Zacks Small-Cap Research

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# BrainStorm Cell Therapeutics, Inc. (BCLI-NASDAQ)

## *BCLI: Phase 3 Trial of NurOwn® to Initiate Soon…*

Based on our probability adjusted DCF model that takes into account potential future revenues from NurOwn® in ALS, BCLI is valued at $15/share. This model is highly dependent upon continued clinical success of NurOwn® and will be adjusted accordingly based upon future clinical results.

| | |
|---|---|
| Current Price (08/23/17) | $3.98 |
| **Valuation** | **$15.00** |

## OUTLOOK

On August 16, 2017, BrainStorm Cell Therapeutics, Inc. (BCLI) held a conference call to provide a business update. The company is in the final stages of preparation before launching the Phase 3 clinical trial of NurOwn® in patients with amyotrophic lateral sclerosis (ALS). The trial will enroll approximately 200 patients randomized 1:1 to receive 3 injections of NurOwn® or placebo over a 6 month time period. The company is focused on "fast progressors", as judged by a rapid decline in the ALSFRS-R score during the 3-month lead-in to randomization. We believe the trial will take approximately 18-20 months to conduct, and the company has indicated there are a large number of patients that are eager to sign up for the trial, thus we do not believe there will be any issues with patient enrollment.

## SUMMARY DATA

| | |
|---|---|
| **52-Week High** | **$4.94** |
| **52-Week Low** | **$2.09** |
| **One-Year Return (%)** | **57.94** |
| **Beta** | **2.29** |
| **Average Daily Volume (sh)** | **96,571** |
| **Shares Outstanding (mil)** | **19** |
| **Market Capitalization ($mil)** | **$75** |
| **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 A | 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 | -$0.06 A | -$0.09 E | -$0.09 E | -$0.34 E |
| 2017 | | | | | -$0.42 E |
| 2018 | | | | | -$0.43 E |

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## WHAT'S NEW

**Financial Update**

On August 14 2017, BrainStorm announced financial results for the second quarter of 2017. As expected, the company did not report any revenue. Net loss for the second quarter of 2017 was $1.0 million, or $0.06 per share. R&D expenses were $0.44 million in the second quarter of 2017 compared to $0.15 million in the second quarter of 2016. The increase was due to an increase in payroll and stock-based compensation partially offset by a decrease in costs related to the U.S. clinical trial. G&A expenses were $0.64 million in the second quarter of 2017 compared to $0.83 million in the second quarter of 2016. The decrease was primarily due to a decrease in stock-based compensation. The company exited the second quarter of 2017 with cash, cash equivalents, and marketable securities of $6.7 million.

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.

On July 21, 2017, BrainStorm announced that the company has received a $16 million grant from the California Institute for Regenerative Medicine (CIRM) to help fund the company's upcoming Phase 3 clinical trial of NurOwn® in patients with amyotrophic lateral sclerosis (ALS).

As of August 7, 2017, BrainStorm had approximately 18.8 million shares of common stock outstanding. When factoring in the 5.9 million warrants and 0.9 million stock options the fully diluted share count is approximately 25.6 million.

**Business Update**

*Awarded $16 Million Grant From CIRM to Help Fund Phase 3 Trial*

On July 21, 2017, BrainStorm announced that the company has received a $16 million grant from the California Institute for Regenerative Medicine (CIRM) to help fund the company's upcoming Phase 3 clinical trial of NurOwn® in patients with amyotrophic lateral sclerosis (ALS). CIRM was established in 2004 through Proposition 71: the California Stem Cell Research and Cures Initiative. While CIRM initially concerned itself with supporting scientists who were just entering the stem cell field, the organization has recently launched CIRM 2.0, which aims to make funding available sooner for projects that are likely to bring stem cell therapies to patients with unmet medical needs (e.g., late stage clinical trials).

The grant helps the company in two important ways: 1) it removes the funding overhang that had been dragging on the stock as investors were unsure of how the company was going to fund the upcoming Phase 3 clinical trial, and 2) it validates NurOwn® and provides significant credibility to the data that the company has compiled thus far from the ALS program.

In addition to the grant from CIRM, the company also recently announced a non-dilutive grant from the Israel Innovation Authority (formerly the Office of the Chief Scientist) for $2.1 million to support the development of NurOwn®, thus the company is continuing to successfully obtain capital in a shareholder friendly way.

