

BrainStorm Cell Therapeutics Submits IND Amendment to FDA, Paving the Way for Phase 3b Trial of NurOwn® in ALS

NEW YORK, April 10, 2025 /PRNewswire/ -- BrainStorm Cell Therapeutics Inc. (NASDAQ: BCLI), a leading biotechnology company developing cutting-edge cell therapies for neurodegenerative diseases, today announced the submission of an Investigational New Drug (IND) amendment to the U.S. Food and Drug Administration (FDA) for NurOwn®, the company's autologous mesenchymal stem cell therapy for amyotrophic lateral sclerosis (ALS). This important milestone sets the stage for the initiation of BrainStorm's highly anticipated Phase 3b clinical trial, which has been designed in collaboration with the FDA under a Special Protocol Assessment (SPA). The SPA agreement confirms that the Phase 3b trial design and statistical analysis plan is in line with the FDA's expectations, validating its potential to support a future Biologics License Application (BLA). The company now anticipates a swift regulatory review of the IND, clearing the way for the trial's launch.

"The submission of this IND amendment, together with the FDA's agreement on our trial design under the SPA, represents a major step toward bringing NurOwn to ALS patients," said Chaim Lebovits, CEO of BrainStorm Cell Therapeutics. "We are optimistic about a rapid review and are eager to initiate our Phase 3b study, which we are undertaking to support regulatory approval."

The Phase 3b trial is expected to enroll approximately 200 ALS participants and will be conducted in two parts. In Part A, patients will receive three doses of either NurOwn or placebo over a 24-week double-blind period. Following this, all participants - including those initially in the placebo group - will have the opportunity to transition to an open-label extension (Part B), receiving three additional doses of NurOwn over another 24 weeks. The trial's primary efficacy endpoint will assess changes in ALSFRS-R scores from baseline to week 24, comparing disease progression between the NurOwn and placebo groups. The successful completion of Part A will position BrainStorm for a BLA submission, accelerating NurOwn's path to potential approval.

ALS remains a devastating disease with limited therapeutic options, and BrainStorm is committed to working closely with regulatory agencies, clinical investigators, and the ALS community to ensure the rapid and rigorous evaluation of NurOwn. The company will provide updates as the FDA review progresses.

[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. is a leading developer of innovative autologous adult stem cell therapeutics for debilitating neurodegenerative diseases. The Company holds the rights to clinical development and commercialization of the NurOwn® technology platform used to produce autologous MSC-NTF cells through an exclusive, worldwide licensing agreement. Autologous MSC-NTF cells have received Orphan Drug designation status from the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA) for the treatment of amyotrophic lateral sclerosis (ALS). Brainstorm has completed a Phase 3 pivotal trial in ALS (NCT03280056); this trial investigated the safety and efficacy of repeat-administration of autologous MSC-NTF cells and was supported by a grant from the California Institute for Regenerative Medicine (CIRM CLIN2-0989). Brainstorm completed under an investigational new drug application a Phase 2 open-label multicenter trial (NCT03799718) of autologous MSC-NTF cells in progressive multiple sclerosis (MS) and was supported by a grant from the National MS Society (NMSS).

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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