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BrainStorm Cell Therapeutics, Inc. (BCLI-NASDAQ)

BCLI: Awaiting BrainStorm's Decision on NurOwn® BLA Filing…

Based on our probability adjusted DCF model that takes into account potential future revenues from NurOwn® in ALS, MS, and Alzheimer's, BCLI is valued at $10.00/share. This model is highly dependent upon continued clinical success of NurOwn® and will be adjusted accordingly based upon future clinical results.

Current Price (03/04/21)	$3.64
Valuation	**$10.00**

OUTLOOK

BrainStorm Cell Therapeutics, Inc. (BCLI) has completed a Phase 3 trial of NurOwn in patients with amyotrophic lateral sclerosis (ALS) and reported results in November 2020 that showed the trial did not reach statistical significance for the primary endpoint. However, an analysis of a pre-specified subgroup of patients with ALSFRS scores >35 showed a clinically meaningful treatment response with 34.6% of NurOwn-treated patients being counted as responders compared to only 15.6% of placebo-treated patients. The company is continuing to discuss the full data set with ALS experts and regulatory advisors before deciding whether to submit a BLA. In addition, the company is preparing a manuscript for publication of the full data set from the Phase 3 trial.

SUMMARY DATA

52-Week High	**$17.73**
52-Week Low	**$3.64**
One-Year Return (%)	**-47.70**
Beta	**0.16**
Average Daily Volume (sh)	**1,317,919**
Shares Outstanding (mil)	**36**
Market Capitalization ($mil)	**$130**
Short Interest Ratio (days)	**N/A**
Institutional Ownership (%)	**23**
Insider Ownership (%)	**22**
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 2018 Estimate	**N/A**
P/E using 2019 Estimate	**N/A**

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

ZACKS ESTIMATES

Revenue
(In millions of $)

	Q1 (Mar)	Q2 (Jun)	Q3 (Sep)	Q4 (Dec)	Year (Dec)
2020	0 A	0 A	0 A	0 A	0 A
2021	0 E	0 E	0 E	0 E	0 E
2022					50 E
2023					150 E

Earnings per Share

	Q1 (Mar)	Q2 (Jun)	Q3 (Sep)	Q4 (Dec)	Year (Dec)
2020	-$0.29 A	-$0.25 A	-$0.14 A	-$0.39 A	-$1.07 A
2021	-$0.27 E	-$0.25 E	-$0.24 E	-$0.24 E	-$0.99 E
2022					$0.00 E
2023					$1.89 E

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WHAT'S NEW

Business Update

Awaiting BrainStorm's Decision on NurOwn® BLA Filing

BrainStorm Cell Therapeutics, Inc. (BCLI) has completed a Phase 3 clinical trial for NurOwn as a treatment for amyotrophic lateral sclerosis (ALS) and in November 2020 announced topline results showing that the trial did not reach statistical significance for the primary endpoint, a responder analysis examining the percentage of participants that experienced a 1.25 point per month improvement in the post-treatment Amyotrophic Lateral Sclerosis Functional Rating Scale – Revised (ALSFRS-R) slope. However, an analysis of a pre-specified subgroup of patients with ALSFRS-R scores ≥ 35, of which approximately 30% of trial participants were included, showed a clinically meaningful treatment response with NurOwn with 34.6% of responders compared to 15.6% of responders in placebo-treated participants (*P*=0.288).

On February 22, 2021, BrainStorm announced feedback from the FDA following a meeting with senior leadership in which the agency concluded from their initial review of the Phase 3 data that it does not provide the threshold of substantial evidence that the FDA is seeking to support a Biologics License Application (BLA). The FDA also advised that the recommendation does not preclude BrainStorm from proceeding with a BLA submission.

Following release of the FDA feedback, we spoke with BrainStorm's management to try to understand what the company's plans will be. First, the company is not planning to perform another Phase 3 clinical trial. Second, the company will continue to speak with principal investigators from the Phase 3 trial, ALS Key Opinion Leaders (KOLs), and regulatory advisors before deciding on whether to file a BLA. Third, the company will be submitting a manuscript for publication that will include the full data set from the Phase 3 trial and which the company believes will speak for itself. The overarching theme from BrainStorm is that the company will be sharing the totality of the data from the Phase 3 trial with leading ALS experts from around the world and taking all feedback under advisement before deciding on the best path forward.

