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Biogen Inc Public Comment on ICER Draft Scope: Disease-Specific Treatments for Friedreich's Ataxia

Biogen Inc appreciates the opportunity to provide comments on the Institute for Clinical and Economic Review's (ICER's) Draft Scope for the assessment of disease-specific treatments for Friedreich ataxia (FA). We commend ICER for recognizing the substantial unmet need in FA, proposing use of the adapted ultra-rare disease framework and seeking to incorporate benefits beyond health that are particularly relevant for individuals living with this progressive, multisystem condition.

We respectfully offer the following recommendations to strengthen the clinical and economic assessment framework and ensure that the review reflects the current evidence base and the lived experience of patients and caregivers affected by FA.

1. Population: Anchor Subgroups to mFARS Ranges and Age of Onset

Biogen supports ICER's proposal to evaluate treatment-effect modification by age of onset, which is the strongest known predictor of disease progression in FA. Given the substantial clinical heterogeneity of FA, we strongly recommend that subgroup analyses also incorporate validated modified Friedreich Ataxia Rating Scale (mFARS) score ranges, which have provided a clinically meaningful framework for characterizing disease progression and functional burden in both the MOXIe clinical trial and the Friedreich's Ataxia Clinical Outcome Measures Study (FACOMS) natural history registry.

Analysis of 1115 participants in FACOMS demonstrated that patients with early-onset disease (<8 years) experienced progression approximately 50% faster than patients with typical-onset disease (8-14 years) and more than twice as fast as those with intermediate-onset disease (15-24 years).[1] Importantly, mFARS severity ranges correspond to clinically meaningful changes in patient function. Analyses from the MOXIe study and FACOMS registry of patients with FA demonstrate substantial increases in mobility impairment between mFARS score ranges of 40-49 and 50-59, with severe gait impairment increasing from approximately 18%-36% to 64%-75% of patients across these thresholds.[2] Mobility- (walking, falling, quality of sitting position) and dexterity-related activities of daily living (ADLs; cutting food and handling utensils, personal hygiene, and dressing) deteriorate steeply between mFARS 40 and ≥60[3, 4]; bulbar symptoms (speech, swallowing, breathing) emerge in approximately 88%-93% of patients with FA, with loss of swallowing and speech function occurring ≈2-3 years prior to death.[5] Notably, the Joint Nordic HTA-Bodies (JNHB) December 2024 assessment of omaveloxolone independently used the same age-of-onset strata (≤7, 8-14, 15-24, ≥25 years) and concluded this framework was appropriate for modeling disease progression in FA.[6]

While measures such as ambulation status may provide useful descriptive information regarding disease burden and functional impairment, mFARS-based severity categories offer a more comprehensive characterization of disease status than ambulation alone and are less likely to create

artificial distinctions between patients with similar underlying disease burden. For these reasons, we encourage ICER to prioritize established age-of-onset strata and mFARS-anchored severity categories when evaluating heterogeneity in treatment outcomes and disease progression.

2. Model Structure: Consideration of Published FA Disease Progression Models

Biogen encourages ICER to consider published FA disease progression models that are grounded in longitudinal natural history evidence and externally assessed by health technology assessment bodies. A multivariable mFARS progression model derived from FACOMS data incorporated 4 age-of-onset subgroups (0-7, 8-14, 15-24, and ≥25 years) and their respective different rates of progression, reflecting the established heterogeneity of disease progression observed in FA natural history studies.[1] A propensity score–matching analysis between patients receiving omaveloxolone in the MOXIe open-label extension (OLE) and matched FACOMS natural history controls showed that omaveloxolone was associated with a 55% reduction in mFARS progression over 3 years (3.0 vs 6.6 mFARS points; difference −3.6 points; P=.0001).[7] The resulting relative progression parameter (0.454) has subsequently been used in published economic modeling, in which omaveloxolone treatment was projected to delay progression to mFARS 50 (11.3 vs 8.1 years) and mFARS 60 (21.7 vs 15.2 years).[2]

The JNHB independently adopted and validated this same age-of-onset stratification and progression parameter (0.454) in its assessment of omaveloxolone and concluded that the model structure was suitable for evaluating the decision problem, confirming that this model architecture reflects the international methodological consensus for FA health economic modeling.[6] Accordingly, we encourage ICER to consider this published framework as a relevant basis for economic modeling, with the understanding that progression varies by age group, and to evaluate key assumptions through scenario and sensitivity analyses.

