“BlackfinBio” Launched With Aim of Developing Gene Therapy Treatments for Rare and Ultra-rare Disorders, Including SPG47

Startup includes venture philanthropy equity stake for Cure AP-4 and goal to hold first human trial for SPG47 gene therapy developed by University of Sheffield

NEWBURYPORT, MA, USA, December 13, 2023 /EINPresswire.com/ -- Cure AP-4 co-founders Kasey and Chris Edwards have entered into a business partnership which includes the University of Sheffield and Cure AP-4. BlackfinBio will focus on taking promising proof-of-concept gene therapy programs into human clinical trials using a platform approach developed by Principal Investigator Mimoun Azzouz, PhD of the Sheffield Institute for Translational Neuroscience (SiTraN). BlackfinBio aims to advance gene therapy research for additional disorders of the CNS in the future by leveraging insights gained from its lead program for SPG47.

Pathological variants in the AP4B1, AP4E1, AP4M1 and AP4S1 genes cause AP-4-associated hereditary spastic paraplegia (AP-4 HSP), named SPG47, SPG50, SPG51 and SPG52, respectively. Disease symptoms become apparent in infancy, generally presenting with loss of muscle tone progressing to spasticity and loss of lower and, sometimes, upper limb function, accompanied by global developmental disability, and seizures. Cure AP-4 (formerly Cure SPG47) was co-founded in 2016 by Kasey & Chris Edwards and Angela & Kevin Duffy after their toddler daughters, Robbie Edwards and Molly Duffy, were diagnosed with SPG47.

Cure AP-4’s financial sponsorship of Dr. Azzouz’s SPG47 gene therapy proof-of-concept research has resulted in an equity stake for the non-profit. “The initial priority was to develop a therapeutic to halt the trajectory of the disease threatening the futures of Robbie and Molly,” said Chris Edwards. “Launching our own venture became the most expedient way to advance the promising treatment developed by Dr. Azzouz and the University of Sheffield.” The Edwards family has agreed to provide seed capital for the venture, with Chris Edwards serving as interim Chief Business Officer and Board Member.

Dr. Azzouz and his team have developed a comprehensive pre-clinical data package supporting efficacy of the experimental AAV9 gene therapy treatment in knockout mice. Toxicity studies are currently underway, as are plans for a phase 1 trial in human SPG47 patients.

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