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Request for Proposals:
Blood-Based Biomarkers for Friedreich Ataxia

Letters of intent (LOI) application deadline: May 1, 2026

Background and Rationale

FARA seeks to support a precompetitive effort to identify and validate blood-based protein biomarkers that can accelerate decision-making during therapeutic development and support regulatory approval of new therapeutics for Friedreich ataxia (FA). Biomarkers that provide early evidence of therapeutic impact are a high priority for drug developers and can help determine go/no-go decisions earlier in therapy development. Despite published reports of differentially expressed plasma proteins in people with FA compared with unaffected individuals, no systematic study has yet identified and validated robust blood-based protein biomarkers suitable for clinical-trial use.

For drug developers, the most impactful biomarkers will be those that track disease progression and / or response to therapeutic effects (i.e., monitoring biomarkers). Frataxin is an excellent biomarker when a therapy is expected to increase frataxin levels; however, monitoring biological processes downstream of frataxin is also essential. Relevant FA biology includes inflammation and neuroinflammation (with activated microglia and astrocytes observed in human postmortem tissue and inflammatory markers detectable in CSF), neurodegeneration (noting that plasma neurofilament light chain [NfL] in FA is typically elevated only in children and young adults and declines by ~40 years of age), ferroptosis, mitochondrial dysfunction, and broader metabolic dysregulation. Biomarkers that reflect these (and other FA-specific) processes and change longitudinally in alignment with disease status or treatment are likely to be informative.

Biomarker Characteristics Required

- Relate directly to the underlying biology of FA, reflecting pathological processes known to contribute to disease progression.

- Be downstream of frataxin, capturing biological consequences of frataxin deficiency to ensure relevance across diverse therapeutic mechanisms (including those not expected to increase frataxin levels).
- Enable monitoring of both treatment response and disease progression, providing measurable, longitudinal changes that align with therapeutic effects or the natural history of disease.

Scope

FARA will accept proposals that aim to (1) identify new blood-based biomarkers for FA or (2) validate putative biomarkers with a robust, evidence-supported connection to FA pathophysiology. Validation studies must include longitudinal data. The proposed development path should not be encumbered by intellectual property restrictions that might impede Biomarker development for clinical trial applications.

Proposals with targeted and/or exploratory discovery approaches will be considered. Untargeted discovery approaches are acceptable, provided they include well-defined objectives tied to biomarker development and a clear plan for transitioning findings to validation using human plasma/serum samples.

Projects may include animal or cell-based data collection when such studies help elucidate disease-relevant pathways or strengthen the rationale for a targeted biomarker strategy in human samples.

Applicants are encouraged to review and leverage datasets already available to identify common signatures that inform biomarker prioritization and validation.

Applicants are strongly encouraged to use existing plasma/serum samples from previous interventional or natural history studies (see “Available Resources”).

Available Resources

- Plasma/Serum samples from FARA-supported natural history studies, including longitudinal samples.
- Associated metadata (e.g., neurological examination data and other clinical features).
- Details on access procedures will be provided upon request; applicants should indicate required sample characteristics in their proposals.

Budget and Timeline

- Project period: up to 2 years.
- Annual budget should not exceed US $250,000.

Review, Feedback, and Revision Process

Proposals will be reviewed by key opinion leaders (KOLs) in FA. Applicants may be asked to revise their plans based on expert feedback to strengthen scientific rigor, feasibility, and alignment with the goals of this initiative.

Eligibility and Submission Instructions

Eligibility criteria and requirements for Letters of Intent and Full Applications are available on the FARA website www.curefa.org . Applicants should follow the posted instructions for formatting, submission platform, and deadlines, and direct any questions to the contacts provided there. Grant application process: https://www.curefa.org/research/grant-program/grant-application-process/

Letters of intent must be submitted via the FARA grant submission portal at https://webportalapp.com/sp/login/fara_grants

Important Dates

LOI application deadline: May 1, 2026 11:59pm ET

Full application submission deadline: July 1, 2026 11:59pm ET

Award notification: October 1, 2026

Start date: November 1, 2026