

Mandos Health

January 2022

To The NPC1 Community,

This communication marks the beginning of a new year and six months since we announced our acquisition of adrabetadex*.

At that time, we shared our plans for taking on not just the drug, but also our intentions to support the large North American Expanded Access Program (“EAP”) that had served the community for years, but which was at risk to close in October of 2021. We heard and recognized how important it was to prioritize finding a way to continue the EAP so that the patients who were already being treated could continue without interruption. We expressed our desire to open outreach efforts and discuss engagement, learning, and collaboration opportunities with the many passionate patients, families, and physicians in the NPC community, as well as the regulators and other key stakeholders critical to the health of the community. We also said we’d begin efforts to gather and analyze the data available to use to help advance understanding of adrabetadex and NPC. Finally, we made a commitment to providing regular updates to the community on our progress against all those plans.

We are thrilled to have been able to progress on all those efforts throughout 2021. The EAP shutdown was averted and physicians within the geographic reach of that program continue to support patients in need of maintaining access to adrabetadex. With that crucial community milestone achieved, we are now solely focused on moving the adrabetadex program forward and will leverage both our team’s experience and passion for collaboration with the community to do so with urgency. We are excited to share many ‘firsts’ for this program with the community today, and in future communications.

Get to know us

Our leadership team collectively has over 200 years of experience and has been intimately involved with more than 100 drug approvals across 17 therapeutic areas including CNS (central nervous system) and rare and ultra-orphan disease drug or biologic therapies. We have executives that have worked in every stage of development from early research through commercialization and members of the team with significant experience working with (or at) FDA.

We are all very proud to work on this program. Over the coming months we will be excited to share more about our team and collaborators.

What's next

Our 'leave no stone unturned' initiative has focused on gathering, aggregating, and reviewing the totality of adrabetadex data that is available to us, as well as additional NPC natural history data. This approach has prioritized creating a better understanding of the risk/benefit across the various NPC subgroups highlighted in the literature and has already begun to pay off.

The first step was to understand and define as best as possible the natural history of the disease for each subtype to help provide a clearer understanding of risk/benefit of adrabetadex for regulators. The patient groups and clinicians with whom we've worked have brought much passion to this effort and helped us identify and access several additional U.S. and International databases. To our understanding, these data sets have not been integrated in such a review before and we are excited to do this.

The second step was to look for commonalities of response among adrabetadex users by analyzing the totality of clinical data available to us. This is something that also had not been done before at this scale. We are examining both the CNS and systemic manifestations of NPC in our analyses and look forward to sharing our findings in those areas.

These larger datasets and analyses will allow us to achieve our goal of bringing regulators the broadest, highest quality dataset to inform our discussions to move the program forward.

Finally, we would be remiss not to share some of our insights. Excitingly, our analyses so far have consistently shown that the earlier patients were treated in the progression of their disease, the better the opportunity for positive outcomes. For us, this has clearly reinforced the importance of efforts related to newborn screening and other approaches to help identify NPC earlier in life and we join with the community to expand support for those initiatives.

External presentations

We are also excited to announce that the first abstract from our own analyses was accepted for virtual ePoster presentation at the upcoming 18th Annual WORLDSymposium, being held February 7-11, 2022. This presentation, authored in collaboration with two external leaders in the field, marks important progress against our stated goals to share with the broader community what we learn in our ongoing analysis of the adrabetadex data. We anticipate expanding our publications and conference plans throughout 2022 and look forward to the opportunity to further discuss and share our learnings with both the community and regulatory agencies.

As you can probably tell, we're excited about 2022. Thank you to everyone in this passionate community who has reached out in support. We look forward to staying in touch, moving the adrabetadex program forward, and working with many of you through the year.

Sincerely,

Scott Riccio
EVP, Patient & Community Engagement
Mandos, LLC
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