Zacks Small-Cap Research

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BrainStorm Cell Therapeutics, Inc. (BCLI-NASDAQ)

BCLI: Phase 3 Trial of NurOwn® in Final Stages of Preparation…

Based on our probability adjusted DCF model that takes into account potential future revenues from NurOwn® in ALS, BCLI is valued at $13/share. This model is highly dependent upon continued clinical success of NurOwn® and will be adjusted accordingly based upon future clinical results.

Current Price (06/13/17)	$4.10
Valuation	**$13.00**

OUTLOOK

On May 15, 2017, BrainStorm Cell Therapeutics, Inc. (BCLI) announced financial results for the first quarter of 2017 and provided a business update. The company exited the quarter with approximately $8.3 million in cash, cash equivalents, and marketable securities. We estimate this is sufficient to fund operations into the second quarter of 2018.

The company is currently in the final planning stages for a Phase 3 clinical trial of NurOwn® in patients with amyotrophic lateral sclerosis (ALS). The trial is expected to enroll approximately 200 patients and will take place at six leading ALS clinical sites in the U.S. and Israel. The trial will focus on faster-progressing patients, who demonstrated superior outcomes in the Phase 2 trial of NurOwn®.

SUMMARY DATA

52-Week High	**$4.71**
52-Week Low	**$2.09**
One-Year Return (%)	**71.55**
Beta	**2.27**
Average Daily Volume (sh)	**82,254**
Shares Outstanding (mil)	**19**
Market Capitalization ($mil)	**$77**
Short Interest Ratio (days)	**N/A**
Institutional Ownership (%)	**8**
Insider Ownership (%)	**24**
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 2016 Estimate	**-5.8**
P/E using 2017 Estimate	**-5.5**

Risk Level	**Above Avg.,**
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)
2016	0 A	0 A	0 A	0 A	0 A
2017	0 A	0 E	0 E	0 E	0 E
2018					0 E
2019					0 E

Earnings per Share

(EPS is operating earnings before non-recurring items)

	Q1 (Mar)	Q2 (Jun)	Q3 (Sep)	Q4 (Dec)	Year (Dec)
2015	-$0.10 A	-$0.05 A	-$0.09 A	-$0.03 A	-$0.27 A
2016	-$0.10 A	-$0.10 E	-$0.10 E	-$0.09 E	-$0.38 E
2017					-$0.42 E
2018					-$0.43 E

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WHAT'S NEW

Business Update

Phase 3 Study of NurOwn® in ALS Set to Get Underway

On December 19, 2016, BrainStorm announced that the company had a successful End-of-Phase 2 meeting with the U.S. FDA in which the FDA accepted the key elements of the Phase 3 program for NurOwn® in amyotrophic lateral sclerosis (ALS). The planned Phase 3 clinical trial will be a randomized, double blind, placebo controlled, multi-dose trial that will take place at six leading ALS centers in both the U.S. and Israel. We anticipate approximately 200 patients being enrolled in the trial randomized 1:1 to NurOwn® or placebo. Cells will be extracted from each patient one time prior to treatment, with all administrations of NurOwn® derived from the same extraction of cells. As in previous studies, there will be a 3-month run-in period prior to the first treatment with two additional NurOwn® treatments occurring two and four months following the first treatment. The primary outcome of the study is the ALSFRS-R score responder analysis. The company is focusing the trial on faster-progressing ALS patients since these patients demonstrated superior outcomes in the Phase 2 trial of NurOwn®. We anticipate the trial initiating in the second quarter of 2017.

The company recently announced that City of Hope's Center for Biomedicine and Genetics would produce clinical supplies of NurOwn® for all U.S. medical centers involved in the Phase 3 study. On May 16, 2017, the company announced that it had initiated training of the cell manufacturing team that will be producing clinical supplies of NurOwn®.

