BrainStorm Announces Topline Results from NurOwn® Phase 3 ALS Study

Clinical trial did not meet statistical significance in primary efficacy endpoint
NurOwn® showed a clinically meaningful treatment response compared to placebo in a pre-specified subgroup
CSF biomarker analyses confirmed NurOwn resulted in a statistically significant increase of neurotrophic factors and reduction in neurodegenerative and neuroinflammatory biomarkers
Company management to host conference call and live webcast today at 8:30 AM ET

NEW YORK, Nov. 17, 2020 /PRNewswire/ -- BrainStorm Cell Therapeutics Inc. (NASDAQ: BCLI), a leading developer of adult stem cell therapies for neurodegenerative diseases, announced today topline results from the Company's randomized, double-blind placebo-controlled Phase 3 trial evaluating NurOwn® (MSC-NTF cells) as a treatment for Amyotrophic lateral sclerosis (ALS). Results from the trial showed that NurOwn® was generally well tolerated in this population of rapidly progressing ALS patients. While showing a numerical improvement in the treated group compared to placebo across the primary and key secondary efficacy endpoints, the trial did not reach statistically significant results.

The Phase 3 clinical trial's primary efficacy endpoint, a responder analysis evaluating the proportion of participants who experienced a 1.25 points per month improvement in the post-treatment Revised Amyotrophic Lateral Sclerosis Functional Rating Scale (ALSFRS-R) slope, was powered on assumed treatment response rates of 35% on NurOwn versus 15% on Placebo. These estimates were based on available historical clinical trial data and the NurOwn Phase 2 data. The primary endpoint was achieved in 34.7% of NurOwn participants versus 27.7% for Placebo (p=0.453). Therefore, the trial met the expected 35% NurOwn treatment group efficacy response assumption, however the high placebo response exceeded placebo responses observed in contemporary ALS trials. The secondary efficacy endpoint measuring average change in ALSFRS-R total score from baseline to Week 28, was -5.52 with NurOwn versus -5.88 on Placebo, a difference of 0.36 (p= 0.693).

In an important, pre-specified subgroup with early disease based on ALSFRS-R baseline score ³ 35, NurOwn demonstrated a clinically meaningful treatment response across the primary and key secondary endpoints and remained consistent with our pre-trial, data-derived assumptions. In this subgroup, there were 34.6% responders who met the primary endpoint definition on NurOwn and 15.6% on Placebo (p=0.288), and the average change from baseline to week 28 in ALSFRS-R total score was -1.77 on NurOwn and -3.78 on Placebo (p=0.198), an improvement of 2.01 ALSFRS-R points favoring NurOwn.

Cerebrospinal fluid (CSF) biomarker analyses confirmed that treatment with NurOwn resulted in a statistically significant increase of neurotrophic factors and reduction in neurodegenerative and neuroinflammatory biomarkers that was not observed in the placebo treatment group. We also carried out pre-specified statistical modeling designed to predict clinical response with high sensitivity and specificity based on ALS biomarkers and ALS Function and confirmed that NurOwn treatment outcomes could be predicted by baseline ALS function as well as key CSF neurodegenerative and neuroinflammatory biomarkers.

Dr. Merit Cudkowicz, one of the Principal Investigators of this trial and the Julianne Dorn Professor of Neurology at Harvard Medical School and the Director of the Healey Center for ALS and Chair of Neurology at Mass General Hospital said, "We found a clinically meaningful response to NurOwn in a pre-specified group of patients (greater than or equal to 35 ALSFRS-R at baseline). A change in pre- to post- treatment slope of 1.25 or more is substantial and clinically important. Given the heterogeneity of ALS, it is not surprising that measurement of treatment effect may be influenced by disease severity including the behavior of disease progression rates at the lower end of the scale. It is important to fully explore this finding. In addition, NurOwn was observed to have its clear intended biological effects with important changes in the pre-specified disease and drug related biomarkers."

"This clinical trial included a more severely affected ALS population compared to other recent ALS clinical trials. We identified a superior treatment response in a pre-specified subgroup of patients with less advanced disease. We are in active discussions with the FDA who have expressed their eagerness to review the data and have committed to prioritize review of this data. The FDA will review the data to see if there is a path forward to support approval" said Chaim Lebovits, Chief Executive Officer of BrainStorm. "We would like to sincerely thank the patients, their families and caregivers, investigators and staff who participated in this study, as their dedication and hard work allowed for the study's on-time completion despite the ongoing COVID-19 pandemic. I also want to thank the California Institute for Regenerative Medicine (CIRM) for their enormous support to conduct this trial."

