BrainStorm Announces New Survival Data from Expanded Access Program Cohort: Remarkably 90% of ALS Patients Survived Over 5 Years with NurOwn®

NEW YORK, June 16, 2025 /PRNewswire/ -- BrainStorm Cell Therapeutics Inc. (NASDAQ: BCLI), a leading developer of adult stem cell therapies for neurodegenerative diseases, today announced new survival data from 10 participants in its Expanded Access Program (EAP) for NurOwn® (autologous MSC-NTF cells) in amyotrophic lateral sclerosis (ALS). The EAP enrolled 10 participants who had previously completed the Phase 3 clinical trial.

The analysis reviewed survival from the time of first symptom onset through participation in the Phase 3 trial, EAP Periods 1 and 2, followed by additional survival data collected through publicly available records.

EAP participants presented at the start of the Phase 3 trial relatively early in their disease course, with Mean ALSFRS-R at Baseline of 35.8 (Range: 32 – 40), followed by Mean ALSFRS-R at the start of the EAP of 31.4 (Range: 27 – 38; with one outlier score of 13).

Key findings include:

- 90% of participants (9/10) survived more than five years from the onset of ALS symptoms, compared to published estimates indicating that approximately 10% of individuals with ALS survive beyond five years. Notably, the single death in the cohort occurred following elective euthanasia.
- The median survival observed in the EAP cohort was 6.8 years (range: 6 to 7 years) from symptom onset.
- Given that approximately 10% of individuals with ALS survive beyond five years[1], the probability of observing such extended survival in 9 out of 10 participants purely by chance is extremely low. This strongly suggests that the survival outcomes in this cohort are unlikely to be the result of random variation alone.

"These survival data provide encouraging real-world insights into the long-term experience of ALS patients treated with NurOwn under expanded access," said Chaim Lebovits, President and CEO of BrainStorm Cell Therapeutics. "We believe these findings add further evidence to support the upcoming Phase 3b clinical trial of NurOwn, to be conducted under an FDA Special Protocol Assessment (SPA). Our goal remains to generate high quality and conclusive data to demonstrate the benefit of NurOwn in people living with ALS."

Bob Dagher, MD, Chief Medical Officer of BrainStorm Cell Therapeutics, stated: "The fact that 9 out of 10 EAP participants surpassed five years of survival from first symptom - in contrast with the very modest expected survival pattern typically seen in ALS - represents a clinically meaningful observation. Furthermore, 6 out of 10 patients are still alive, even post seven years from symptom onset. These data further strengthen the scientific rationale to pursue our pivotal Phase 3b trial of NurOwn in ALS, under full alignment with the FDA guidance."

The NurOwn EAP was designed to provide compassionate access to NurOwn for eligible ALS participants who completed the Phase 3 trial. The observed durability of survival in this cohort, as reflected in the publicly sourced survival data, underscores the importance of advancing the upcoming registrational Phase 3b controlled clinical trial.

About NurOwn®

The NurOwn® technology platform (autologous MSC-NTF cells) represents a promising investigational therapeutic approach to targeting disease pathways important in neurodegenerative disorders. MSC-NTF cells are produced from autologous, bone marrow-derived mesenchymal stem cells (MSCs) that have been expanded and differentiated ex vivo. MSCs are converted into MSC-NTF cells by growing them under patented conditions that induce the cells to secrete high levels of neurotrophic factors (NTFs). Autologous MSC-NTF cells are designed to effectively deliver multiple NTFs and immunomodulatory cytokines directly to the site of damage to elicit a desired biological effect and ultimately slow or stabilize disease progression.

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 second 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), 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.

CONTACTS

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

Media:
Uri Yablonka, Chief Business Officer
Phone: +1 917-284-2911
uri@brainstorm-cell.com

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[1] National Institute of Neurological Disorders and Stroke (NINDS), NIH. *Amyotrophic Lateral Sclerosis (ALS) Fact Sheet*. https://www.ninds.nih.gov/health-information/disorders/amyotrophic-lateral-sclerosis-als

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

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