



NIH Resources

The NINDS also strongly encourages researchers to use these NIH developed materials for NINDS-sponsored research, when appropriate. Utilization of these resources will enable greater consistency for NINDS-sponsored research studies. These tools are free of charge.

- National Institutes of Health (NIH) Toolbox
- Quality of Life in Neurological Disorders (Neuro-QOL)
- Patient-Reported Outcomes Measurement Information System (PROMIS)

Suicidal Ideation

Investigators should review the FDA’s ["Guidance for Industry: Suicidal Ideation and Behavior: Prospective Assessment of Occurrence in Clinical Trials"](#) for the most up-to-date information about suicidal ideation and behavior. One scale that FDA suggests is the Columbia Suicide Severity Rating Scale (C-SSRS) (available at <http://www.cssrs.columbia.edu>)

Disease/Domain	Recommendations
Myotonic Dystrophy	<p>These instruments and elements are recommended for use in DM studies:</p> <p>Core elements:</p> <p>See Start-Up Resources Listing document (DM Start-Up Resource Listing)</p> <p>Supplemental – Highly Recommended instruments:</p> <p>Manual Muscle Testing-Using the Medical Research Council Muscle Grading Scale, Bedside Myotoniaⁱ, Epworth Sleepiness Scaleⁱ, Myotonic Dystrophy Health Index (MDHI)ⁱⁱ</p>
Participant / Subject Characteristics; Demographics	<p>Core: Demographicsⁱⁱⁱ, General Core</p> <p>Supplemental – Highly Recommended: None</p> <p>Supplemental: Demographics</p> <p>Exploratory: None</p>
Participant / Subject Characteristics; Social Status	<p>Core: Social Statusⁱⁱⁱ</p> <p>Supplemental – Highly Recommended: None</p> <p>Supplemental: Social Status</p> <p>Exploratory: None</p>



Myotonic Dystrophy CDE Highlight Summary Document

Disease/Domain	Recommendations
Participant/Subject History and Family History; General Health History	Core: Medical History ⁱⁱⁱ Supplemental – Highly Recommended: None Supplemental: Family History, Medical History Exploratory: None
Assessments and Examinations; Imaging Diagnostics	Core: None Supplemental – Highly Recommended: None Supplemental: Magnetic Resonance Imaging (MRI), Cardiac Magnetic Resonance Imaging Exploratory: None
Assessments and Examinations; Laboratory Tests and Biospecimens / Biomarkers	Core: None Supplemental – Highly Recommended: None Supplemental: Laboratory Tests and Tracking ^{iv} , Muscle Biopsy and Autopsy Tissue, Mutation Analysis Exploratory: None
Assessments and Examinations; Non-Imaging Diagnostics	Core: None Supplemental – Highly Recommended: Electrocardiogram (ECG) Supplemental: Echocardiogram, Electrocardiogram (ECG), Electrophysiology, Holter Examination Exploratory: None
Assessments and Examinations; Other Clinical Data	Core: None Supplemental – Highly Recommended: None Supplemental: None Exploratory: Non-Dystrophic Myotonia Interactive Voice Response (IVR) Diary
Assessments and Examinations; Physical/Neurological Examination	Core: None Supplemental – Highly Recommended: Bedside Myotonia, Epworth Sleepiness Scale (ESS) Supplemental: None Exploratory: Evoked Myotonia through Electrical Stimulation
Treatment / Intervention Data; Therapies	Core: None Supplemental – Highly Recommended: None Supplemental: Respiratory Interventions Exploratory: None



Disease/Domain	Recommendations
Outcomes and Endpoints; Pain	<p>Core: None</p> <p>Supplemental – Highly Recommended: None</p> <p>Supplemental: McGill Pain Questionnaire</p> <p>Exploratory: None</p>
Outcomes and Endpoints; Pediatric	<p>Core: None</p> <p>Supplemental – Highly Recommended: None</p> <p>Supplemental: None</p> <p>Exploratory: Pediatric Quality of Life Inventory (PEDIQL), Pediatric Quality of Life Individual Neuromuscular Module</p>
Outcomes and Endpoints; Performance Measures	<p>Core: None</p> <p>Supplemental – Highly Recommended: None</p> <p>Supplemental: Wechsler Abbreviated Scale of Intelligence (WASI)</p> <p>Exploratory: None</p>
Outcomes and Endpoints; Pulmonary Function Testing/Respiratory Status	<p>Core: None</p> <p>Supplemental – Highly Recommended: Pulmonary Function Testing</p> <p>Supplemental: Measures of Gas Exchange</p> <p>Exploratory: None</p>
Outcomes and Endpoints; Quality of Life	<p>Core: None</p> <p>Supplemental – Highly Recommended: None</p> <p>Supplemental: Individualized Neuromuscular Quality of Life Questionnaire (INQoL), Short Form 36-Item Health Survey (SF-36)</p> <p>Exploratory: Quality of Life in Neurological Disorders (Neuro-QOL), Patient-Reported Outcomes Measurement Information System (PROMIS), Sickness Impact Profile (SIP)</p>
Outcomes and Endpoints; Muscle Strength Testing	<p>Core: None</p> <p>Supplemental – Highly Recommended: Manual Muscle Testing-Using the Medical Research Council Muscle Grading Scale</p> <p>Supplemental: Grip Strength Fatigue, Hand Held Dynamometry, Maximum Voluntary Isometric Contraction Testing (MVICT)</p> <p>Exploratory: None</p>



Myotonic Dystrophy CDE Highlight Summary Document

Disease/Domain	Recommendations
Outcomes and Endpoints; Multifactorial Patient Reported Burden of Disease	<p>Core: None</p> <p>Supplemental – Highly Recommended: Myotonic Dystrophy Health Index (MDHI)²</p> <p>Supplemental: None</p> <p>Exploratory: None</p>
Outcomes and Endpoints; Functional Status	<p>Core: None</p> <p>Supplemental – Highly Recommended: None</p> <p>Supplemental: 10 Meter Timed Walk, 2 Minute Walk Test, 6 Minute Walk Test, Purdue Pegboard, Stair Climb, Timed Up and Go (TUG), Nine Hole Peg Test, Barthel Index, Gross Motor Function Measure (GMFM), Jebsen Taylor Hand Function Test</p> <p>Exploratory: None</p>

ⁱ Supplemental – Highly Recommended for QOL, functional status, therapy

ⁱⁱ Supplemental – Highly Recommended for use as a primary or secondary outcome measure during DM clinical trials
 Supplemental – Highly Recommended as a marker for disease severity for patients with DM

ⁱⁱⁱ Contains some Core CDEs

^{iv} Supplemental – Highly Recommended for clinical trials