BrainStorm Cell Therapeutics Announces Second Quarter 2022 Financial Results and Provides a Corporate Update

BrainStorm to submit a Biologics License Application (BLA) to the U.S. Food and Drug Administration (FDA) for NurOwn® for the treatment of ALS

New clinical analyses reinforce the conclusions from NurOwn's® Phase 3 clinical trial

Conference call and webcast at 8:00 a.m. Eastern Time today

NEW YORK, Aug. 15, 2022 /PRNewswire/ -- BrainStorm Cell Therapeutics Inc. (NASDAQ: BCLI), a leading developer of adult stem cell therapeutics for neurodegenerative diseases, today announced financial results for the second quarter ended June 30, 2022, and provided a corporate update. The company also announced its decision to submit a Biologics License Application (BLA) to the U.S. Food and Drug Administration for NurOwn® for the treatment of amyotrophic lateral sclerosis (ALS).

BrainStorm announces decision to submit a BLA to the FDA for NurOwn® for the treatment of ALS

"Brainstorm Cell Therapeutics is at a pivotal moment as a company as we finalize the regulatory filing for NurOwn® in the treatment of ALS. The continued analysis and the feedback received from the many scientific presentations of NurOwn's® Phase 3 data have uncovered key insights that furthered our understanding of the product mechanism of action and therapeutic potential and strengthened the conclusions of NurOwn's® efficacy," said Chaim Lebovits, Chief Executive Officer. "After carefully considering these learnings, the totality of the evidence from NurOwn's® clinical studies, and the feedback received from key opinion leaders and the broader ALS community, we will submit a Biologics License Application to the FDA. We are deeply grateful to the ALS clinical experts, members of the ALS community and faithful investors for their contribution to the development of NurOwn® and what it may mean to those living with ALS. Their contributions and commitment made our current progress possible and continue to inspire us as we prepare for the considerable work ahead. We intend to provide additional updates upon learning whether the FDA files our BLA submission."

New clinical analyses strengthen the conclusions from NurOwn's® Phase 3 clinical trial

A correction was made to the *Muscle and Nerve* publication from December 2021 describing the results of NurOwn's® Phase 3 clinical trial in ALS following new clinical analyses which strengthen the Company's original conclusions from the trial. The correction results in a statistically significant treatment difference (p=0.050) of more than 2 points for an important secondary endpoint, average change from baseline in ALSFRS-R, in the pre-specified efficacy subgroup of participants with a baseline score of at least 35. Analyses reported in the original publication utilized an efficacy model that unintentionally deviated from the trial's pre-specified statistical analysis plan by erroneously incorporating interaction terms between the subgroup and treatment. The newly published results, which includes supporting information to the publication, employ the efficacy model as pre-specified in the trial's statistical analysis plan, correcting the analyses. The correction also relates to the other subgroup analyses published for this endpoint, demonstrating that all subgroups with ALSFRS-R baseline scores of at least 26 to 35 showed a statistically significant benefit following treatment with NurOwn® (p≤0.050) on this secondary endpoint.

