BrainStorm Cell Therapeutics Presented Positive Survival Data from NurOwn® Expanded Access Program at 2024 Annual NEALS Meeting

Biomarker data suggest ALS patients may benefit from longer-term treatment with NurOwn

Poster highlighting design of planned Phase3b NurOwn trial also presented

NEW YORK, Oct. 28, 2024 /PRNewswire/ -- BrainStorm Cell Therapeutics Inc. (NASDAQ: BCLI), a leading developer of cellular therapies for neurodegenerative diseases, today announced the presentation of two posters featuring NurOwn® (MSC-NTF0 or debamestrocel) at the 2024 Annual Northeastern Amyotrophic Lateral Sclerosis Consortium (NEALS) Meeting, which took place virtually October 21 – 24. The posters 'Debamestrocel Long-Term Benefits on Survival and Neurodegeneration in ALS Expanded Access Program' and 'An Overview of The Phase 3b Clinical Trial of Debamestrocel in ALS' highlight the results achieved with ALS patients who participated in the Expanded Access Program (EAP) for NurOwn and summarize the details of BrainStorm's upcoming Phase 3b trial in ALS.

Debamestrocel Long-Term Benefits on Survival and Neurodegeneration in ALS Expanded Access Program(Bob Dagher et al)

- Ten participants (6 debamestrocel, 4 placebo) from Brainstorm's prior Phase 3 clinical trial (BCT-002-US) were enrolled in an open label Expanded Access Program (EAP). The EAP spanned two 28-week periods, with a break in time between the periods. Participants received an intrathecal dose of Debamestrocel every 8 weeks, for a maximum of 6 doses over the 2 periods.
- Baseline characteristics from 10 EAP participants, captured at the time they entered the Phase 3 trial, were matched against a comparable cohort from the PRO-ACT historical database using propensity score matching (PSM).
- Matching covariates for PSM included time since disease onset, pre-baseline ALSFRS-R slope, age, Slow/Forced Vital Capacity (SVC/FVC), and site of onset, with a 10:1 matching ratio. A Kaplan-Meier (KM) plot was generated, and a log rank test (LRT) was performed to compare survival between the two groups. A longitudinal plot of neurofilament light (NfL) was generated to assess long-term effects on neurodegeneration.
- At the last available visit in the EAP, 9/10 participants were alive. The survival curves revealed a statistically significant difference in favor of debamestrocel (LRT, p= 0.0379) with a median survival time of 46.6 months for the debamestrocel group compared to 41.1 months for the matched control.
- Among the six 'early-start' participants (those who had received Debamestrocel during the Phase 3 study), a continual reduction in NfL was observed. In contrast, for those who received placebo in the Phase 3, the group median NfL change was 37% by the end of phase 3, indicating worsening neurodegeneration. However, after these participants received debamestrocel in the EAP, the majority showed a stabilization in NfL levels.

"We were pleased to provide this important update from the NurOwn EAP with the ALS community at this year's NEALS meeting," said Bob Dagher, M.D., Executive VP and Chief Medical Officer at Brainstorm. "The encouraging findings suggest that among the 10 ALS participants who received NurOwn during the EAP, there was a long-term survival benefit when compared with the matched control group. As previously reported, we also observed reductions in neurofilament light (NfL) which is an important biomarker marker in ALS, measuring neurodegeneration and neural cell death. The consistent reductions in NfL observed both during the randomized Phase 3 trial and in the subsequent EAP periods, indicate that patients treated with NurOwn continued to see benefits from the extended treatment. These data align with our understanding of NurOwn's mechanism of action."

An Overview of The Phase3b Clinical Trial of Debamestrocel in ALS (Bob Dagher et al)

- Up to approximately 200 participants with ALS are expected to enroll in the two-part trial, to receive 3 doses of either debamestrocel or placebo for 24 weeks (Part-A), followed by an open label period of receiving 3 doses debamestrocel for another 24 weeks (Part B).
- Participants in both treatment arms will be able to receive standard of care while on study.
- The key entry criteria will include: age 18 to 75 years old, ALS diagnosis defined by the revised El Escorial criteria as laboratory-supported probable, clinically probable, or definite, symptom onset within 24 months of screening, ≥2 points on each item of the ALSFRS-R, ALSFRS-R total score ≤45, and upright Slow Vital Capacity (SVC) ≥65% of predicted.
- The primary efficacy endpoint will be a comparison of change in ALSFRS-R from baseline to week-24 (i.e. at the end of Part A) for debamestrocel vs. placebo.
- Other outcome assessments will include CAFS (Clinical Assessment of Function & Survival), SVC, HHD (Hand-Held Dynamometry) ALSAQ-40 questionnaire, the ZBI (Zarit Burden Interview), and survival.
- CSF and blood samples will be collected for analysis of biomarkers of neuroinflammation, neurodegeneration, and neuroprotection. A sample using an oral swab can be collected for DNA evaluation of ALS-related genes (optional).

Dr. Dagher added, "A key priority for BrainStorm is to confirm NurOwn's efficacy in the upcoming Phase 3b trial. Our goal is to conclusively demonstrate the treatment's benefits in early-stage ALS patients, hence, we have set the entry criteria to specifically target people living with ALS in the early stage of their disease. We have secured an agreement with the US FDA on a Special Protocol Assessment (SPA), thereby significantly derisking the regulatory aspects of this program. We have also aligned with the FDA on the Chemistry, Manufacturing, and Controls (CMC) aspects of the trial. We look forward to providing further updates as we advance our trial preparations."

Copies of the posters can be viewed online by registered attendees of the NEALS Meeting and will be available on the BrainStorm corporate website.

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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