Phase 2 Clinical Trial Data of NurOwn® in Progressive MS Will Be Presented at the 37th Congress of the European Committee for Treatment and Research in Multiple Sclerosis (ECTRIMS)

- Research Demonstrates Safety and Tolerability, Relevant CSF Biomarker Outcomes, and Preliminary Evidence of Efficacy
- Trial Sponsor BrainStorm Cell Therapeutics Plans Further Clinical Development in Progressive MS

NEW YORK, Oct. 14, 2021 /PRNewswire/ -- BrainStorm Cell Therapeutics Inc. (NASDAQ: BCLI), a leading developer of cellular therapies for neurodegenerative diseases, will present findings from a multicenter, open label clinical trial of NurOwn® in progressive multiple sclerosis. The study, "Phase 2 Safety and Efficacy Study of Intrathecal MSC-NTF cells in Progressive Multiple Sclerosis," will be delivered in an oral presentation today at the fully digital 37th Congress of the European Committee for Treatment and Research in Multiple Sclerosis (ECTRIMS).

The Phase 2 clinical trial was designed to evaluate intrathecal administration of NurOwn (autologous MSC-NTF cells) in participants with progressive MS. The study achieved the primary endpoint of safety and tolerability. It demonstrated a reduction of neuroinflammatory biomarkers and an increase in neuroprotective biomarkers in the cerebrospinal fluid (CSF) and consistent improvement across MS functional outcome measures, including measures of walking, upper extremity function, vision and cognition.

"We were pleased that this study demonstrated safety, preliminary evidence of efficacy and relevant biomarker outcomes in patients with progressive multiple sclerosis, in an area of high unmet need," said Jeffrey Cohen, M.D., Director of Experimental Therapeutics at the Cleveland Clinic Mellen Center for MS and principal investigator for the trial. "These results should be confirmed in a randomized placebo-controlled trial."

The study was sponsored by Brainstorm Cell Therapeutics with additional financial support for biomarker analyses from the National Multiple Sclerosis Society Fast-Forward Program. It was conducted at four U.S. MS centers of excellence:

- Cleveland Clinic Mellen Center for MS, Cleveland;
- Icahn School of Medicine at Mount Sinai, New York;
- Keck School of Medicine of the University of Southern California, Los Angeles; and
- Stanford University School of Medicine, Palo Alto, Calif.

"We very much appreciate the tremendous collaboration among many premier organizations, for their generous sharing of expertise, support and data, which enabled the important balance between scientific rigor and ethical treatment of progressive MS participants in the trial," said Ralph Kern, M.D., MHSc., President and Chief Medical Officer, Brainstorm Cell Therapeutics. "We are holding discussions with key MS experts, and seeking guidance from the FDA to determine next steps for the development of NurOwn in progressive MS."

"The National MS Society is pleased to support the biomarker portion of this study through our commercial funding program Fast Forward," said Mark Allegretta, Ph.D., Vice President, Research. "We're encouraged to see evidence that the biomarker analysis showed proof of concept for detecting neuroprotection and reduced inflammation."

About the trial

The Phase 2 open-label study evaluated the safety and efficacy of intrathecal administration of autologous MSC-NTF cells in patients with primary or secondary progressive MS. The primary study endpoint was safety and tolerability. Secondary efficacy endpoints included: timed 25-foot walk (T25FW); 9-Hole Peg Test (9-HPT); Low Contrast Letter Acuity (LCLA); Symbol Digit Modalities Test (SDMT); 12 item MS Walking Scale (MSWS-12); as well as cerebrospinal fluid (CSF) and blood biomarkers. Clinical efficacy outcomes were compared with matched (n=48) participants in the Comprehensive Longitudinal Investigation of Multiple Sclerosis (CLIMB) registry, Tanuja Chitnis, MD Brigham and Women's Hospital and the Ann Romney Center for Neurologic Diseases, and 255 patient randomized double blind placebo controlled NN-102 SPRINT-MS Study, courtesy NIH/NINDS, PI: Robert J. Fox, MD, MS, FAAN, Cleveland Clinic, CTR: NCT01982942. Baseline characteristics from these two cohorts were similar allowing for comparison of efficacy results, comparisons with SPRINT-MS were

with the placebo arm of this study.

Mean age of participants was 47 years, 56% were female, and mean baseline EDSS score was 5.4. 18 participants were treated, 16 (80%) received all 3 treatments and completed the entire study; 2 study discontinuations were due to procedure-related adverse events. No deaths or treatment-related adverse events due to worsening of MS were observed.

In responder analyses, 14% and 13% of MSC-NTF treated participants showed at least a 25% improvement in T25FW and 9-HPT (combined hands) respectively, compared to 5% and 0% in matched CLIMB patients and 9% and 3% in SPRINT. Twenty-seven percent (27%) showed at least an 8-letter improvement in LCLA (binocular, 2.5% threshold) and 67% showed at least a 3-point improvement in SDMT, compared to 6% and 18% in CLIMB and 13% and 35% in SPRINT, respectively.

Mean improvements of +0.10 ft/sec in T25FW and -0.23 sec in 9-HPT (combined hands), were observed in MSC-NTF treated participants, compared to a mean worsening of -0.07 ft/sec and +0.49 sec in CLIMB and -0.06 ft/sec and +0.28 sec in SPRINT, respectively. MSC-NTF treated participants showed a mean improvement of +3.3 letters in LCLA (binocular, 2.5% threshold) and 3.8 points in SDMT, compared to a mean worsening of -1.07 letters in LCLA (binocular, 2.5% threshold) and mean improvement of +0.10 in SDMT, in CLIMB and -0.6 and -0.1 in SPRINT. In addition the MSFC-4 Composite Z-score of T25W, 9-HPT, SDMT and LCLA showed a 0.18 point improvement in MSC-NTF treated participants, while CLIMB and SPRINT showed decreases of -0.02 and -0.05.

Furthermore, 38% of treated patients showed at least a 10-point improvement in the MSWS-12 a patient reported outcome that evaluates the impact of MS on walking function, whereas this outcome was not evaluated in CLIMB or SPRINT.

CSF biomarkers obtained at 3 consecutive time points, showed increases in neuroprotective molecules (VEGF, HGF, NCAM-1, Follistatin, Fetuin-A) and decreases in neuroinflammatory biomarkers (MCP-1, SDF-1, sCD27 and Osteopontin).

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).

For more information, visit the company's website at www.brainstorm-cell.com.

Safe-Harbor Statement

Statements in this announcement other than historical data and information, including statements regarding future NurOwn® manufacturing and clinical development plans, 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 "may," "should," "would," "could," "will," "expect," "likely," "believe," "plan," "estimate," "predict," "potential," and similar terms and phrases are intended to identify these forward-looking statements. The potential risks and uncertainties include, without limitation, BrainStorm's need to raise additional capital, BrainStorm's ability to continue as a going concern, the prospects for regulatory approval of BrainStorm's NurOwn® treatment

candidate, the initiation, completion, and success of BrainStorm's product development programs and research, regulatory and personnel issues, development of a global market for our services, the ability to secure and maintain research institutions to conduct our clinical trials, the ability to generate significant revenue, the ability of BrainStorm's NurOwn® treatment candidate to achieve broad acceptance as a treatment option for ALS or other neurodegenerative diseases, BrainStorm's ability to manufacture, or to use third parties to manufacture, and commercialize the NurOwn® treatment candidate, obtaining patents that provide meaningful protection, competition and market developments, BrainStorm's ability to protect our intellectual property from infringement by third parties, heath reform legislation, demand for our services, currency exchange rates and product liability claims and litigation; 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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