

Mandos Health

Community Update, May 2025

We are excited to share important updates on our progress as we continue to build a science-driven organization dedicated to NPC.

Here is the overview of topics we cover in this newsletter:

1. **Conference & Meeting Attendance**
2. **Development Program Update**
3. **Expanded Access Program**
4. **Stay up to date with our progress!**

Conference & Meeting Attendance

Mandos Health is proud and excited to be supporting and attending several upcoming medical and patient advocacy conferences:

Ara Parseghian Medical Research Foundation (APMRF), **National Niemann-Pick Disease Foundation (NNPDF)**, the **American Neurological Association (ANA)**, the **Child Neurology Society (CNS)**, and the **International Niemann-Pick Disease Alliance (INPDA)** meetings.

We enjoy engaging all stakeholders with our communications, conference attendance, and patient advocacy organization meetings.

Please contact us if you would like to get together at any of these conferences!

patientadvocacy@mandoshealth.com

Development Program Update

At Mandos Health, our goal is to move adrabetadex forward in its development in the most efficient manner possible and in consideration of all those suffering with NPC1.

We are reviewing and analyzing all possible sources of data regarding adrabetadex and NPC to determine a path forward, including clinical trials, EAP, natural history studies, and registries.

We are getting closer to finishing our assessment.

We are grateful for the FDA's thoughtful collaboration on the development program.

We plan to release a series of publications and provide presentations at these important conferences.

This will include data on the Phase 2b/3 study, our additional clinical analyses and future development plans.

We also look forward to introducing you to a new senior team member, Dr. Jonathan Yong, as SVP Medical Affairs. Jonathan previously led medical affairs for Spinraza, one of the most successful Orphan CNS drug launches. Jonathan was most recently at Cerevel.

Expanded Access Program (EAP) Update

We want to remind you that we remain steadfast in our commitment to the EAP and providing drug supply.

Recently, we invested in securing additional drug product, which allows continued supply of adrabetadex through 2025.

Mandos Health will continue to work with the clinicians, researchers and sites who continue to provide expanded access and care to NPC patients and their families in North America.

Stay up to date with our progress!

Every day we support the NPC and broader rare disease community by advancing research and building relationships with patients, their families, clinicians, policy makers, and everyone striving for care and novel treatment that aligns with patients, accessible to all.

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