# BrainStorm Cell Therapeutics Announces the Presentation of New Analyses from the Phase 3 Trial of NurOwn® in ALS at the 4th Annual ALS ONE Research Symposium

## Pre-specified and post hoc analyses leveraging the published ENCALS model demonstrate a potential treatment effect on ALS disease progression in participants with less severe disease and show that this effect is protected by randomization

NEW YORK, Nov. 29, 2021 /PRNewswire/ -- BrainStorm Cell Therapeutics Inc. (NASDAQ: BCLI), a leading developer of cellular therapies for neurodegenerative diseases, today announced the presentation of new analyses from the Phase 3 trial of NurOwn at the 4th Annual ALS ONE Research Symposium. The presentation, which will be delivered Tomorrow by Dr. Jonathan Katz, a co-principal investigator on the trial and Chair of the Neurology Department and Director of the Forbes Norris ALS Clinic at the California Pacific Medical Center, showed that although the Phase 3 trial did not reach statistical significance on the primary and secondary endpoints, pre-specified and post hoc analyses leveraging different methods of exploring the heterogeneity of baseline disease in the trial, revealed the potential for a meaningful treatment effect across endpoints when focusing on a subset of participants with less severe disease at baseline.

**Additional key findings from the presentation included:**

- The observed potential treatment effect of NurOwn on ALS disease progression in participants with less severe disease was protected by randomization.
- The Phase 3 trial of NurOwn in ALS included a higher percentage of participants with advanced ALS at baseline (ALSFRS-R ≤ 25) compared to other trials, resulting in a lower baseline mean.
- In the subgroup of participants that are predicted by the ENCALS model to have long to very long survival, NurOwn treated participants had a greater percentage of responders compared to placebo (33% vs 14%).
- In the pre-specified subgroup of participants with ALSFRS-R greater than or equal to 35 at baseline, NurOwn had a greater percentage of responder compared to placebo (34.6% versus 15.6%).
- Analyses that focus both on baseline ALSFRS-R and ENCALS model categories suggest that efficacy measurements are impacted in participants with more severe disease.

"These compelling analyses add to the positive momentum behind our ALS program, and we were pleased to have the opportunity to share them with the clinical community at this year's ALS ONE Research Symposium," said Stacy Lindborg, PhD, Executive Vice President and Chief Development Officer, Brainstorm Cell Therapeutics. "We are very encouraged by the observation that there appears to be a meaningful treatment effect in patients with less severe disease at baseline that is consistent when using baseline ALSFRS-R scores and the ENCALS model which incorporates additional important disease characteristics that have been shown to be predictive of survival time.  We believe this is an important finding, especially since this effect was protected by randomization. Looking ahead, we are eager to share additional analyses from our Phase 3 trial with the clinical community through a peer reviewed publication and remain committed to pursuing the best and most expeditious path forward to bring NurOwn to patients with ALS."

The slides from the presentation will be available on the Publications page of Brainstorm's corporate website following the conclusion of the ALS ONE Research Symposium.

**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.

**Contacts:**

**Investor Relations**:
John Mullaly
LifeSci Advisors, LLC
Phone: +1 617-429-3548
jmullaly@lifesciadvisors.com

**Media**:
Mariesa Kemble
kemblem@mac.com

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