CHOPS Foundation initiates AI-driven therapeutics discovery program with Unravel Biosciences

Innovative program will generate the first clinical molecular data for CHOPS syndrome and related disorders

Logo of CHOPS Foundation Rare Diseases

PISTOIA, ITALY, January 5, 2024 /EINPresswire.com/ -- Fondazione CHOPS Malattie Rare ETS ("CHOPS Foundation") announced its partnership with Unravel Biosciences, Inc. ("Unravel") to establish a concerted drug discovery and development program with the goal of rapidly derisking novel therapeutic mechanisms in patients using available drugs. The CHOPS Foundation triggered the first phase of patient stratification and in silico drug screening, thanks to an investment of about $70,000 dollars coming from its awareness campaign.

CHOPS syndrome is a complex genetic disorder caused by mutations in the AFF4 gene. CHOPS is an acronym for a list of symptoms including cognitive impairment, coarse facial features, heart defects, obesity, lung (pulmonary) involvement, short stature, and skeletal abnormalities. This syndrome was discovered in 2015 with 33 cases documented around the world, 4 of which are Italian.

Unravel, a Boston, MA-based pioneering therapeutics company leveraging a machine-learning network model of whole body human health and a deep understanding of true molecular function of over 40,000 molecules 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 CHOPS syndrome. Unravel will lead a multipronged approach to repurposing FDA-approved drugs and other readily available compounds for treating CHOPS syndrome by first collecting RNA and generating RNA profiles from families and patients around the world and screen them to identify potential treatments. Uniquely, this accelerated drug discovery program also involves a group of patients affected by a similar genetic condition: Cornelia De Lange Syndrome.

“Our objective is to discover a cure”, motivates Manuela Mallamaci, president and co-Founder of CHOPS Foundation. “The beginning of this research comes after a complex path, necessary to guarantee scientific precision and transparency. I thank all the members of the Foundation, especially our medical and scientific advisory board, chaired by Dr. Ian Krantz from Children’s Hospital of Philadelphia and our legal consultants, lawyer Marco del Pinto, Mariavittoria Michelacci and Francesco Faccioli. I thank all the people who contributed a donation. We are confident in our ability to rapidly help CHOPS syndrome patients through this exciting and novel therapeutic discovery approach”, explains Manuela Mallamaci, president of Fondazione CHOPS Malattie Rare ETS and the mother of Mario, a 2 year old child diagnosed with CHOPS syndrome.

“The CHOPS Foundation’s strategic approach to tackle an ultra-rare disorder by joining forces with Cornelia de Lange syndrome families and leverage Unravel’s BioNAV™ platform to rapidly identify therapeutic mechanisms that can be tested clinically, addresses many of the key challenges in orphan drug development,” said Richard Novak, PhD, Unravel’s CEO and Co-Founder. “Unravel is thrilled to partner with the CHOPS Foundation in their vision for innovative drug development for CHOPS and Cornelia de Lange syndrome patients.”

About CHOPS Foundation

Fondazione CHOPS Malattie Rare was established on May 13th 2023, after a successful crowdfunding campaign in Italy “Un aiuto per Mario e non solo” (https://gofund.me/aad116be). It is the first and only non-profit organization with the mission of driving and accelerating targeted therapies for CHOPS Syndrome, an ultra-rare and multi-organ neurodevelopmental genetic disease. Together with the Unravel project, CHOPS Foundation has just opened a request for applications. More at the Foundation website.

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. Both new mechanisms are being tested in upcoming clinical trials using the off-target mechanisms of approved drugs. The rareSHIFT program provides platform access to foundation and biotech partners to enable unprecedented acceleration of therapeutics development and clinical derisking. More at www.unravel.bio and www.rareshift.org

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