The feedback from the FDA was noticed by at least one member of Congress. Jeff Fortenberry is the U.S. Congressman from the 1st Congressional District of Nebraska and the ranking member of the Agriculture, Rural Development, Food and Drug Administration (FDA), and Related Agencies Subcommittee. That subcommittee is responsible for overseeing a number of government agencies and programs, including the FDA. Mr. Fortenberry, who's brother-in-law passed away from ALS, tweeted the following regarding the FDA's formal feedback to BrainStorm (although it should be noted the FDA did not issue any decision on "approving…NurOwn"):

> "My brother-in-law's death from #ALS connected me to so many courageous person suffering from this cruel disease. The @US_FDA just shattered hope, not approving the treatment NurOwn because it only helped some. We can't take much more. Is our government here to help?"

Clearly, the ALS community is important to Mr. Fortenberry, who has sponsored the "Accelerating Access to Critical Therapies for ALS Act", which has garnered 300 co-sponsors. That bill aims to streamline the approval process for treatments of neurodegenerative diseases such as ALS. Mr. Fortenberry expanded upon his views regarding ALS drug development in the Feb. 26, 2021 "Fort Report", a publication his office publishes each week, where he commented that the current drug approval regulatory process "fails to understand that for complex diseases like ALS incremental progress can be monumental" and that "The FDA would rather have a drug with a 3% benefit to every patient than a drug that helps 20% of patients substantively." The report also quoted Dr. Robert Brown, who was a Principal Investigator for the Phase 3 NurOwn trial, as saying, "The FDA is far more restrictive with treatments for neurodegenerative disease like ALS than those for HIV or cancer."

In another twist to the story, on March 2, 2021, the FDA took the unusual step of issuing a statement titled "Update on Amyotrophic Lateral Sclerosis Drug Development" and that "given the tremendous public interest" it was "important to provide high-level information about the status of the NurOwn development program." We believe the release of the statement was likely driven by what we assume to be a massive number of inquiries to the agency regarding the potential approval of NurOwn. The agency stated that the Phase 3 data presented by BrainStorm did not support the proposed clinical benefit, however it should be noted that there was no indication that the FDA has informed the company not to file a BLA.

At this point it's not worth speculating about a potential outcome without knowing whether BrainStorm is going to file a BLA or not. Even if the company does file a BLA, it is going to be an uphill climb to approval, although having a number of strong patient advocacy groups will be helpful, as it no doubt was for Sarepta in gaining approval for eteplirsen.

Investor Presentation Highlights Phase 3 Data

Recently, Dr. Stacy Lindborg, BrainStorm's Executive Vice President and Head of Global Clinical Research, presented some of the data from the Phase 3 trial at an investor conference. The following chart gives the baseline disease characteristics for the Phase 3 trial. While many of the characteristics were very similar between the two treatment groups, the NurOwn cohort had a baseline ALSFRS-R score an average of 1 point lower than the placebo cohort.

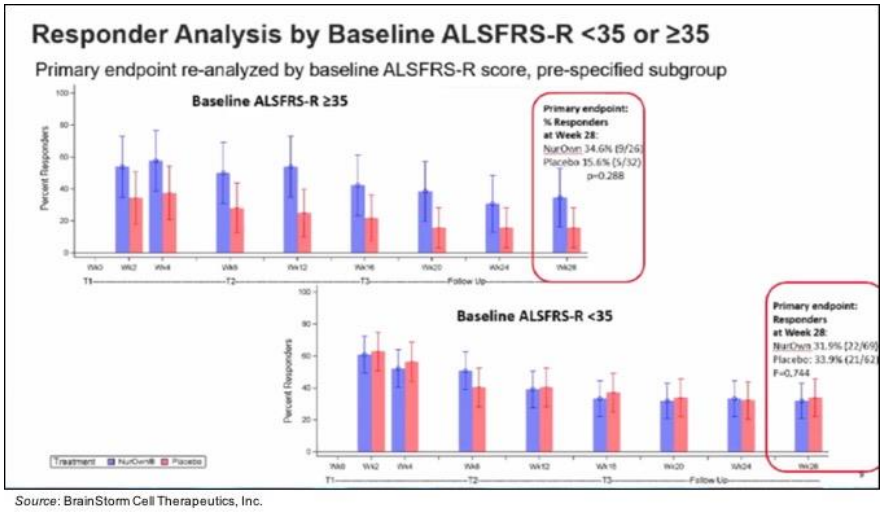
Baseline Disease Characteristics (mITT Population)

