BrainStorm Cell Therapeutics Announces Second Quarter 2023 Financial Results and Provides Corporate Update

FDA advisory committee (ADCOM) meeting to discuss NurOwn® for Amyotrophic Lateral Sclerosis (ALS) scheduled for September 27, 2023

BrainStorm's Biologics License Application (BLA) for NurOwn has a Prescription Drug User Fee Act (PDUFA) action date targeted to occur by December 8, 2023

Company prepares for ADCOM date presentation and builds resources for FDA review and decision

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

NEW YORK, Aug. 14, 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 second quarter ended June 30, 2023 and provided a corporate update.

BrainStorm's immediate priorities are to prepare for the upcoming ADCOM meeting to review the BLA for NurOwn®, scheduled for September 27, and complete preparations for commercial launch. The Company's senior team is working with expert consultants to ensure it will deliver a compelling presentation to the ADCOM and is prepared to address the questions that the FDA and members of the committee might raise.

Dr. Stacy Lindborg, Co-Chief Executive Officer of BrainStorm commented, "We look forward to discussing NurOwn's full dataset at the forthcoming ADCOM meeting. Our clinical program has generated complex results, and the ADCOM meeting will provide us with the opportunity for a thoughtful discussion with scientists, ALS experts, FDA reviewers, advocates, and patients. We have full confidence in the data we have compiled, and believe that a comprehensive analysis of our results strongly supports NurOwn's clinically meaningful effectiveness. In addition, we continue to share our data with the ALS community at scientific meetings and recently delivered an important presentation at the 2023 ALS and Related Motor Neuron Diseases Gordon Research Conference The data from this new analysis showed that treatment with NurOwn significantly elevated markers of neuroprotection and lowered markers of neuroinflammation and neurodegeneration, including neurofilament light (NfL). Reductions in plasma NfL are believed to be a predictor of clinical benefit in ALS."

Second Quarter 2023 and Recent Highlights

Clinical and regulatory

- The U.S. Food and Drug Administration (FDA) notified BrainStorm that a meeting of the Cellular, Tissue and Gene Therapies Advisory Committee to review the BLA for NurOwn® has been scheduled for September 27, 2023. In addition, BrainStorm's BLA for NurOwn has been assigned a PDUFA action date targeted to occur by December 8, 2023.
- In July 2023, new biomarker data from the Phase 3 trial of NurOwn were presented at the2023 ALS and Related Motor Neuron Diseases Gordon Research Conference. These data show that treatment with NurOwn significantly elevated markers of neuroprotection and lowered markers of neuroinflammation and neurodegeneration, including NfL over time compared to placebo in all trial participants. It is believed that reductions in plasma NfL are reasonably likely to predict clinical benefit in ALS.

Corporate

- In July 2023, BrainStorm closed a registered direct offering with a single institutional investor raising gross proceeds, before deducting the placement agent's fees and other offering expenses, of approximately $7.5 million.
- In July 2023, BrainStorm appointed Dr. Bob Dagher as Executive Vice President and Chief Development Officer. Dr. Dagher will be responsible for the portfolio strategy and advancement of clinical development plans towards regulatory approval, including the expansion of NurOwn into new diseases and the translation of pre-clinical research into first-in-human trials. He will serve on BrainStorm's executive leadership team reporting to Dr. Lindborg.
- In June 2023, BrainStorm appointed Nir Naor, CPA, CFA, MBA (IMD), LL.M, as Board Member, chairman of its Audit Committee and member of its Governance, Nomination and Compensation Committee. Mr. Naor replaced Mr. Malcolm Taub, who retired from the same roles after 14 years of service on BrainStorm's Board of Directors.
- In April 2023, BrainStorm appointed Dr. Kirk Taylor as Executive Vice President and Chief Medical Officer, continuing a targeted capability build that was launched with the promotion of Dr. Lindborg to Co-CEO in January 2023. The capability build is being implemented to hire and bring expertise inside BrainStorm in preparation for anticipated growth.

Financial Results for the Quarter Ended June 30, 2023

Cash, cash equivalents, and short-term bank deposits were approximately $.75 million as of June 30, 2023, compared to $3.0 million as of December 31, 2022. In July 2023, subsequent to the end of the quarter, BrainStorm raised gross proceeds of approximately$7.5 million in a registered direct offering.

Research and development expenses for the three months endedJune 30, 2023 and 2022 were approximately $2.8 million and $5.1 million, respectively.

General and administrative expenses for the three months ended June 30, 2023 and 2022 were approximately $2.7 million and $2.5 million, respectively.

Net loss for the three months ended June 30, 2023 was approximately $5.3 million, as compared to a net loss of approximately $7.0 million for the three months ended June 30, 2022.

Net loss per share for the three months ended June 30, 2023 and 2022 was $0.13 and $0.19, respectively.

Conference Call and Webcast, 8:00 a.m. Eastern Time Today

Participant Numbers:	
Toll Free:	
	888-506-0062
International:	
	973-528-0011
Participant Access Code:	569863
Webcast URL:	
	https://rb.gy/hblmt

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://shorturl.at/eko26 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 until August 28, 2023.

