Azafaros Secures € 132M in Oversubscribed Series B Financing to Advance Phase 3 Clinical programs of innovative therapies in lysosomal storage disorders

- **Financing round led by Jeito Capital and co-led by Forbion Growth, with participation from Seroba, Pictet Group and other existing investors**
- **Proceeds to fund two Phase 3 pivotal programs with nizubaglustat, lead asset in Niemann-Pick disease Type C (NPC) and GM1/GM2 gangliosidoses as well as expanding the Azafaros pipeline to other indications**
- **Nizubaglustat has been awarded Orphan Drug Designation in both the US and Europe as well as Fast-track status in the US. The company expects to initiate both Phase 3 studies later this year**

Leiden, Netherlands, 13 May 2025 – Azafaros, a clinical-stage company focused on developing disease-modifying therapeutics to offer new treatment options to patients with rare lysosomal storage disorders, announces the completion of an oversubscribed €132M Series B financing led by Jeito Capital, co-led by Forbion Growth and with additional participation from Seroba, Pictet Group and existing investors Forbion Ventures, Schroders Capital and BioGeneration Ventures (BGV).

This financing enables Azafaros to accelerate the development of its lead product nizubaglustat, scheduled to enter Phase 3 studies for Niemann-Pick disease Type C (NPC) and GM1/GM2 gangliosidoses later this year as well as expanding the Azafaros pipeline to other indications.

Rachel Mears, Partner at Jeito Capital; Julien Elric, Senior Principal at Jeito Capital; and Audrey Cacaly, Principal at Forbion Growth will also join Azafaros's Board of Directors as Board members.

Founded in 2018 by BGV, leveraging science from Leiden University and Amsterdam UMC, Azafaros is led by a team of highly experienced industry experts in rare disease drug development and commercialization. The company is developing a first-in-class dual-acting drug candidate to offer new treatment options to patients with lysosomal storage disorders, a group of severe rare genetic diseases that often cause progressive neurodegeneration and, in many cases, fatal outcomes.

"*This successful Series B round marks a significant milestone for Azafaros, allowing us to accelerate the development of nizubaglustat and leverage our scientific understanding and competencies to bring additional candidates into development*," **said Stefano Portolano, Chief Executive Officer at Azafaros.** "*The fact that we have been able to attract leading life sciences investors to join our existing, strong group of specialist investors is a testament to the impressive accomplishments of the team and the large unmet medical need that currently exists for patients with these hugely debilitating neurological diseases. We look forward to bringing nizubaglustat to patients*."

"*Azafaros has been impressive in its execution with nizubaglustat poised to begin Phase 3 clinical development and the potential to significantly improve the lives of NPC and GM1/GM2 patients. We are excited to support and accelerate the Azafaros team in this important next step in the Company's clinical development journey. Leading this round further demonstrates Jeito's commitment to making a meaningful difference in patients' lives by pursuing much*

needed benefits for those suffering from rare diseases," **said Rachel Mears, Partner at Jeito Capital.**

"*Azafaros exemplifies the kind of science-driven, mission-focused company we seek to back. Nizubaglustat has the potential to fundamentally change the treatment landscape for rare genetic diseases, and we are proud to support their journey as they move closer to delivering real hope and this much needed treatment option to patients and families,*" **noted Audrey Cacaly, Principal at Forbion Growth**.

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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 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 Jeito Capital, Forbion Growth, Seroba, Pictet Group and a syndicate of leading Dutch and Swiss existing investors including Forbion Ventures, BioGeneration Ventures (BGV), BioMedPartners, Asahi Kasei Pharma Ventures, and Schroders Capital.

About Jeito Capital

Jeito Capital is a global leading Private Equity fund with a patient benefit driven approach that finances and accelerates the development and growth of ground-breaking medical innovation. Jeito empowers and supports managers through its expert, integrated, multi-talented team and through the investment of significant capital to ensure the growth of companies, building market leaders in their respective therapeutic areas with accelerated patients' access globally, especially in Europe and the United States. Jeito has built a diversified portfolio of clinical biopharmas with cutting-edge innovations addressing high unmet needs. Jeito Capital is based in Paris with a presence in Europe and the United States. For more information, please visit www.jeito.life or follow us on LinkedIn.

About Forbion

Forbion is a leading global venture capital firm with deep expertise in Europe and offices in Naarden, The Netherlands, Munich, Germany and Boston, USA. Forbion invests in innovative biotech companies, managing approximately €5 billion across multiple fund strategies that cover all stages of (bio-) pharmaceutical drug development. In addition, Forbion's team consists of over 30 investment professionals who have built an impressive performance track record since the late nineties with 128 investments across 11 funds. Forbion typically selects impactful investments that will positively affect the health and well-being of people and the planet, as well as meet its financial return objectives. The firm is a signatory to the United Nations Principles for Responsible Investment. Forbion operates a joint venture with BGV, the manager of seed and early-stage funds, especially focused on Benelux and Germany.

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