DDT #000090 **DDT QUALIFICATION DEFERRAL**

Jeffrey Statland, MD
Assistant Professor of Neurology
University of Kansas Medical Center
4330 Shawnee Mission Parkway, Ste 323
Fairway, KS 66205
Email: jstatland@kumc.edu

Regarding: DDT #000090 Letter of Intent for the Facioscapulohumeral Muscular Dystrophy Functional Composite (FSHD-COM)

Dear Dr. Statland,

We have completed our review of your letter of intent (LOI) submission for DDT #000090 received on November 20th, 2017, by the Clinical Outcomes Assessments (COA) Qualification Program.

The submission included a letter of intent for the FSHD-COM, proposed as an assessment of gait, mobility, and trunk and arm function in ambulatory FSHD patients.

Following careful consideration of your submission, we have concluded that we are unable to accept the FSHD-COM instrument into the COA qualification program. The proposed instrument is a composite of performance outcome measures and clinical examination findings, some of which have already been used to support clinical trial endpoints. Therefore, we do not view the FSHD-COM as filling a measurement gap. Additionally, several of the suggested composite measures evaluate aspects of neurologic function in a manner that does not directly reflect patients' ability to perform their daily activities.

Please note that COAs are not required to be qualified to be used in clinical trials to support drug development. Although we are unable to accept this tool into our qualification program, we are offering the following advice and feedback that may be helpful to you and others who are considering the use of this tool in clinical trials.

- We suggest only the inclusion of concepts that are areas of concern for patients with FSHD. These concepts should represent clinically meaningful concepts that are distinct, clearly defined, and non-overlapping. Response options should have a limited number of categories that represent clinically meaningful differences in patient status.

- Any measure that you plan to develop should reflect the current state of the patient, without necessitating a comparison to a previous time.
- Items should be scored in a manner such that a score change (both improvement and decline) reflects FSHD patients' ability to perform their daily activities (e.g., mobility, activities of daily living, or personal hygiene tasks).
- Please take into consideration the inclusion of clinically meaningful upper body performance outcome measures in order to best capture the proposed concepts of interest and clinical benefit for FHSD patients.
- You should consider the use of a patient reported outcome (PRO) to complement other measures that you plan to develop to provide the patient perspective as well as help to assess meaningful change among affected patients.

Should you wish to discuss this further or require any clarification, please contact the Clinical Outcome Assessments Staff at COADDTQualification@fda.hhs.gov.

Sincerely,

Elektra J. Papadopoulos -S

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Elektra Papadopoulos, MD, MPH
Associate Director
Clinical Outcome Assessments Staff
Office of New Drugs
Center for Drug Evaluation and Research

William Dunn -S

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Billy Dunn, MD
Director
Division of Neurology Products
Office of New Drugs
Center for Drug Evaluation and Research