BrainStorm Cell Therapeutics Announces Full Year 2022 Financial Results and Provides a Corporate Update

Biologics License Application for NurOwn® for the treatment of ALS to be discussed in an upcoming FDA Advisory Committee Meeting

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

NEW YORK, March 30, 2023 /PRNewswire/ -- BrainStorm Cell Therapeutics Inc. (NASDAQ: BCLI), a leading developer of adult stem cell therapeutics for neurodegenerative diseases, today announced financial results for the fiscal year ended December 31, 2022 and provided a corporate update.

"Our priority in 2023 is to advance NurOwn® through the regulatory process as expeditiously as possible, including making preparations for our upcoming Advisory Committee Meeting," said BrainStorm's President and Chief Executive Officer (CEO) Chaim Lebovits and Co-CEO Dr. Stacy Lindborg in a joint statement. "The ADCOM will provide an invaluable opportunity for an open and thoughtful discussion among BrainStorm, regulators, ALS experts, and other key stakeholders on both the urgent need for new ALS therapies and the robust and intricate dataset that we believe supports NurOwn's approval. As we move towards this important event, our clinical trial results and experienced team give us confidence in our ability to secure a successful outcome and execute on our mission of improving the lives of individuals with ALS."

Fourth Quarter 2022 and Recent Highlights

U.S. Food and Drug Administration (FDA) notified BrainStorm in a written communication that the Agency will hold an Advisory Committee Meeting (ADCOM) to review the company's Biologics License Application (BLA) for NurOwn for the treatment of amyotrophic lateral sclerosis (ALS).

- To meet its goal of proceeding to an ADCOM as expeditiously as possible, BrainStorm utilized the FDA's File Over protest procedure to return the BLA to active review and filed an amendment which responds to most of the outstanding questions previously posed by the FDA. The Agency notified Brainstorm that it will set a date for the ADCOM as well as a Prescription Drug User Fee Act (PDUFA) target action date in due course.
- In January 2023, Dr. Stacy Lindborg was promoted to Co-CEO, launching a targeted capability build designed to position BrainStorm for sustained success.
- A presentation at the 2023 MDA Clinical and Scientific Conference delivered by Dr. Lindborg featured post hoc sensitivity analyses from NurOwn's Phase 3 ALS trial. The presentation showed that a floor effect was observed in the PRO-ACT database, and a pattern of a plateau in ALSFRS-R total score was accompanied by scale items of 0 suggesting measurement challenges in those with advanced ALS due to the floor effect of the ALSFRS-R in the NurOwn phase 3 trial and historical studies which are included in the PRO-ACT database. Analyses conducted in participants not impacted by the floor effect at baseline of the NurOwn phase 3 trial revealed statistically significant, clinically meaningful effects with NurOwn on the primary and key secondary endpoints.
- Announced a partnership with the Northeast Amyotrophic Lateral Sclerosis (NEALS) Consortium, The ALS Association and I AM ALS to provide biospecimens from NurOwn's placebo-controlled Phase 3 ALS trial to for use by the research community. The specimens are being submitted to the NEALS biorepository in connection with a $500,000 grant previously awarded to BrainStorm by The ALS Association and I AM ALS, to support biomarker research.
- Additional analyses from the Phase 3 trial of NurOwn in ALS were featured in a presentation at the 21st Annual NEALS Meeting. These analyses further strengthened the body of evidence supporting a clinically meaningful treatment effect with NurOwn in ALS. Two complementary post-hoc sensitivity analysis methods showed that, after controlling for the impact of the ALSFRS-R floor effect, participants treated with NurOwn had a higher rate of clinical response and less function lost across 28 weeks compared to placebo. The presentation was co-delivered by Dr. Lindborg and Merit Cudkowicz, MD, MSC, Chief of Neurology at Massachusetts General Hospital, Julieanne Dorn Professor of Neurology at Harvard Medical School, and Director of the Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital.
- Biomarker data from the Phase 3 trial of NurOwn in ALS were featured in a presentation delivered by Dr. Lindborg at the 5th Annual ALS ONE Research Symposium. The data showed NurOwn modulated pathways related to neurodegeneration, neuroinflammation, and neuroprotection, with changes that were consistent regardless of a participant's level of disease progression at baseline. These data provide further evidence of NurOwn's multifaceted mechanism of action and of the importance of accounting for ALSFRS-R floor effects when evaluating clinical endpoints.
- Findings from the Phase 3 trial of NurOwn in ALS, including biomarker data and analyses accounting for the

ALSFRS-R floor effect, were presented at the 13th Annual California ALS Research Summit by Dr. Lindborg. The presentation demonstrated that NurOwn had significantly better outcomes in analyses controlling for the floor effect. Outcomes that aligned with historical data and power calculations of the trial.

- Biomarker data from the Phase 2 trial of NurOwn in progressive multiple sclerosis were presented at the 38th Congress of the European Committee for Treatment and Research in Multiple Sclerosis (ECTRIMS). The data showed robust increases in levels of neuroprotective biomarkers in cerebrospinal fluid with NurOwn treatment, thereby providing important biological context for clinical outcome data showing large, clinically meaningful improvements in some trial participants, as defined by response criteria, across all endpoints measured. These observed improvements diverged from what was seen in matched patients with progressive MS from the Comprehensive Longitudinal Investigation of Multiple Sclerosis (CLIMB) registry. The presentation was delivered by Jeffrey Cohen, MD, Hazel Prior Hostetler Endowed Chair and Professor of Neurology, Cleveland Clinic Lerner College of Medicine, Director, Experimental Therapeutics, Mellen Center for MS Treatment and Research.

