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BrainStorm Cell Therapeutics, Inc.

(BCLI-NASDAQ)

BCLI: Receives SPA Agreement from FDA on Design of Phase 3b Trial of NurOwn™...

Based on our probability adjusted DCF model that takes into account potential future revenues from NurOwn® in ALS, BCLI is valued at \$2.50/share. This model is highly dependent upon continued clinical success of NurOwn® and will be adjusted accordingly based upon future clinical results.

Current Price (04/11/24) \$0.58
Valuation \$2.50

OUTLOOK

On April 9, 2024, BrainStorm Cell Therapeutics, Inc. (BCLI) announced it received written agreement from the U.S. Food and Drug Administration (FDA), under a Special Protocol Assessment (SPA), on the design for a Phase 3b trial of NurOwn™ in amyotrophic lateral sclerosis (ALS). The SPA agreement with the FDA validates the clinical trial protocol and statistical analysis plan and helps to de-risk certain regulatory aspects of the NurOwn clinical program. The company also recently announced the peer-reviewed publication of Phase 3 biomarker data in *Muscle and Nerve*. The study suggests that NurOwn may impact key biomarkers in ALS that are predictive of disease and discusses the identification of three biomarkers that are predictive of clinical outcomes in NurOwn-treated patients. The protocol for the Phase 3b trial includes an analysis of biomarkers of neuroinflammation, neurodegeneration, and neuroprotection.

SUMMARY DATA

52-Week High \$3.20
52-Week Low \$0.15
One-Year Return (%) -79.63
Beta 0.28
Average Daily Volume (sh) 1,530,202

Shares Outstanding (mil) 68
Market Capitalization (\$mil) \$40
Short Interest Ratio (days) N/A
Institutional Ownership (%) 14
Insider Ownership (%) 5

Annual Cash Dividend \$0.00
Dividend Yield (%) 0.00

5-Yr. Historical Growth Rates

Sales (%) N/A
Earnings Per Share (%) N/A
Dividend (%) N/A

P/E using TTM EPS N/A

P/E using 2024 Estimate N/A

P/E using 2025 Estimate N/A

Risk Level High
Type of Stock Small-Growth
Industry Med-Biomed/Gene

ZACKS ESTIMATES

Revenue

(In millions of \$)

	Q1 (Mar)	Q2 (Jun)	Q3 (Sep)	Q4 (Dec)	Year (Dec)
2023	0 A	0 A	0 A	0 A	0 A
2024	0 E	0 E	0 E	0 E	0 E
2025					0 E
2026					0 E

Earnings per Share

	Q1 (Mar)	Q2 (Jun)	Q3 (Sep)	Q4 (Dec)	Year (Dec)
2023	-\$0.14 A	-\$0.28 A	-\$0.03 A	\$0.01 A	-\$0.40 A
2024	-\$0.07 E	-\$0.07 E	-\$0.06 E	-\$0.07 E	-\$0.28 E
2025					-\$0.24 E
2026					-\$0.24 E

WHAT'S NEW

Business Update

Receives SPA Agreement with FDA

On April 9, 2024, BrainStorm Cell Therapeutics, Inc. (BCLI) [announced](#) that it received written agreement from the U.S. Food and Drug Administration (FDA) under a Special Protocol Assessment (SPA) on the design for a Phase 3b trial of NurOwn™ in amyotrophic lateral sclerosis (ALS). A SPA is a process through which drug developers meet with the FDA to reach an agreement on the size and design of certain clinical trials to ensure they adequately address scientific and regulatory requirements such that they can support marketing approval. A SPA agreement indicates the FDA concurs with the adequacy and acceptability of specific elements of a protocols overall design to ensure that the trial conducted under the protocol can be considered an adequate and well-controlled study to support marketing approval.

