Cell-free microRNA as a Prognostic Biomarker for Amyotrophic Lateral Sclerosis (ALS)

(No. T4-2038)

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Overview

ALS is a severe neurodegenerative disorder with no cure and highly variable disease progression, which complicates clinical trial design and therapeutic development. This technology offers a novel, minimally invasive prognostic tool that measures circulating levels of miR-181â€”a brain- and spinal cord-enriched microRNAâ€”to stratify patients by disease severity. When combined with NfL, it provides a powerful RNAâ€“protein biomarker pair to enhance clinical trial precision and reduce variability.

Applications

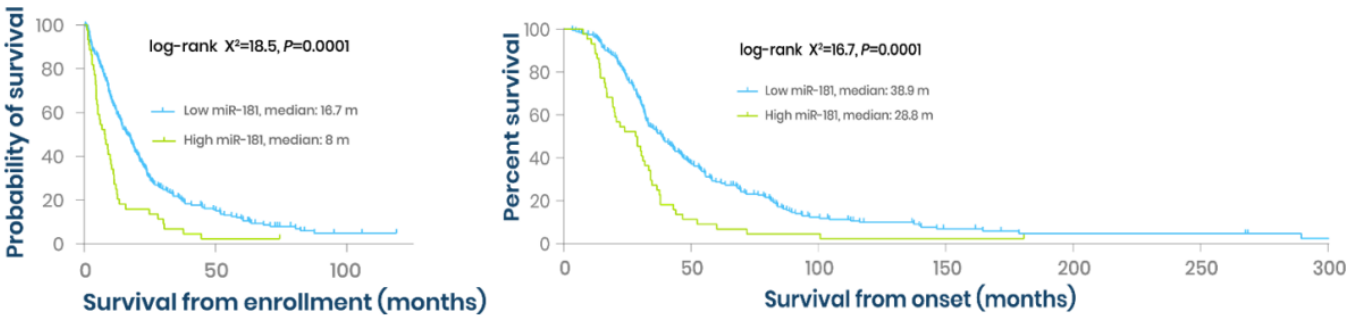
- Prognostic tool â€“ Predicts disease progression in ALS patients
- Clinical trial optimization by patient stratification â€“ Enables a more precise and balanced grouping of patients in clinical trials
- Therapeutic monitoring â€“ Assesses response to ALS treatments as a pharmacodynamic biomarker

Differentiation

- Minimally invasive, blood-based biomarker
- Improves accuracy of ALS prognosis
- Validated in two large patient cohorts

Development Stage

The biomarker has been validated in large, independent ALS patient cohorts using next-generation sequencing. Its prognostic value, alone and in combination with NfL, has been robustly demonstrated, positioning it at an advanced research stage with strong potential for clinical validation.



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miR-181 is a prognostic biomarker of ALS - Kaplanâ€“Meier curves on 248 ALS patients: 204 patients with subthreshold (light blue) versus 44 patients with suprathreshold (green) miR-181 levels from enrollment or onset.

References

Magen et al., Nat Neurosci, 2021. https://doi:10.1038/s41593-021-00936-z [1]

Benatar et al., eBioMedicine, 2024. https://doi.org/10.1016/j.ebiom.2024.105323 [2]

Patent Status

USA Published: Publication Number: 2022-0220559-A1