Replay Numbers:	
Toll Free:	877-481-4010
International:	919-882-2331
Replay Passcode:	
	48916

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, which is under FDA review. 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 Phase 3 pivotal trial of NurOwn did not reach statistical significance on the primary or secondary endpoints, likely due to a "floor effect," which confounds measurement of disease progression in patients with more advanced disease. A thorough analysis of NurOwn Phase 3 data shows evidence of clinically meaningful effectiveness in ALS participants who have not progressed to advanced levels of disease progression. In a pre-specified group of participants with an ALSFRS-R score ³35, there was a larger treatment effect across all endpoints with NurOwn compared to placebo, which aligned with historical trials and the study power assumptions and resulted in a statistically significant difference on a key endpoint (change from baseline in ALSFRS-R). Additionally, a post-hoc sensitivity analysis of patients across threshold of >26 through ≥35 on the ALSFRS-R highlighted that NurOwn-treated patients retain, on average, two points of function more compared to placebo–clinically meaningful preservation and important for quality of life for a person living with ALS and their loved ones.

NurOwn's clinical program also included most robust cerebrospinal fluid (CSF) biomarker study ever done in ALS, strong and consistent biomarker data, which are predictive of clinical response in the trial, span pathways that are important to ALS (neuroinflammation, neurodegeneration, neuroprotection), and align with NurOwn's mechanism of action. Biomarker data in all trial participants showed consistent biological patterns of NurOwn reducing markers of inflammation and neurodegeneration and increasing neuroprotective markers relative to placebo. Biomarker patterns were consistent across all NurOwn-participants, including in those with Advanced ALS disease where clinical scales, such as the ALS Functional Rating Scale, have demonstrated measurement challenges. Three CSF biomarkers were predictive of clinical outcomes in NurOwn-treated participants–NfL, galectin-1, latency associated peptide of TGF-beta1 (LAP or TGF-b).

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.

BrainStorm previously announced the FDA's intention to hold an ADCOM meeting to review NurOwn for the treatment of ALS.

BrainStorm filed a BLA for NurOwn on September 9, 2022, and received a Refusal to File (RTF) letter from FDA on November 8, 2022. Following a Type A meeting and subsequent discussions with the FDA BrainStorm requested that CBER utilize the FDA's "File Over Protest" procedure, which offers the shortest amount of time to complete the regulatory process. The BLA was filed over protest, allowing completion of the regulatory process in the shortest time possible, and active review resumed on February 7, 2023.

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 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).

Notice Regarding Forward-Looking Statements

This press release contains "forward-looking statements" that are subject to substantial risks and uncertainties, including statements regarding the upcoming 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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BRAINSTORM CELL THERAPEUTICS INC. AND SUBSIDIARIES
INTERIM CONDENSED CONSOLIDATED BALANCE SHEETS
U.S. dollars in thousands
(Except share data)

	June 30, 2023	December 31, 2022
	Unaudited	Audited
	U.S. $ in thousands	

ASSETS

Current Assets:		
Cash and cash equivalents	$ 546	$ 772
Short-term deposit (Note 4)	202	2,211
Other accounts receivable	93	91
Prepaid expenses and other current assets (Note 5)	372	32
Total current assets	1,213	3,106
Long-Term Assets:		
Prepaid expenses and other long-term assets	22	23
Operating lease right of use asset (Note 6)	3,713	4,389
Property and Equipment, Net	799	933
Total Long-Term Assets	4,534	5,345
Total assets	$ 5,747	$ 8,451
LIABILITIES AND STOCKHOLDERS' EQUITY (DEFICIT)		
Current Liabilities:		
Accounts payables	$ 5,190	$ 6,224
Accrued expenses	90	84
Operating lease liability (Note 6)	1,354	1,427
Other accounts payables	1,057	1,065
Total current liabilities	7,691	8,800
Long-Term Liabilities:		
Operating lease liability (Note 6)	1,938	2,666
Total long-term liabilities	1,938	2,666
Total liabilities	$ 9,629	$ 11,466
Stockholders' Equity:		
Stock capital: (Note 7)	12	12
Common Stock of $0.00005 par value - Authorized: 100,000,000 shares June 30, 2023 and December 31, 2022 respectively; Issued and outstanding: 40,926,745 and 36,694,078 shares at June 30, 2023 and December 31, 2022 respectively.		
Additional paid-in-capital	204,431	194,910
Treasury stocks	(116)	(116)
Accumulated deficit	(208,209)	(197,821)
Total stockholders' equity (deficit)	(3,882)	(3,015)
Total liabilities and stockholders' equity (deficit)	$ 5,747	$ 8,451

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,	
	2023	2022	2023	2022
	Unaudited		Unaudited	
Operating expenses:				
Research and development, net (Note 8)	$ 5,718	$ 7,729	$ 2,794	$ 5,113
General and administrative	4,882	5,337	2,655	2,478

Operating loss	(10,600)	(13,066)	(5,449)	(7,591)
Financial expenses (income), net	(212)	(665)	(120)	550
Net loss	$ (10,388)	$ (12,401)	$ (5,329)	$ (7,041)
Basic and diluted net loss per share from continuing operations	$ (0.27)	$ (0.34)	$ (0.13)	$ (0.19)
Weighted average number of shares outstanding used in computing basic and diluted net loss per share	38,224,230	36,461,667	39,696,665	36,486,180

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/2023-08-14-BrainStorm-Cell-Therapeutics-Announces-Second-Quarter-2023-Financial-Results-and-Provides-Corporate-Update