BrainStorm Cell Therapeutics Submits Special Protocol Assessment (SPA) Request to FDA for Phase 3b Trial of NurOwn® in ALS

NEW YORK, Feb. 23, 2024 /PRNewswire/ -- BrainStorm Cell Therapeutics Inc. (NASDAQ: BCLI), a leading developer of adult stem cell therapeutics for neurodegenerative diseases, today announced that it has submitted a Special Protocol Assessment (SPA) request to the U.S. Food and Drug Administration (FDA) for a Phase 3b study of NurOwn®, its investigational treatment for amyotrophic lateral sclerosis (ALS).

The submission follows an in-person meeting that BrainStorm held with the FDA in December 2023. The interactions leading up to this submission have provided guidance on advancing the planned Phase 3b trial. Participating in an SPA program would allow BrainStorm to reach consensus with the FDA on its clinical trial design, including the proposed statistical analysis, before the study is initiated. This would help ensure that the study would be considered adequate to support a future marketing evaluation. The response is expected within 45 days of submission.

"Having an SPA in place is an important next step in advancing our development program for NurOwn," said Chaim Lebovits, President and Chief Executive Officer of BrainStorm. "We believe that the SPA will potentially de-risk certain regulatory aspects of the program. We look forward to working with the Agency to complete the SPA."

Stacy Lindborg, Ph.D., Co-CEO of BrainStorm added, "BrainStorm remains deeply committed to the ALS community. We continue to believe that NurOwn, if approved, has the potential to make a substantial difference in the lives of those affected by this devastating disease. We are grateful for the FDA's ongoing guidance and look forward to finalizing the SPA of this registration enabling trial."

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 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 harvested from each person with ALS and are manufactured using an innovative and proprietary process to secrete neurotrophic factors to target specific neurodegenerative diseases. The lead program for NurOwn is for the treatment of ALS. BrainStorm's long-term commitment to ALS is demonstrated in preclinical research and a series of clinical studies, all of which have been published in peer-reviewed journals.

The NurOwn clinical program has generated valuable insights into the pathology of ALS, as well as disease progression and treatment. Since the initial Phase 3 readout, BrainStorm has shared the full dataset through rigorous peer-reviewed analysis, including: quantification of Floor Effect, which had been noted, but never before explored in depth; evaluation of multiple pre-specified biomarkers, collected at seven different points across 20 weeks during the trial, allowing a longitudinal view; and analysis of genetic data, which represents one of the first ALS trials to prospectively invoke pharmacogenomic analysis of clinical outcome, offering great promise for the development of future treatments for ALS.

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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