Start-up Resource –NINDS Neuromuscular Diseases 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 Neuromuscular Diseases was developed in 2011. The Core data elements to be used by an investigator when beginning a research study in this disease/disorder are listed 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.

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

Core CDEs for all NINDS Studies[1]:

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

[1] Note: Education year count C00015 is no longer a general Core CDE

Core CDEs for Neuromuscular Disease Studies:

Domain; Sub-Domain	Data element	CDE ID
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 text	C00322
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	C18769
Disease/Injury Related Events; History of Disease/Injury Event	Medical history taken date and time	C00314
Disease/Injury Related Events; History of Disease/Injury Event	Diagnosis first given date and time	C08007
Disease/Injury Related Events; History of Disease/Injury Event	Spectroscopy water reference data acquire indicator	C12613
Participant/Subject Characteristics; Social Status	Education level USA type	C00012
Disease/Injury Related Events; History of Disease/Injury Event	Clinical trial previous participation indicator	C18253

Supplemental – Highly Recommended CDEs for Neuromuscular Disease Studies:

Domain; Sub-Domain	Data element	CDE ID
Assessments and Examinations; Laboratory Tests and Biospecimens/Biomarkers	Biopsy amyloid present on Congo red stain indicator	C12236
Assessments and Examinations; Laboratory Tests and Biospecimens/Biomarkers	Biopsy and autopsy fiber abnormality type	C12525
Assessments and Examinations; Laboratory Tests and Biospecimens/Biomarkers	Biopsy and autopsy type 1 predominance fiber percentage value	C12526
Assessments and Examinations; Laboratory Tests and Biospecimens/Biomarkers	Biopsy and autopsy type 2 predominance fiber percentage value	C12527
Assessments and Examinations; Laboratory Tests and Biospecimens/Biomarkers	Biopsy and autopsy histochemical stains diagnostic abnormalities present type	C12249
Assessments and Examinations; Laboratory Tests and Biospecimens/Biomarkers	Biospecimen fragment collect count	C12277
Assessments and Examinations; Non-Imaging Diagnostics	ECG atrial arrhythmia type	C04500
Assessments and Examinations; Non-Imaging Diagnostics	ECG assessment date and time	C04501
Assessments and Examinations; Non-Imaging Diagnostics	ECG global result type	C04502
Assessments and Examinations; Non-Imaging Diagnostics	ECG heart rate	C04503
Assessments and Examinations; Non-Imaging Diagnostics	ECG heart rhythm result type	C04504
Assessments and Examinations; Non-Imaging Diagnostics	ECG left ventricular hypertrophy indicator	C04505

Domain; Sub-Domain	Data element	CDE ID
Assessments and Examinations; Non-Imaging Diagnostics	ECG right ventricular hypertrophy indicator	C04506
Assessments and Examinations; Non-Imaging Diagnostics	ECG previous myocardial infarction indicator	C04508
Assessments and Examinations; Non-Imaging Diagnostics	ECG QT interval	C04511
Assessments and Examinations; Non-Imaging Diagnostics	ECG QTc interval	C04512
Assessments and Examinations; Non-Imaging Diagnostics	ECG ST segment abnormality indicator	C04513
Assessments and Examinations; Non-Imaging Diagnostics	ECG T wave abnormality indicator	C04514
Assessments and Examinations; Non-Imaging Diagnostics	ECG ventricular arrhythmia type	C04515
Assessments and Examinations; Non-Imaging Diagnostics	ECG atrial arrhythmia other text	C18776
Assessments and Examinations; Non-Imaging Diagnostics	ECG complete bundle branch block indicator	C04507
Assessments and Examinations; Non-Imaging Diagnostics	ECG heart rhythm result other text	C18777
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
Assessments and Examinations; Laboratory Tests and Biospecimens/Biomarkers	Mutational analysis performed family member indicator	C12784
Assessments and Examinations; Laboratory Tests and Biospecimens/Biomarkers	Mutational analysis performed indicator	C12944

