BrainStorm Cell Therapeutics Announces FDA Advisory Committee Meeting to Review NurOwn® Biologics License Application Scheduled for September 27, 2023

PDUFA target action date set to occur by December 8, 2023

NEW YORK, June 6, 2023 /PRNewswire/ -- BrainStorm Cell Therapeutics Inc. (NASDAQ: BCLI), a leading developer of adult stem cell therapeutics for neurodegenerative diseases, today announced that the U.S. Food and Drug Administration (FDA) will convene a meeting of the Cellular, Tissue and Gene Therapies Advisory Committee (ADCOM) to review the Biologics License Application (BLA) for NurOwn®, its investigational mesenchymal stem cell therapy for the treatment of amyotrophic lateral sclerosis (ALS). The advisory committee meeting has been scheduled for September 27, 2023, and will be available for live streaming. In addition, BrainStorm's BLA for NurOwn has a Prescription Drug User Fee Act (PDUFA) action date targeted to occur by December 8, 2023.

"We are encouraged by the regulatory flexibility that the FDA has shown over the last year in ALS broadly, and with respect to NurOwn in particular, and believe an Advisory Committee meeting is good for patients," said Chaim Lebovits, BrainStorm President & CEO. "We are of course deeply committed to the scientific and regulatory process, which includes continuing research to confirm the results of the NurOwn clinical program and are working with ALS experts in designing a rigorous clinical study to answer important questions about this therapy and inform further research on ALS."

Stacy Lindborg, Ph.D., BrainStorm co-CEO, commented: "We welcome the opportunity to present our data at the forthcoming ADCOM. We remain confident in NurOwn and believe our data support regulatory approval. As is the case with most ALS research, our clinical program generated complex results, which deserve a thoughtful and holistic review by scientists, ALS experts, FDA reviewers, advocates, and patients. We believe this approach honors the needs of those living with ALS and offers the greatest promise for BrainStorm to fulfill our commitment to the ALS community."

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, which is under FDA review. 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 Phase 3 pivotal trial NurOwn did not reach statistical significance on the primary or secondary endpoints, likely due to a "floor effect," which confounds measurement of disease progression in patients with more advanced disease. A thorough analysis of NurOwn Phase 3 data shows evidence of clinically meaningful effectiveness in ALS participants who have not progressed to advanced levels of disease progression. In a pre-specified group of participants with an ALSFRS-R score ³35, there was larger treatment effect across all endpoints with NurOwn compared to placebo, which aligned with historical trials and the study power assumptions. With a statistically significant difference on a key endpoint (change from baseline in ALSFRS-R). Additionally, a post-hoc sensitivity analysis of patients across threshold of >26 through ≥35 on the ALSFRS-R highlighted that NurOwn-treated patients retain, on average, two points of function more compared to placebo - clinically meaningful preservation and important for quality of life for a person living with ALS and their loved ones.

NurOwn's clinical program also included the largest cerebrospinal fluid (CSF) biomarker study ever done in ALS, strong and consistent biomarker data, which are predictive of clinical response in the trial, span pathways that are important to ALS (neuroinflammation, neurodegeneration, neuroprotection), and align with NurOwn's mechanism of action. Biomarker data in all trial participants showed consistent biological patterns of NurOwn reducing markers of inflammation and neurodegeneration, and increasing neuroprotective markers relative to placebo. Biomarker patterns were consistent across all NurOwn-participants, including in those with Advanced ALS disease where clinical scales, such as the ALS Functional Rating Scale, have demonstrated measurement challenges. Three CSF biomarkers were predictive of clinical outcomes in NurOwn-treated participants– neurofilament light (NfL), galectin-1, latency associated peptide of TGF-beta1 (LAP or TGF-b).

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.

BrainStorm previously announced the FDA intention to hold an ADCOM meeting to review NurOwn for the treatment of ALS. The company filed a BLA for NurOwn on September 9, 2022, and received a Refusal to File (RTF) letter from FDA on November 8, 2022. Following a Type A meeting and subsequent discussions with the FDA, BrainStorm requested that CBER utilize the FDA's "File Over Protest" procedure, which offers the shortest amount of time to complete the regulatory process. The BLA active review was resumed on February 7, 2023.

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 MS and was supported by a grant from the National MS Society (NMSS).

Safe-Harbor Statement

Statements in this announcement other than historical data and information, including statements regarding BrainStorm's Type A meeting with the FDA and the clinical development of NurOwn® as a therapy for the treatment of ALS, constitute "forward-looking statements" and involve risks and uncertainties that could cause BrainStorm Cell Therapeutics Inc.'s actual results to differ materially from those stated or implied by such forward-looking statements. Terms and phrases such as "intend," "should," "could," "will," "believe," "potential," and similar terms and phrases are intended to identify these forward-looking statements. The 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, the impacts of the COVID-19 pandemic on our clinical trials, supply chain, and operations, 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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