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

BrainStorm has entered the final planning stage for the Phase 3 clinical trial of NurOwn® in patients with ALS, which includes accomplishing the following activities:

- 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 and that it had initiated training of the cell manufacturing team that will be producing clinical supplies of NurOwn®.

- BrainStorm has selected Worldwide Clinical Trials as its Clinical Research Organization for the Phase 3 trial of NurOwn. Worldwide has extensive expertise and experience in managing pivotal Phase 3 clinical trials, particularly in the field of ALS and neurology.

- The company has signed definitive agreements with Massachusetts General Hospital and California Pacific Medical Center, with Drs. Merit Cudkowicz and Robert G. Miller agreeing to participate as principal investigators.

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®. According to the company, approximately 50-60% of ALS patients could be considered "fast progressors". Management has indicated there is a strong interest in the trial from patients and physicians, and there are already a substantial number of patients that are ready to enroll in the trial. Thus, we do not anticipate there being any issues with patient enrollment. We believe it will take approximately 18-20 months from the time patient's first begin enrolling until topline data is announced.

*Edavarone Approval Shows FDA Eagerness to Approve New ALS Treatments*

On May 5, 2017, the U.S. Food and Drug Administration (FDA) announced the approval of edaravone for the treatment of amyotrophic lateral sclerosis (ALS). This is the first medication approved for the treatment of ALS since riluzole in 1995. As stated in the press release, the FDA was eager to engage with the drug's developer, Mitsubishi Tanabe Pharmaceuticals Corp., following the approval of the drug in Japan, since there are so few treatment options for ALS patients in the U.S.

We believe the news of edaravone's approval is a positive for BrainStorm. It shows the FDA is willing and eager to work with a company to get an ALS treatment approved in a timely fashion and it shows the type of efficacy data that the FDA is looking for in an ALS treatment. Lastly, BrainStorm's data for NurOwn® is highly comparable to the edaravone data, which is encouraging given that BrainStorm's data is based on a single dose of NurOwn® while edaravone is required to be infused 64 times.

Edaravone

Edaravone is a potent scavenger of oxygen radicals. While the underlying mechanisms responsible for causing ALS are unknown, it is believed that oxidative stress plays some role in the development of the disease. This is supported by the fact that mutations in superoxide dismutase 1 (SOD1) cause familial ALS. SOD1 is responsible for converting superoxide radicals to oxygen and hydrogen peroxide.

In an ALS mouse model that involves a mutation in SOD1, administration of edaravone resulted in reduced motor decline and preserved motor neurons in the spinal cord. Similar results were seen in a rat model of ALS. Based on these results, edaravone was tested in ALS patients in three different clinical trials.

Edaravone Clinical Trials

Edaravone was originally tested in a Phase 2 clinical trial involving 20 ALS patients. The study was an open-label comparison study that evaluated patients before and after treatment with edaravone. Results showed a statistically significant difference in the change in ALS function rating scale revised (ALSFRS-R) before treatment (4.7 points) compared to during the treatment period (2.3 points; $P$=0.036).

The first Phase 3 clinical trial of edaravone was conducted in 205 patients randomized to receive edaravone (n=101) or placebo (n=104) (Abe *et al*., 2014). Treatment consisted of i.v. infusions given over 60 min for the first 14 days of cycle 1 (followed by 14 days off drug), and then 10 of the first 14 days during cycles two through six, with 14 days off drug following treatment in each cycle. The primary endpoint was the change in ALSFRS-R during the 24-week treatment period. Results showed that the change in ALSFRS-R scores were -5.70 and -6.35 in the edaravone and placebo groups, respectively, which did not represent a statistically significant different ($P$=0.411). No serious adverse events were reported and the level and frequency of adverse events were similar between the two treatment groups. A post-hoc analysis suggested that edaravone could be efficacious in a restricted subgroup

