians, and advocacy groups who are partnering with us to advance this promising therapy."

About the NAVIGATE trial

The two 18-month randomized 2 to 1, double-blind, placebo-controlled trials will recruit patients at approximately 35 sites across 15 countries worldwide, including in the US, Europe and Latin America. The studies are expected to enroll around 70 patients. The primary endpoint for both trials is the change from baseline to Month 18 in the Scale for the Assessment and Rating of Ataxia (SARA), with both total and functional SARA scores evaluated.

For more information on the Phase 3 program, please visit www.azafaros.com. To enquire about trial participation, email: medinfo@azafaros.com (if a professional) or patientadvocacy@azafaros.com (if a patient or caregiver). To protect privacy, avoid including identifying information in the initial message.

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 disease type C (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 disease Type C (NPC)

Niemann-Pick disease Type C 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 disease can happen throughout the lifespan of an affected individual, from prenatal life through adulthood.

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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