

# Mandos Health

June 2023

## To the NPC1 Community,

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

1. Community Update
2. Partnerships
3. Development Program Update
4. Expanded Access Program

### Community Update

Recently, Mandos attended the 2023 MICHAEL, MARCIA, AND CHRISTA PARSEGHIAN SCIENTIFIC CONFERENCE FOR NIEMANN-PICK TYPE C RESEARCH, June 10-13, Tucson, AZ

<https://parseghianfund.nd.edu/events/annual-conference/>.

Over one hundred attendees representing NPC research, foundations, industry, caregivers, and families attended the conference. HOPE WINS was the conference theme in honor of Michael, Marcia, Christa, and others who have lost their battle with NPC disease.

At Mandos, we are proud to support the efforts of the Ara Parseghian Medical Research Fund to advance NPC research. The conference creates a forum for NPC patients & families, communities, non-profit organizations, and industry from around the world to meet and discuss existing and new directions for the community.

This event is vital to the long-term success and goals of this community. Mandos was proud to support and connect with all facets of the NPC community to advance the research and development of adrabetadex (VTS-270) for patients living with NPC.



Picture above (Right): Starting from the left to right, Toni Mathieson (NPUK/INPDA), Sandy Cowie (INPDA), Conan Donnelly (INPDR) Blake Denison (Mandos)

Mandos will be attending and sponsoring the NNPDR Family Support & Medical Conference, July 20-22, Orlando, Florida. <https://www.nnpdrconf.org/>.

## Partnership Update



We are excited to announce that in January, we struck a partnership with INPDR Gateway Ltd to gain access to their NPC patient registry. We will now have access to real-world data from more than three hundred patients with NPC will provide Mandos with evidence to accelerate our research.

Links to press releases:

[INPDR-Gateway-Press-Release-1-June-2023.pdf](#)

[INPDR and Mandos Health Press Release - Business Wire](#)

## Development Program Update

Success to us means leaving 'no stone left unturned.' We are committed to generating and reviewing all clinical and supporting data to evaluate the effects of VTS-270 to inform our path forward.

Our plans are in place to support a laser-focused evaluation of clinical, biomarker, natural history, and real-world data. As far as we are aware, this analysis will be the first of its kind in NPC. We couldn't be more grateful to our scientific, clinical, and FDA collaborators. Of course, all of this is possible because of the passionate NPC community and patients we aspire to help and our team, who have been working tirelessly to support this work.

We look forward to sharing our analysis and findings with the NPC community and thought leaders after we receive advice from the regulatory agencies.

We know everyday 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 EAP. Recently, we invested in securing an additional drug product manufacturing site which allows continued supply of VTS-270 through 2025.

## Stay up to date with our progress

We are committed to building a science-driven organization that is laser focused on NPC. We enjoy engaging all stakeholders with updated communications through conference attendance and patient advocacy organization meetings.

Stay up to date with us at <https://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

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