**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 [http://www.cssrs.columbia.edu](http://www.cssrs.columbia.edu))

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**Disease/Domain** | **Recommendations**
---|---
Congenital Muscular Dystrophy | These instruments and elements are recommended for use in CMD studies:
| Core elements: | See Start-Up Resources Listing document ([CMD Start-Up Resource Listing](#))
| Supplemental – Highly Recommended instruments: | 6 Minute Walk, Bayley Scales of Infant Development\(^i\), Egen Klassifikation Scale Version 2 (EK2)\(^ii\), Modified Hammersmith Functional Motor Scale for Children with Spinal Muscular Atrophy (MHFMS-SMA/MHFMS-Extend)\(^iii\), North Star Ambulatory Assessment (NSAA)\(^iv\), Manual Muscle Testing-Using the Medical Research Council Muscle Grading Scale, Wechsler Abbreviated Scale of Intelligence (WASI)\(^v\), Wechsler Intelligence Scale for Children-IV (WISC-IV)\(^vi\), Wechsler Preschool and Primary Scale of Intelligence (WPPSI-III), Quality of Life in Neurological Disorders (Neuro-QOL), Patient-Reported Outcomes Measurement Information System (PROMIS)

Participant / Subject Characteristics; Demographics | Core: Demographics\(^vii\), General Core
| Supplemental – Highly Recommended: | None
| Supplemental: | Demographics
| Exploratory: | None

| Participant / Subject Characteristics; Social Status | Core: Social Status\(^vii\)
| Supplemental – Highly Recommended: | None
| Supplemental: | Social Status
| Exploratory: | None

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# Congenital Muscular Dystrophy CDE Highlight Summary Document

<table>
<thead>
<tr>
<th>Disease/Domain</th>
<th>Recommendations</th>
</tr>
</thead>
</table>
| Participant/Subject History and Family History; General Health History | **Core**: Surgical History\(^{vii}\)  
**Supplemental – Highly Recommended**: None  
**Supplemental**: Family History, Intake Medical History, Interval Medical History, Prenatal and Perinatal History, Surgical History  
**Exploratory**: None |
| Assessments and Examinations; Imaging Diagnostics | **Core**: None  
**Supplemental – Highly Recommended**: None  
**Supplemental**: Brain Magnetic Resonance Imaging\(^{viii}\), Dual-Energy X-Ray Absorptiometry (DEXA), Muscle Imaging  
**Exploratory**: Cardiac Magnetic Resonance Imaging, Diffusion Tensor Imaging |
| Assessments and Examinations; Laboratory Tests and Biospecimens / Biomarkers | **Core**: Muscle Biopsies and Autopsy Tissue\(^{vii}\)  
**Supplemental – Highly Recommended**: None  
**Supplemental**: Muscle Biopsies and Autopsy Tissue, Peripheral Nerves – Biopsies and Autopsies, Skin Biopsies for Qualification of Intraepidermal Nerve Fibers  
**Exploratory**: None |
| Assessments and Examinations; Non-Imaging Diagnostics | **Core**: None  
**Supplemental – Highly Recommended**: None  
**Supplemental**: Electrophysiology, Short Scalp Electroencephalography (EEG)  
**Exploratory**: Echocardiogram |
| Assessments and Examinations; Physical/Neurological Examination | **Core**: None  
**Supplemental – Highly Recommended**: None  
**Supplemental**: None  
**Exploratory**: Electrical Impedance Myography (EIM) |
| Treatment / Intervention Data; Drugs | **Core**: Prior and Concomitant Medications\(^{vii}\)  
**Supplemental – Highly Recommended**: None  
**Supplemental**: Prior and Concomitant Medications  
**Exploratory**: None |
### Congenital Muscular Dystrophy CDE Highlight Summary Document

