NeuroSense Investor Relations

NeuroSense Therapeutics Announces Transformative Phase 2b MicroRNA Data, Highlighting PrimeC's Promise as a Disease-Modifying ALS Treatment

New miRNA findings unveiled at the AAN, reveal key biological mechanisms behind PrimeC's clinical benefit in ALS.

CAMBRIDGE, Mass., April 9, 2025 /PRNewswire/ -- NeuroSense Therapeutics, Ltd. (NASDAQ: NRSN), a leading clinical-stage biotechnology company focused on developing treatments for severe neurodegenerative diseases, today announced promising new findings from its Phase 2b PARADIGM clinical trial. The data highlights the significant impact of PrimeC, the company's investigational therapy for the treatment of amyotrophic lateral sclerosis (ALS), on microRNA (miRNA) modulation.

These data are particularly significant because they:

- Represent an important development in understanding how the therapy may slow disease progression in people living with ALS.
- Reveal the consistent effect of PrimeC on altering the expression of miRNAs associated with ALS, reinforcing its potential role in addressing key pathological processes in ALS and underscoring its potential as a transformative, multi-targeted therapeutic.
- Align with the previously reported clinical benefits, including the 33% reduction in disease progression and 58% improvement in survival rates observed with PrimeC treatment.

"We are excited by these results from the PARADIGM trial, which provide further compelling support of PrimeC's potential to modify disease progression in people living with ALS," said Alon Ben-Noon, CEO of NeuroSense Therapeutics. "The ability of PrimeC to affect miRNA expression and the underlying disease pathways not only validates our approach but positions PrimeC as a promising therapeutic candidate for ALS."

Key Findings:

- **Consistent downregulation of 161 miRNAs in PrimeC-treated patients**
  In ALS patients treated with PrimeC, the study observed a profound and consistent downregulation of 161 mature miRNAs across all time points in the double-blind period. In contrast, no significant changes were detected in the placebo arm, emphasizing PrimeC's targeted biological activity and potential for disease modification.
- **Clinical relevance: miRNA modulation and disease progression**
  PrimeC treatment led to the significant downregulation of ALS-related miRNAs, including **miR-199** and **miR-181**, both of which are associated with ALS disease progression and survival. Notably, both miR-199 and miR-181 are upregulated in ALS. miR-199 is associated with neuroinflammation and reduced neuronal survival, while miR-181 correlates with a higher risk of mortality. This downregulation of both miRNAs in patients corresponds with the already reported improvement in clinical function.

This research, conducted in collaboration with Prof. Noam Shomron of Tel Aviv University's Faculty of Medical and Health Sciences, a leader in microRNA research and functional genomics, was presented yesterday by Jeffrey Rosenfeld, M.D., Ph.D., Professor of Neurology and Associate Chairman of Neurology at Loma Linda University School of Medicine, during a Late Breaker session at the 77th Annual American Academy of Neurology (AAN) Meeting, in San Diego, CA.

"What stood out to us was the consistent modulation of key miRNAs closely associated with ALS biology," said Prof. Shomron. "Observing these changes as part of a rigorous and well-controlled analysis was particularly compelling. It speaks to the potential of PrimeC to directly influence key molecular mechanisms in ALS, in a manner that is both data-driven and unbiased."

As NeuroSense advances PrimeC's clinical development, the company remains focused on deepening mechanistic insights and identifying miRNA-based biomarkers for predicting and monitoring treatment response. The strength of the Phase 2b PARADIGM miRNA data provides a solid foundation for the next phase of development, including further investigations into PrimeC's impact on miRNA maturation and its relevance in other neurodegenerative indications.

About MicroRNAs

MicroRNAs (miRNAs) are crucial regulators of gene expression, controlling cellular processes such as differentiation, proliferation, and apoptosis. Dysregulation of miRNA are known to contribute to neurodegenerative diseases such as ALS, exacerbating inflammation, impairing neuronal survival, and disrupting critical cellular pathways.

About ALS

Amyotrophic lateral sclerosis ("ALS") is an incurable neurodegenerative disease that causes complete paralysis and death within 2-5 years from diagnosis. Every year, more than 5,000 people are diagnosed with ALS in the U.S. alone, with an annual disease burden of $1 billion. The number of people living with ALS is expected to grow by 24% by 2040 in the U.S. and EU.

About PrimeC

PrimeC, NeuroSense's lead drug candidate, is a novel extended-release oral formulation composed of a unique fixed-dose combination of two FDA-approved drugs: ciprofloxacin and celecoxib. PrimeC is designed to synergistically target several key mechanisms of ALS that contribute to motor neuron degeneration, inflammation, iron accumulation and impaired ribonucleic acid ("RNA") regulation to potentially inhibit the progression of ALS. NeuroSense completed a Phase 2a clinical trial which met its safety and efficacy endpoints including reducing functional and respiratory deterioration and statistically significant changes in ALS-related biological markers indicating PrimeC's biological activity. PrimeC was granted Orphan Drug Designation by the U.S. Food and Drug Administration and the European Medicines Agency.

About NeuroSense

NeuroSense Therapeutics, Ltd. is a clinical-stage biotechnology company focused on discovering and developing treatments for patients suffering from debilitating neurodegenerative diseases. NeuroSense believes that these diseases, which include amyotrophic lateral sclerosis (ALS), Alzheimer's disease and Parkinson's disease, among others, represent one of the most significant unmet medical needs of our time, with limited effective therapeutic options available for patients to date. Due to the complexity of neurodegenerative diseases and based on strong scientific research on a large panel of related biomarkers, NeuroSense's strategy is to develop combined therapies targeting multiple pathways associated with these diseases.

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Forward-Looking Statements

This press release contains "forward-looking statements" that are subject to substantial risks and uncertainties. All statements, other than statements of historical fact, contained in this press release are forward-looking statements. Forward-looking statements contained in this press release may be identified by the use of words such as "anticipate," "believe," "contemplate," "could," "estimate," "expect," "intend," "seek," "may," "might," "plan," "potential," "predict," "project," "target," "aim," "should," "will" "would," or the negative of these words or other similar expressions, although not all forward-looking statements contain these words. Forward-looking statements are based on NeuroSense Therapeutics' current expectations and are subject to inherent uncertainties, risks and assumptions that are difficult to predict and include statements regarding the potential of PrimeC. Further, certain forward-looking statements are based on assumptions as to future events that may not prove to be accurate. The future events and trends may not occur and actual results could differ materially and adversely from those anticipated or implied in the forward looking statements. These risks include the risk that regulatory approvals for PrimeC will be delayed or not obtained in Canada or elsewhere; unexpected R&D costs or operating expenses, insufficient capital to complete development of PrimeC, the timing of expected regulatory and business milestones, risks associated with meeting with the FDA and Health Canada to determine the best path forward following the results from PARADIGM clinical trial, including a delay in any such meeting; the potential for PrimeC to safely and effectively target ALS; preclinical and clinical data for PrimeC; the uncertainty regarding outcomes and the timing of current and future clinical trials; timing for reporting data; the development and commercial potential of any product candidates of Neurosense; the ability of NeuroSense to remain listed on Nasdaq; and other risks and uncertainties set forth in NeuroSense's filings with the Securities and Exchange Commission (SEC). You should not rely on these statements as representing our views in the future. More information about the risks and uncertainties affecting NeuroSense is contained under the heading "Risk Factors" in the Annual Report on Form 20-F filed with the Securities and Exchange Commission on April 7, 2025 and NeuroSense's subsequent filings with the SEC. Forward-looking statements contained in this announcement are made as of this date, and NeuroSense undertakes no duty to update such information except as required under applicable law.

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