



Message to the Niemann-Pick Community

We are thrilled to share with you a significant milestone on the Azafaros Phase 2 RAINBOW trial: last week we announced full enrollment of the study of nizubaglustat in GM2 and NPC patients.

This achievement is a testament to the incredible dedication and determination of the GM2 and NPC community, who played a vital role in making this milestone possible. We extend our heartfelt gratitude to all those who participated in the study, helping us advance our mission to provide solutions for these rare genetic diseases.

The study enrolment was completed at sites in Brazil and we regret that we were not able to enroll patients at US sites. With RAINBOW now closed to enrolment, there are unfortunately no actively recruiting studies with nizubaglustat in the US at the moment. We are now working to initiate phase 3 studies globally, including the US. We will keep this community updated as we continue our work with regulatory authorities to design and implement clinical studies.

We look forward to continuing working with the US Niemann-Pick community to advance research, and ultimately bring new solutions to patients and families.

Thank you for being a part of this journey. We look forward to keeping you informed about our future initiatives and our ongoing commitment to the GM2 and NPC community. Your continued support and dedication are invaluable to us, and we will do our utmost to make the road ahead as promising as possible.

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Wishing you a wonderful holiday season and a wonderful 2024. The Azafaros team.