3. Health State Utilities: Adopt Disease-Specific Vignette-Derived Values

We encourage ICER to carefully evaluate the limitations of generic utility mapping approaches in FA and to consider disease-specific utility estimates as part of the base-case analysis. The JNHB's own analysis noted that generic instruments such as EQ-5D and SF-36 may not adequately capture important manifestations of FA, including bulbar dysfunction, and concluded that assumptions regarding the relationship between mFARS and quality of life (QOL) are highly uncertain.[6] There is also clear heterogeneity across published health-state utility estimates in FA, reflecting differences in instruments, populations, and disease-state anchoring.[8-13] We therefore encourage ICER to assess the full range of available utility evidence and to support additional research to better characterize QOL by mFARS-defined disease severity.

4. Outcomes of Interest: Confirm Ambulatory Years and Include Bulbar Endpoints

Biogen supports the inclusion of functional outcomes, mobility, ADLs, cardiomyopathy, and QOL within the assessment framework. We respectfully recommend that:

- Ambulatory years be included as a predefined outcome of interest rather than a pending measure, given the importance of mobility preservation to patients and caregivers and the availability of published supporting data linking disease severity to mobility milestones[2]
- Bulbar outcomes, including speech, swallowing, and respiratory function, be explicitly identified as patient-important outcomes. These manifestations are common in FA, are

strongly associated with disease progression, and contribute substantially to loss of independence and caregiver burden, with loss of swallowing and speech function occurring shortly before death[5]

- Assessment of cardiac safety consider the totality of available evidence regarding omaveloxolone, including reported cardiovascular monitoring data and characterization of observed laboratory findings within their clinical context, as 52 weeks of treatment with omaveloxolone 150 mg/day did not produce clinically significant changes in blood pressure, heart rate, ECG, or echocardiographic parameters, and mild BNP elevations were observed without fluid retention.[14] No cardiac safety issues were observed through OLE Year 4[15]

5. Comparators and Assessment of Value

Nomlabofusp's BLA submission is anticipated in June 2026 and no approval has been granted at the time of this writing. Its evidence base will be nascent during ICER's review, and the head-to-head comparison with omaveloxolone should be treated as a scenario analysis rather than a co-base case.

6. Societal Perspective and Benefits Beyond Health

Biogen strongly supports activation of the societal co-base case. FA imposes particularly substantial lifeline societal consequences in patients with earlier disease onset, who experience more severe and rapidly progressive disease, resulting in lost educational attainment, early exit from the workforce, and caregiver burden that are not fully captured within a healthcare-sector perspective alone. The JNHB assessment recognized these broader consequences by incorporating educational support, transportation costs, productivity loss, and caregiver costs within its economic framework.[6] Importantly, caregiver burden increases markedly with disease progression: the proportion of patients requiring caregiving rises from 18% at mFARS 0-10 to 100% at mFARS ≥50, while average caregiving requirements increase from 6 hours per week at lower disease severity to ≈70 hours per week at mFARS 50-60 and 24 hours/day for the week for mFARS ≥90.[6] These findings illustrate that slowing disease progression may generate meaningful benefits beyond direct healthcare utilization through preservation of patient independence, reduced caregiver burden, and improved participation in education and employment. Formal modeling that incorporates caregiver time and patient productivity loss by mFARS range demonstrates that societal perspective materially affects the cost-effectiveness estimate. Accordingly, we encourage ICER to fully evaluate these broader societal consequences as part of its adapted framework for serious ultra-rare diseases.

Conclusion

Biogen appreciates ICER’s thoughtful approach to evaluating therapies for FA and its commitment to incorporating patient-centered outcomes and broader elements of value. We respectfully submit these recommendations to help ensure that the assessment reflects the heterogeneity of FA, the clinical significance of disease progression, and the substantial burden experienced by patients and caregivers.

Thank you for your consideration. We welcome continued engagement throughout the review process.