More NurOwn-treated participants had ALSFRS-R baseline scores <35

	NurOwn N=95	Placebo N=94	Total N=189
Males (n, %)	68 (71.6)	59 (62.8)	127 (67.2)
Pre-treatment slope (Mean, SD)	-1.7 (0.75)	-1.6 (0.82)	
Baseline ALSFRS-R (Mean, SD)	30.3 (6.5)	31.4 (6.1)	30.9 (6.3)
ALSFRS-R < 35 (n, %)	69 (72.6)	62 (66.0)	131 (69.3)
ALSFRS-R >=35 (n, %)	26 (27.4)	32 (34.0)	58 (30.7)
Baseline SVC (% predicted) (Mean, SD)	76.2 (21.9)	75.8 (19.9)	76.0 (20.9)
Months since diagnosis (months)(Mean, SD)	6.8 (4.35)	6.1 (4.80)	6.4 (4.58)
Months since symptom onset (months)(Mean, SD)	19.6 (5.17)	19.1 (4.90)	19.4 (5.03)
Use of Riluzole (n, %)	65 (68.4)	56 (59.6)	121 (64.0)
El Escorial Possible (n, %)	6 (6.3)	6 (6.4)	12 (6.3)
Lab-supported Probable (n, %)	15 (15.8)	23 (24.5)	38 (20.1)
Probable (n, %)	24 (25.3)	31 (33.0)	55 (29.1)
Definite (n, %)	50 (52.6)	34 (36.2)	84 (44.4)

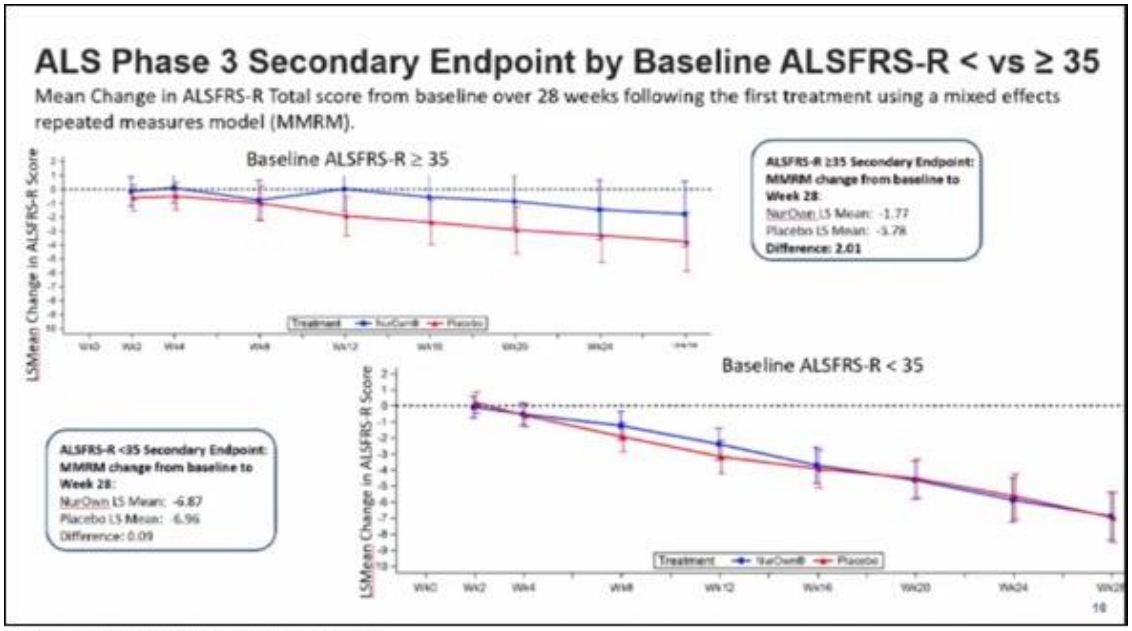
Source: BrainStorm Cell Therapeutics, Inc.

As mentioned previously, an examination of patients stratified by an ALSFRS-R score ≥ 35 showed a greater separation between NurOwn- and placebo treated subjects that was consistent over time, as shown in the following chart. For patients with an ALSFRS-R score < 35 there was no separation between treatment groups.



Source: BrainStorm Cell Therapeutics, Inc.