The Phase 3b trial will be a two-part, multicenter study that will enroll ALS patients who are earlier in the course of their disease (onset of ALS symptoms, including limb weakness, within the prior 24 months), with all ALSFRS-R items ≥ 2 at screening, and upright slow vital capacity $\geq 65\%$ of predicted for gender, height, and age. Part A of the trial will have a double blind, placebo controlled period of 24 weeks. Up to approximately 200 patients are expected to be enrolled and randomized 1:1 to receive NurOwn or placebo. There will be a screening period of six to nine weeks, during which time eligible participants will undergo a single bone marrow aspiration to procure the mesenchymal stem cells (MSCs) to produce NurOwn for the duration of the trial. Participants will receive either NurOwn or placebo via three repeated intrathecal injections once every eight weeks. All participants who complete Part A will be eligible to enroll into Part B, an open label extension period of 24 weeks where all participants will receive three repeated injections of NurOwn once every eight weeks. The primary endpoint of the trial will be the change in the Revised Amyotrophic Lateral Sclerosis Functional Rating (ALSFRS-R) total score from baseline to Week 24. Cerebrospinal fluid (CSF) and blood samples will be collected for analysis of biomarkers of neuroinflammation, neurodegeneration, and neuroprotection. An independent Data Monitoring Committee will be established to monitor the safety of trial participants. The company is planning to initiate the trial after meeting with investigator, securing study site Institutional Review Board approvals, engaging with appropriate members of the ALS community.

Peer-Reviewed Publication on Biomarker Data from Phase 3 Trial

On April 10, 2024, BrainStorm [announced](#) the peer-reviewed publication of Phase 3 biomarker data in *Muscle and Nerve*. The study suggests that NurOwn treatment may impact key biomarkers in ALS patients that are predictive of disease progression ([Lindborg et al., 2024](#)).

Analysis of biomarker data showed that NurOwn treatment impacted multiple CSF biomarkers compared with placebo, with the majority (64%) of 45 biomarkers analyzed showing a significant overall treatment effect with NurOwn compared to placebo in the direction predicted to be beneficial. The following image lists the different biomarkers with overall significant treatment effects.

^c Biomarkers with overall significant treatment effect: Fetuin-A, hsa-miR-146a-5p, hsa-miR-146b-5p, IL-37, LAP, MCP-1, MSR1, OPG, S100B, SDF-1a for Neuroinflammation; DR6, NfL, pNfH and TWEAK for Neurodegeneration; BDNF, Clusterin/ApoJ, galectin-1, G-CSF, GDF-15, HGF, NMNAT1 and VEGF for Neuroprotection; Follistatin, hsa-miR-124-3p, hsa-miR-132-3p, hsa-miR-19b-3p, hsa-miR-20a-5p, hsa-miR-30b-5p and hsa-miR-34a-5p for Other category. NfL, pNfH, hsa-miR-19b-3p and hsa-miR-20a-5p are not significant for the population with ALSFRS-R ≥ 25 at baseline.

Source: Lindborg et al., 2024

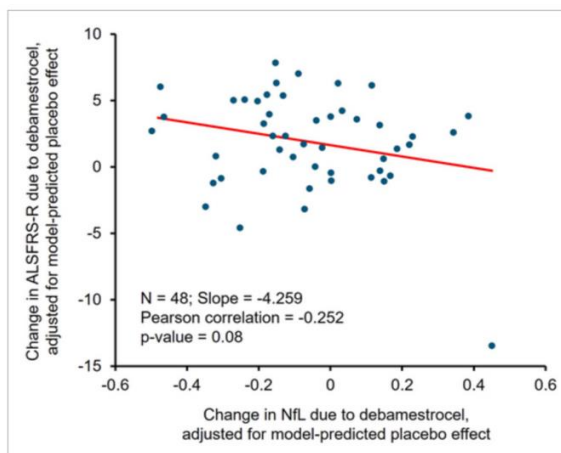
The impact of NurOwn treatment on many biomarkers was rapid, with the largest magnitude of change relative to baseline occurring at the first posttreatment measurement (i.e., two weeks after the first treatment) while for others

the largest impact was not seen until Week 20. For example, NurOwn-treated patients had the largest reduction in MCP-1 compared to placebo at Week 2 (-31% vs. -0.2%) while the neurodegenerative biomarkers NfL and phosphorylated neurofilament heavy chain (pNfH) both had the largest reduction observed from baseline at Week 20 (NfL = -11% vs. -1.6% ($P < 0.05$); pNfH = -13.1% vs. -6.4%).

Genetic analysis was also performed on 124 of 189 participants. Sixty-two percent of participants (77/124) had a positive UNC13A C risk allele (Genotype AC 47%, CC 15%), while the remaining 38% had the wild-type AA genotype. The UNC13A risk allele was previously identified as a susceptibility locus for sporadic ALS ([van Es et al., 2009](#)).

