

Mandos Health

December 1, 2021

To The NPC1 Community,

In the spirit of the season, we start this message with gratitude. We are grateful as always for the support from the entire NPC community, highlighted this month by international collaborators stepping in and helping us access additional important data for our analyses. We are grateful as well to the entire Mandos team, who are working tirelessly to ensure that we examine the totality of the data as expeditiously as possible. Our analysis is augmented by individual insights gleaned from countless conversations with investigators, patients, and families, enabling us to ultimately help advance the understanding of NPC1 and the potential use of adrabetadex*.

We are pleased now to report that the final stage of transition from Mallinckrodt to Mandos is complete. We are, as always, appreciative of the efforts of the Mallinckrodt team working on VTS-270 to make the transition as smooth and seamless for us and, more importantly, for patient, families, and clinical sites. The last doses for the VTS-301 study were administered and the study closeout process is well underway. We were thrilled to be able to support the patients who wanted to continue with their adrabetadex treatments following the conclusion of their clinical trial participation. We are most appreciative for the preparation and vigilance from everyone involved with the Expanded Access Program (EAP) who enabled those patients who met the eligibility criteria for continued benefit to enroll into the expanded access program and continue treatment without interruption.

As part of our monthly communication to help keep the community up to date on our EAP efforts, we are happy to share the most current dashboard summary below.

Summary DASHBOARD of EAP Efforts

(as of December 1, 2021)

Current Sites

14 distinct sites are currently active in EAP

New/Pending Sites

5 pending new sites for EAP

Current patients enrolled in Expanded Access Program

50 patients

Collaboration

We were thrilled to attend the NPC Newborn Screening Clinical Roundtable, a group of nearly 40 NPC and newborn screening experts who gathered virtually to solidify the role of newborn screening for NPC and gain consensus on recommendations for the timing of intervention following a positive diagnosis at birth. NPC patient and community organizations from the U.S., Canada, U.K., Germany, Switzerland, and Australia attended the meeting that was organized by the Firefly Fund.

Development

Our efforts to gain access to additional analysis opportunities from both U.S. and international databases continue to advance, and we look forward to presenting and publishing our analyses starting in early 2022. Completing these important analyses, and ultimately readying them for presentation, will position Mandos to formally request meetings with regulatory health authorities, including FDA, to discuss our findings and potential paths forward for adrabetadex.

We look forward to keeping the community informed as we continue to march forward in this work.

Sincerely,

Scott Riccio
EVP, Patient & Community Engagement
Mandos, LLC
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**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