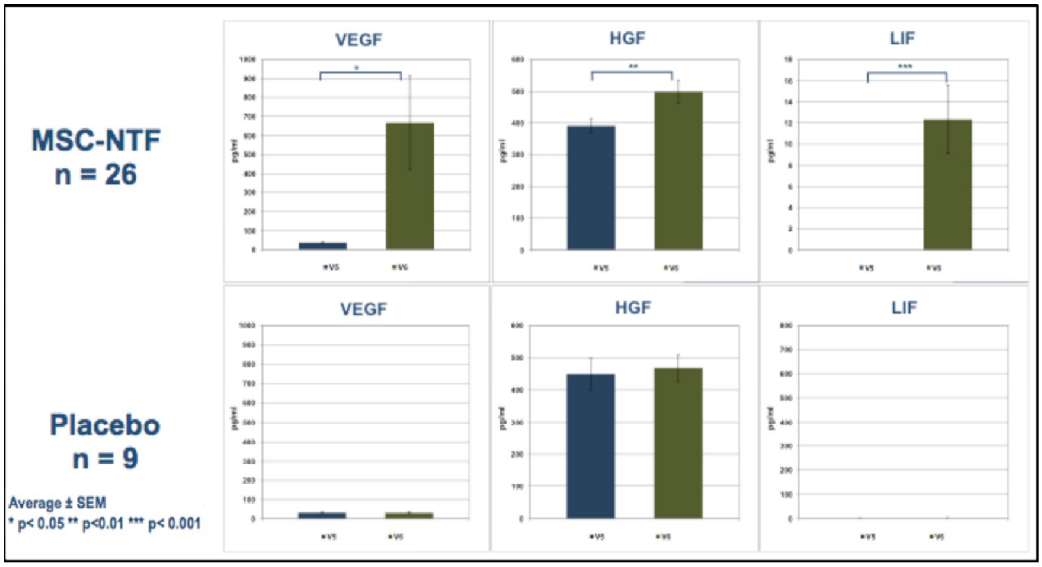
In addition, the cryopreservation process has been validated, which involved a comparison of NurOwn® derived from fresh mesenchymal stem cells (MSCs) to NurOwn® derived from cryopreserved MSC. The results showed that MSCs stored in liquid nitrogen for prolonged periods of time continued to maintain their characteristics after thawing. This was an important milestone for the company as it will streamline the process of producing NurOwn®, will decrease the number of aspiration procedures for the patients taking part in the multi-dose Phase 3 study, and lower the cost of goods for NurOwn®.

New Data Presented From Phase 2 NurOwn® Trial

On December 9, 2016, Dr. James Berry presented data from the Phase 2 clinical trial of NurOwn® in patients with amyotrophic lateral sclerosis (ALS) at the 27th International Symposium on ALS/MND. Dr. Berry was a Principal Investigator of that trial. Below we highlight some of the data, including increased levels of neurotrophic factors in the cerebrospinal fluid (CSF) of patients following NurOwn® treatment as well as statistically significant changes in the rate of disease progression in patients whose disease was rapidly progressing.

Increased Levels of Neurotrophic Factors in Patients Treated with NurOwn®

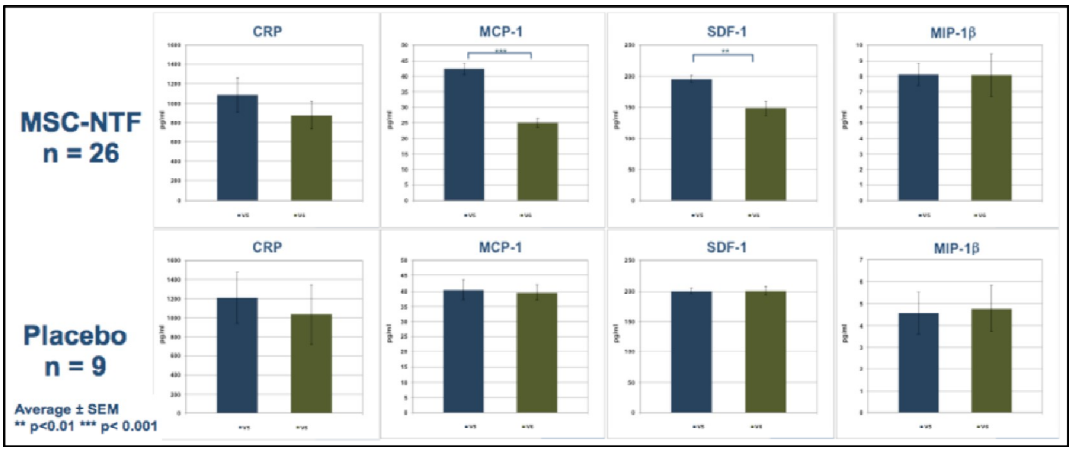
Treatment of patients in the Phase 2 trial involved a single intrathecal injection of NurOwn® cells. Analysis of the CSF was performed to analyze whether increased levels of neurotrophic factors could be detected, which would also indicate the cells retained biological activity following injection. The following graphs show that there was a significant increase in the levels of vascular endothelial growth factor (VEGF), hepatocyte growth factor (HGF), and leukemia inhibitor factor (LIF) in patients treated with NurOwn® following treatment with no change in the neurotrophic factors seen in placebo-treated patients.