A prespecified statistical model identified CSF levels of NfL, TGF- β 1, and galectin-1 as being predictive of clinical outcomes for NurOwn-treated participants. Less neurodegeneration (lower NfL) and neuroinflammation (higher TGF- β 1) at baseline and higher neuroprotection posttreatment (increase in galectin-1 from baseline) were all associated with slower functional decline in NurOwn-treated participants. When the UNC13A C risk status was included in the model it was identified as statistically contributing to the prediction of clinical outcomes in the trial in an additive manner to the biomarker terms. UNC13A genotypes AC and CC were associated with slower functional decline compared with AA for participants treated with NurOwn.

NurOwn treatment significantly reduced NfL levels from baseline compared to placebo. To better understand the impact the reduction in NfL levels had on clinical endpoints, causal inference models (natural disease models) were used to adjust observed NurOwn treatment effects. Due to the ALSFRS-R floor effect, the analysis was conducted in a subgroup of patients with no evidence of floor effect at baseline (N=49). The following graph shows that reductions in NfL were associated with less functional loss from baseline following NurOwn treatment.



Source: Lindborg et al., 2024

The reduction in NfL from baseline at Week 20 (-11% NurOwn vs. -1.6% placebo; $P < 0.05$) was smaller in magnitude compared to Tofersen (-60% Tofersen vs. 20% placebo; $P < 0.01$) ([Miller et al., 2022](#)). However, using the same causal inference model as Biogen and the FDA ([FDA Briefing Document on Tofersen](#)), it was shown that larger reductions in NfL were associated with smaller changes in ALSFRS-R, which is consistent with published Tofersen data (although with a lower correlation).

It is encouraging that BrainStorm was able to identify biomarkers that correlated with slower disease progression as this provides support to the notion that NurOwn is having a positive impact in ALS patients. The company will again be conducting biomarker analyses in the upcoming Phase 3b trial that will examine markers associated with neuroprotection, neurodegeneration, and neuroinflammation.

Financial Update

On April 1, 2024, BrainStorm announced financial results for 2023. As anticipated, the company did not report any revenues during 2023. Net R&D expenses for 2023 were \$10.8 million compared to \$14.0 million in 2022. The decrease was due to decreased costs associated with the Phase 3 trial and payroll expenses. G&A expenses were \$10.7 million in 2023 compared to \$10.9 million in 2022. The decrease was primarily due to decreased stock-based compensation, rent costs, depreciation, and public relations activities partially offset by increased payroll expenses and travel, consultant, and stock costs.

BrainStorm exited 2023 with approximately \$1.3 million in cash and cash equivalents and the company will need to raise capital before conducting the Phase 3b trial. As of March 27, 2024, the company had approximately 68.3 million shares outstanding and, when factoring in stock options and warrants, a fully diluted share count of 74.3 million.