Domain; Sub-Domain	Data element	CDE ID
Assessments and Examinations; Laboratory Tests and Biospecimens/Biomarkers	Mutational analysis results available indicator	C12783
Outcomes and End Points; Pulmonary Function Testing/Respiratory Status	Pulmonary function test date and time	C11098
Outcomes and End Points; Pulmonary Function Testing/Respiratory Status	Pulmonary function test position type	C11100
Outcomes and End Points; Pulmonary Function Testing/Respiratory Status	Pulmonary function test mouth apparatus type	C11101
Outcomes and End Points; Pulmonary Function Testing/Respiratory Status	Pulmonary function test not done reason	C12298
Outcomes and End Points; Pulmonary Function Testing/Respiratory Status	Pulmonary Function Test seat position type	C12301
Outcomes and End Points; Pulmonary Function Testing/Respiratory Status	Pulmonary function test equipment manufacturer name	C12303
Outcomes and End Points; Pulmonary Function Testing/Respiratory Status	Pulmonary function test equipment model name	C12304
Outcomes and End Points; Pulmonary Function Testing/Respiratory Status	Pulmonary function test equipment software program name	C12305
Outcomes and End Points; Pulmonary Function Testing/Respiratory Status	Pulmonary function complete exhalation indicator	C12310

Domain; Sub-Domain	Data element	CDE ID
Outcomes and End Points; Pulmonary Function Testing/Respiratory Status	Pulmonary function index of lung function type	C12311
Outcomes and End Points; Pulmonary Function Testing/Respiratory Status	Pulmonary function index of lung function best trial measurement	C12312
Outcomes and End Points; Pulmonary Function Testing/Respiratory Status	Pulmonary function maximal pressure trial difference indicator	C12313
Outcomes and End Points; Pulmonary Function Testing/Respiratory Status	Pulmonary function forced vital capacity cough exhalation indicator	C12314
Outcomes and End Points; Pulmonary Function Testing/Respiratory Status	Pulmonary function forced vital capacity leak indicator	C12315
Outcomes and End Points; Pulmonary Function Testing/Respiratory Status	Pulmonary function forced vital capacity large value range indicator	C12316
Outcomes and End Points; Pulmonary Function Testing/Respiratory Status	Pulmonary function forced vital capacity early termination indicator	C12317
Outcomes and End Points; Pulmonary Function Testing/Respiratory Status	Pulmonary function forced vital capacity peak flow indicator	C12318
Outcomes and End Points; Pulmonary Function Testing/Respiratory Status	Pulmonary function lung function measurement	C12516
Outcomes and End Points; Pulmonary Function Testing/Respiratory Status	Pulmonary function test not done other text	C18833

Domain; Sub-Domain	Data element	CDE ID
Assessments and Examinations; Laboratory Tests and Biospecimens/Biomarkers	Tissue specimen collection anatomic site	C12285
Assessments and Examinations; Laboratory Tests and Biospecimens/Biomarkers	Tissue specimen section thickness measurement	C12286
Assessments and Examinations; Laboratory Tests and Biospecimens/Biomarkers	Tissue specimen collection anatomic site other text	C18830
Assessments and Examinations; Laboratory Tests and Biospecimens/Biomarkers	Tissue specimen collection date and time	C12230
Assessments and Examinations; Laboratory Tests and Biospecimens/Biomarkers	Tissue specimen section count	C12287
Assessments and Examinations; Laboratory Tests and Biospecimens/Biomarkers	Tissue specimen size measurement	C12229
Assessments and Examinations; Laboratory Tests and Biospecimens/Biomarkers	Histochemical stains used type	C12235
Assessments and Examinations; Laboratory Tests and Biospecimens/Biomarkers	Histochemical stains used other text	C18828
Assessments and Examinations; Laboratory Tests and Biospecimens/Biomarkers	Lab specimen collection date and time	C01701
Assessments and Examinations; Laboratory Tests and Biospecimens/Biomarkers	Lab test abnormality significance type	C01707

Domain; Sub-Domain	Data element	CDE ID
Assessments and Examinations; Laboratory Tests and Biospecimens/Biomarkers	Lab test name	C01705
Assessments and Examinations; Laboratory Tests and Biospecimens/Biomarkers	Lab test result value	C01706
Assessments and Examinations; Laboratory Tests and Biospecimens/Biomarkers	Lab test other text	C18731
Assessments and Examinations; Laboratory Tests and Biospecimens/Biomarkers	Lab test result status	C01709
Assessments and Examinations; Laboratory Tests and Biospecimens/Biomarkers	Lab test result unit of measure	C01711
Assessments and Examinations; Laboratory Tests and Biospecimens/Biomarkers	Specimen histochemical stain diagnostic abnormality type	C12278
Assessments and Examinations; Laboratory Tests and Biospecimens/Biomarkers	Specimen source type	C12226
Outcomes and End Points; Pulmonary Function Testing/Respiratory Status	Trial number	C10171

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 Neuromuscular Disease Studies:

Manual Muscle Testing

For the complete list of NINDS CDE recommendations for NMD, please see the NINDS CDE website.