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Larimar Therapeutics Comments on ICER's Draft Background and Scope: Disease-Specific Treatments for Friedreich's Ataxia

Larimar Therapeutics appreciates the opportunity to comment on the Institute for Clinical and Economic Review's (ICER) Draft Background and Scope for its assessment of disease-specific treatments for Friedreich's ataxia (FA). FA is a rare, progressive, and systemic disease with neurologic deterioration caused by a genetic defect on both alleles of chromosome 9 that results in decreased production of the critical mitochondrial protein, frataxin, leading to lower tissue frataxin levels in affected individuals.[1,2] On average most patients with FA only produce about 20-40% of the frataxin levels seen in homozygous healthy people.[3,4] Not having enough frataxin leads to a myriad of debilitating symptoms, including unsteady posture and frequent falling. Patients will often present before the age of 14 and symptoms are progressive, typically causing patients to be wheelchair bound 7-10 years after the initial diagnosis.[5,6] Patients with FA may experience impairment of musculoskeletal function, vision, hearing, and speech, and may develop diabetes, osteoporosis, and cardiomyopathy. Unfortunately, patients with FA have a life expectancy of only 30-50 years, with the most common cause of death being heart disease.[7] Lower frataxin levels are closely associated with disease severity and progression.[8,9] This underscores the importance of considering the role of frataxin deficiency when evaluating therapies for FA and highlights why outcomes related to frataxin restoration may provide meaningful context for interpreting clinical benefit. Although omaveloxolone is the first approved therapy for FA, it does not address the root cause of the disease, low levels of frataxin, and it is not approved for children under 16 years of age. There remains no approved therapy specifically designed to increase frataxin levels and directly address frataxin deficiency.[1,2] Consequently, patients, caregivers, clinicians, and advocates continue to identify a substantial unmet need for therapies that address the biology underlying disease progression.[10]

Larimar appreciates ICER's recognition of the significant unmet need in FA and its proposal to assess therapies under the adaptation of the ICER Value Assessment Framework for treatments of serious ultra-rare conditions. FA natural history data can provide important context for interpreting outcomes when randomized or long-term trial data are limited. However, such comparisons should be used cautiously and account for key prognostic differences, including baseline severity, ambulation status, GAA repeat length, and other population characteristics. ICER's approach to economic evaluation by using both the health care system and modified societal perspectives seems reasonable, as FA imposes substantial patient and caregiver burden beyond direct medical costs, including unpaid caregiving, productivity loss, disability-related costs, and loss of independence.[11]

Larimar also supports ICER's proposed lifetime horizon within the comparative value analyses, as therapies designed to restore frataxin may generate benefits over many years through slowed progression, delayed loss of function, and reduced caregiver burden. Importantly, uncertainties around durability, treatment waning, discontinuation, disease stage, and utility mapping should be explored in scenario analyses.

Larimar respectfully recommends the following focused refinements to the final scope to ensure that ICER's assessment reflects the biology of FA, the realities of evidence generation in ultra-rare diseases, the distinct therapeutic approaches under review, and the outcomes most meaningful to patients, clinicians and caregivers.

1. Use of precise terminology and recognition of distinct therapeutic approaches in FA

The draft scope identifies "no disease-modifying therapy" as a comparator and evaluates both omaveloxolone and nomlabofusp as interventions of interest. Larimar understands ICER's intent to distinguish active FA-directed therapies from supportive care alone. However, the final scope should avoid implying that all interventions have the same type, mechanism, magnitude, or evidentiary basis of disease modification.

Omaveloxolone is an approved disease-specific therapy for FA. The FDA-approved prescribing information (PI) describes a 48-week randomized, double-blind, placebo-controlled study in patients 16 to 40 years of age and reports statistically significant benefit on mFARS versus placebo at Week 48.[12] The PI also describes exploratory longer-term evidence using a post hoc propensity-matched comparison versus untreated natural history controls,

while cautioning that these analyses should be interpreted carefully because they were conducted outside of a controlled study and may be subject to confounding.[13] As stated in the PI, the mechanism by which omaveloxolone exerts its effect in patients with FA is unknown, but the described pharmacologic activity does not directly target frataxin deficiency.

To address the needs of patients with FA, Larimar is developing nomlabofusp, a recombinant fusion protein designed to directly address the root cause of the disease, frataxin deficiency.[14] We do this by attaching a cell penetrating peptide to the frataxin molecule allowing the delivery of the protein across the cell membrane and into the mitochondria.

Requested scope revision: Replace “no disease-modifying therapy” with “supportive care without active FA-directed therapy” or “no active FA-directed disease-specific therapy,” and add language stating that ICER will evaluate the mechanism, nature, and evidentiary basis of disease modification separately for each intervention.