Other Second Quarter 2022 and Recent Highlights

- Presented new analyses from NurOwn's® Phase 3 ALS trial that showed a treatment effect in participants predicted by the ENCALS model to have intermediate to very long survival. These analyses confirmed the importance of avoiding potentially misclassifying treatment responses due to the ALSFRS-R floor effect and were presented in a poster presentation at the 2022 European Network for the Cure of ALS (ENCALS) Meeting. The presentation was delivered by Dr. Jonathan Katz, 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.
- Biomarker analyses from NurOwn's® Phase 3 ALS trial were the subject of an abstract presented at the 2022 American Academy of Neurology Congress by Dr. James Berry, MD, MPH, principal investigator on the trial and Director of the Massachusetts General Hospital Multidisciplinary ALS Clinic and Chief of the Division of ALS and Motor Neuron Diseases. Results of the analyses showed significant changes across multiple cerebrospinal fluid (CSF) biomarkers following NurOwn® treatment, with the strongest effects observed on biomarkers related to neuroprotection and neuroinflammation.
- Presented a summary of analyses from the Phase 3 trial of NurOwn® in ALS that highlighted the ability of biomarkers to predict clinical treatment response and provide a window into the complex biological pathways underlying disease progression. The presentation was delivered at the ALS Drug Development Summit by BrainStorm's President and Chief Medical Officer, Dr. Ralph Kern, MD, MHSc.
- Reported new results from the open-label Phase 2 study of NurOwn® in progressive multiple sclerosis (MS) that highlighted post-treatment improvements in monocular and binocular low contrast letter acuity (LCLA) outcomes (1.25% and 2.5% thresholds). In contrast, matched patients from the long-term Comprehensive Longitudinal Investigation of Multiple Sclerosis (CLIMB) study and participants from the placebo arm of the SPRINT study showed worsening in LCLA outcomes over a similar time period. The results were presented in a poster presentation delivered by Dr. Kern at the 2022 Consortium of Multiple Sclerosis Centers (CMSC) Meeting.
- Presented biomarker analyses from the open-label Phase 2 study of NurOwn® in progressive MS showing consistent post-treatment reductions in CSF inflammatory biomarkers that may be relevant to disease progression and treatment response. The presentation was delivered by Dr. Christopher Lock, PhD, Clinical Associate Professor, Neurology and Neurological Studies, at Stanford School of Medicine, at the CMSC 2022 Meeting.
- Preclinical *in-vitro* data that showed NurOwn® cells maintaining their neurotrophic and immunomodulatory effects in the presence of Siponimod, an S1P modulator recently approved for the treatment of secondary progressive MS, were presented in a poster presentation at the CMSC 2022 Meeting. The presentation was delivered by Dr. Sidney Spector, MD, PhD, Senior Vice President, Medical Affairs and Global Strategy at BrainStorm Cell Therapeutics.
- Reported preclinical data from a murine lung injury model (the bleomycin model) that demonstrated intrathecal administration of NurOwn®-derived exosomes (Exo MSC-NTF) may have potential as a clinical therapy for inflammatory pulmonary pathologies and display superior macrophage immunomodulation compared to naïve mesenchymal stem cell-derived exosomes (Exo-MSC). The data were presented at the International Society for Extracellular Vesicles (ISEV) 2022 Annual Meeting by Haggai Kaspi, PhD, Preclinical Research Manager at BrainStorm Cell Therapeutics.
- Preclinical *in vitro* data demonstrating the superior anti-inflammatory effects of Exo MSC-NTF compared to Exo-MSC were featured in a poster at the International Society of Cell & Gene Therapy (ISCT) 2022 Meeting. The poster was presented by Dr. Kim Thacker, Senior Vice President, Medical Affairs and Clinical Innovation at BrainStorm Cell Therapeutics.
- Strengthened executive team in preparation for anticipated growth and corporate development, with the appointment of Netta Blondheim-Shraga, PhD, as VP of Research & Development and Antal Pearl-Lendner, Adv., as Chief Legal Counsel

Financial Results for the Second Quarter Ended June 30, 2022

Cash, cash equivalents, and short-term bank deposits were approximately $12.2 million as of June 30, 2022, compared to $18.4 million as of March 31, 2022.

Research and development expenses for the three months ended June 30, 2022, and 2021 were approximately $5.1 million and $3.6 million, respectively.

General and administrative expenses for the three months ended June 30, 2022, and 2021 were approximately $2.5 million.

Net loss for the three months ended June 30, 2022, was approximately $7.0 million, as compared to a net loss of approximately $6.3 million for the three months ended June 30, 2021.

Net loss per share for the three months ended June 30, 2022, and 2021 was $0.19 and $0.17, respectively.

Conference Call and Webcast

August 15, 2022, at 8:00 a.m. Eastern Time

Participant Numbers:
Toll Free: 888-506-0062
International: 973-528-0011
Participant Access Code: 955552

Webcast URL: https://bit.ly/3cXwQkt

Those that wish to listen to the replay of the conference call can do so by dialing the numbers below. The replay will be available for 14 days.

Toll Free: 877-481-4010
International: 919-882-2331
Replay Passcode: 46290

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).

