

## Introduction to Azafaros

We would like to first thank the INPDA for the opportunity to introduce Azafaros to the international Niemann Pick community. We know from experience how strong and well-organized this community is, and we are looking forward to collaborating with you on possible treatments for Niemann Pick disease.

Azafaros was founded in 2018 by a team of experts who share a deep understanding of rare genetic disease mechanisms. The Dutch-Swiss startup aims to develop new treatments for patients suffering from rare genetic metabolic disorders by combining a wealth of experience in developing new therapies with the latest scientific insights.

The company's lead clinical program is AZ-3102, an orally available small molecule. Currently in the clinical development phase, AZ-3102 is a potential treatment for the rare lysosomal storage diseases, including Niemann Pick type C disease, GM1 and GM2 Gangliosidoses. It has already completed a successful first-in-human clinical study in healthy participants and delivered positive safety, tolerability, and pharmacodynamic data. [Azafaros Presents Positive Clinical and Preclinical Data Supporting Development of Lead Compound AZ-3102 in Lysosomal Storage Disorders at the 18th Annual WORLDSymposium™ Conference](#)

Following the initial Orphan Drug Designation (ODD) for GM2 in January, the Food and Drug Administration (FDA) in March granted AZ-3102 the same status for Niemann-Pick disease type C (NP-C). The designation was based on promising preclinical data of AZ-3120 from a NP-C mouse model, recently presented at the 18th Annual WORLDSymposium™. [Azafaros Announces FDA Grant of Orphan Drug Designation for AZ-3102 in the Treatment of Niemann-Pick Disease](#)

We hope to give updates as we continue to develop our programs over the coming months and in the meantime are looking forward to attending the annual NNPDF Family Support & Medical Conference and INPDA Biennial Meeting in Orlando in July 2022.

For more information: [www.azafaros.com](http://www.azafaros.com)

For any questions, please contact [info@azafaros.com](mailto:info@azafaros.com), [patientadvocacy@azafaros.com](mailto:patientadvocacy@azafaros.com)

For INPDA member organizations:

Gisela Linthorst, Head of Patient engagement

[Gisela.linthorst@azafaros.com](mailto:Gisela.linthorst@azafaros.com)