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# CURE AP-4 ENGAGES UNRAVEL BIOSCIENCE IN PIONEERING AI-DRIVEN DRUG DISCOVERY FOR ULTRA-RARE CNS DISEASE

*Partnership will generate clinical data from patients globally to accelerate identifying and developing therapeutics for AP-4 hereditary spastic paraplegias.*

NEWBURYPORT, MA, December 5, 2023 /EINPresswire.com/ -- Cure AP-4 announced its decision to partner with Unravel BioSciences, Inc. ("Unravel") to identify potential therapeutics for AP-4 hereditary spastic paraplegia.Unravel, a pioneering therapeutics company leveraging a machine-learning network model of whole body human health to accelerate the development of drugs for complex diseases, will utilize its in silico predictive drug discovery platform called BioNAV™ to accelerate drug discovery for AP-4 by collecting RNA data from families and patients around the world to identify potential treatments specific to patient subgroups.

First described in 2011, AP-4-associated hereditary spastic paraplegia (AP-4-HSP) is a group of four neurodevelopmental and slowly progressive neurological disorders that generally present with global developmental delay, moderate to severe intellectual disability, impaired/absent speech, small head size (microcephaly), seizures and progressive motor symptoms. Low muscle tone (hypotonia) in infancy develops into high muscle tone (hypertonia), resulting in spasticity of the legs that leads to the inability to walk (non-ambulation) and wheelchair reliance. Spasticity may progress to the upper extremities, leading to the partial or total loss of use of all four limbs and torso (tetraplegia). To date, there is no approved treatment for any of the four AP-4-HSPs.

"The innovative technology offered by Unravel Bio is a game changer for the rare disease community," said Kasey Edwards, Cure AP-4 Non-profit Co-Founder and Community Coordinator, "It presents a timelier and much more affordable option than some other therapeutic solutions. Additionally, Unravel is enabling patients with all 4 genes of AP-4, from all around the world, the ability to participate for the first time in research focused on both drug discovery and identification of commonalities and differences amongst these patients to better personalize therapeutic response, This offers hope to a greater reach of patients,"

The funding will support Unravel to generate RNAseq data from diverse AP-4-HSP patients across the globe to stratify patients by therapeutic response and deploy its BioNAV™ computational platform for in-depth in silico compound screening and target discovery. Additionally, RNAseq data from mouse models will be used to plan preclinical studies for each patient subgroup. This initiative aims to uncover and forecast effective treatments that would

benefit patients through rapid repurposing of existing drugs, while strategically enabling Unravel to uncover new targets and develop novel therapeutic molecules for AP-4-HSP.

"We are excited to partner with Cure AP-4 to generate clinical data to understand the disorders and identify a strategic plan for clinical translation of drug candidates to a global patient population," said Richard Novak, PhD., Co-founder and CEO of Unravel. "The foundation is thinking very creatively about how to most efficiently treat all four groups of patients, and Unravel is honored to enable this highly innovative accelerated approach to drug discovery for a challenging and complex disorder."

About Cure AP-4

The Cure AP-4 non-profit organization was founded in 2016 by families of two AP-4-HSP patients. The purpose of this organization is to study and seek a cure for all AP-4-HSP disorders. We aim to improve the quality of life for children impacted by AP-4 HSP by accelerating the research for cures and treatments and providing support for patient therapies critical to their well-being and rehabilitation.

About Unravel Biosciences

Unravel Biosciences is the first rapid prototyping therapeutics company, integrating AI systems biology computation with rapid in vivo screening and clinical validation of discovered targets with unprecedented efficiency. Unravel leverages its proprietary BioNAV™ platform combining target and drug discovery, preclinical screening and patient stratification to find treatments for complex diseases. Unravel's platform discovered RVL002, a first-in-class new small molecule targeting mitochondrial metabolism with applications in multiple CNS and metabolic disorders, and RVL027, a molecule targeting a novel mechanism to treat dystonias. The rareSHIFT program provides platform access to foundation and biotech partners. More at www.unravel.bio and www.rareshift.org

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