When looking at mean change in ALSFRS-R from baseline over time, there is an approximately 2-point difference in the ALSFRS-R score that favored the NurOwn-treated group compared to placebo in patients with a baseline ALSFRS-R score ≥ 35. Similar to the responder analysis, there is no difference in treatment groups when examining patients with an ALSFRS-R score < 35.



Source: BrainStorm Cell Therapeutics, Inc.

We can understand how investors might look at a two-point difference in ALSFRS-R and conclude that it's not meaningful. However, it's important to keep in mind that each point on the ALSFRS-R scale represents preserved physical function, and small changes in functional ability can have an outsized effect on a patient's overall quality of life. For example, the following shows the scoring criteria for a patient's ability to swallow. A two-point change in swallowing ability can mean the difference between independently being able to feed themselves (Score of 3) and relying on a feeding tube and the assistance of a caregiver (Score of 1).

4 – Normal eating habits
3 – Early eating problems – occasional choking
2 – Dietary consistency changes
1 – Needs supplemental tube feeding
0 – NPO (exclusively parenteral or enteral feeding)

Additional examples of how a change in ALSFRS-R can have a direct impact on a patient's life include:

- For every 1-point decrease in the ALSFRS-R there was a 7% increase in the risk of death or tracheostomy (Kaufmann *et al*., 2005).
- Each point decline in the ALSFRS-R equates to an approximately 7% reduction in a patient's quality of life (Ilse *et al*., 2015).
- Slowing the decline in ALSFRS-R by 16.5% is equal to a prolongation of 4-5 months median survival (Leigh *et al*., 2004).
- Depression is common among ALS patients, with one study showing the occurrence of depression being significantly associated with physical functioning as measured by the ALSFRS-R (Körner *et al*., 2015).

We believe there is ample evidence to support how important the preservation of physical function, as shown through a decrease in the rate of decline of the ALSFRS-R, is to ALS patients and that treatments that can slow the decline in ALSFRS-R could have a meaningful impact on a patient's life.

Conclusion

We're in a "wait and see" mode regarding the future of NurOwn in ALS as the company continues to gather as much information as possible regarding a potential BLA filing. While we wait for updated guidance from the company, we remind investors that results from the Phase 2 clinical trial of NurOwn in progressive MS should be reported by the end of the first quarter of 2021. Our valuation remains at $10 per share.

PROJECTED FINANCIALS

Brainstorm Cell Therapeutics	2020 A	Q1 E	Q2 E	Q3 E	Q4 E	2021 E	2022 E	2023 E
MSC-NTF Stem Cells	**$0**	**$0**	**$0**	**$0**	**$0**	**$0**	**$50**	**$150**
YOY Growth	-	-	-	-	-	-	-	-
Total Revenues	**$0**	**$0**	**$0**	**$0**	**$0**	**$0**	**$50**	**$150**
YOY Growth	-					-	-	-
Cost of Goods / Services	$0.0	$0	$0	$0	$0	$0.0	$5.0	$15.0
Product Gross Margin	-	-	-	-	-	-	*90.0%*	*90.0%*
R&D	$22.3	$6.0	$6.0	$6.0	$6.0	$24.0	$30.0	$33.0
% R&D	-	-	-	-		-	-	-
SG&A	$9.4	$2.8	$2.8	$2.9	$3.0	$11.5	$15.0	$17.0
% SG&A	-	-	-	-	-	-	-	-
Operating Income	($31.7)	($8.8)	($8.8)	($8.9)	($9.0)	($35.5)	$0.0	$85.0
Net Other Income	($0.1)	$0.0	$0.0	$0.0	$0.0	$0.1	$0.0	$0.0
Pre-Tax Income	($31.8)	($8.8)	($8.8)	($8.9)	($9.0)	($35.4)	$0.0	$85.0
Taxes	$0	$0	$0	$0	$0	$0	$0	$0
Tax Rate	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*
Net Income	($31.8)	($8.8)	($8.8)	($8.9)	($9.0)	($35.4)	$0.0	$85.0
Net Margin	-	-	-	-	-	-	-	-
Reported EPS	**($1.07)**	**($0.27)**	**($0.25)**	**($0.24)**	**($0.24)**	**($0.99)**	**$0.00**	**$1.89**
YOY Growth	-	-	-	-	-	-	-	-
Wt. Avg Shares Outstanding	29.8	33.0	35.5	37.0	38.0	35.9	40.0	45.0

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

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