C-Path's RDCA-DAP Webinar, "The Use and Development of DHTs with Patient Advocacy Groups" a Hit, with Keynote, Patient Advocacy Panel, and Engaged QAs

Critical Path Institute's Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP®), part of C-Path's Rare and Orphan Disease Programs, recently hosted a webinar, "The Use and Development of Digital Health Technologies (DHTs) with Patient Advocacy Groups." RDCA-DAP Scientific Director Heidi Grabenstatter, Ph.D., M.S., moderated, along with guest speaker Laurent Servais, M.D., Ph.D., from the University of Oxford, UK, and University of Liege, Belgium.

Servais's presentation, "Bringing Digital Outcome from an Idea to a Regulatory-qualified Endpoint, from Duchenne to Angelman and Many Others," was followed by a Q&A panel featuring patient advocates Allyson Berent, DVM, DACVIM, CSO at the Foundation for Angelman Syndrome Therapeutics, Eric Camino, Ph.D., Vice President, Research and Clinical Innovation at Parent Project Muscular Dystrophy, Spela Mirosevic, Ph.D., Co-founder and President at CTNNB1 Foundation and Sakshi Sardar, Ph.D., Senior Director, Digital and Precision Medicine at C-Path.

Each patient advocacy leader shared learnings and insights based on the stage of DHT development from their respective program, and described the unique approach necessary given the complexity of the disorder that they represent. Emphasis was placed on how powerful a small change can be for a family caring for or an individual with a neuromuscular or neurodevelopmental disorder. Dr. Berent used the examples of her daughter's decrease in falls and the ability to ambulate to the restroom or elsewhere in the home without constant assistance, while the family knew she was safe. Dr. Camino spoke to the specificity of the device and its ability to measure several different aspects of improved muscle weakness (e.g., distance traveled, gait/stride length, and stride velocity) using sensors, thus, allowing for the validation of the digitally-derived endpoints with the Stride Velocity 95^{th} percentile assessment.

Dr. Mirosevic, meanwhile, discussed the enthusiasm of the CTNNB1 Syndrome community and that nearly 100% of responders to a recent survey had replied that they were interested in participating in a research study focused on development of DHTs that would be beneficial for the neurodevelopmental disorder. This comes with knowledge that the currently available fine and gross motor assessments are inadequate at the detection of clinically meaningful change in pediatric patients with neuromuscular delay.

Dr. Sardar discussed forward-looking approaches that aim to utilize standard protocols in the development of DHTs, and Dr. Servais was encouraging that those projects, while in their infant stages, could be very important to the field. A key theme was that development of DHTs requires the input of clinicians, engineers, regulators, and patient advocates that are experts in the specific disease area. The approach needs to begin with patients' symptoms in mind and align the underlying pathophysiology of the disease, the capabilities of the sensors, and the current regulatory guidance. The need to reflect on how to capture additional unmet measurement needs that go beyond the currently measured ones, particularly in relation to regulatory decision-making, was emphasized.

"The Rare and Orphan Disease Program and RDCA-DAP team were thrilled with the response to the webinar," Dr. Grabenstatter explained. "We'd like to thank the guest speakers for their engaging presentations and commentary and are grateful to the attendees that asked compelling questions to help drive

the Q&A panel discussion.”

You can view the recording in its entirety below: