BrainStorm to Present Biomarker Insights Supporting NurOwn's Mechanism of Action and Clinical Impact at the 2025 ALS Drug Development Summit

- *Presentation of biomarker findings from cerebrospinal fluid analyses*
- *Workshop on adaptive and decentralized ALS trial design*
- *Panel on clinical trial access and the role of patient and caregiver engagement*

NEW YORK, May 6, 2025 /PRNewswire/ -- BrainStorm Cell Therapeutics Inc. (NASDAQ: BCLI), a leading developer of cellular therapies for neurodegenerative diseases, today announced its participation in the 4th Annual ALS Drug Development Summit, to take place May 12-14, 2025, in Boston, MA. The company's senior leadership team will participate in sessions that highlight insights and expertise gained throughout the NurOwn® development program, contributing to the broader conversation on the future of ALS clinical research.

Netta Blondheim-Shraga, PhD, Senior Vice President of Research & Development at BrainStorm, will present new insights into cerebrospinal fluid (CSF) biomarker pathways associated with Debamestrocel (NurOwn), the company's investigational cell therapy, including their relationship to clinical outcomes and disease heterogeneity in ALS.

"Biomarker data from our prior Phase 3 study suggest a potential multimodal mechanism of action and deepen our understanding of how NurOwn may influence ALS disease pathways," said Blondheim-Shraga. "These insights are helping to refine the biomarkers that will be further evaluated in the upcoming Phase 3b trial of NurOwn, under a Special Protocol Assessment (SPA) agreement with the U.S. Food and Drug Administration (FDA)."

Dr. Bob Dagher, Executive Vice President and Chief Medical Officer, will moderate a workshop session focused on adaptive and decentralized clinical trial models that have the potential to improve enrollment, increase geographic reach, and reduce burden on people living with ALS.

Dr. Dagher commented, "There is a growing imperative to modernize ALS trial design to ensure both scientific rigor and real-world feasibility. Our upcoming Phase 3b trial reflects key learnings from our previous Phase 3 study, particularly in refining patient selection criteria and optimizing trial design to better evaluate the therapeutic benefit of our cell therapy platform."

Mary Kay Turner, Senior Vice President of Global Patient Advocacy & Public Affairs, will lead a panel discussion titled "ALS Clinical Trials and Access to Investigational Therapies Through Expanded Access Programs: Perspectives from Patients, Caregivers, Advocacy, and Biopharma - And How the Path to Diagnosis Shapes These Decisions." The conversation will explore how the diagnostic journey influences trial decision-making, the need for early patient involvement in shaping protocols, and strategies to improve accessibility and trust in clinical research.

BrainStorm's participation in this year's summit marks a critical juncture for the company as it prepares to launch its Phase 3b clinical trial of NurOwn, supported by a Special Protocol Assessment (SPA) from the U.S. Food and Drug Administration (FDA), which affirms that the trial's design and statistical analysis are adequate to support a future marketing application if successful.

About Brainstorm Cell Therapeutics Inc.
BrainStorm Cell Therapeutics Inc. (NASDAQ: BCLI) is a leading developer of autologous adult stem cell therapies for debilitating neurodegenerative diseases. The company's proprietary NurOwn® platform uses autologous mesenchymal stem cells (MSCs) to produce neurotrophic factor-secreting cells (MSC-NTF cells), designed to deliver targeted biological signals that modulate neuroinflammation and promote neuroprotection.
NurOwn® is BrainStorm's lead investigational therapy for amyotrophic lateral sclerosis (ALS) and has received Orphan Drug designation from both the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA). A Phase 3 trial in ALS (NCT03280056) has been completed, and a confirmatory Phase 3b trial is set to launch under a Special Protocol Assessment (SPA) agreement with the FDA. The NurOwn clinical program has generated valuable insights into ALS disease biology, including pharmacogenomic response associated with the UNC13A genotype, biomarker data collected at seven longitudinal time points, and a comprehensive analysis of the "Floor Effect" — a critical challenge in measuring clinical outcomes in advanced ALS. BrainStorm has published its findings in multiple peer-reviewed journals. In addition to ALS, BrainStorm has completed a Phase 2 open-label multicenter trial (NCT03799718) of MSC-NTF cells in progressive multiple sclerosis (MS), supported by a grant from the National MS Society. BrainStorm is also advancing a proprietary, allogeneic exosome-based platform designed to deliver therapeutic proteins and nucleic acids. The company recently received a Notice of Allowance from the U.S. Patent and Trademark Office for a foundational patent covering its exosome technology, further strengthening BrainStorm's growing IP portfolio in this emerging area of regenerative medicine. To learn more, visit www.brainstorm-cell.com.

Notice Regarding Forward-Looking Statements

This press release contains "forward-looking statements" that are subject to substantial risks and uncertainties, including statements regarding meetings with the U.S. Food and Drug Administration (FDA), Special Protocol Assessment (SPA), ADCOM meeting related to NurOwn, the timing of a PDUFA action date for the BLA for NurOwn, the clinical development of NurOwn as a therapy for the treatment of ALS, the future availability of NurOwn to patients, and the future success of BrainStorm. 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 BrainStorm's current expectations and are subject to inherent uncertainties, risks and assumptions that are difficult to predict. These potential risks and uncertainties include, without limitation, management's ability to successfully achieve its goals, BrainStorm's ability to raise additional capital, BrainStorm's ability to continue as a going concern, prospects for future regulatory approval of NurOwn, whether BrainStorm's future interactions with the FDA will have productive outcomes, and other factors detailed in BrainStorm's annual report on Form 10-K and quarterly reports on Form 10-Q available at http://www.sec.gov. These factors should be considered carefully, and readers should not place undue reliance on BrainStorm's forward-looking statements. The forward-looking statements contained in this press release are based on the beliefs, expectations, and opinions of management as of the date of this press release. We do not assume any obligation to update forward-looking statements to reflect actual results or assumptions if circumstances or management's beliefs, expectations or opinions should change, unless otherwise required by law. Although we believe that the expectations reflected in the forward-looking statements are reasonable, we cannot guarantee future results, levels of activity, performance, or achievements.

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