

Overview

Working group: Facioscapulohumeral muscular dystrophy (FSHD)

The National Institute of Neurological Disorders and Stroke (NINDS) Facioscapulohumeral muscular dystrophy (FSHD) Common Data Elements (CDE) project was comprised of a Working Group (WG) of researchers from the FSHD community.

The development of the FSHD Common Data Elements (CDEs) was a multi-tiered process which required review, compilation and re-review of instruments and case report forms (CRFs) by means of monthly meetings. The main goal of the project was to facilitate the reproduction of FSHD studies universally. The group began by making initial recommendations to include specific FSHD instrument, measures and CRF-CDEs, which involved the review of sample CRF templates for Demographics, Social Status and Medical History. This also included reviewing CDEs from CRF templates created by neuromuscular disease working groups in order to begin the process of developing appropriate CRFs for FSHD. Through the series of conference calls, the WG compiled a list of specific instruments to be included in the recommendations. CDE classifications (i.e., Core, Supplemental, etc.) were decided at the final stage of the process.

Summary of Outcome Measures

READ ME: This is a recommendations summary document of the instruments/measures/case report forms, sorted alphabetically. Details of the recommendations follow this spreadsheet in the form of information documents (e.g., Notices of Copyright) or case report forms and CDE details.

Instrument / Scale Name / CRF template <i>Name and acronym of the instrument/measure that is recommended for inclusion in the CDEs</i>	Domain	Subdomain	Classification <i>(e.g., Core, Supplemental (also those highly-recommended), Exploratory)</i>
2 minute walk test	Outcomes and End Points	Functional Status	Exploratory
6 min walk test	Outcomes and End Points	Functional Status	Supplemental - Highly Recommended
Brief Pain Inventory	Outcomes and End Points	Quality of Life	Supplemental

Electrical Impedance Myography (EIM)	Outcomes and End Points	Device	Exploratory
Individualized Neuromuscular Quality of Life Questionnaire (INQoL)	Outcomes and End Points	Quality of Life	Supplemental
Manual Muscle Testing (MMT)	Outcomes and End Points	Muscle Strength Testing	Supplemental - Highly Recommended
Maximum Voluntary Isometric Contraction (MVICT)	Outcomes and End Points	Muscle Strength Testing	Supplemental - Highly Recommended
McGill Pain Questionnaire (MPQ) short form	Outcomes and End Points	Emotional and Cognitive Status	Supplemental
Neuro-QOL	Outcomes and End Points	Quality of Life	Exploratory
Pinch Grip Dynamometry	Outcomes and End Points	Muscle Strength Testing	Supplemental
Pulmonary Function Testing	Outcomes and End Points	Pulmonary Function Testing/Respiratory Status	Supplemental— Highly Recommended for clinical trials
Range of Motion (ROM)/ Goniometry	Outcomes and End Points	Functional Status	Supplemental
Short Form Health Survey-36 (SF-36)	Outcomes and End Points	Quality of Life	Supplemental
Stair climb (Time to climb 4 stairs)	Outcomes and End Points	Functional Status	Supplemental
Time to rise from a chair	Outcomes and End Points	Functional Status	Exploratory

Timed up and go	Outcomes and End Points	Functional Status	Supplemental
World Health Organization Disability Assessment Schedule II (WHO - DAS II)	Participant/Subject History and Family History	Epidemiology/Environmental History	Exploratory

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CRF Name	Domain	Subdomain	Classification <i>(e.g., Core, Supplemental (also those highly-recommended), Exploratory)</i>
DEXA Lean Muscle Mass	Assessments and Examinations	Imaging Diagnostics	Supplemental - Highly Recommended in clinical studies looking at lean muscle mass
Electrocardiogram (ECG)	Assessments and Examinations	Non-Imaging Diagnostics	Supplemental - Highly Recommended
Hand Held Dynamometry	Outcomes and End Points	Muscle Strength Testing	Supplemental
Laboratory Tests and Tracking	Assessments and Examinations	Laboratory Tests and Biospecimens/Biomarkers	Supplemental - Highly Recommended in clinical trials

Magnetic Resonance Imaging (MRI)	Assessments and Examinations	Imaging Diagnostics	Supplemental
Muscle Biopsies and Autopsy Tissue	Assessments and Examinations	Laboratory Tests and Biospecimens/Biomarkers	Exploratory
Mutation Analysis	Assessments and Examinations	Laboratory Tests and Biospecimens/Biomarkers	Supplemental
Polysomnography	Assessments and Examinations	Physical / Neurological Examination	Supplemental
Pregnancy - Prenatal and Perinatal History	Participant/Subject History and Family History	General Health History	Supplemental
Prior and Concomitant Medication	Treatment/Intervention Data	Drugs	Supplemental - Highly Recommended in most clinical studies
Respiratory Interventions	Treatment / Intervention Data	Therapies	Exploratory