

NBIAcure

NEWSLETTER

~ February 2024 ~



PKAN Gene Therapy Updates

This past December, we hosted two information sessions alongside our collaborators at the UMass Horae Gene Therapy Center to discuss the ongoing progress toward gene therapy for PKAN. We were delighted and encouraged by how many families attended the sessions and brought questions. To learn more about the fundraising trajectory for the project, please visit lovingloic.org.

As fundraising for gene therapy progresses, so does the work in the lab. Before any human clinical studies can begin, pre-clinical animal studies are required to gather preliminary safety information. Our team conducted mouse studies of this nature a few years ago, with promising results. As we move in the direction of a human trial, we're undertaking new studies to clarify the best gene therapy product.

What are we looking for?

We're looking to see which product and dose works best in mice. The dose means how much virus is administered and the product means which promoter is used. The promoter is a DNA sequence that helps control how much protein the cell makes.

How long is it going to take?

It's difficult to say for sure, but an experiment of this scale will probably take 1-2 years. The good news is that other clinical trial planning and preparations will happen concurrently.

How will this study help us to get to clinical trial?

The study is proof-of-concept to the FDA that our proposed treatment will accomplish the desired goal in an animal model, without toxic side effects, and reveals certain specifications of the treatment before we move to human trial.



School of
MEDICINE



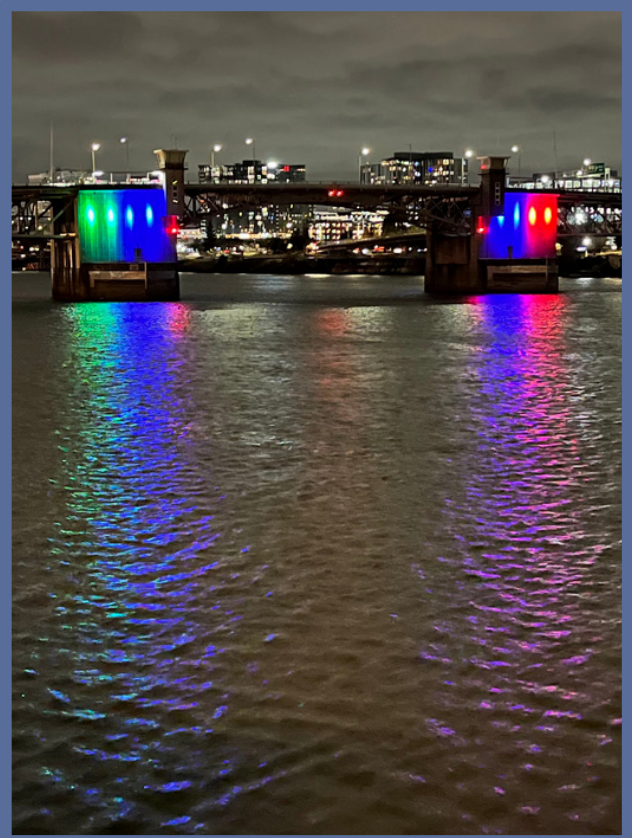


Rare Disease Day

February 29th, 2024



OHSU's rare disease community of clinicians, researchers, staff, and students don their zebra accessories for an all-campus gathering.



Portland's Morrison bridge illuminated in the NBIAcure colors to commemorate Rare Disease Day.

Rare Disease Day is a movement to raise awareness for rare diseases and promote healthcare equity for the 300 million people worldwide who live with a rare disease. This year, Rare Disease Day is taking place on the rarest date on the calendar, February 29th!

At OHSU, we've honored the occasion by gathering with our rare disease colleagues from all across campus for a zebra-themed photo. We also lit up the Morrison bridge in Portland with the NBIAcure colors for the last few nights of February. To learn more about all of the many ways to support, visit rarediseaseday.org.



29 FEB 2024 RARE DISEASE DAY



SHARE YOUR COLOURS