2. Elevation of frataxin restoration from a general biomarker to a central and potentially surrogate outcome

The draft scope lists “changes in FA biomarkers” under “Other Outcomes.” Larimar believes this framing is too narrow. FA is caused by frataxin deficiency, and the lower a person’s frataxin levels, the earlier the age of onset of disease, the faster the rate of disease progression, and the shorter the time to loss of ambulation.[8] Recently published data support the potential use of skin frataxin concentrations as a reasonably likely surrogate endpoint.[14] Skin frataxin concentration, related pharmacodynamic measures, and exposure-response relationships are central to understanding nomlabofusp’s mechanism of action and potential clinical benefit.[15]

Requested scope revision: Replace “changes in FA biomarkers”, with “frataxin restoration including skin frataxin concentrations” and move it from "Other Outcomes" to the main outcomes section.

3. Specification of patient-important outcome measurements and evaluation of safety through a benefit-risk lens

Larimar appreciates that ICER’s draft scope includes a broad and clinically relevant list of patient-important outcomes, including function, mobility and need for assistive devices, activities of daily living, fatigue, and others. This list appropriately recognizes that FA is a multisystem disease and that meaningful treatment benefit may extend beyond ambulation alone.

Larimar recommends that ICER retain this broad outcomes framework and further specify how these outcomes will be measured and incorporated into the clinical and economic assessment. Relevant and clinically validated measures may include, but are not limited to, published data on mFARS total score, mFARS subdomains like Upright Stability Subscore, FARS-Activities of Daily Living, 9-Hole Peg Test, fatigue measures such as MFIS, and ambulation status where available.[16,17] Particular attention should be given to mobility, balance, independence, and preservation of function, as well as the population in which each instrument is being used.[10] In younger patients with FA, the Upright Stability Score may be an especially relevant measure.[18]

Larimar also agrees that ICER should evaluate serious adverse events, discontinuations due to adverse events, and treatment emergent events (i.e., liver injury, injection site reactions and anaphylaxis in some study subjects who received nomlabofusp in a prior study followed by a prolonged pause before re-initiating nomlabofusp administration). However, safety should be interpreted in clinical context. ICER should consider adverse event timing, reversibility, need for monitoring laboratory values (e.g., aminotransferases, B-type natriuretic peptide, and serum lipids), risk mitigation, modified dosing strategies, patient education, treatment discontinuation, and patient and caregiver preferences regarding benefit-risk tradeoffs.[13] Safety conclusions should distinguish between manageable risks and risks that materially alter the long-term benefit-risk profile.

Larimar also notes that the stakeholder input section refers to ICER’s interest in refining its understanding of the clinical effectiveness and value of “preventive treatments.” Because this review concerns disease-specific treatments for FA, Larimar recommends revising this wording to avoid confusion.

Requested scope revision: I. Retain the broad list of patient-important outcomes and add language specifying that ICER will evaluate FA-specific instruments and clinically meaningful endpoints, (eg, Upright Stability Subscore). II. Add language stating that safety will be evaluated in the context of disease severity, risk mitigation strategies (e.g. strategies for mitigating anaphylaxis), monitoring, dosing strategies, reversibility, treatment discontinuation, and patient/caregiver preferences. III. Replace "preventive treatments" with "disease-specific treatments or prevention of deterioration for Friedreich's ataxia."

4. Alignment on age-based population analyses with each therapy's evidence base, label, and regulatory context

The draft scope defines the population of interest as adolescents and adults with FA and states that treatment in younger patients may be explored as a scenario. The pivotal omaveloxolone evidence base was generated in patients aged 16 to 40, and omaveloxolone is currently approved for patients aged 16 years and older.[12,13] FA, however, often begins in childhood or adolescence, and earlier onset is associated with faster progression, earlier loss of ambulation, and greater lifelong burden.[8] These observations suggest that initiation early in the disease course with a therapy that replaces frataxin may have a profound impact on the health and well-being of patients with FA over their lifetime. Therefore, ICER should avoid applying a single age-based framework that either extrapolates evidence beyond the populations studied or excludes populations that are clinically central to FA.

Where evidence, regulatory context, or the final indication support evaluation of younger patients, ICER should assess those populations transparently and separately from older adolescent and adult populations. Where evidence is limited, ICER should clearly describe uncertainty rather than assuming comparability across age groups or applying results from one age group to another without justification.

