

Hawaii Patient at <u>Center for Rare Neurological Diseases</u> became 2nd Patient in the World Treated with Groundbreaking ASO (Antisense Oligonucleotide) Therapy for DRPLA Genetic Disease

Kuri grew up in Hawaii just like any normal child until she was around 9 years old when she began to lose her balance and motor skills. Mom also noticed that she is not keeping up with schoolwork and noted the decline in her cognitive skills and ability. Soon, the nightmares of frequent seizures set in. Genetic testing showed that Kuri suffered from a rare genetic disorder known as Dentatorubral-pallidoluysian atrophy (DRPLA).



DRPLA is caused by a mutation in the ATN1
gene which provides instructions for making a protein called atrophin-1. DRPLA is a rare
genetic disorder. See Kuri's Story. There is currently no cure or even hope for DRPLA until
2024 February when the first patient in New York was treated with Groundbreaking ASO
Technology. Kuri recently became the second DRPLA patient in the world to receive this ASO
technology therapy generously provided by n-lorem Foundation.

n-Lorem is a "non-profit committed to discovering and providing personalized experimental treatments, for free, for life, to the most isolated and desperate of patients. We mount a drug discovery program to develop a drug that is targeted for that single patient."

Hawaii's neuroscience specialists and researchers at <u>Center for Rare Neurological Diseases</u> and <u>Clinical Research Center</u> at Hawaii Pacific Neuroscience are glad to join n-Lorem to volunteer their time to provide the needed research care for Kuri **for free for life** so hope is restored for this precious family.

Please support Kuri by making a donation to <u>Hawaii Pacific Neuroscience Foundation</u>. For progress and to follow Kuri's journey, <u>Cure Disease Kuri Channel</u>



"Our specialists & researchers in Hawaii are honored to be part of Kuri's journey and contribute to this worldwide effort to find a cure for DRPLA" said NIH trained neurologists and researcher Kore Kai Liow, MD, Principal Investigator at Clinical Research Center where he oversees research programs in various neurological disorders including rare neurological conditions like DRPLA affecting people in Hawaii & the Pacific Regions. Questions: Info@HawaiiNeuroscience.com