Conclusion

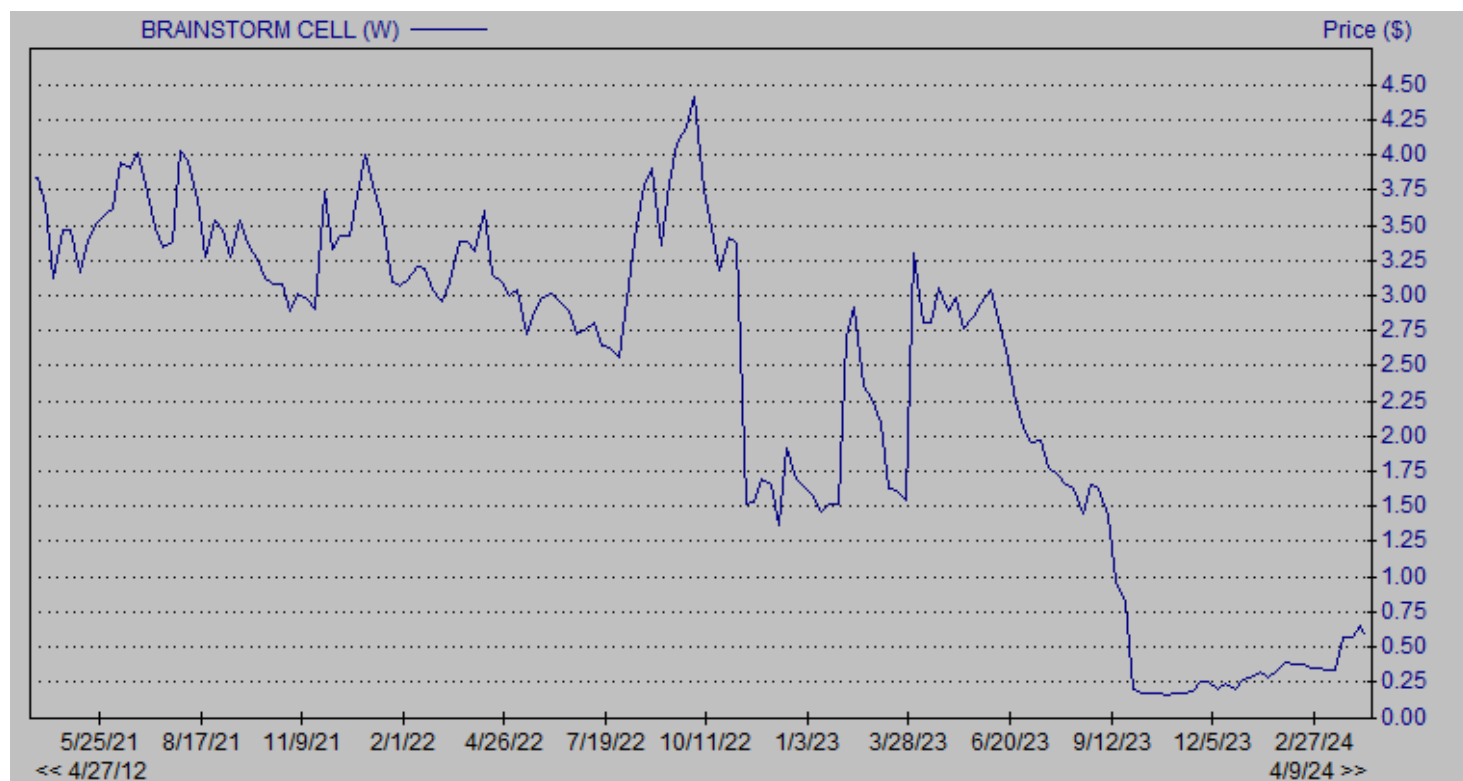
Getting a SPA agreement with the FDA is an important regulatory milestone and we look forward to updates as the company moves closer to initiating the Phase 3b trial. The biomarker data published by the company is intriguing and it provides important insight into the potential mechanisms for how NurOwn is helping ALS patients. We have adjusted our model to account for the de-risked trial design along with the necessary capital that will need to be raised in order to fund the Phase 3b study. Our valuation now stands at \$2.50 per share.

PROJECTED FINANCIALS

Brainstorm Cell Therapeutics	2023 A	Q1 E	Q2 E	Q3 E	Q4 E	2024 E	2025 E	2026 E
MSC-NTF Stem Cells	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0
YOY Growth	-	-	-	-	-	-	-	-
Total Revenues	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0
YOY Growth	-	-	-	-	-	-	-	-
Cost of Goods / Services	\$0.0	\$0	\$0	\$0	\$0	\$0.0	\$0.0	\$0.0
Product Gross Margin	-	-	-	-	-	-	-	-
R&D	\$10.7	\$2.0	\$2.5	\$3.0	\$3.5	\$11.0	\$13.0	\$15.0
% R&D	-	-	-	-	-	-	-	-
SG&A	\$10.7	\$2.5	\$2.7	\$2.8	\$2.9	\$10.9	\$11.0	\$11.5
% SG&A	-	-	-	-	-	-	-	-
Operating Income	(\$21.4)	(\$4.5)	(\$5.2)	(\$5.8)	(\$6.4)	(\$21.9)	(\$24.0)	(\$26.5)
Net Other Income	\$4.2	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0
Pre-Tax Income	(\$17.2)	(\$4.5)	(\$5.2)	(\$5.8)	(\$6.4)	(\$21.9)	(\$24.0)	(\$26.5)
Taxes	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0
Tax Rate	0%	0%	0%	0%	0%	0%	0%	0%
Net Income	(\$17.2)	(\$4.5)	(\$5.2)	(\$5.8)	(\$6.4)	(\$21.9)	(\$24.0)	(\$26.5)
Net Margin	-	-	-	-	-	-	-	-
Reported EPS	(\$0.40)	(\$0.07)	(\$0.07)	(\$0.06)	(\$0.07)	(\$0.28)	(\$0.24)	(\$0.24)
YOY Growth	-	-	-	-	-	-	-	-
Wt. Avg Shares Outstanding	43.1	65.0	70.0	90.0	90.0	78.8	100.0	110.0

Source: Zacks Investment Research, Inc. David Bautz, PhD

HISTORICAL STOCK PRICE



Source: Zacks SCR

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