BrainStorm Announces NurOwn® Expanded Access Program

NurOwn® will be made available for ALS patients who completed all Phase 3 clinical trial assessments and meet specific eligibility criteria

NEW YORK, Dec. 14, 2020 /PRNewswire/ -- BrainStorm Cell Therapeutics Inc. (NASDAQ: BCLI), a leader in developing innovative autologous cellular therapies for highly debilitating neurodegenerative diseases, announced today the initiation of a NurOwn® (MSC-NTF cells) Expanded Access Program (EAP) for patients with amyotrophic lateral sclerosis (ALS) who completed the Company's recently concluded pivotal Phase 3 clinical trial and meet specific eligibility requirements.

The protocol for the EAP was developed in partnership with the United States Food and Drug Administration (FDA) to provide access to NurOwn for Phase 3 clinical trial participants who meet specific eligibility criteria. Initially, patients less severely affected by ALS, as measured by the Revised ALS Functional Rating Scale (ALSFRS-R), will be the first to receive treatment. This approach is informed by recently announced topline data from the Company's Phase 3 clinical trial. According to the FDA, EAPs, alternatively known as "compassionate use" programs, provide a pathway for patients to receive an investigational medicine for a serious disease or condition outside of a clinical trial.

"This expanded access program is an appropriate and welcome next step in following up the exciting results of the Phase 3 study; it is widely anticipated and deeply appreciated by our ALS patients," said Robert Brown, MD DPhil, Leo P. and Theresa M. LaChance Chair in Medical Research, and chair, department of neurology, University of Massachusetts Medical School and UMass Memorial Medical Center, and one of the Principal Investigators in the NurOwn® Phase 3 study.

Through the newly announced EAP, the six clinical centers of excellence participating in the Phase 3 NurOwn® trial will each have the opportunity to treat ALS patients who completed the trial. These six centers are: University of California, Irvine; Cedars-Sinai Medical Center; California Pacific Medical Center; Massachusetts General Hospital; University of Massachusetts Medical School; and Mayo Clinic. EAP treatment of ALS patients who have completed the Phase 3 clinical trial will not interfere with data or regulatory timelines. The Dana Farber Cancer Institute will initially manufacture the investigational therapy, assisted by BrainStorm personnel who will be on-site.

Chaim Lebovits, CEO of BrainStorm, commented, "We are pleased to have the opportunity to treat additional patients with NurOwn through this Expanded Access Program, which was strongly advocated for by members of the ALS advocacy community. We recognize the urgency with which people with ALS want and need access to new potential treatments. This EAP is an important next step to providing an immediate option for some patients. We remain committed to rapidly advancing NurOwn through clinical development and regulatory review in the hope that the greatest number of people living with ALS may benefit."

Brian Wallach, person living with ALS and Co-Founder of I AM ALS, added, "The entire ALS community has worked tirelessly to find pathways to access promising investigational therapies because ALS waits for no one. The launch of this EAP represents an important step forward and precedent for realizing the vision of access to promising investigational therapies for all, and I am thankful to see it come to fruition."

Nancy Frates, ALS Advocate and TED Speaker remarked, "Recently our family took pause as we reflected on 1 year of living without our Peter. Continuing my promise to my son, I remain committed to his mission to #StrikeOutALS for the ALS patients of today and tomorrow. What has always been clear, is the importance of the community to work together to advance all legitimate potential treatments. I support the efforts of Brainstorm, as they take this important step toward realizing the potential promise of NurOwn in ALS. This Expanded Access Program provides real hope."

"On behalf of the largest ALS community in the United States, which includes three of the NurOwn clinical trial sites, the Golden West Chapter is extremely grateful to Brainstorm and our clinic partners for establishing and executing an Early Access Program for those who participated in the Phase 3 trial of NurOwn. Enabling early access, while the data review continues, is an extraordinary gesture of support and compassion for those living with ALS, and reflects an understanding of the ALS community's urgent need for an effective therapy. I applaud Brainstorm for taking this important step, and I look forward to learning the results of their full data analysis," said Fred Fisher, President & CEO of Golden West Chapter of The ALS Association.

About NurOwn®

NurOwn® (autologous MSC-NTF) cells represent 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. 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. BrainStorm has fully enrolled a Phase 3 pivotal trial of autologous MSC-NTF cells for the treatment of amyotrophic lateral sclerosis (ALS). BrainStorm also recently received U.S. FDA acceptance to initiate a Phase 2 open-label multicenter trial in progressive MS and enrollment began in March 2019.

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 fully enrolled a Phase 3 pivotal trial in ALS (NCT03280056), investigating 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 is intended to support a filing for U.S. FDA approval of autologous MSC-NTF cells in ALS. BrainStorm also recently received U.S. FDA clearance to initiate a 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 at www.brainstorm-cell.com.

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