



## Summary of Core Recommendations: Duchenne Muscular Dystrophy / Becker Muscular Dystrophy CDEs

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### Start-up Resource –NINDS Duchenne Muscular Dystrophy/Becker Muscular Dystrophy (DMD/BMD) CDE Recommendations

The National Institute of Neurological Disorders and Stroke (NINDS) and other Federal agencies and international organizations have the common mission of developing data standards for clinical research. Through the efforts of subject-specific working groups, topic-driven data elements have been created. The first set of Common Data Elements (CDEs) for Duchenne Muscular Dystrophy and Becker Muscular Dystrophy was developed in 2012. The Core data elements to be used by an investigator when beginning a research study in this resource document. All other recommendations are listed on the website and should be considered based on study type.

Each CDE or instrument could be classified according to the definitions below:

**General Core:** A data element that is required for all NINDS funded studies.

**Disease Core:** A data element that collects essential information applicable to any disease-specific study, including all therapeutic areas. The NINDS and its appointed working groups assign the disease “Core” classification based on the current clinical research best practices. In each case, the disease Core CDEs are a small subset of the available CDEs, where it is anticipated that investigators will need to collect the disease Core CDEs on any type of study. These are required for all disease-specific studies.

**Disease Supplemental - Highly Recommended:** A data element which is essential based on certain conditions or study types in clinical research studies. In most cases, these have been used and validated in the disease area. These data elements are strongly recommended for the specified disease condition, study type or design.

**Disease Supplemental:** A data element which is commonly collected in clinical research studies. Use depends upon the study design, protocol or type of research involved. These are recommended, but not required, for studies.

**Disease Exploratory:** A data element that requires further validation but may fill current gaps in the CDEs and/or substitute for an existing CDE once validation is complete. Such data elements show great promise but require further validation before they are ready for prime-time use in clinical research studies. They are reasonable to use with the understanding that it has limited validation in the target group.



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<p><b>National Institute of Health (NIH) Resources:</b>  <i>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.</i></p>	<ul style="list-style-type: none"> <li>• NIH Toolbox</li> <li>• Quality of Life in Neurological Disorders (Neuro-QOL)</li> <li>• Patient-Reported Outcomes Measurement Information System (PROMIS)</li> </ul>
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### Core CDEs for all NINDS Studies<sup>1</sup>:

Domain/Sub-Domain	CDE Name	CDE ID	Study Type
Participant Characteristics; Demographics	Birth date	C00007	All studies
Participant Characteristics; Demographics	Ethnicity USA category	C00020	All studies
Participant Characteristics; Demographics	Race USA category	C00030	All studies
Participant Characteristics; Demographics	Birth sex assigned type	C58676	All studies
Participant Characteristics; Demographics	Gender identity type	C58677	All studies
Participant History and Family History; General Health History	Medical history condition text	C00322	All studies
Participant History and Family History; General Health History	Medical history condition SNOMED CT code	C00313	All studies

### Supplemental – Highly Recommended CDEs for Duchenne Muscular Dystrophy / Becker Muscular Dystrophy Studies:

Domain; Sub-Domain	Data element	CDE ID
History of Disease/Injury Event	Symptoms first appeared date and time	C08006
History of Disease/Injury Event	Diagnosis first given date and time	C08007
Participant/Subject History and Family History; General Health History	Medical history global assessment indicator	C00315

<sup>1</sup> Note: Education year count C00015 is no longer a general Core CDE

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Domain; Sub-Domain	Data element	CDE ID
Participant/Subject History and Family History; General Health History	Family history medical condition indicator	C00721
Participant/Subject History and Family History; General Health History	Family history medical condition relative type	C00722
Participant/Subject History and Family History; General Health History	Family history medical condition relative other text	C18679
Assessments and Examinations; Laboratory Tests and Biospecimens/Biomarkers	Gene mutation detected result type	C12785
Assessments and Examinations; Laboratory Tests and Biospecimens/Biomarkers	Gene mutation detected digenic result specify	C18873
Participant/Subject History and Family History; General Health History	Body system category	C00312
Participant/Subject History and Family History; General Health History	Body system category other text	C18666
Assessments and Examinations; Vital Signs and Other Body Measures	Heart rate	C01521
Participant/Subject History and Family History; General Health History	Medical history assessment indicator	C18254
Participant/Subject History and Family History; General Health History	Medical history condition end date and time	C00316
Participant/Subject History and Family History; General Health History	Medical history condition start date and time	C00317
Participant/Subject History and Family History; General Health History	Medical history condition ongoing indicator	C00319
Participant/Subject History and Family History; General Health History	Medical history taken date and time	C00314
Assessments and Examinations; Laboratory Tests and Biospecimens/Biomarkers	Mutational analysis results available indicator	C12783
Assessments and Examinations; Laboratory Tests and Biospecimens/Biomarkers	Mutational analysis performed family member indicator	C12784

