Friedreich’s Ataxia (FA) CDE Highlight Summary

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 Columbia Suicide Severity Rating Scale Website).

<table>
<thead>
<tr>
<th>Domain; Sub-Domain</th>
<th>Recommendations</th>
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</thead>
<tbody>
<tr>
<td><strong>Friedreich’s Ataxia</strong></td>
<td>These instruments and elements are recommended for use in all FA studies:</td>
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<tr>
<td><strong>Core instruments:</strong></td>
<td>Friedreich Ataxia Rating Scale (FARS), International Cooperative Ataxia Rating Scale (ICARS), Scale for the Assessment and Rating of Ataxia (SARA), Low Contrast Letter Acuity, Timed 25-Foot Walk, 9-Hole Pegboard</td>
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<tr>
<td><strong>Core elements:</strong></td>
<td>Friedreich’s Ataxia Start-Up Resource Listing</td>
</tr>
<tr>
<td><strong>Exploratory:</strong></td>
<td>None</td>
</tr>
<tr>
<td><strong>Participant Characteristics; Demographics</strong></td>
<td>Core: Demographics, General Core</td>
</tr>
<tr>
<td><strong>Supplemental:</strong></td>
<td>Demographics, General Core</td>
</tr>
<tr>
<td><strong>Exploratory:</strong></td>
<td>Demographics, General Core</td>
</tr>
<tr>
<td><strong>Participant Characteristics; Social Status</strong></td>
<td>Core: Social Status</td>
</tr>
<tr>
<td><strong>Supplemental:</strong></td>
<td>Social Status</td>
</tr>
<tr>
<td><strong>Exploratory:</strong></td>
<td>Social Status</td>
</tr>
<tr>
<td><strong>Participant History and Family History; General Health History</strong></td>
<td>Core: Behavioral History, History Data Source Reliability, Medical History</td>
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<tr>
<td><strong>Supplemental:</strong></td>
<td>Behavioral History, Family History, History Data Source Reliability, Medical History, Pediatric Developmental History, Pregnancy History, Prenatal and Perinatal History</td>
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<tr>
<td><strong>Exploratory:</strong></td>
<td>History Data Source Reliability, Pediatric Developmental History, Prenatal and Perinatal History</td>
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<td>Recommendations</td>
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| Disease/Injury Related Events; History of Disease/Injury Event | Core: Medical History of Friedreich’s Ataxia<sup>ix</sup>  
Supplemental: Medical History of Friedreich’s Ataxia  
Exploratory: None |
| Assessments and Examinations; Physical/Neurological Examination | Core: Physical Examination<sup>x</sup>  
Supplemental: Physical Examination  
Exploratory: None |
| Assessments and Examinations; Vital Signs and Other Body Measures | Core: Vital Signs<sup>xi</sup>  
Supplemental: Vital Signs  
Exploratory: None |
| Assessments and Examinations; Laboratory Tests and Biospecimens/Biomarkers | Core: Laboratory Tests<sup>xii</sup>  
Supplemental: Laboratory Tests  
Exploratory: None  
**Guideline Documents:** Plasma Collection Guidelines, Urine Collection Guidelines |
| Assessments and Examinations; Imaging Diagnostics | Core: Cardiac and Magnetic Resonance Imaging<sup>xiii</sup>  
Supplemental: Cardiac and Magnetic Resonance Imaging  
Exploratory: None |
| Assessments and Examinations; Non-Imaging Diagnostics | Core: Echocardiogram, Electrocardiogram, Holter Exam  
Supplemental: None  
Exploratory: None |
| Treatment/Intervention Data; Drugs | Core: Prior and Concomitant Medications<sup>xiv</sup>  
Supplemental: Prior and Concomitant Medications  
Exploratory: None |
| Outcomes and Endpoints; Summary of All Outcome Recommendations | Core: None  
Supplemental: None  
Exploratory: None  
**Recommendations:** FA CDE Instrument Recommendations by Outcome Domain and Classification |
<table>
<thead>
<tr>
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<th>Recommendations</th>
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| Outcomes and Endpoints; Activities of Daily Living / Performance | **Core:** Activities of Daily Living and Gait<sup>xv</sup>  
**Supplemental:** Activities of Daily Living and Gait, PaTaKa Speech Test, PROMIS, NIH Toolbox  
**Exploratory:** Acoustic Analysis of Speech, Barthel Index, Functional Independence Measure, Jebsen-Taylor Hand Functioning Test, Stride Analysis and Gait Variability |
| Outcomes and Endpoints; Ataxia and Performance Measures | **Core:** Friedreich's Ataxia Rating Scale (FARS), International Cooperative Rating Scale (ICARS), Nine Hole Peg Test, Scale for the Assessment and Rating of Ataxia (SARA), Sloan Low Contrast Letter Acuity, Timed 25-Foot Walk (T25FW)  
**Supplemental:** Assessment of Intelligibility of Dysarthic Speech (AIDS), Boston Diagnostic Aphasia Exam (BDAE-III), Friedreich's Ataxia Impact Scale (FAIS), Modified Fatigue Impact Scale (MFIS), Tardieu Scale  
**Exploratory:** Bladder Control Scale (BLCS), Bowel Control Scale (BWCS), Delis Kaplan Executive Function System (DKEFS), Impact of Visual Impairment Scale (IVIS), MOS Pain Effects Scale (PES), Phonemic Verbal Fluency (PVF) |
| Outcomes and Endpoints; Quality of life | **Core:** None  
**Supplemental:** Pediatric Quality of Life Inventory (PEDSQL), 36-Item Short Form Health Survey (SF-36), Short Form Health Survey 10 for Children (SF-10), Quality of Life in Neurological Disorders (Neuro-QOL)  
**Exploratory:** None |
| Outcomes and Endpoints; Clinical Event End Points | **Core:** Cardiac End Points  
**Supplemental:** None  
**Exploratory:** None |

<sup>1</sup>CRF has Core, Supplemental, and Exploratory elements  
<sup>2</sup>CRF has Core, Supplemental, and Exploratory elements  
<sup>3</sup>CRF has Core, Supplemental, and Exploratory elements  
<sup>4</sup>CRF has Core and Supplemental elements  
<sup>5</sup>CRF has Core, Supplemental, and Exploratory elements  
<sup>6</sup>CRF has Core, Supplemental, and Exploratory elements  
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<sup>13</sup>CRF has Core and Supplemental elements  
<sup>14</sup>CRF has Core and Supplemental elements  
<sup>15</sup>CRF has Core and Supplemental elements