Requested scope revision: State that ICER will align age-based population and scenario analyses with each therapy's available clinical evidence, regulatory status, and final labeled or indicated population, and will distinguish evidence generated in older adolescents and adults from evidence generated in younger pediatric patients where applicable and will also transparently describe uncertainty when extrapolating across age groups.

5. Treatment of indirect comparisons between nomlabofusp and omaveloxolone

Larimar recognizes that ICER intends to evaluate omaveloxolone and nomlabofusp in the same review. However, these therapies differ in mechanism of action, route of administration, development program, endpoint strategy, regulatory context, and patient populations (e.g., differences in disease severity, age, etc.).

The final scope should state that any comparison between nomlabofusp and omaveloxolone will account for differences in mechanism of action, trial design, study populations, baseline disease severity, age, ambulation status, outcome measures, follow-up duration, and type of evidence available. Network meta-analysis or indirect treatment comparison should be conducted only if the underlying data are sufficiently comparable. If not, evidence should be synthesized qualitatively and interpreted cautiously.[19] Similarly, the draft scope lists "nomlabofusp added to omaveloxolone" as an intervention of interest. Combination use may be clinically relevant, but it should not be treated as a core intervention unless sufficient evidence exists to evaluate its safety.

Requested scope revision: Add language stating that indirect comparisons will be considered exploratory unless trial populations (e.g., baseline disease-severity), endpoints, follow-up duration, age eligibility, and outcome definitions are sufficiently comparable. Clarify that concomitant use of nomlabofusp and omaveloxolone will be evaluated only if sufficient evidence of the safety of the concomitant use of these 2 products is available.

Sincerely,

Carole Ben-Maimon, MD

CEO of Larimar Therapeutics

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June 15, 2026

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Re: Public Comment on ICER Draft Background and Scope – Disease-Specific Treatments for Friedreich's Ataxia (May 26, 2026)

Dear Ms. Piltch and the ICER Review Team,

On behalf of the Friedreich's Ataxia Research Alliance (FARA) and the National Ataxia Foundation (NAF), thank you for the opportunity to comment on ICER's Draft Background and Scope for the review of disease-specific treatments for Friedreich's ataxia (FA). FARA and NAF are leading nonprofit organizations dedicated to advancing FA research and advocating for the FA community. We have engaged extensively with patients, caregivers, clinicians, researchers, and pharmaceutical companies for over two decades and are uniquely positioned to provide input on this review.

We offer the following comments to strengthen the scope and ensure it reflects the full complexity of FA and the realities of the current evidence landscape.

1. Overarching Concern: Insufficient Clinical Evidence to Achieve the Stated Aims
While FARA recognizes the value of evaluating the health and economic outcomes of FA treatments, **we have significant concerns about ICER's ability to conduct the stated aims of this review at this time.** Nomlabofusp has not yet received FDA approval, clinical trials are ongoing, and there are no published data from randomized controlled trials available to be abstracted. For omaveloxolone, while approved, additional trials remain underway, and long-term data are still maturing. Critically, **there are no head-to-head studies comparing these interventions**, making the proposed comparative clinical evidence review and any indirect treatment comparisons premature. We encourage ICER to carefully consider whether sufficient evidence exists to support a rigorous and balanced assessment at this time and to recognize the unique realities of rare disease drug development when determining the appropriate timing and methodology for its review. A review based on insufficient data could have detrimental consequences to access to, and future investment in, rare disease treatments.

2. Background: Framing of FA and Symptom Characterization
The draft states, "There are no available cures for FA." While technically accurate, this framing sets an unrealistic benchmark and risks undervaluing treatments that slow or halt disease progression. Given the chronic, multisystem nature of FA, **patients will likely need multiple therapies** addressing different aspects of the disease. We recommend revising this language to emphasize the critical need for disease-modifying treatments and the therapeutic value of slowing progression — a benefit that 80% of patients ranked as the most important outcome[1].

Additionally, the background section should include **fatigue, vision loss, hearing loss, and mental health** as key symptoms that are meaningful to patients. Ninety-two percent of individuals with FA report fatigue among the top three symptoms impacting daily quality of life (63%)[1]. Vision and hearing loss, prominent in later disease stages, profoundly affect independence and activities of daily living and should be acknowledged as core features of the disease. Depression and anxiety are common mental issues reported by individuals with FA based on the chronic and progressive nature of the disease with major impact on quality of life.

