

Perlara PBC Announces Patient Advocacy Group Partnerships



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SAN FRANCISCO, Jan. 10, 2017 /PRNewswire/ -- Perlara PBC, a global patient portal and drug discovery platform company for the 1 in 10 families affected by rare disease, today announced the formation of two PerlQuest partnerships with rare disease family organizations. The first PerlQuest is with the California-based Grace Science Foundation to cure NGLY1 Deficiency, a metabolic disease related to proteasome-mediated degradation. The second PerlQuest is with the Arizona-based Wylder Nation Foundation to cure Niemann-Pick Type A (NPA)/Acid Sphingomyelinase Deficiency, a lysosomal storage disorder.

PerlQuests are drug discovery collaborations funded directly by patient groups, who in exchange receive joint ownership of the resulting data and inventions. PerlQuests begin with a natural history study of the disease in model organisms, progress to a drug-repurposing screen, and culminate in a high-throughput drug screening campaign to discover novel compounds suitable for preclinical validation in mice. "The goal of the PerlQuest model is to democratize venture philanthropy and make it possible for any patient advocacy group anywhere in the world to be equal partners in their own personalized drug discovery journey," said Ethan Perlstein, Ph.D., CEO of Perlara PBC.

"We've worked closely with the Perlara team since the company's formation. We are proud to be a launch partner of PerlQuests and are excited about the drug leads that will emerge," said Matt Wilsey, President of Grace Science Foundation. "Perlara's nimble platform allows it to quickly iterate and in turn cut down the time when NGLY1 patients can receive a life-saving treatment."

"Perlara's unique drug discovery platform and business model is a perfect fit for advancing our efforts in discovering existing compounds that can be repurposed, and new novel compounds that can be optimized for treating Niemann-Pick Type A," said Steven Laffoon, Co-Founder and President of Wylder Nation Foundation.

Last year, Perlara PBC announced its first commercial deal, a research collaboration with and investment by Novartis for three lysosomal storage disorders, starting with Niemann-Pick Type C.

About Perlara PBC

Perlara PBC is a scientific discovery Public Benefit Corporation that works with families and drug companies to find treatments for diseases previously believed to be too rare to cure. For general information and updates, please visit us at perlara.com, connect with us at @PerlaraPBC on Twitter and at www.facebook.com/PerlaraPBC on Facebook, and learn specifically about PerlQuests at www.perlara.com/perlquests and www.perlara.com/faqs.

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