BrainStorm Cell Therapeutics Announces Agreement with FDA on a Special Protocol Assessment (SPA) for Phase 3b Trial in ALS

Conference call and webcast at 8:00 a.m. Eastern Time today

NEW YORK, April 9, 2024 /PRNewswire/ -- BrainStorm Cell Therapeutics Inc. (NASDAQ: BCLI), a leading developer of adult stem cell therapeutics for neurodegenerative diseases, today announced that it received written agreement from the U.S. Food and Drug Administration (FDA), under a Special Protocol Assessment (SPA), on the design for a Phase 3b trial of NurOwn® in amyotrophic lateral sclerosis (ALS).

The SPA agreement with the FDA validates the clinical trial protocol and statistical analysis of the planned Phase 3b trial of NurOwn, demonstrating their adequacy for addressing objectives that support a future BLA (Biologics License Application) in ALS.

"We are pleased to have reached an agreement with the FDA on key elements of the Phase 3b trial design that provides a potential path forward towards obtaining regulatory approval," said Chaim Lebovits, President and Chief Executive Officer of BrainStorm. "We believe that having this SPA in place will help de-risk certain regulatory aspects of the NurOwn clinical program. BrainStorm's ultimate goal is to provide a new treatment option that can help patients afflicted with ALS, and we believe that the SPA potentially brings us one step closer to this goal. We appreciate the Agency's engagement and guidance during the SPA process and look forward to moving forward with the study."

BrainStorm anticipates commencement of the Phase 3b study in 2024, after reviewing the protocol with investigators, securing study site Institutional Review Board approvals, and engaging with appropriate members of the ALS community.

Stacy Lindborg, Co-CEO of BrainStorm, commented, "We have worked with leading neurologists, scientists, and members of the ALS community to create a robust study designed to evaluate the effectiveness and safety of NurOwn. This trial builds on valuable insights from our earlier studies and will enroll people living with ALS who are earlier in the course of their disease. Based on the evidence generated on NurOwn to date, we have a trial that we're optimistic will be positive. Over the past year, we've assembled an excellent team in clinical development that is highly qualified to execute this trial with excellence. We've been parallel processing to enable us to deliver the first dose in the trial in 2024, and we're anxious to get started."

The Phase 3b trial (Study BCT-006-US) will be a two-part, multicenter, study designed to assess the efficacy and safety of NurOwn in patients with ALS. The entry criteria will enroll participants earlier in the course of their disease, having the onset of ALS symptoms, including limb weakness, within the prior 24 months, all ALSFRS-R items □2 at Screening, and upright slow vital capacity >=65% of predicted for gender, height and age. Patients will also be allowed to receive concomitant treatment of an approved standard of care.

Part-A is a double blind, placebo-controlled period of 24 weeks duration. Up to approximately 200 patients are planned to be enrolled and randomized 1:1 to NurOwn or placebo treatment groups. There will be a screening period of six to nine weeks, during which eligible participants will undergo a single bone marrow aspiration procedure to procure the mesenchymal stem cells (MSCs) that will be used to manufacture each participant's NurOwn treatment for the duration of the trial. Patients will then be randomized 1:1 and treated with NurOwn or placebo via three repeated intrathecal injections, once every eight weeks. All eligible patients who complete Part-A will have the option of entering Part-B, open-label extension period of 24 weeks duration, where all participants will receive three repeated intrathecal injections of NurOwn, once every eight weeks.

The primary efficacy endpoint is the change in the Revised Amyotrophic Lateral Sclerosis Functional Rating (ALSFRS-R) total score from baseline to Week 24. Primary inference from the trial will be based on a p-value from the combined assessment of function and survival (CAFS) to account for mortality observed in the trial. Cerebrospinal Fluid (CSF) and blood samples will be collected for analysis of biomarkers of neuroinflammation, neurodegeneration, and neuroprotection. An independent Data Monitoring Committee (DMC) will monitor the safety of the trial participants.

Special Protocol Assessment
A Special Protocol Assessment (SPA) is a process in which drug developers may ask to meet with the FDA to reach an 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. A 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. A SPA agreement does not indicate FDA concurrence on every protocol detail.

Conference Call and Webcast

BrainStorm management will host a conference call and webcast at 8:00 a.m. ET today to discuss the SPA and planned Phase 3 program, after which investment community questions will be answered. Investors may participate in the conference call by clicking the link below or dialing the following numbers:

Participant Numbers:
Toll-Free: 1-888-407-2553
International: 1-347-487-1157
(No entry code needed)
Webcast URL: https://veidan.activetrail.biz/NurOwnProgram-2024

Those that wish to listen to the replay of the conference call can do so by dialing the numbers below. The replay will be available until April 24, 2024.

Teleconference Replay Number:
International: 1-888-254-7270

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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