



## 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](#)).

Domain; Sub-Domain	Recommendations
Friedreich’s Ataxia	<p><b>These instruments and elements are recommended for use in all FA studies:</b></p> <p><b>Core instruments:</b>            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</p> <p><b>Core elements:</b> <a href="#">Friedreich's Ataxia Start-Up Resource Listing</a></p> <p><b>Exploratory:</b> None</p>
Participant Characteristics; Demographics	<p><b>Core:</b> Demographics<sup>i</sup>, General Core<sup>ii</sup></p> <p><b>Supplemental:</b> Demographics, General Core</p> <p><b>Exploratory:</b> Demographics, General Core</p>
Participant Characteristics; Social Status	<p><b>Core:</b> Social Status<sup>iii</sup></p> <p><b>Supplemental:</b> Social Status</p> <p><b>Exploratory:</b> Social Status</p>
Participant History and Family History; General Health History	<p><b>Core:</b> Behavioral History<sup>iv</sup>, History Data Source Reliability<sup>v</sup>, Medical History<sup>vi</sup></p> <p><b>Supplemental:</b> Behavioral History, Family History, History Data Source Reliability, Medical History, Pediatric Developmental History<sup>vii</sup>, Pregnancy History, Prenatal and Perinatal History<sup>viii</sup></p> <p><b>Exploratory:</b> History Data Source Reliability, Pediatric Developmental History, Prenatal and Perinatal History</p>



Domain; Sub-Domain	Recommendations
<b>Disease/Injury Related Events; History of Disease/Injury Event</b>	<p><b>Core:</b> Medical History of Friedreich's Ataxia<sup>ix</sup></p> <p><b>Supplemental:</b> Medical History of Friedreich's Ataxia</p> <p><b>Exploratory:</b> None</p>
<b>Assessments and Examinations; Physical/ Neurological Examination</b>	<p><b>Core:</b> Physical Examination<sup>x</sup></p> <p><b>Supplemental:</b> Physical Examination</p> <p><b>Exploratory:</b> None</p>
<b>Assessments and Examinations; Vital Signs and Other Body Measures</b>	<p><b>Core:</b> Vital Signs<sup>xi</sup></p> <p><b>Supplemental:</b> Vital Signs</p> <p><b>Exploratory:</b> None</p>
<b>Assessments and Examinations; Laboratory Tests and Biospecimens/ Biomarkers</b>	<p><b>Core:</b> Laboratory Tests<sup>xii</sup></p> <p><b>Supplemental:</b> Laboratory Tests</p> <p><b>Exploratory:</b> None</p> <p><b>Guideline Documents:</b> Plasma Collection Guidelines, Urine Collection Guidelines</p>
<b>Assessments and Examinations; Imaging Diagnostics</b>	<p><b>Core:</b> Cardiac and Magnetic Resonance Imaging<sup>xiii</sup></p> <p><b>Supplemental:</b> Cardiac and Magnetic Resonance Imaging</p> <p><b>Exploratory:</b> None</p>
<b>Assessments and Examinations; Non-Imaging Diagnostics</b>	<p><b>Core:</b> Echocardiogram, Electrocardiogram, Holter Exam</p> <p><b>Supplemental:</b> None</p> <p><b>Exploratory:</b> None</p>
<b>Treatment/Intervention Data; Drugs</b>	<p><b>Core:</b> Prior and Concomitant Medications<sup>xiv</sup></p> <p><b>Supplemental:</b> Prior and Concomitant Medications</p> <p><b>Exploratory:</b> None</p>
<b>Outcomes and Endpoints; Summary of All Outcome Recommendations</b>	<p><b>Core:</b> None</p> <p><b>Supplemental:</b> None</p> <p><b>Exploratory:</b> None</p> <p><b>Recommendations:</b> FA CDE Instrument Recommendations by Outcome Domain and Classification</p>



Domain; Sub-Domain	Recommendations
<b>Outcomes and Endpoints; Activities of Daily Living / Performance</b>	<p><b>Core:</b> Activities of Daily Living and Gait<sup>xv</sup></p> <p><b>Supplemental:</b> Activities of Daily Living and Gait, PaTaKa Speech Test, PROMIS, NIH Toolbox</p> <p><b>Exploratory:</b> Acoustic Analysis of Speech, Barthel Index, Functional Independence Measure, Jebsen-Taylor Hand Functioning Test, Stride Analysis and Gait Variability</p>
<b>Outcomes and Endpoints; Ataxia and Performance Measures</b>	<p><b>Core:</b> 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)</p> <p><b>Supplemental:</b> Assessment of Intelligibility of Dysarthric Speech (AIDS), Boston Diagnostic Aphasia Exam (BDAE-III), Friedreich's Ataxia Impact Scale (FAIS), Modified Fatigue Impact Scale (MFIS), Tardieu Scale</p> <p><b>Exploratory:</b> 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)</p>
<b>Outcomes and Endpoints; Quality of life</b>	<p><b>Core:</b> None</p> <p><b>Supplemental:</b> Pediatric Quality of Life Inventory (PEDIQL), 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)</p> <p><b>Exploratory:</b> None</p>
<b>Outcomes and Endpoints; Clinical Event End Points</b>	<p><b>Core:</b> Cardiac End Points</p> <p><b>Supplemental:</b> None</p> <p><b>Exploratory:</b> None</p>

<sup>i</sup> CRF has Core, Supplemental, and Exploratory elements

<sup>ii</sup> CRF has Core, Supplemental, and Exploratory elements

<sup>iii</sup> CRF has Core, Supplemental, and Exploratory elements

<sup>iv</sup> CRF has Core and Supplemental elements

<sup>v</sup> CRF has Core, Supplemental, and Exploratory elements

<sup>vi</sup> CRF has Core and Supplemental elements

<sup>vii</sup> CRF has Supplemental and Exploratory elements

<sup>viii</sup> CRF has Supplemental and Exploratory elements

<sup>ix</sup> CRF has Core and Supplemental elements

<sup>x</sup> CRF has Core and Supplemental elements

<sup>xi</sup> CRF has Core and Supplemental elements

<sup>xii</sup> CRF has Core and Supplemental elements

<sup>xiii</sup> CRF has Core and Supplemental elements

<sup>xiv</sup> CRF has Core and Supplemental elements

<sup>xv</sup> CRF has Core and Supplemental elements