



Fall ~ Winter, 2022

Opus Genetics Newsletter

Happy holidays from the entire team at Opus Genetics! It has been a busy year here at Opus. We'd like to take an opportunity as we close out 2022 to recap the progress of this past year and share some updates as we look ahead to the New Year.

LCA5

We are pleased to share that the FDA (US Food and Drug Administration) has reviewed our IND (Investigational New Drug application), which allows us to move forward with in-human trials, and is [allowing us to proceed](#) with the phase 1b/2a clinical trial. We plan to start enrolling patients in 2023. Additional information about the study can be found at [clinicaltrials.gov](#). For any interested families, we recommend speaking with your physician for more information.

We wanted to provide a few details about this trial that were decided in conjunction with the FDA.

First, there will be three doses tested in the trial as part of a dose-escalation design. Three adult participants will be treated within each dose cohort for a total of nine anticipated participants in the study. Also, pediatric participants will not be included at this time. After completion of the first three cohorts in adults, we will ask our independent data monitoring committee to review all data and provide their recommendation as to the suitability of including pediatric participants in our trial. If the committee recommends moving to the pediatric population, we will open a dialogue with the FDA to determine the next steps.



RDH12

Earlier this year we shared information about the preclinical work we have done for RDH12 and our plans for starting clinical trials. After reviewing our submission, FDA provided guidance for additional preclinical studies required prior to proceeding to a first-in-human trial. We are now performing those additional studies and anticipate filing the full IND with the FDA in early 2024. We have several opportunities planned in 2023 to connect with the RDH12 community and will continue to update on our progress as we have more information.

NMNAT1

In early 2023, FDA will be giving us guidance on our preclinical, manufacturing, and clinical study plans for NMNAT1. FDA's feedback will help guide our next steps before we can prepare an IND.

Opus Sponsors Uni-Rare Natural History Study

The Uni-Rare Study is a new natural history study for approximately 1,500 people with mutations in one of more than 300 rare genes associated with inherited retinal diseases (IRDs). This two-part study includes a patient registry and a natural history study. With Opus' support, patients with RDH12 will be eligible to participate in the natural history portion of the Uni-Rare study. For more information on the Uni-Rare Study, please visit www.clinicaltrials.gov.

Opus in the Community

Dinner in the Dark: Vice President of Regulatory Affairs & Medical Writing, Sarah Tuller, recently had the opportunity to attend Dinner in the Dark hosted by [Hope in Focus](#).



Boston VisionWalk: On October 15, 2022 the local Opus team enjoyed a beautiful walk around Artesani Park as part of the 15th annual Boston VisionWalk hosted by the Foundation Fighting Blindness.



RDH12 Scientific Workshop: Several Opus team members had the opportunity to participate in the 2nd Global RDH12 Scientific Conference in Baltimore, MD on November 14th, 2022. At the workshop, patients, caregivers, regulators, clinicians, scientists, and biotechnology companies engaged in lively and productive discussions regarding the most relevant endpoints to



guide clinical trial design. Many thanks to Eyes on the Future, the RDH12 Fund for Sight, and the RDH12 Alliance for organizing.

FFB National Chapter Webinar: Vice President of Clinical Development, Erin O'Neil, participated in the Foundation Fighting Blindness National Chapter Vision Webinar on Emerging Therapies & Research for IRDs on November 12th, 2022. A replay can be viewed [here](#).

Upcoming Events

Rare Disease Day

February 28th, 2023

Foundation Fighting Blindness Speaker Series

6 – 8pm March 28, 2023

Opus HQ, 8 Davis Drive, Durham, NC 27709

Mingle with Opus Genetics team and hear updates regarding our programs and scientific developments.

18th Annual Triangle VisionWalk

9:30am April 29, 2023

Pullen Park, 520 Ashe Ave, Raleigh, NC 27606

The Opus team is looking forward to the upcoming 18th annual [Foundation Fighting Blindness Triangle VisionWalk](#) in Raleigh, NC. We'll see you there!



Stay Connected

To stay informed of our work, please visit <https://opusgtx.com> where you can sign up for updates. We love hearing from the community- please don't hesitate to reach out to us with questions via email at patientsupport@opusgtx.com

You can also follow us on the following social media channels.

