

# Spinal Muscular Atrophy CDE Revision History Document

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# Spinal Muscular Atrophy CDE Revision History Document

## 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.

# Spinal Muscular Atrophy CDE Revision History Document

## 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)

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

**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

## Spinal Muscular Atrophy CDE Revision History Document

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

## Spinal Muscular Atrophy CDE Revision History Document

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

## Spinal Muscular Atrophy CDE Revision History Document

- 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

## Spinal Muscular Atrophy CDE Revision History Document

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

## Spinal Muscular Atrophy CDE Revision History Document

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

## Spinal Muscular Atrophy CDE Revision History Document

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.