BrainStorm Cell Therapeutics to Unveil Phase 3b NurOwn® Trial Design at MDA Clinical and Scientific Conference

Planned Phase 3b trial Designed to Confirm Efficacy and Safety of NurOwn in mild-to-moderate ALS patients

NEW YORK, Feb. 27, 2024 /PRNewswire/ -- BrainStorm Cell Therapeutics Inc. (NASDAQ: BCLI), a leading developer of adult stem cell therapeutics for neurodegenerative diseases, today announced that it will present a poster (#M201) outlining the proposed design for a Phase 3b trial of NurOwn® in amyotrophic lateral sclerosis (ALS) at the MDA Clinical and Scientific Conference, on Monday, March 4 between 6-8 p.m. at the Hilton Orlando Hotel, Florida. The design is for a two-part, multicenter, Phase 3b study to assess the efficacy and safety of NurOwn in participants with ALS. Eligible patients will initially enter a 24-week randomized, double-blind, placebo-controlled period (Part A), followed by a 24-week open-label extension period (Part B). The entry criteria will enroll people living with mild-to-moderate ALS. The primary endpoint will be based on the Revised Amyotrophic Lateral Sclerosis Functional Rating Scale (ALSFRS-R).

BrainStorm recently submitted a Special Protocol Assessment (SPA) request to the U.S. Food and Drug Administration (FDA) for this Phase 3b study of NurOwn. A SPA agreement would indicate concurrence by FDA with the adequacy and acceptability of the overall protocol design for the planned Phase 3b study, intended to support a future marketing application. The details are currently under review by the FDA and the company plans to finalize the details following the receipt of input from the Agency.

"We are committed to doing everything we can to make NurOwn available to ALS patients in need and we are pleased to present an outline of our planned confirmatory Phase 3b trial," said Chaim Lebovits, President and Chief Executive Officer of BrainStorm. "Together with our advisors, we have designed a trial that we believe will generate robust and conclusive data. Our goal is to develop an efficacious treatment option that, if approved, can help patients and generate value for our stakeholders."

Bob Dagher, MD, commented, "We have given careful consideration to a Phase 3b trial that will conclusively demonstrate the efficacy and safety of NurOwn and support a new NDA. Our previous studies have provided us with valuable insight into the NurOwn treatment effect. We believe that by incorporating these learnings into the design of a confirmatory study we can maximize the prospects of a successful outcome. The planned study will enroll mild-to-moderate ALS patients, as we believe that this is the appropriate patient population in which the course of the disease can be accurately measured using ALSFRS-R. We are grateful for the FDA's ongoing engagement and look forward to its feedback and to finalizing the SPA."

The abstract for this presentation can be viewed here. Brainstorm will post a copy of the poster to its corporate website at the conclusion of the presentation.

Special Protocol Assessment
A Special Protocol Assessment (SPA) is a process in which drug developers may ask to meet with the FDA to reach agreement on the design and size of certain clinical trials to determine if they adequately address scientific and regulatory requirements for a study that could support marketing approval. An SPA agreement indicates concurrence by FDA with the adequacy and acceptability of specific critical elements of overall protocol design for a study intended to support a future marketing application. These elements are critical to ensuring that the trial conducted under the protocol can be considered an adequate and well-controlled study that can support marketing approval. Feedback on these issues provides the greatest benefit to companies in planning late-phase development strategy. An SPA agreement does not indicate FDA concurrence on every protocol detail.

About BrainStorm Cell Therapeutics Inc.
BrainStorm Cell Therapeutics Inc. is a leading developer of innovative autologous adult stem cell therapeutics for debilitating neurodegenerative diseases. BrainStorm 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 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), and another grant from the ALS Association and I AM ALS. BrainStorm completed under an investigational new drug application a Phase 2 open-label multicenter trial (NCT03799718) of autologous MSC-NTF cells in

progressive 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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