"The findings from this clinical trial demonstrated that NurOwn treatment was associated with a clinically meaningful treatment response and consistent biomarker effects in known ALS disease pathways and that the ability of the clinical trial to demonstrate treatment effects compared to placebo are influenced by baseline disease status, as revealed through ALS function and key biomarkers. We are committed to advancing discussions with the FDA to identify regulatory pathways that may support NurOwn in ALS," commented Ralph Kern MD MHSc, President and CMO of Brainstorm. "In addition to planned

scientific engagements, biosamples from this study will be shared through the NEALS biorepository to enable additional scientific discovery efforts. We want to thank our partners, I AM ALS, and ALSA, who kindly supported the biomarker study".

"The consistency of effect observed across NurOwn treated patients, including within pre-specified subgroups, highlights an important treatment effect in a fatal disease with very limited treatment options. The placebo response observed in this trial is unprecedented and the ability to show treatment benefit in this context provides evidence of the clinical value of NurOwn. The robust changes in biomarkers of Neurodegeneration, including NfL and MCP-1, which allows identification of likely responders prior to treatment is encouraging", said Stacy Lindborg PhD, EVP and Head of Global Clinical Research. "More detailed analyses will be shared at upcoming scientific conferences and in subsequent publications. We are committed to learning as much as we can from this trial and to partner with the ALS community to progress our collective understanding of ALS, which in turn will help us to continue to bring forward new treatments for this unrelenting disease."

Study Design

The Phase 3 NurOwn® trial was a multi-center, placebo-controlled, randomized, double-blind trial designed to evaluate the safety and efficacy of NurOwn® in 189 ALS patients. It was conducted at six centers of excellence: University of California Irvine (Dr. Namita Goyal); Cedars-Sinai Medical Center (Dr. Matthew Burford); California Pacific Medical Center (Prof. Robert Miller); Massachusetts General Hospital (Prof. Merit Cudkowicz, Dr. James Berry); University of Massachusetts Medical School (Prof. Robert Brown) and Mayo Clinic (Prof. Anthony Windebank, Dr. Nathan Staff). Potential participants with ALS were screened during an 18-week run-in period and those who were rapid progressors (defined as patients with at least a 3 point decrease in ALSFRS-R score during the run-in period) were randomized 1:1 to receive three intrathecal injections (8 weeks between each injection) of NurOwn® or placebo. Participants were followed for 28 weeks after treatment. The primary endpoints of the trial were safety assessments and a responder analysis of the rate of decline in ALSFRS-R score over 28 weeks, where response was defined as participants with a ³ 1.25 points/month improvement in the post-treatment versus pre-treatment slope in ALSFRS-R at 28 weeks following the first treatment. Secondary endpoints included the percentage of patients with disease progression halted or improved, ALSFRS-R change from baseline, combined analysis of function and survival, slow vital capacity, tracheostomy-free survival, overall survival and cerebrospinal fluid biomarker measurements. For more information on the trial, visit https://clinicaltrials.gov/ct2/show/NCT03280056.

Conference Call and Webcast Details

BrainStorm's management team will host a call and webinar to discuss the Phase 3 data today at 8.30 AM EST. The call can be accessed by dialing the numbers below:

Participant Numbers:	Toll Free: 877-407-9205 International: 201-689-8054

Those interested in listening to the conference call live via the internet may do so by visiting the "Investors & Media" page of BrainStorm's website at www.ir.brainstorm-cell.com and clicking on the conference call link.

Event Link:	Webcast URL: https://www.webcaster4.com/Webcast/Page/2354/38723
Webcast Replay Expiration:	Wednesday, November 17, 2021

There will also be a replay of the call which can be accessed by using the webcast link above or by dialing the numbers below. The replay will be available for 14 days.

Replay Number:	Toll Free: 877-481-4010 International: 919-882-2331 Replay Passcode: 38723
Teleconference Replay Expiration:	Tuesday, December 01, 2020

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 can 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 status designation 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 the Phase 3 pivotal trial in ALS

(NCT03280056); this trial investigated repeat-administration of autologous MSC-NTF cells at six U.S. sites supported by a grant from the California Institute for Regenerative Medicine (CIRM CLIN2-0989). The pivotal study was intended to support a filing for FDA approval of autologous MSC-NTF cells in ALS and discussion of potential regulatory pathways for approval are planned with the U.S. FDA. BrainStorm is also conducting an FDA-cleared Phase 2 open-label multicenter trial in progressive multiple sclerosis (MS). The Phase 2 study of autologous MSC-NTF cells in patients with progressive MS (NCT03799718) started enrollment in March 2019.

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

Safe-Harbor Statement

Statements in this announcement other than historical data and information, including statements regarding the topline results from the NurOwn® Phase 3 ALS study and future clinical trial enrollment and data, 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, the regulatory approval potential of BrainStorm's NurOwn® treatment candidate, the 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, if approved, to achieve broad acceptance as a treatment option for ALS or other neurodegenerative diseases, BrainStorm's ability 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 BrainStorm's need to raise additional capital, BrainStorm's ability to continue as a going concern; 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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https://ir.brainstorm-cell.com/2020-11-17-BrainStorm-Announces-Topline-Results-from-NurOwn-R-Phase-3-ALS-Study