Safe-Harbor Statement

Statements in this announcement other than historical data and information, including statements regarding future BLA submission, 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, BrainStorm's plan to submit a BLA for NurOwn® to the FDA, prospects that the FDA will accept BrainStorm's BLA for NurOwn® for filing and review, prospects that the FDA does not view BrainStorm's *NurOwn®* product candidate to have demonstrated adequate safety or effectiveness, prospects for future regulatory approval 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 BrainStorm's products, if approved, and services, the ability to secure and maintain research institutions to conduct BrainStorm's 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 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, health reform legislation, demand for our services, currency exchange rates and product liability claims and litigation; the impacts of the COVID-19 pandemic and additional strains of COVID-19 or any other health epidemic on our clinical trials, supply chain, and operations; potential delays in any planned or anticipated review or interactions with the FDA due to disruptions at, or inadequate funding of, the FDA; the impact of global economic and political developments on our business, including rising inflation and capital market disruptions; the current conflict in Ukraine, economic sanctions and economic slowdowns or recessions that may result from such development; 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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BRAINSTORM CELL THERAPEUTICS INC. AND SUBSIDIARIES
INTERIM CONDENSED CONSOLIDATED BALANCE SHEETS (UNAUDITED)
U.S. dollars in thousands

(Except share data)

	June 30, 2022	December 31, 2021
	U.S. $ in thousands	
ASSETS		
Current Assets:		
Cash and cash equivalents	$ 9,005	$ 18,856
Short-term deposit (Note 4)	3,239	3,238
Other accounts receivable	117	86
Prepaid expenses and other current assets (Note 5)	445	1,100
Total current assets	12,806	23,280
Long-Term Assets:		
Prepaid expenses and other long-term assets	27	27
Operating lease right of use asset (Note 6)	5,056	4,781
Property and Equipment, Net	1,072	1,189
Total Long-Term Assets	6,155	5,997
Total assets	$ 18,961	$ 29,277
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current Liabilities:		
Accounts payables	$ 5,292	$ 3,700
Accrued expenses	44	83
Operating lease liability (Note 6)	1,478	1,461
Other accounts payables	1,032	1,073
Total current liabilities	7,846	6,317
Long-Term Liabilities:		
Operating lease liability (Note 6)	3,264	3,618
Total long-term liabilities	3,264	3,618
Total liabilities	$ 11,110	$ 9,935
Stockholders' Equity:		
Stock capital: (Note 7)	12	12
Common Stock of $0.00005 par value - Authorized: 100,000,000 shares June 30, 2022 and December 31, 2021 respectively; Issued and outstanding: 36,486,180 and 36,401,413 shares at June 30, 2022 and December 31, 2021 respectively.		
Additional paid-in-capital	193,900	192,990
Treasury stocks	(116)	(116)
Accumulated deficit	(185,945)	(173,544)
Total stockholders' equity	7,851	19,342
Total liabilities and stockholders' equity	$ 18,961	$ 29,277

The accompanying notes are an integral part of the consolidated financial statements.

BRAINSTORM CELL THERAPEUTICS INC. AND SUBSIDIARIES
INTERIM CONDENSED CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS (UNAUDITED)
U.S. dollars in thousands
(Except share data)

	Six months ended June 30,		Three months ended June 30,	
	2022	2021	2022	2021
	Unaudited		Unaudited	
Operating expenses:				
Research and development, net (Note 8)	$ 7,729	$ 7,940	$ 5,113	$ 3,599
General and administrative	5,337	5,328	2,478	2,522
Operating loss	(13,066)	(13,268)	(7,591)	(6,121)
Financial expenses (income), net	(665)	(119)	550	148

Net loss	$ (12,401)	$ (13,149)	$ (7,041)	$ (6,269)
Basic and diluted net loss per share from continuing operations	$ (0.34)	$ (0.36)	$ (0.19)	$ (0.17)
Weighted average number of shares outstanding used in computing basic and diluted net loss per share	36,461,667	36,056,391	36,486,180	36,318,561

The accompanying notes are an integral part of the consolidated financial statements.

SOURCE BrainStorm Cell Therapeutics Inc

Additional assets available online: Photos (1)

https://ir.brainstorm-cell.com/2022-08-15-BrainStorm-Cell-Therapeutics-Announces-Second-Quarter-2022-Financial-Results-and-Provides-a-Corporate-Update?dm_i=40VQ%2C1H727%2C73ZN21%2C5FG6U%2C1