Azafaros to Present at the Piper Sandler Healthcare Conference 2026

Leiden, Netherlands, [25 November 2026] – Azafaros, a private company building a portfolio to become a leader in lysosomal storage disorders (LSDs), focused on addressing neurological symptoms, today announced that Stefano Portolano, M.D, Azafaros' Chief Executive Officer, will present at the 38th Annual Piper Sandler Healthcare Conference, taking place on December 1–3, 2026, at the Lotte New York Palace in New York City, USA.

The presentation will take place on **Tuesday, December 1, 2026, at 10:10 a.m Eastern Time (EST)** and will provide an overview of Azafaros' progress and development strategy, including its Navigate Phase 3 clinical program evaluating nizubaglustat for the treatment of Niemann-Pick type C disease (NPC) and GM1/GM2 gangliosidoses.

Dr. Portolano and Chief Financial Officer, Amy Sullivan, will also be available for one-on-one meetings with institutional investors during the conference.

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 nizubaglustat 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 and other selected countries. Topline data from both Phase 3 studies are anticipated in 2028.

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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