

NBIAcure

NEWSLETTER

- FEBRUARY 2018 -



40 Patient consults from all over the world completed remotely by our team in the past year



46 OHSU students/faculty/family who volunteered their blood as healthy controls for NBIA research



20 Mouse pups that were born into our PKAN mouse colony in January



372 Pages written for three grants that were submitted this past month



4 Academic papers currently in the process of being written or in the final stages of editing

Hello!

Joining our team, Dolly Zhen is taking over for Rachel in the lab, and Katrina Wakeman has started to work with participants of the natural history studies.



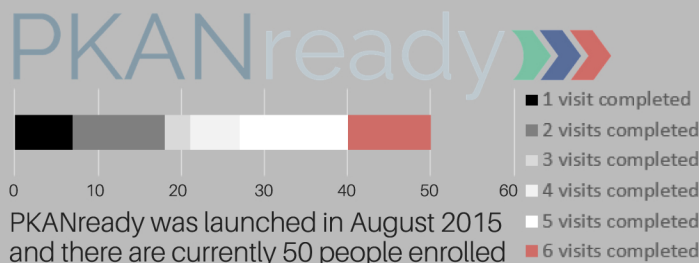
Goodbye..

This past month we were all sad to say goodbye to Rachel Fox and Leila Schwanemann.

Leila is now loving life in Southern California and Rachel is already busy shoveling snow in Boston! They both made invaluable contributions to NBIA research and we wish them the best of luck!

Natural History Studies

We currently have two natural history studies actively enrolling participants, a third one in development and more coming down the line! Visit our website to learn why these studies are important to get ready for future clinical trials and how you or your child can enroll.



BPANready

Currently in development and expected to launch in late winter or early spring.

Funding Updates

In January, we were awarded four grants and one large donation to continue our research.

- PKAN grant by the Collins Medical Trust
- PKAN grant by the Friends of Doernbecher
- BPAN grant by the NBIA Disorders Association
- BPAN grant by the University of Pennsylvania Orphan Disease Center
- INAD donation of ~\$30,000 by the Jacob Phillips Memorial Fund

New Publications

- Lee, JH, Gregory A, Hogarth P, Rogers C, Hayflick SJ. (2018) Looking Deep into the Eye of the Tiger in Pantothenate Kinase Associated Neurodegeneration. American Journal of Neuroradiology.
- Hayflick SJ, Kurian MA, and Hogarth P (2018) Neurodegeneration with Brain Iron Accumulation in Handbook of Clinical Neurology, Geschwind, Paulson and Klein, Eds., Elsevier Oxford UK. Vol. 147:293-305

The CoA-Z Story

Our team's goal since the day Dr. Haylick saw her first patient with PKAN has been to try to find a treatment for all NBIA disorders. This past year, we had a breakthrough in the lab that has moved us closer to this goal.

What is CoA-Z?

In collaboration with Dr. Sibon in the Netherlands, we've developed a compound called "CoA-Z" (pronounced 'co-A-zee') that we believe holds great promise for PKAN. You can visit our website to learn the science behind why we believe this compound may help people with PKAN.

When will CoA-Z be ready for clinical testing?

We've completed most of the work to get to this point with grant funding, but we have much left to do. We need to raise \$2 million in order to make larger batches of

the compound to test in humans and start clinical trials. This money is being raised through a nonprofit called the Spoonbill Foundation.



Why was Spoonbill created?

We are determined to eventually make CoA-Z available to everyone who needs it at the lowest cost possible. Rather than partnering with a pharmaceutical company who could raise the price of the compound just to make money, we decided to form a nonprofit foundation instead. We don't want families to pay anything more than the actual cost of manufacturing.

Why the name Spoonbill?

Spoonbills are birds with brilliant white plumage that commonly nest and breed in the Netherlands, and represent the strong collaboration between our team and Dr. Sibon's team in Groningen. The first three letters, S-P-O, also correspond to the first names of Susan, Penny, and Ody!

Interestingly, spoonbills also raise their offspring longer than most birds, which is a fitting reflection of PKAN families who do the same.

What step is the process at?

We are using the money raised through your donations and fundraising for Spoonbill to have two companies test different methods of producing the compound in large quantities. They are testing to see if they can create large batches of CoA-Z that are still safe, pure and effective. We've heard positive updates from both companies.

Fun facts about CoA-Z

- 1.) We are working with chef-scientists to figure out a way to deliver this compound so it's tasty enough for kids to take by mouth but can also be easily delivered via a g-tube.
- 2.) Right now two flavors called "green apple" and "red grape" are vying for top choice.

Spoonbill Fundraising Update

Thanks to everyone's creative and exhaustive fundraising, you have raised \$251,940 so far, with \$19,484 coming in just this past month alone!

Patient Spotlight

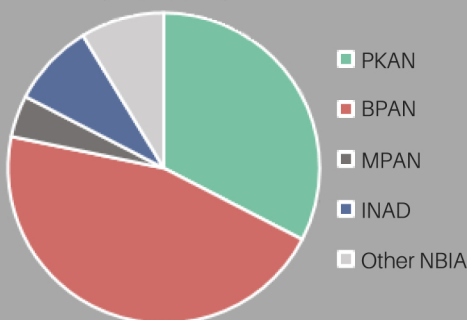


Meet adorable, 4 year-old twins Aubrey and Angela. Our team had the pleasure of meeting these two, along with their older brother, Daron, and their parents during a clinical visit last month

Who has come to see us?

Newly diagnosed children and adults come to our clinic from all over the world to learn about their condition and get management advice. Most of these families continue to keep in touch for additional guidance over the years.

Clinical Appointments (since 2015)



International Visitors

Canada	4
India	3
Australia	1
Turkey	1
Ukraine	1

States travelled from to visit the NBIAcure team