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Domain; Sub-Domain	Data element	CDE ID
Assessments and Examinations; Laboratory Tests and Biospecimens/Biomarkers	Mutational analysis performed indicator	C12944
Outcomes and Endpoints; Pulmonary Function Testing/Respiratory Status	Pulmonary function complete exhalation indicator	C12310
Outcomes and Endpoints; Pulmonary Function Testing/Respiratory Status	Pulmonary function index of lung function type	C12311
Outcomes and Endpoints; Pulmonary Function Testing/Respiratory Status	Pulmonary function index of lung function best trial measurement	C12312
Outcomes and Endpoints; Pulmonary Function Testing/Respiratory Status	Pulmonary function maximal pressure trial difference indicator	C12313
Outcomes and Endpoints; Pulmonary Function Testing/Respiratory Status	Pulmonary function forced vital capacity cough exhalation indicator	C12314
Outcomes and Endpoints; Pulmonary Function Testing/Respiratory Status	Pulmonary function forced vital capacity leak indicator	C12315
Outcomes and Endpoints; Pulmonary Function Testing/Respiratory Status	Pulmonary function forced vital capacity large value range indicator	C12316
Outcomes and Endpoints; Pulmonary Function Testing/Respiratory Status	Pulmonary function forced vital capacity early termination indicator	C12317
Outcomes and Endpoints; Pulmonary Function Testing/Respiratory Status	Pulmonary function forced vital capacity peak flow indicator	C12318
Outcomes and Endpoints; Pulmonary Function Testing/Respiratory Status	Pulmonary function lung function measurement	C12516
Outcomes and Endpoints; Pulmonary Function Testing/Respiratory Status	Pulmonary function test best trial number	C18319
Outcomes and Endpoints; Pulmonary Function Testing/Respiratory Status	Pulmonary function test date and time	C11098

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Domain; Sub-Domain	Data element	CDE ID
Outcomes and Endpoints; Pulmonary Function Testing/Respiratory Status	Pulmonary function test position type	C11100
Outcomes and Endpoints; Pulmonary Function Testing/Respiratory Status	Pulmonary function test mouth apparatus type	C11101
Outcomes and Endpoints; Pulmonary Function Testing/Respiratory Status	Pulmonary function test not done reason	C12298
Outcomes and Endpoints; Pulmonary Function Testing/Respiratory Status	Pulmonary Function Test seat position type	C12301
Outcomes and Endpoints; Pulmonary Function Testing/Respiratory Status	Pulmonary function test equipment manufacturer name	C12303
Outcomes and Endpoints; Pulmonary Function Testing/Respiratory Status	Pulmonary function test equipment model name	C12304
Outcomes and Endpoints; Pulmonary Function Testing/Respiratory Status	Pulmonary function test equipment software program name	C12305
Outcomes and Endpoints; Pulmonary Function Testing/Respiratory Status	Trial number	C10171
Assessments and Examinations; Vital Signs and Other Body Measures	Vital signs date and time	C01519
Assessments and Examinations; Vital Signs and Other Body Measures	Walking ability status	C10698
Assessments and Examinations; Vital Signs and Other Body Measures	Weight measurement	C01541
Assessments and Examinations; Vital Signs and Other Body Measures	Weight unit of measure	C01581
Treatment/Intervention Data; Drugs	Medication prior or concomitant start date and time	C02016
Treatment/Intervention Data; Drugs	Medication prior or concomitant end date and time	C02008

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### General Core for all Studies:

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](#)).

### Core Instruments for all Neuromuscular Diseases:

[Manual Muscle Testing](#)

### Supplemental – Highly Recommended Duchenne Muscular Dystrophy / Becker Muscular Dystrophy Instruments:

1. [Vignos Lower Extremity Scale](#)
2. [Brooke Upper Extremity Scale](#)
3. [Time to rise from supine](#)
4. [Time to climb 4 standard stairs](#)
5. [Time to walk/run 30 feet/10 meters](#)
6. [Time to rise from a chair/ time to rise from a chair](#)

For the complete list of NINDS CDE recommendations for DMD/BMD, please see the [NINDS CDE website](#).