FDA Review of Citizen Petition Offers a Fresh Look at NurOwn®'s Evidence of Treatment Effectiveness

BrainStorm to Continue with Planned Phase 3b Trial and Remains Committed to Advancing Access for People Living with ALS

NEW YORK, July 8, 2025 /PRNewswire/ -- BrainStorm Cell Therapeutics Inc. (NASDAQ: BCLI), a leading developer of adult stem cell therapeutics for neurodegenerative diseases, today acknowledged that the U.S. Food and Drug Administration's (FDA) consideration of a Citizen Petition requesting a new review of the data supporting NurOwn will provide a critical opportunity to reaffirm its potential as therapy for amyotrophic lateral sclerosis (ALS). A Citizen Petition, submitted to the FDA under the Federal Food, Drug, and Cosmetic Act (21 CFR § 10.30), is a regulatory process that allows any interested party to formally request the Agency to take action on specific matters, such as reviewing data, issuing new guidance, or taking enforcement action. In this case, the Citizen Petition is requesting that the FDA review the data supporting NurOwn.

BrainStorm has consistently supported the integrity and scientific validity of its data and believes a comprehensive review of all evidence is essential, especially as the regulatory landscape for rare diseases with unmet needs continues to evolve. Although BrainStorm was not involved in drafting or submitting this petition or its contents, the company welcomes the FDA's willingness to reevaluate existing data. It endorses any process that thoroughly assesses the scientific evidence related to potential treatment options for people living with ALS.

"We respect the FDA's independent review process and welcome its consideration of this request," said Chaim Lebovits, President and Chief Executive Officer of BrainStorm. "We continue to stand firmly behind the scientific integrity of BrainStorm's data and remain committed to working collaboratively with the FDA and the ALS community to advance the development of safe and effective therapies."

BrainStorm is committed to continuing alignment with FDA and will proceed with its Phase 3b ENDURANCE trial of NurOwn under an FDA Special Protocol Assessment (SPA), designed to generate additional robust data and confirm the product's efficacy and safety in early stage ALS patients. In parallel, the company supports exploration of potential regulatory pathways that may allow appropriate access to NurOwn for individuals living with ALS today.

Bob Dagher, M.D., Chief Medical Officer at BrainStorm, stated, "A new review of the NurOwn data offers an important chance to ensure all available evidence is fully considered. We remain dedicated to rigorous science and to increasing access for ALS patients in need."

Mary Kay Turner, Senior Vice President, Advocacy and Public Affairs at BrainStorm, added, "The ALS community has been a powerful voice in advocating for new approaches to treatment. We stand with advocates in supporting efforts that prioritize both data-driven decision-making and urgency for patients facing this devastating disease."

ALS is a rapidly progressive neurodegenerative disease that leads to the loss of motor neurons, severely impairing voluntary muscle movement. Despite recent advances in care, effective disease-modifying treatment options remain limited.

Peer-reviewed analyses have demonstrated clinically meaningful benefits in individuals with less-advanced ALS:

- In the subgroup of participants with baseline ALSFRS-R scores of 35 and above, NurOwn showed statistically significant functional improvement versus placebo.
- Post-hoc analyses revealed NurOwn-treated participants retained an average of two more ALSFRS-R points than placebo recipients - representing meaningful functional preservation.

BrainStorm recently announced new survival data from 10 participants who had previously completed the Phase 3 clinical trial and subsequently entered its Expanded Access Program (EAP). Notably, participants survived more than five years from ALS symptom onset – well above published estimates suggesting that only 10% of people diagnosed with ALS reach that benchmark. The median survival in the group was 6.8 years.

These results strongly suggest that the observed survival outcomes in the EAP patients are unlikely to be due to chance alone and further underscore the need for continued scientific exploration.

BrainStorm will continue to engage transparently with the FDA, clinicians, advocacy groups, and the broader ALS community, and will provide updates as appropriate.

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.

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