BrainStorm's NurOwn® Data Selected as Breakthrough Science for Presentation at ISCT 2025 Meeting

New findings highlight impact of UNC13A genotype on treatment response in ALS

NEW YORK, April 29, 2025 /PRNewswire/ -- BrainStorm Cell Therapeutics Inc. (NASDAQ: BCLI), a leading developer of cellular therapies for neurodegenerative diseases, in collaboration with leading ALS research centers, today announced the acceptance of new pharmacogenomic data for oral presentation of new findings on NurOwn® at the International Society for Cell & Gene Therapy (ISCT) 2025 Annual Meeting, to take place from May 6 - 10, 2025 in New Orleans. The data highlight the impact of the UNC13A genotype on clinical outcomes for amyotrophic lateral sclerosis (ALS) patients treated with NurOwn® (debamestrocel).

The presentation was featured in the ISCT public announcement, which indicated that the data were "carefully reviewed and selected by the ISCT 2025 Planning Faculty, to explore the latest breakthroughs in the clinical translation of Mesenchymal Stem/Stromal Cells and how they will shape the future of cell therapies."

"We look forward to sharing the latest scientific data on NurOwn at this year's ISCT meeting. These new results are particularly meaningful as they explore a genetic factor that may help predict treatment response. The feedback received from the ALS experts on these new data continues to be encouraging as we advance in our mission to address the unmet needs of patients with this devastating disease," said Chaim Lebovits, President and CEO of BrainStorm.

Mr. Lebovits added, "Our immediate priority is to launch the Phase3b trial of NurOwn to build on our clinical data and demonstrate its potential in early ALS. We are enjoying positive communication with the FDA, have submitted an IND amendment, and are implementing the SPA exactly as agreed with the Agency. We're fully prepared to begin the study."

Dr. Bob Dagher, Executive Vice President & Chief Medical Officer at BrainStorm, commented, "These latest results offer additional scientific insights into our completed Phase 3 NurOwn trial and increase our understanding of treatment effects in patient subpopulations. These types of analysis provide important information for the development of future ALS treatments and add to the growing body of evidence supporting NurOwn®." Dr. Dagher added, "This represents a first-of-its-kind pharmacogenomic analysis from the Phase 3 trial in ALS and is among the first ALS studies to analyze how genetic differences, specifically the UNC13A genotype, affect patient response to therapy. Understanding how genetics can influence treatment response will help move us closer to truly personalized medicine for ALS."

Presentation details:

Title:	Debamestrocel Effect on Clinical and Biomarker Endpoints by UNC13A Genotype in Phase 3 ALS Trial
Lead Author	Bob Dagher, MD
Oral Session:	Mesenchymal Stem/Stromal Cells #1
Date and time:	Date & Time: Thursday, May 8, 2025 | 9:00 – 10:00 a.m. U.S. Central Time.
Location:	Ernest N. Morial Convention Center, 900 Convention Center Boulevard, New Orleans, Louisiana 70130

A copy of the poster will be available on the BrainStorm corporate website, at the conclusion of the ISCT meeting.

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.

CONTACT:
Michael Wood
Phone: +1 646-597-6983
mwood@lifesciadvisors.com

Logo: https://mma.prnewswire.com/media/1166536/BrainStorm_Logo.jpg

SOURCE BrainStorm Cell Therapeutics Inc.

Additional assets available online: Photos (1)

https://ir.brainstorm-cell.com/2025-04-29-BrainStorms-NurOwn-R-Data-Selected-as-Breakthrough-Science-for-Presentation-at-ISCT-2025-Meeting