<table>
<thead>
<tr>
<th>Disease/Domain</th>
<th>Recommendations</th>
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</thead>
<tbody>
<tr>
<td><strong>Treatment / Intervention Data; Therapies</strong></td>
<td><strong>Core:</strong> None&lt;br&gt;<strong>Supplemental – Highly Recommended:</strong> None&lt;br&gt;<strong>Supplemental:</strong> Respiratory Interventions&lt;br&gt;<strong>Exploratory:</strong> None</td>
</tr>
<tr>
<td><strong>Outcomes and Endpoints; Functional Status</strong></td>
<td><strong>Core:</strong> None&lt;br&gt;<strong>Supplemental – Highly Recommended:</strong> 6 Minute Walk Test, Bayley Scales of Infant Development&lt;sup&gt;x&lt;/sup&gt;, Egen Klassifikation Scale Version 2 (EK2), Modified Hammersmith Functional Motor Scale for Children with Spinal Muscular Atrophy (MHFMS-SMA/MHFMS-Extend), North Star Ambulatory Assessment (NSAA)&lt;br&gt;<strong>Supplemental:</strong> Alberta Infant Motor Scale (AIMS), Brooke Upper Extremity Scale, Goniometry, Pediatric Evaluation of Disability Inventory (PEDI)&lt;sup&gt;+&lt;/sup&gt;, Stair Climb, Vignos Lower Extremity Scale&lt;br&gt;<strong>Exploratory:</strong> 10 Meter Timed Walk, 2 Minute Walk Test, Barthel Index, Gross Motor Function Measure (GMFM-88, GMFM-66), Jebsen Taylor Hand Function Test, Nine Hole Peg Test, Timed Up and Go (TUG)</td>
</tr>
<tr>
<td><strong>Outcomes and Endpoints; Muscle Strength Testing</strong></td>
<td><strong>Core:</strong> None&lt;br&gt;<strong>Supplemental – Highly Recommended:</strong> Manual Muscle Testing-Using the Medical Research Council Muscle Grading Scale&lt;br&gt;<strong>Supplemental:</strong> Grip Strength Fatigue, Hand Held Dynamometry, Maximum Voluntary Isometric Contraction Testing (MVICT), Pinch Strength&lt;br&gt;<strong>Exploratory:</strong> None</td>
</tr>
<tr>
<td><strong>Outcomes and Endpoints; Neuropsychological Testing</strong></td>
<td><strong>Core:</strong> None&lt;br&gt;<strong>Supplemental – Highly Recommended:</strong> Wechsler Abbreviated Scale of Intelligence (WASI), Wechsler Intelligence Scale for Children-IV (WISC-IV), Wechsler Preschool and Primary Scale of Intelligence (WPPSI-III)&lt;br&gt;<strong>Supplemental:</strong> Peabody Picture Vocabulary Test 4th Edition (PPVT-4), Purdue Pegboard, Wechsler Individual Achievement Test-III (WIAT-III)&lt;br&gt;<strong>Exploratory:</strong> None</td>
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<tr>
<td><strong>Outcomes and Endpoints; Performance Measures</strong></td>
<td><strong>Core:</strong> None&lt;br&gt;<strong>Supplemental – Highly Recommended:</strong> None&lt;br&gt;<strong>Supplemental:</strong> Motor Function Measure (MFM)&lt;br&gt;<strong>Exploratory:</strong> None</td>
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</table>
## Congenital Muscular Dystrophy CDE Highlight Summary Document

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<th>Disease/Domain</th>
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<tbody>
<tr>
<td>Outcomes and Endpoints; Pulmonary Function</td>
<td></td>
</tr>
<tr>
<td>Testing/Respiratory Status</td>
<td>Core: None</td>
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<tr>
<td></td>
<td><strong>Supplemental – Highly Recommended:</strong> Pulmonary Function Testing</td>
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<td></td>
<td><strong>Supplemental:</strong> Measures of Gas Exchange</td>
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<tr>
<td></td>
<td><strong>Exploratory:</strong> None</td>
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<tr>
<td>Outcomes and Endpoints; Quality of Life</td>
<td></td>
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<tr>
<td></td>
<td>Core: None</td>
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<tr>
<td></td>
<td><strong>Supplemental – Highly Recommended:</strong> Quality of Life in Neurological Disorders (Neuro-QOL), Patient-Reported Outcomes Measurement Information System (PROMIS)</td>
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<tr>
<td></td>
<td><strong>Supplemental:</strong> None</td>
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<tr>
<td></td>
<td><strong>Exploratory:</strong> None</td>
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</tbody>
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1. Supplemental – highly recommended for developmental, psychological, and neuropsychological studies of infants and toddlers up to 42 months old. Highly recommended as a means of characterizing study participants.
2. Specifically for studies involving adolescents and adults with CMD.
3. Supplemental - highly recommended for studies analyzing motor function - age limit 2+
4. For studies with ambulatory CMD patients.
5. Supplemental - highly recommended for psychological and neuropsychological CMD studies for ages 6 years and up; Recommended for other types of CMD studies as a way to characterize the study population.
6. Supplemental – highly recommended for psychological and neuropsychological studies for ages 6 to 16 years; Recommended for other types of studies as a way to characterize the study population.
7. Contains some Core CDEs.
8. The elements on this CRF are Supplemental - highly recommended for dystroglycanopathies; Supplemental for MDC1A; and Exploratory for all other congenital muscular dystrophies.
9. Supplemental - highly recommended for developmental, psychological, and neuropsychological studies of infants and toddlers up to 42 months old. Supplemental - highly recommended as a means of characterizing study participants.
10. Particularly appropriate in assessing functional capabilities in CMD children in terms of both current status and change over time.