***Source*: BrainStorm Cell Therapeutics, Inc.**

In animal models of ALS, treatment with VEGF leads to improvements in motor function, protection of motor neurons, and increased survival (Zheng *et al*., 2004). Increased levels of HGF were shown to reduce motor neuron degeneration and increase survival in a mouse model of ALS (Sun *et al*., 2002). LIF was also shown to reduce motor neuron degeneration in a mouse model of ALS (Azari *et al*., 2001).

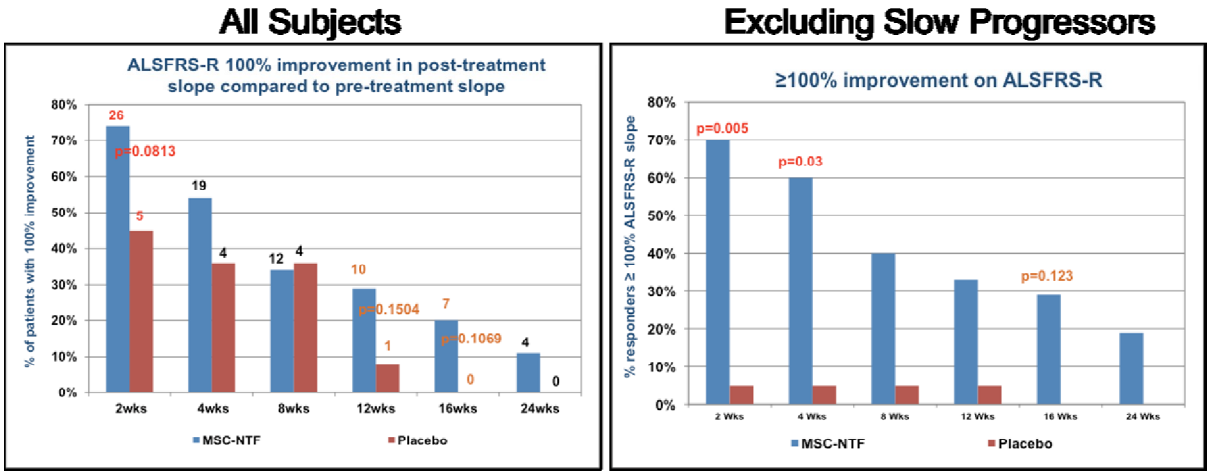
In addition to an increase in neurotrophic factors, there was also a statistically significant decrease in the inflammatory markers monocyte chemoattractant protein-1 (MCP-1) and stromal cell derived factor 1 (SDF-1). MCP-1 is produced by activated microglia (Sargsyan *et al*., 2009) and is known to be involved in various diseases of the central nervous system. MCP-1 was found to be significantly increased in serum and CSF of ALS patients. Blocking the interaction between SDF-1 (CXCL12) and its receptor (CXCR4) increases survival in a mouse model of ALS (Rabinovich-Nikitin *et al*., 2016). Furthermore there was also a statistically significant and meaningful inverse correlation between the increase in NTFs and the decrease in inflammatory factors. The decrease in MCP-1 correlated significantly with reduction in disease progression, suggesting a direct effect of the cells by a paracrine mechanism.



