Spinal Muscular Atrophy CDE Revision History Document

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December 2024 Revisions

The Notices of Copyright were updated on the SMA Data Standards webpage in the following Assessments and Examinations; Physical/Neurological Examination Domain; Subdomains:

- Outcomes and Endpoints; Functional Outcomes
    - Gross Motor Function Measure (GMFM-88, GMFM-66)

September 2024 Revisions

The Pediatric Evaluation of Disability Inventory (PEDI) Notice of Copyright was added to the Outcomes and Endpoints; Pediatric Domain; Subdomain on the SMA Data Standards webpage.

The Notices of Copyrights were updated on the SMA Data Standards webpage in the following Outcome and Endpoints Domain, Subdomains:

- Outcomes and Endpoints; Functional Outcomes Domain; Subdomain
    - 10 Meter Timed Walk
    - 6 Minute Walk Test
    - North Star Ambulatory Assessment (NSAA)
    - Purdue Pegboard
    - Timed Up and Go (TUG)
- Outcomes and Endpoints; Muscle Strength Testing Domain; Subdomain
    - Pinch Strength
    - Manual Muscle Testing-Using the Medical Research Council Muscle Grading Scale
    - Maximum Voluntary Isometric Contraction (MVIC)
- Outcomes and Endpoints; Pediatric Domain; Subdomain
    - Pediatric Evaluation of Disability Inventory (PEDI)
    - Bayley Scales of Infant Development (Bayley III, BSID)
- Outcomes and Endpoints; Quality of Life/Patient Reported Outcomes Domain; Subdomain
    - Canadian Occupational Performance Measure (COPM)
    - Nine Hole Peg Test
    - Pediatric Quality of Life Inventory (PedsQL)
    - Short Form 36-Item Health Survey (SF-36)

The SMA Highlight Summary Document was updated to include the Pediatric Evaluation of Disability Inventory (PEDI) Notice of Copyright in the Outcomes and Endpoints; Pediatric Domain; Subdomain classified as Supplemental

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January 2020 Revisions

- General Core CRF updated to replace 'Gender' question with ‘Sex assigned at birth' and 'Gender identity’.
- Two new CDEs added to CRF: C58676 (Sex assigned at birth) and C58677 (Gender identity). C00035 has been removed
- Start-Up document updated to reflect these changes.

April 2015 Revisions

No changes.

March 2015 Revisions

- Classification changes to CDEs in SMA per feedback from NMD Oversight Committee.

February 2015 Revisions

No changes.

January 2015 Revisions

- Posting of revised 508 compliant Start-Up and Highlight Summaries.
- Addition of Dr. Glen Nuckolls and removal of Dr. John Porter to the SMA Acknowledgments page.

November 2014 Revisions

No changes.

September 2014 Revisions

No changes.

August 2014 Revisions

No changes.

July 2014 Revisions

No changes.

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June 2014 Revisions

No changes.

May 2014 Revisions

No changes.

April 2014 Revisions

No changes.

March 2014 Revisions

No changes.

February 2014 Revisions

No changes.

January 2014 Revisions

Overview

There have been major changes to the structure and content of the Spinal Muscular Atrophy CDE materials in version 1.1 compared to version 1.0, including the following:

Form Status Changes:

New Forms:

- 10 Minute Walk Test
- 6 Minute Walk Test
- Grip Strength
- North Star Ambulatory Assessment (NSAA)
- Pinch Strength
- Purdue Pegboard
- Timed Up and Go (TUG)
- Manual Muscle Testing (MMT)-Using the Medical Research Council (MRC) Grading Scale
- Maximum Voluntary Isometric Contraction Testing (MVICT)
- Short Form-36 Health Survey (SF-36)

- Canadian Occupational Performance Measure (COPM)
- Nine Hole Peg Test
- Pediatric Quality of Life Inventory (PEDSQOL)

Updated Forms:

- None

Removed Forms:

- None

Detailed Form Revisions:

Additional specific changes to the CRF modules are detailed in the table included on the subsequent page.

