BrainStorm Cell Therapeutics to Present New Biomarker Data Suggesting ALS Patients May Benefit From Longer-Term Treatment with NurOwn

The data will be presented at the Annual ALS Drug Development Summit, which is focused on identifying transformative ALS targets, seeking translational biomarkers and propelling more clinical approvals

NEW YORK, May 20, 2024 /PRNewswire/ -- BrainStorm Cell Therapeutics Inc. (NASDAQ: BCLI), a leading developer of adult stem cell therapeutics for neurodegenerative diseases, will present new biomarker data suggesting that ALS patients may benefit from longer-term treatment with debamestrocel (NurOwn®). The Company will share the data with an international audience of patient advocacy groups, physicians, research organizations, industry representatives, key thought leaders and decision makers dedicated to ALS research at The 3rd Annual ALS Drug Development Summit, to take place May 21 to 23, 2024 in Boston MA. Stacy Lindborg PhD will deliver a presentation on new biomarker data from the NurOwn Expanded Access Program (EAP) along with data from the Phase 3 trial.

"We are very pleased to share these important outcome data from the NurOwn EAP with the ALS community," said Dr Stacy Lindborg, Member of BrainStorm's Board of Directors. "The data demonstrate a consistent reduction of neurofilament light (NfL) from baseline among participants who were randomized to receive NurOwn in the Phase 3 study. This reduction in NfL observed during the randomized Phase 3 trial, as well as in the subsequent EAP periods, indicate that patients treated with NurOwn during the Phase 3 study see benefits from the extended treatment. Furthermore, participants initially randomized to placebo in Phase 3 and were later treated with NurOwn during the EAP showed stabilization in Period 1 of the EAP, followed by reductions in NfL in Period 2 over their Phase 3 baseline levels. We looked forward to confirming this finding in the planned Phase 3b study."

Presentation Title: **Promising Longer-Term Biomarker Data from NurOwn Program in ALS: Spotlight on NfL in EAP Extension Cohort**
Speaker: Stacy Lindborg, PhD
Date/ time: May 22, 9am ET
Location: Hyatt Regency, Boston MA

Highlights

- A fixed sample of participants in the Phase 3 NurOwn trial, who met eligibility criteria, had the opportunity to enroll in an FDA approved Expanded Access Program (EAP). The EAP was conducted over two periods of 28 weeks each, during which participants could receive a total of 6 doses of NurOwn, 3 doses of NurOwn in each period. All participants in the EAP received NurOwn, including participants randomized to placebo in the Phase 3 trial.
- 13 Cerebrospinal fluid (CSF) samples were drawn during Phase 3 trial and the EAP. Levels of neurofilament light chain (NfL) in CSF were monitored during Phase 3 and subsequent EAP periods. NfL is an important biomarker marker in ALS, which measures neurodegeneration and neural cell death. NfL values has been shown through published studies to be associated with clinical progression, and treatment driven reductions in NfL were the basis for a recent ALS drug approval.
- Recently published NfL data from the Phase 3 trial showed that participants treated with NurOwn had an 11% decline from baseline in NfL. Participants randomized to placebo had NfL values similar to baseline across the trial. (Lindborg et al. Muscle and Nerve 2024).
- 10 trial participants who completed Phase 3 were enrolled in the EAP. 8 participants completed EAP period 1, and 6 completed EAP period 2. The participants in the EAP had lower NfL values at Phase 3 baseline as compared to the entire Phase 3 population.
- The new EAP data to be presented at the ALS Summit showed that, for participants randomized to NurOwn, there was a 4% decrease from baseline in NfL in Phase 3 and a 27% and 36% decrease from baseline, at the ends of Period 1 and Period 2 of the EAP, respectively. These results suggest continual benefit from extended treatment of NurOwn.
- For participants randomized to placebo and subsequently treated with NurOwn in the EAP, there was a 37% increase in NfL from baseline to study end in Phase 3. Following treatment with NurOwn in the EAP, the same patients had a 17% increase in NfL from baseline during Period 1 and a 5% decrease from baseline in NfL during Period 2.
- BrainStorm hopes to confirm these results in the planned Phase 3b trial of NurOwn.

Workshop and Panel

Antonio Trejo, VP of Regulatory Affairs at BrainStorm, will participate in a workshop **Reinventing the Surrogate Landscape: Innovating Regulatory Acceptable Endpoints Most Meaningful to People Living with ALS** at 2pm on May 21. The co-presenters in the workshop will be Christopher Ocampo, Senior Medical Director, AbbVie; Angela Genge, Director, ALS Centre of Excellence, McGill University; and Marjan Sepassi, Vice President, Medical Affairs, Clene Nanomedicine.

Mary Kay Turner, Senior Vice President, Global Patient Advocacy & Public Affairs and Kylan Morris Senior Manager, Patient Advocacy at Brainstorm, will participate in a panel discussion **Leveraging Insight from Real-Life Experiences: How Do People Living with ALS Evaluate Which Trial They Want to be Enrolled in?** at 4pm on May 22.

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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