

Duchenne Muscular Dystrophy (DMD) / Becker Muscular Dystrophy (BMD) CDE Highlight Summary

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 [Columbia Suicide Severity Rating Scale Website](#)).

Disease; Domain	Recommendations
DMD/BMD	<p>These instruments and elements are recommended for use in all Duchenne Muscular Dystrophy and Becker Muscular Dystrophy studies: 10 Meter Timed Walk, Brooke Upper Extremity Scale, , Vignos Lower Extremity Scale</p> <p>Core Elements: See Start-Up Listing Document (DMD/BMD Start-Up Resource Listing)</p>
Participant / Subject History and Family History/General Health History	<p>Core: General Core, Medical History</p> <p>Supplemental – Highly Recommended: Family Historyⁱ</p> <p>Supplemental: Family history, Medical history, Surgical history</p> <p>Exploratory: None</p>
Assessments and Examinations / Laboratory Tests and Biospecimens / Biomarkers:	<p>Core: None</p> <p>Supplemental – Highly Recommended: Mutation Analysisⁱⁱ</p> <p>Supplemental: Laboratory Tests Recommendations Table and Guidelines for Use; Mutation Analysis</p> <p>Exploratory: None</p>
Assessments and Examinations / Vital Signs and Other Body Measures	<p>Core:</p> <p>Supplemental – Highly Recommended: Vital signsⁱⁱⁱ</p> <p>Supplemental: Vital signs</p> <p>Exploratory: Vital signs</p>

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Treatment / Intervention Data / Devices	<p>Core: None</p> <p>Supplemental – Highly Recommended: External Devices and other Treatments</p> <p>Supplemental: External Devices and other Treatments</p> <p>Exploratory: None</p>
Treatment / Intervention Data/Drugs	<p>Core: None</p> <p>Supplemental – Highly Recommended: None</p> <p>Supplemental: Prior and Current Medications</p> <p>Exploratory: None</p>
Treatment/Intervention Data; Therapies	<p>Core: None</p> <p>Supplemental – Highly Recommended: None</p> <p>Supplemental: GI Therapies</p> <p>Exploratory: None</p>
Outcomes and Endpoints / Summary of All Outcome Recommendations	<p>Core: None</p> <p>Supplemental – Highly Recommended: None</p> <p>Supplemental: DMD and BMD Clinical Outcome Recommendations</p> <p>Exploratory: None</p>
Outcomes and Endpoints / Functional Status	<p>Core: 10 Meter Timed Walk, Brooke Upper Extremity Scale, Vignos Lower Extremity Scale</p> <p>Supplemental – Highly Recommended: None</p> <p>Supplemental: 6 Minute Walk Test, North Star Ambulatory Assessment (NSAA)</p> <p>Exploratory: Jebsen-Taylor Hand Functioning Test, Nine Hole Peg Test, Pediatric Evaluation of Disability Inventory (PEDI), Pediatric Orthopedic Society of North America Measure (PODCI)</p>
Outcomes and Endpoints / Muscle Strength Testing	<p>Core: None</p> <p>Supplemental – Highly Recommended: None</p> <p>Supplemental: Manual Muscle Testing-Using the Medical Research Council Muscle Grading Scale, Maximum Voluntary Isometric Contraction Testing (MVICT), Pinch Strength</p> <p>Exploratory: None</p>

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Outcomes and Endpoints / Pulmonary Function Testing / Respiratory Status	<p>Core: None</p> <p>Supplemental – Highly Recommended: Pulmonary Function Testing^{iv}</p> <p>Supplemental: None</p> <p>Exploratory: Pulmonary Function Testing</p>
Outcomes and Endpoints / Quality of Life	<p>Core: None</p> <p>Supplemental – Highly Recommended: None</p> <p>Supplemental: Pediatric Quality of Life Inventory (PEDIQL)</p> <p>Exploratory: Canadian Occupational Performance Measure (COPM), Quality of Life in Neurological Disorders (Neuro-QOL)</p>

ⁱ Supplemental – Highly Recommended for a study based on the reliability and validity of the measure in the DMD population

ⁱⁱ Supplemental – Highly Recommended for genotype/phenotype studies and therapeutic trials

ⁱⁱⁱ Supplemental – Highly Recommended for a study based on the reliability and validity of the measure in the DMD population

^{iv} Supplemental – Highly Recommended for a study based on the reliability and validity of the measure in the DMD population