1 Spinal Muscular Atrophy CDE Revision History Table

CRF Module/ Guideline	Current Version	Previous Version	Change Classification	Change Type	Description of Change
10 Minute Walk Test	2.0	1.0	Major	Notice of Copyright	A Notice of Copyright was created for this form and will replace the existing form
6 Minute Walk Test	2.0	1.0	Major	Notice of Copyright	A Notice of Copyright was created for this form and will replace the existing form
Grip Strength	2.0	1.0	Major	Notice of Copyright	A Notice of Copyright was created for this form and will replace the existing form

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CRF Module/ Guideline	Current Version	Previous Version	Change Classification	Change Type	Description of Change
North Star Ambulatory Assessment (NSAA)	2.0	1.0	Major	Notice of Copyright	A Notice of Copyright was created for this form and will replace the existing form
Pinch Strength	2.0	1.0	Major	Notice of Copyright	A Notice of Copyright was created for this form and will replace the existing form
Purdue Pegboard	2.0	1.0	Major	Notice of Copyright	A Notice of Copyright was created for this form and will replace the existing form
Timed Up and Go (TUG)	2.0	1.0	Major	Notice of Copyright	A Notice of Copyright was created for this form and will replace the existing form
Manual Muscle Testing (MMT)-Using the Medical Research Council (MRC) Grading Scale	2.0	1.0	Major	Notice of Copyright	A Notice of Copyright was created for this form and will replace the existing form
Maximum Voluntary Isometric Contraction Testing (MVICT)	2.0	1.0	Major	Notice of Copyright	A Notice of Copyright was created for this form and will replace the existing form

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CRF Module/ Guideline	Current Version	Previous Version	Change Classification	Change Type	Description of Change
Short Form-36 Health Survey (SF-36)	2.0	1.0	Major	Notice of Copyright	A Notice of Copyright was created for this form and will replace the existing form
Canadian Occupational Performance Measure (COPM)	2.0	1.0	Major	Notice of Copyright	A Notice of Copyright was created for this form and will replace the existing form
Nine Hole Peg Test	2.0	1.0	Major	Notice of Copyright	A Notice of Copyright was created for this form and will replace the existing form
Pediatric Quality of Life Inventory (PEDSQOL)	2.0	1.0	Major	Notice of Copyright	A Notice of Copyright was created for this form and will replace the existing form

September 2013 Revisions

Overview

The Spinal Muscular Atrophy (SMA) CDE Materials were previously listed under the outline for Neuromuscular Disease on the NINDS website. The NINDS website has recently expanded to support 4 new diseases and the Spinal Muscular Atrophy CDE Materials have been removed from the Neuromuscular Disease outline to create a new disease area featuring these materials. The materials listed in this disease area will be considered version 1.0.

A Start-up Resource Listing was added to the CMD disease area to provide investigators with a set of CORE data elements to be used during a research study.

Spinal Muscular Atrophy CDE Revision History Document

Form Status Changes:

New Forms:

None

Updated Forms:

- Demographics
- Social Status
- Family History-SMA
- Medical History-SMA
- Prenatal and Perinatal History-SMA
- Surgical and Hospitalization History-SMA
- Medical History of SMA
- Motor Milestones and Current Level of Function – SMA
- Physical Examination
- Vital Signs
- Biomarkers Sample Data Guidelines
- Biospecimen Collection Guidelines for Use
- Fat Aspirate Tissue Specimens
- Gene Table and Guidelines for Use
- Laboratory Tests and Tracking
- Laboratory Tests Recommendations Table and Guidelines for Use
- Muscle Biopsy and Autopsy Tissue
- Mutation Analysis
- Nerve Biopsy
- NINDS Repository Spinal Muscular Atrophy Data Elements
- Skin Biopsies for Qualification of Intraepidermal Nerve Fibers

- Brain Magnetic Resonance Imaging (MRI)
- Cardiac Magnetic Resonance Imaging (MRI)
- Diffusion Tensor Imaging (DTI)
- Dual-Energy X-Ray Absorptiometry (DEXA)
- Magnetic Resonance Imaging (MRI)
- Magnetic Resonance Spectroscopy (MRS)
- Echocardiogram
- Electrocardiogram (ECG)
- Electrophysiology
- Holter Examination
- External Devices - SMA
- Gastrointestinal Therapies
- Respiratory Interventions
- SMA Adult Clinical Outcomes Recommendations by Domain and Classification - SMA
- Cognitive Guidelines for Use
- Measures of Gas Exchange
- Pulmonary Assessment Master Outline and Guidelines for Use
- Pulmonary Function Testing
- Pediatric Cognitive Instrument Recommendations by Domain and Classification
- Pediatric Cognitive Instrument Table
- SMA Pediatric Clinical Outcomes Recommendations by Domain and Classification - SMA
- Death Report