***Source*: BrainStorm Cell Therapeutics, Inc.**

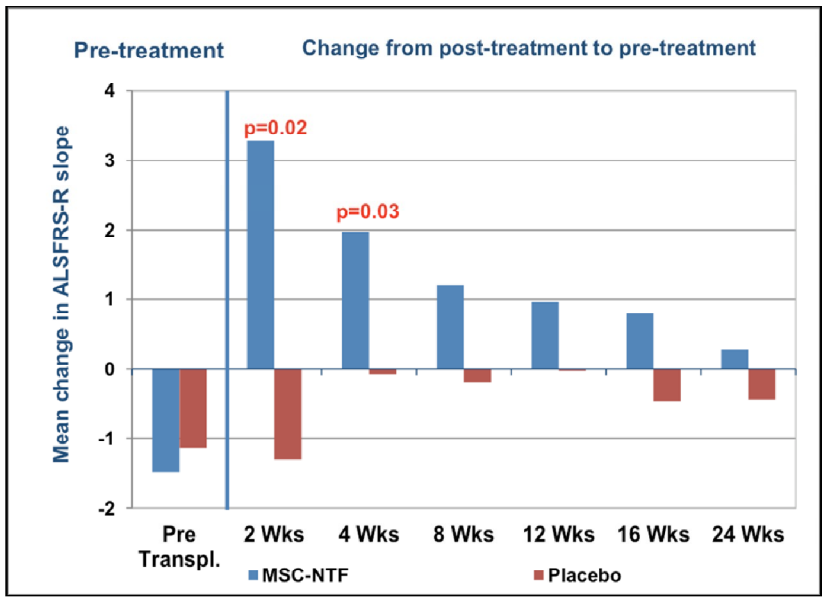
NurOwn® Effects Most Pronounced in Rapid Progressors

A predefined subgroup analysis was performed that excluded patients who were “slow progressors”, defined as ≤ two point decline in the ALSFRS-R from screening to baseline. The ALSFRS-R is a scoring system that measures gross motor tasks, fine motor tasks, bulbar functions, and respiratory functions to quantify the rate of ALS progression. The following graphs show that many more patients in the NurOwn® treatment group had a halt in decline or improvement in ALSFRS-R following treatment (≥100% improvement in rate of decline of ALSFRS-R), which was particularly evident when the slow progressors were excluded.



***Source*: BrainStorm Cell Therapeutics, Inc.**

The following graph shows that patients treated with NurOwn® actually showed an average <u>improvement</u> in the change in ALSFRS-R of approximately three points at two weeks following treatment, with the average change in the rate of decline still positive all the way out to 24 weeks! This was for a group of patients that had an average decline of approximately -1.3 points in the pre-treatment period. For a terminal illness that is marked by steady decline for virtually all patients, these results are very encouraging.



***Source*: BrainStorm Cell Therapeutics, Inc.**

We believe that the results presented by Dr. Berry show that NurOwn® cells are active following administration (as shown by an increase in neurotrophic factors and decrease in inflammatory factors in the CSF) and result in clinically meaningful changes in the course of the disease (as shown by the percentage of patients with ≥100% improvement in rate of decline of ALSFRS-R), particularly in those patients that were progressing most rapidly. Since these results were obtained with only one administration of NurOwn® we anticipate that multiple administrations of NurOwn® could extend the results seen for an even greater period of time.

<u>Preclinical Data Shows Potential for NurOwn® in Treatment of Autism</u>

On April 19, 2017, BrainStorm announced the publication of a preclinical study evaluating the use of NurOwn® in a mouse model of autism in the journal *Behavioral Brain Research* (Perets *et al*., 2017). Mice in the study were injected into the cerebral lateral ventricles with NurOwn® cells or placebo and evaluated through a series of behavioral tests. The BTBR mouse model of autism was used in the study. Results showed that:

- **MSC and NurOwn® transplantation improved social behavior**: BTBR mice injected with a single dose of either mesenchymal stem cells (MSC) or NurOwn® spent significantly more time initiating nose to nose social interaction with a stranger male mouse compared to placebo-treated BTBR mice (*P*<0.01). This effect was maintained for six months.

- **NurOwn® transplantation ameliorated repetitive behaviors for six months**: BTBR mice injected with a single dose of NurOwn® showed significantly lower levels of self-grooming compared to placebo-treated for up to six months. The effect was only significant for MSC-treated BTBR mice for one month.

- **NurOwn® treatment, but not MSC, decreased cognitive rigidity**: BTBR mice injected with NurOwn® showed significant improvement in their flexibility to adjust to changes in their environment one month after treatment. This effect was not seen in MSC-treated BTBR mice.