Financial Results for the Year Ended December 31, 2022

Cash, cash equivalents, and short-term bank deposits were approximately $3 million as of December 31, 2022, compared to $22 million as of December 31, 2021.

Research and development expenses for the twelve months ended December 31, 2022, and 2021 were approximately $14 million and $15.2 million, respectively.

General and administrative expenses for the twelve months ended December 31, 2022, and 2021 were approximately $10.9 million and $9.3 million, respectively.

Net loss for the twelve months ended December 31, 2022, was approximately $24.3 million, as compared to a net loss of approximately $24.5 million for the twelve months ended December 31, 2021.

Net loss per share for the twelve months ended December 31, 2022, and 2021 was $0.66 and $0.68, respectively.

Conference Call and Webcast

March 30, 2023, at 8:00 a.m. Eastern Time

Participant Numbers:
Toll Free: 877-545-0523
International: 973-528-0016
Entry Code: 943129

Webcast URL: https://bit.ly/40EuACf

Those interested in listening to the conference call live via the internet may do so by using the webcast link above or by visiting the "Investors & Media" page of BrainStorm's website at https://ir.brainstorm-cell.com/events-and-presentations and clicking on the conference call link.

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.

Replay Numbers:

Toll Free: 877-481-4010
International: 919-882-2331
Replay Passcode: 47908

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.

BrainStorm filed the BLA for ALS on September 9, 2022. BrainStorm received a Refusal to File letter from FDA

on November 8, 2022, and requested a Type A meeting on December 9, 2022, which was held on January 11, 2023. The perspective shared by the FDA review team reflected what was in the previously issued RTF letter. Conversations with the FDA continued, following the Type A meeting, on the best pathway to resolve the outstanding questions that remained. During these discussions, BrainStorm was presented with multiple options to return the BLA to regulatory review, which included the regulatory procedure to File over Protest. With the commitment by FDA to accept amendments that were filed to address items raised in the FTF letter, BrainStorm notified the FDA on February 6, 2023 of our decision to request the FDA to file the NurOwn BLA for ALS over Protest.

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 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 BrainStorm's Type A meeting with the FDA and the clinical development of NurOwn® as a therapy for the treatment of ALS, 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 "intend," "should," "could," "will," "believe," "potential," and similar terms and phrases are intended to identify these forward-looking statements. The 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, the impacts of the COVID-19 pandemic on our clinical trials, supply chain, and operations, 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.
CONSOLIDATED BALANCE SHEETS
U.S. dollars in thousands
(Except share data)

	December 31,	
	2022	2021
	U.S. $ in thousands	
ASSETS		
Current Assets:		
Cash and cash equivalents	$ 772	$ 18,856
Short-term deposit (Note 8)	2,211	3,238
Other accounts receivable	91	86
Prepaid expenses and other current assets (Note 4)	32	1,100
Total current assets	$ 3,106	$ 23,280
Long-Term Assets:		
Prepaid expenses and other long-term assets	$ 23	$ 27
Operating lease right of use asset (Note 5)	4,389	4,781
Property and Equipment, Net (Note 6)	933	1,189
Total Long-Term Assets	$ 5,345	$ 5,997
Total assets	$ 8,451	$ 29,277
LIABILITIES AND STOCKHOLDERS' EQUITY (DEFICIT)		
Current Liabilities:		
Accounts payables	$ 6,224	$ 3,700
Accrued expenses	84	83
Operating lease liability (Note 5)	1,427	1,461
Other accounts payables	1,065	1,073
Total current liabilities	$ 8,800	$ 6,317
Long-Term Liabilities:		
Operating lease liability (Note 5)	2,666	3,618
Total long-term liabilities	$ 2,666	$ 3,618
Total liabilities	$ 11,466	$ 9,935
Stockholders' Equity (deficit):		
Stock capital: (Note 9)	12	12
Common Stock of $0.00005 par value - Authorized: 100,000,000 shares at December 31, 2022 and December 31, 2021 respectively; Issued and outstanding: 36,694,078 and 36,401,413 shares at December 31, 2022 and December 31, 2021 respectively.		
Additional paid-in-capital	194,910	192,990
Treasury stocks	(116)	(116)
Accumulated deficit	(197,821)	(173,544)
Total stockholders' equity (deficit)	$ (3,015)	$ 19,342
Total liabilities and stockholders' equity (deficit)	$ 8,451	$ 29,277

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

U.S. dollars in thousands
(Except share data)

	Year ended December 31,	
	2022	**2021**
	U.S. $ in thousands	
Operating expenses:		
Research and development, net (Note 10)	$ 13,956	$ 15,235
General and administrative	10,866	9,304
Operating loss	(24,822)	(24,539)
Financial income, net	545	82
Net loss	$ (24,277)	$ (24,457)
Basic and diluted net loss per share	$ (0.66)	$ (0.68)
Weighted average number of shares outstanding used in computing basic and diluted net loss per share	36,509,060	36,181,753

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

Logo: https://mma.prnewswire.com/media/1166536/BrainStorm_Logo.jpg

SOURCE BrainStorm Cell Therapeutics Inc.

Additional assets available online: Photos (1)

https://ir.brainstorm-cell.com/2023-03-30-BrainStorm-Cell-Therapeutics-Announces-Full-Year-2022-Financial-Results-and-Provides-a-Corporate-Update