FSHD Society and Critical Path Institute Collaborate to Collect Clinical Trial Data

TUCSON, Ariz., May 23, 2022 — The FSHD Society, a research-focused patient advocacy nonprofit dedicated to bringing treatments to patients affected by facioscapulohumeral muscular dystrophy (FSHD), has partnered with Critical Path Institute (C-Path) to facilitate the integration of clinical trial data from the control arm of various FSHD-related clinical studies into C-Path's Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP®). These data will come from several companies that have sponsored clinical trials in FSHD.

The integration of these data makes it possible to analyze information from a larger number of patients to generate a more robust and granular understanding of the natural disease progression in FSHD. This is particularly important in rare diseases like FSHD that progress at slow and unpredictable rates.

"RDCA-DAP promotes the sharing of existing patient-level data and encourages the standardization of new data collection," said Alexandre Bétourné, RDCA-DAP Scientific Director. "By integrating such data in a regulatory-grade format suitable for analytics, RDCA-DAP helps accelerate the understanding of disease progression, clinical outcome measures, and biomarkers, and facilitates the development of mathematical models of disease and innovative clinical trial designs."

Organizations participating to date in the FSHD Society's initiative include Acceleron Pharma, recently acquired by Merck & Co., Inc., and the University of Rochester. Acceleron, based in Cambridge, Mass., is contributing control data from its phase 2, randomized, placebo-controlled trial of ACE-083. This therapy was designed to increase the strength and function of specific muscles in FSHD patients. Ninety-five patients were enrolled in the study, which terminated in October of 2019. Acceleron has agreed to provide data collected from the control arm of the study, including total muscle volume (measured by MRI) and quantitative testing of muscle strength.

The University of Rochester trial enrolled 90 patients to assess the effectiveness of albuterol, a beta-adrenergic agonist, to increase the strength of FSHD patients. The study concluded in 2015. The functional data collected in this study included quantitative voluntary isometric contraction testing and muscle mass assessments by dual energy x-ray absorptiometry (DEXA).

"We are grateful to Acceleron and the University of Rochester. Their leadership in the effort to share meticulously collected clinical data to help advance the field of FSHD and future clinical trial design should be applauded," said Jamshid Arjomand, Chief Science Officer of the FSHD Society. "We look forward to expanding the FSHD database in RDCA-DAP as new studies generate additional clinical data. Over time, this project will lead to insights that will improve the design of clinical trials for FSHD."

"This is a great example of how we can collaborate to help accelerate drug development for rare diseases," Bétourné said. "The FSHD society included their community in discussions, landscaped the accessible data and simultaneously engaged C-Path and multiple data contributors, which expedited the sharing of these two datasets."

About the FSHD Society
The FSHD Society is the world's largest research-focused patient organization for facioscapulohumeral muscular dystrophy (FSHD), one of the most prevalent forms of muscular dystrophy. The Society has catalyzed major advancements and is accelerating the development of treatments and a cure to end the pain, disability, and suffering endured by one million people worldwide who live with FSHD. The FSHD Society has transformed the landscape for FSHD research and is committed to making sure that no one faces this disease alone. The Society offers a community of support, news, and information through its website at https://www.fshdsociety.org.

About C-Path
Critical Path Institute (C-Path) is an independent, nonprofit organization established in 2005 as a public and private partnership. C-Path's mission is to catalyze the development of new approaches that advance medical innovation and regulatory science, accelerating the path to a healthier world. An international leader in forming collaborations, C-Path has established numerous global consortia that currently include more than 1,600 scientists from government and regulatory agencies, academia, patient organizations, disease foundations, and hundreds of pharmaceutical and biotech companies. C-Path U.S. is headquartered in Tucson, Arizona, C-Path in Europe is headquartered in Amsterdam, Netherlands and C-Path Ltd. operates from Dublin, Ireland with additional staff in multiple other locations. For more information, visit c-path.org.

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