The results from this study showed that a single treatment of NurOwn® decreased autistic behavior in BTBR mice for up to six months and that NurOwn® was superior to MSC treatment. This study was the first to show improvement across all behavioral phenotypes measurable in mice and the first to show a long-lasting effect of a single treatment for six months.

Financial Update

On May 15, 2017, BrainStorm announced financial results for the first quarter of 2017. As expected, the company did not report any revenue. Net loss for the first quarter of 2017 was $1.8 million, or $0.10 per share. R&D expenses were $0.94 million in the first quarter of 2017 compared to $0.99 million in the first quarter of 2016. The slight decrease was due to a decrease in costs for the Phase 2 clinical trial in the U.S. G&A expenses were $0.83 million in the first quarter of 2017, which was the same as in the first quarter of 2016. The company exited the first quarter of 2017 with cash, cash equivalents, and marketable securities of $8.3 million. We anticipate this being sufficient to fund operations through the second quarter of 2018. On June 13, 2017, BrainStorm announced it received a grant totaling approximately $2.1 million from the Israel Innovation Authority (IIA, formerly the Office of the Chief Scientist). Thus far, BrainStorm has received approximately $7.5 million in total from the IIA since 2007. The company will be required to pay mid-single digit royalties to the IIA based on sales of NurOwn® up to the total cumulative amount of the IIA grants received plus interest.

As of May 5, 2017, BrainStorm had approximately 18.7 million shares of common stock outstanding. When factoring in the 6.3 million warrants and 0.9 million stock options the fully diluted share count is approximately 25.9 million.

Conclusions

We are excited for the Phase 3 multi-dose study of NurOwn® to get underway, as we believe the previous results reported by the company point to the potential for greater efficacy with repeated doses of the drug. Perhaps the most encouraging data reported thus far has to do with the cohort of patients whose disease was progressing the fastest in the pre-treatment period, as these are the patients most in need of an effective treatment and who appeared to respond best to NurOwn® treatment. Thus, there is ample evidence supporting the company's effort to focus on this cohort of patients in the upcoming Phase 3 trial.

Before becoming generic, Riluzole® cost $50,000 per year and was shown to only extend survival of ALS patients by two to three months. The results previously reported by the company indicate that NurOwn® may be able to slow down the progression of ALS, and for some patients it may even lead to disease stabilization. Multiple doses of NurOwn® could increase these effects. As such, we currently model for NurOwn™ to cost $100,000 per year. However, once we learn more about the Hospital Exemption, particularly how much the treatment costs under this program, we may modify our pricing assumptions. We continue to believe that upon approval NurOwn® could generate peak revenues of over $1 billion. Our current valuation for BrainStorm is $13 per share.

PROJECTED FINANCIALS

BrainStorm Cell Therapeutics, Inc.
Income Statement

Brainstorm Cell Therapeutics	2016 A	Q1 A	Q2 E	Q3 E	Q4 E	2017 E	2018 E	2019 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	$2.3	$0.9	$1.0	$1.0	$1.0	$3.9	$5.0	$6.0
% R&D	-	-	-	-	-	-	-	-
SG&A	$2.8	$0.8	$0.8	$0.8	$0.8	$3.2	$4.0	$4.5
% SG&A	-	-	-	-	-	-	-	-
Operating Income	($5.1)	($1.8)	($1.8)	($1.8)	($1.8)	($7.2)	($9.0)	($10.5)
Net Other Income	$0.1	($0.0)	$0.0	$0.0	$0.0	$0.0	$0.1	$0.1
Pre-Tax Income	($5.0)	($1.8)	($1.8)	($1.8)	($1.8)	($7.1)	($8.9)	($10.4)
Taxes	$0	$0	$0	$0	$0	$0	$0	$0
Tax Rate	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*
Net Income	($5.0)	($1.8)	($1.8)	($1.8)	($1.8)	($7.1)	($8.9)	($10.4)
Net Margin	-	-	-	-	-	-	-	-
Reported EPS	**($0.27)**	**($0.10)**	**($0.10)**	**($0.10)**	**($0.09)**	**($0.38)**	**($0.42)**	**($0.43)**
YOY Growth	-	-	-	-	-	-	-	-
Wt. Avg Shares Outstanding	18.7	18.7	18.7	18.7	18.8	18.7	21.0	24.0

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

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