

FOR IMMEDIATE RELEASE

Northwestern University and NORD Examine Physical Function in Rare Diseases

FDA grant-funded work seeks to understand patient physical function in five rare diseases

Chicago, September 4, 2020-- Northwestern University and National Organization for Rare Disorders (NORD) conduct novel research to develop a core outcome set of Clinical Outcome Assessments (COAs) with support with from the U.S. Food and Drug Administration (FDA).

“Our research seeks to fill a gap in regulatory science, examining and developing a set of physical function assessments and outcomes, designed to be used in pharmaceutical drug development,” said David Cella, PhD, Northwestern University Professor and Principal Investigator of the project— Northwestern University Clinical Outcome Assessment Team (NUCOAT).

NUCOAT is conducting a multi-year project, funded by the FDA Center for Drug Evaluation and Research (CDER) division, to develop and validate a set of patient-reported and performance-based physical function COAs appropriate for use across a range of rare diseases. The team of investigators, including NORD Director of Research Vanessa Boulanger, expect the work to result in core outcome sets that span the full range of physical function limitations and severity. The team is currently reviewing literature and conducting interviews to explore physical function limitations, severity, and importance to quality of life for individuals diagnosed with Idiopathic Pulmonary Fibrosis, Myositis, Systemic Sclerosis, Facioscapulohumeral Muscular Dystrophy, and Hepatocellular Carcinoma.

The NUCOAT project seeks to elevate the patient voice through not only the products of their research, but by engaging the rare disease community throughout the research process. The project involves regular engagement of stakeholders including patients, care partners, and seeks to keep the rare disease organizations and community informed and engaged through bi-annual public meetings and dissemination of research findings and milestones on their website.

The success of the NUCOAT project will hopefully lead to scientific progress in drug development and help to advance the FDA’s mission to promote patient-centered drug development in rare disorders, representing a significant, unmet need.

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