Removed Forms:

- NMD Clinical Outcome Recommendations by Domain and Classification

Spinal Muscular Atrophy CDE Revision History Document

Detailed Form Revisions:

Additional specific changes to the CRF modules are detailed in the table included on the subsequent page

2 Spinal Muscular Atrophy CDE Revision History Table

CRF Module/ Guideline	Current Version	Previous Version	Change Classification	Change Type	Description of Change
Demographics	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy
Social Status	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy
Family History-SMA	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy
Medical History-SMA	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy
Prenatal and Perinatal History-SMA	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy

Spinal Muscular Atrophy CDE Revision History Document

CRF Module/ Guideline	Current Version	Previous Version	Change Classification	Change Type	Description of Change
Surgical and Hospitalization History-SMA	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy
Medical History of SMA	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy
Motor Milestones and Current Level of Function – SMA	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy
Physical Examination	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy
Vital Signs	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy
Biomarkers Sample Data Guidelines	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy

Spinal Muscular Atrophy CDE Revision History Document

CRF Module/ Guideline	Current Version	Previous Version	Change Classification	Change Type	Description of Change
Biospecimen Collection Guidelines for Use	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy
Fat Aspirate Tissue Specimens	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy
Gene Table and Guidelines for Use	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy
Laboratory Tests and Tracking	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy
Laboratory Tests Recommendations Table and Guidelines for Use	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy
Muscle Biopsy and Autopsy Tissue	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy

Spinal Muscular Atrophy CDE Revision History Document

CRF Module/ Guideline	Current Version	Previous Version	Change Classification	Change Type	Description of Change
Mutation Analysis	1.0	N/A	Major	Modified Forms	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy
Nerve Biopsy	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy
NINDS Repository Spinal Muscular Atrophy Data Elements	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy
Skin Biopsies for Qualification of Intraepidermal Nerve Fibers	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy
Brain Magnetic Resonance Imaging (MRI)	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy
Cardiac Magnetic Resonance Imaging (MRI)	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy

Spinal Muscular Atrophy CDE Revision History Document

CRF Module/ Guideline	Current Version	Previous Version	Change Classification	Change Type	Description of Change
Diffusion Tensor Imaging (DTI)	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy
Dual-Energy X-Ray Absorptiometry (DEXA)	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy
Magnetic Resonance Imaging (MRI)	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy
Magnetic Resonance Spectroscopy (MRS)	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy
Echocardiogram	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy
Electrocardiogram (ECG)	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy

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CRF Module/ Guideline	Current Version	Previous Version	Change Classification	Change Type	Description of Change
Electrophysiology	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy
Holter Examination	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy
External Devices - SMA	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy
Gastrointestinal Therapies	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy
Respiratory Interventions	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy
SMA Adult Clinical Outcomes Recommendations by Domain and Classification - SMA	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy

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CRF Module/ Guideline	Current Version	Previous Version	Change Classification	Change Type	Description of Change
Cognitive Guidelines for Use	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy
Measures of Gas Exchange	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy
Pulmonary Assessment Master Outline and Guidelines for Use	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy
Pulmonary Function Testing	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy
Pediatric Cognitive Instrument Recommendations by Domain and Classification	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy
Pediatric Cognitive Instrument Table	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy

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CRF Module/ Guideline	Current Version	Previous Version	Change Classification	Change Type	Description of Change
SMA Pediatric Clinical Outcomes Recommendations by Domain and Classification - SMA	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy
Death Report	1.0	N/A	Major	Modified Form	Forms were moved from Neuromuscular Disease to a new disease area for Spinal Muscular Atrophy

January 2013 Revisions

Overview

Form Status Changes

New Forms:

- Spinal Muscular Atrophy (SMA) Coriell Form

Updated Forms:

- None

Removed Forms:

- None

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CRF Module/ Guideline	Current Version*	Previous Version	Change Classification	Change Type	Description of Change
SMA Coriell Form	1.0	N/A	Major	New Form	A copy of unique SMA CDEs were added to the Web site.