Stanford and MD Anderson Researchers Both Win CureAccelerator Live! for Pediatric Rare Diseases

Clinical Repurposing Trials in Vascular Malformations and Osteosarcoma Receive Funding

Chicago, Ill. – June 15, 2021

Dr. Joyce Teng representing Stanford University and Dr. Nancy Gordon representing the University of Texas MD Anderson Cancer Center both won Cures Within Reach's CureAccelerator Live! for Rare Diseases virtual philanthropic pitch event, which was held June 10-11. Dr. Teng's project, "Treating Vascular Malformations in the Face, Head and Neck Using Trametinib," will study complex extracranial arteriovenous malformations in pediatric patients treated with an approved cancer drug. If successful, this project may lead to slower disease progression and improved quality of life for these patients. Dr. Gordon's project, "Enhancing Treatment Response in Recurrent/Metastatic Osteosarcoma with Hydroxychloroquine," will study adding an approved malaria drug to the current recommended drug combination for pediatric patients with relapsed/recurrent osteosarcomas. If successful, this project may enhance the treatment response and lead to the identification of biomarkers in these pediatric patients to help improve survival rates. Both researchers will receive up to $50,000 in funding from Cures Within Reach.

A leading global nonprofit focused on repurposing research as a fast track to impacting patients, Cures Within Reach's CureAccelerator Live! competition showcases clinical repurposing projects that are ready to fund. Held during Global Genes' RARE Drug Development Symposium with the Orphan Disease Center of the University of Pennsylvania, CureAccelerator Live! featured four researchers representing academic medical centers from across the US and Chile, and its attendees voted for the winning rare pediatric disease clinical repurposing trial.

"Arteriovenous malformation is a challenging congenital disorder without any FDA approved treatment," said Dr. Teng. "We are very excited about the potential benefits of repurposing a targeted medial therapy for this debilitating condition."

"Osteosarcoma, a malignant bone tumor, is an orphan disease in a vulnerable patient population," said Dr. Gordon. "Funds provided by Cures Within Reach are essential to further our understanding of why certain patients may or may not benefit from this treatment and allow the developing of biomarkers that could be used to select patients for this treatment or related clinical trials in the future with potential to impact survival."

"These projects highlight the speed and cost-effectiveness that clinical repurposing trials can have on pediatric rare disease patients – and any patient – with unsolved diseases," said Barbara Goodman, President & CEO at Cures Within Reach. "We are thrilled to support both Drs. Teng and Gordon and hope to raise funding for the other two finalists in 2021."

The other finalists who presented their repurposing projects at CureAccelerator Live! included:

- Ignacia Fuentes, PhD of DEBRA Chile: Repurposing a Common Antioxidant Nutraceutical, N-Acetylcysteine, for Wound Treatment in Butterfly Skin
- Joseph Rower, PhD of University of Utah: Repurposing Valganciclovir to Treat Cytomegalovirus-Induced Hearing Loss

Expert panelists representing pharma, clinicians, academia and patient advocacy helped attendees make a more informed voting decision. Panelists included representatives from Advocate Aurora Health, the Charles H. Hood Foundation; Global Genes; and Horizon Therapeutics.

Cures Within Reach is grateful for philanthropic support from the Charles H. Hood Foundation for its pediatric-focused clinical repurposing research. Additional support for this event and all of its Repurposing Communities from Brand Institute, EdgeOne Medical, Healx, Horizon Therapeutics, the Judy Hirsch Foundation, Medidata, Recordati Rare Diseases, Takeda Pharmaceuticals and TerSera Therapeutics.

About Cures Within Reach

Cures Within Reach (CWR) is a US-based philanthropic leader improving patient quality and length of life by leveraging the speed, safety and cost-effectiveness of medical repurposing research: driving more treatments to more patients more quickly. CWR catalyzes research to facilitate and validate already approved therapies for new indications to create clinical impact. CWR provides seed funds for first in-human or pivotal studies that, when successful, allow a catalytic effect of additional follow-on trials and follow-on funding. CWR's 2021 initiatives include focused efforts to impact Pediatrics; Veterans; and Diversity, Equity & Inclusion efforts. CWR currently has a global portfolio of 33 repurposing research projects at 28 institutions in 21 diseases. Visit cureswithinreach.org.

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