

Mandos Health

September 2024

To the NPC1 Community,

We are excited to share important updates on our progress and commitment to supporting novel therapies for Niemann-Pick Type C (NPC) Disease. Here is the overview of topics we cover in this newsletter:

- 1. Community Update**
- 2. Development Program Update**
- 3. Expanded Access Program**

Community Update

This summer, the Mandos Health team attended the [2024 Parseghian Fund Annual Conference](#) on **June 8-11, 2024** at Tucson, AZ and the [NNPDF Family Support & Medical Conference](#) on **July 11-13, 2024** in Salt Lake City, Utah.

Both meetings allowed us to join members of the community, including patients and their families, researchers, clinicians, and other companies focused on this disease.

We were proud to support and connect with all facets of the NPC community to advance the research and development of adobetadex (VTS-270) for patients living with NPC, and we will continue to participate in upcoming meetings.





Development Program Update

We are excited to announce the FDA has accepted our request for a Type C Meeting scheduled in October to discuss the direction of our adrabetadex program's development. We have filed our meeting package and will continue to drive forward our plans to support a laser-focused evaluation of clinical, biomarker, natural history, and real-world data for the program.

We look forward to sharing our data findings with the NPC community and thought leaders after we receive advice from the regulatory agencies, and we plan to release a series of publications and deliver presentations at upcoming medical conferences.

As always, we know every day matters. We couldn't be more grateful for the trust you continue to place in us.

Expanded Access Program (EAP) Update

We remain steadfast in our commitment to the RUSH EAP. Recently, after discussions with the FDA, the EAP age criteria have been expanded to include patients from 3 months to 25 years old.

Mandos Health continues to work with the clinicians, researchers and sites who provide expanded access and care to NPC patients and their families.

Stay up to date with our progress

We are committed to continuing to build a science-driven organization dedicated to NPC.

We enjoy engaging all stakeholders with updated communications through conference attendance and patient advocacy organization meetings.

Stay up to date with us at www.mandoshealth.com

**Adrabetadex is an investigational drug that has not been approved by FDA, and FDA has not found it to be safe and effective for use to treat NPC1 or for any other use.*

Mandos Health

A world of support for all affected by NPC1

Research. Access. Collaboration.

#NoStoneLeftUnturned #TheRightThingToDo #BuiltForPatients