February 2nd, 2025 INAD/PLAN Family Call Notes

Gene Therapy Update

Leena Panwala, President of the INADcure Foundation, opened the call by explaining why the Foundation is sponsoring a gene therapy program. After years of searching for a biotech partner without success, the Foundation decided to sponsor the drug development program independently. Leena noted that companies often overlook rare diseases like INAD, as they typically focus on conditions with larger patient populations and greater profit potential. Over the past two years, the Foundation has assembled a team of scientists, researchers, and manufacturing experts to advance its program. It has been working closely with the INAD/PLAN community to raise the necessary funds.

Neil Hackett, Ph.D., Liz Schwarzbach, Ph.D., and Dr. Darius Adams joined to provide updates on the Foundation's gene therapy program and answer questions from INAD/PLAN families. Dr. Hackett, the program's director, and Liz Schwarzbach, Chair of the Foundation's Scientific and Medical Advisory Board, shared that the program has achieved "proof of concept" in cell and mouse models. Two critical preclinical studies are underway: a toxicity study at Nationwide Children's Hospital to evaluate safety and a dosing study at The Jackson Laboratory to determine the optimal treatment dose.

Once these studies are completed, the Foundation hopes to submit an Investigational New Drug (IND) application to the FDA by September 2025. If approved, the Foundation can move forward with a clinical trial. However, $1.8 million is needed to manufacture the drug for the trial. Since manufacturing takes approximately six months, the Foundation hopes to begin this process soon to avoid delays in starting the trial.

Many families asked about the clinical trial. Dr. Darius Adams explained that these details have not yet been finalized. Specifics like participation criteria (e.g., age, health status, or other factors), the number of children who will be enrolled, and the final trial location (likely New Jersey, near New York City) are still being determined.

The Foundation will continue to update INAD/PLAN families on the progress of its gene therapy program and fundraising efforts.

Fundraising for Gene Therapy

The gene therapy program relies on the continuous support and involvement of the entire INAD/PLAN community. Currently, over 40 INAD/PLAN families from various countries, including Belgium, Brazil, England, France, India, Ireland, Portugal, Singapore, Slovakia, Spain, and the United States, are helping to raise the funds needed to keep the program moving forward.

Significant investment has been made in the advanced stages of our preclinical development.

-Preclinical Investment to Date: $1,743,119

-Looking ahead, we anticipate the following costs for manufacturing and clinical trials.

-Manufacturing: $1.8 million total, with a $500,000 down payment required to commence production.

-Clinical Trial Costs: Estimated to exceed $250,000 per patient.

How You Can Get Involved

🌍 Fundraising Through Our Website -

One of the most simple and impactful ways to support the gene therapy program is by setting up a fundraising page through our website. These pages can be tailored to your family's comfort level, allowing you to share as much or as little as you choose while still making a significant difference. Follow this link and click "Become a Fundraiser", If you have any questions or need support, please reach out to Becca - rdanz@inadcure.org

🎬 "The Zebra and the Bear" Virtual Screening – February 16

We invite you to attend and share our special virtual screening of "The Zebra and the Bear" on February 16. This documentary is a powerful way to help others understand the journey of rare disease families and why our fight for a cure is so urgent. By inviting friends, family, and your broader community to watch, you're not only spreading awareness—you're helping us raise critical funds to move our gene therapy program forward.

Register Here!

🎗️ Rare Disease Day – February 28

Rare Disease Day is an opportunity for us to raise awareness and make our voices heard. Here's how you can participate:

- Wear and Share – Purchase Rare Disease Day merchandise and use it as a conversation starter.
- Spread the Word – Engage your community by sharing your family's story or fundraising page.
- Participate in Local or Virtual Events – Every action helps bring attention to the urgent need for INAD research.

📚 Sharing Resources with Schools

We know every family has different comfort levels when it comes to talking about INAD. That's why we offer a range of school resources—from simple classroom activities to larger awareness campaigns—so you can choose what feels right for your child's school and community.

🔵 Rare Rally for Miracles – Lighting Up the Map: February 16th - 28th

We're launching our Rare Rally for Miracles, an initiative that highlights just how widespread and impactful the INAD community is. By participating, you'll help put INAD families on the map, showing the world why this fight matters. More details to come!

🚦 Additional Support -

If you need any support or facilitation in these ideas above, please reach out to Becca. She is more than happy to help and is willing to do some of the necessary legwork!

💜💙Follow Up Survey -

As a follow-up to Sunday's Quarterly Family Zoom Call, we'd ask for your participation in this survey. In doing so, you will be entered into a $50 Rare Disease Day INADcure Foundation Merch Gift Card. We ask that all submissions be in by February 7th at 9 am EST.