3. Stakeholder Engagement: Include Caregivers as Named Stakeholders
The draft includes a paragraph describing the considerable impact on caregivers, yet **caregivers are not specifically named as stakeholders to be engaged in the review process.** Given the progressive nature of FA and the escalating dependence on caregivers — we strongly recommend that caregivers be explicitly identified as a stakeholder group whose input will be actively sought throughout the review.

4. Populations: Inclusion of Children and Refinement of Subpopulations
We are concerned that limiting the population of interest to **adolescents and adults excludes children with the most severe forms of FA.** Early-onset FA (before age 8) is associated with the fastest rate of disease progression and the most significant clinical burden. Children represent a critical population for whom treatment impact would be most meaningful, and their exclusion risks producing an incomplete assessment of treatment value.

Regarding subpopulations, **age of onset alone is a more clinically meaningful and practical stratification variable** than the combination of age of onset and GAA repeat number. While GAA length correlates with onset age, age of onset is the stronger predictor of disease trajectory and is more readily available in clinical practice. We also recommend removing **pes cavus** as a subpopulation-defining complication. Pes cavus is not a clinically meaningful driver of disease burden or treatment response. Subpopulations based on complications should instead focus on conditions with significant clinical impact, such as **cardiomyopathy and diabetes**.

5. Outcomes: Distinguish Clinically Meaningful Outcomes from Symptoms
The current draft of outcomes is more a list of symptoms than a list of measurable clinical outcomes. We encourage ICER to go deeper in identifying clinically meaningful, measurable outcomes for evaluation. For example:

- **Time to loss of ambulation** or time to use of an assistive device
- **Fatigue** has a significant impact on quality of life and can be assessed via patient reported outcome measures
- **Changes in cardiac function** (e.g., LVEF, LVMI), need for cardiac medications, cardiac hospitalizations, and survival
- **Caregiver need** should be assessed on a spectrum (e.g., hours per day/week, full-time vs. part-time), not as a binary yes/no
- **Education** should be added alongside employment, as many individuals with FA are children, teens and young adults, who often report needing to alter their education due to their disease (reduced hours or missing school due to fatigue or other symptom management, home schooling, in class assistance, etc…). Meaningful outcomes could include assessments of school attendance, amount of supportive services, etc.

- Specify **which FA biomarkers** will be evaluated, as some correlate more strongly with clinical outcomes than others

We also note that **cardiomyopathy appears both as a patient-important outcome and as an adverse event of interest.** This dual listing requires clarification — how will disease-related cardiac progression be distinguished from treatment-related cardiac effects?

6. Benefits Beyond Health and Comparative Value Analyses

We recommend that the "Benefits Beyond Health" section explicitly consider that **slowing or stopping FA progression may result in meaningful reductions in overall healthcare system costs** over a patient's lifetime. This perspective should be incorporated into the value analyses, especially given the document's opening emphasis on the lack of a cure.

Regarding the Scope of Comparative Value Analyses, we offer several recommendations:

- **Societal costs should prominently include loss of employment.** A published analysis of the FACOMS natural history cohort found that despite educational attainment exceeding that of the general US population — with advanced degree completion at nearly twice the national rate — over 70% of adults with FA were unemployed.[2] This pattern — high educational investment followed by progressive loss of employment capacity due to physical disability — represents a significant, quantifiable, and largely unrecognized societal cost that must be captured in ICER's economic model.
- **Healthcare cost estimates should include durable medical equipment (DME)** and necessary modifications to homes and vehicles, which represent substantial out-of-pocket and system costs that are frequently excluded from economic analyses.
- **mFARS is a research tool, not a clinical assessment** used in routine care. While it may serve as a useful modeling framework, ICER should acknowledge this limitation and consider how health states defined by mFARS translate to real-world clinical and functional status.
- The model should **incorporate costs of non-neurological management of FA**, including annual cardiac, diabetes, and scoliosis monitoring, management, medications and related interventions and mental health diagnoses, management and treatment. These represent ongoing, lifelong costs that are integral to the FA care experience.

Conclusion

FARA and NAF appreciate ICER's commitment to engaging the patient community and stakeholders in this review. Friedreich's ataxia is a devastating, progressive, multisystem disease, and any assessment of treatment value must reflect its full complexity — including the diverse symptoms experienced by patients, the profound impact on caregivers and families, and the significant limitations of the current evidence base. We look forward to continued dialogue with ICER and are available to provide additional data, patient input, and clinical context to support a rigorous and meaningful review.

Sincerely,

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