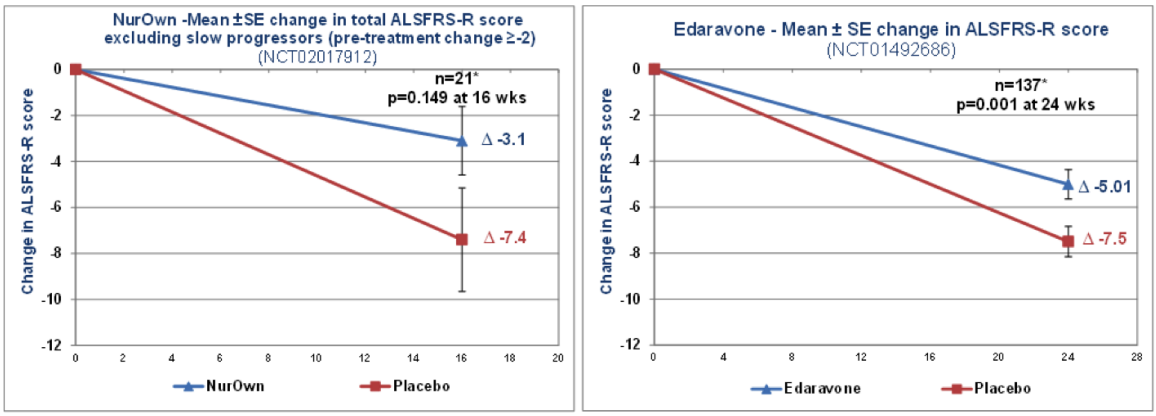
that includes recently diagnosed patients with milder disease symptoms.

Based on the post-hoc analysis, a second Phase 3 clinical trial was conducted that was restricted to patients with a disease duration of <2 years and independent activity of daily living (Tanaka *et al.*, 2015). A total of 134 patients were randomized to receive edaravone (n=68) or placebo (n=66) for six months, with treatment given the same as in the first Phase 3 clinical trial. The change in ALSFRS-R score from baseline at six months was -5.01 in the edaravone group and -7.50 in the placebo group (*P*=0.001). Once again, adverse events were similar between the edaravone and the placebo groups.

The results of the two Phase 3 trials of edaravone show that the drug is efficacious in ALS patients, however due to the small effect size its use may be limited to patients who are newly diagnosed and who have mild symptoms. The drug will be sold under the name Radicava by MT Pharma America, a subsidiary of Mitsubishi Tanabe Pharmaceuticals, and will cost approximately $145,000 per year.

Edaravone vs. NurOwn®

The approval of edaravaone helps to give an idea of what type of results the FDA is looking for in order to gain approval as an ALS treatment. Thus, we feel it is worth comparing the results seen in the pivotal trial of edaravone to those seen with NurOwn®. The following graph shows a comparison between edaravone 24-week data and NurOwn® 16-week data, showing that the two treatments are quite comparable.




*Source*: BrainStorm Cell Therapeutics, Inc.

A couple of things to keep in mind when comparing the data are:

1) The NurOwn® study was conducted in the U.S., while the edaravone study was conducted in Japan, thus differences in the standard of care could contribute to any differences in the rate of change in ALSFRS-R, on top of the inherent differences that would be expected based on the variability of ALS patient progression.
2) The difference in ALSFRS-R at 16 weeks in NurOwn®-treated "fast progressors" compared to placebo-treated was 4.3 points, while the difference between edaravone-treated and placebo-treated patients at 24 weeks was only 2.5 points. It is reasonable to think that if the NurOwn® study had gone out to 24 weeks the difference between NurOwn® and placebo-treated patients would only increase more.
3) The reason that the NurOwn® results were not significant is because only 21 patients were included in the analysis while the edaravone results included 137 patients.
4) The NurOwn® results are based a single treatment. Edaravone was dosed 64 times, with each dosage consisting of a 1 hour intravenous infusion.

*Appointment of Vice President of Patient Advocacy and Government Affairs*

On August 7, 2017, BrainStorm announced the appointment of Mary Kay Turner to the position of Vice President of Patient Advocacy and Government affairs. Ms. Turner will lead the company's outreach program and work directly with patient advocacy groups to better understand patient's needs such that BrainStorm's efforts can be better aligned with the needs of the ALS community. She recently worked at Mitsubishi Tanabe Pharma America as Head of Patient Advocacy and Communications. Prior to Mitsubishi Tanabe, Ms. Turner spent 26 years at Bristol Myers Squibb where she helped establish the company's advocacy function while holding various positions of increasing responsibility in sales leadership, patient advocacy, and government affairs.

**Conclusions**

The non-dilutive CIRM grant is yet another example of the shareholder friendly way in which BrainStorm is continuing to accumulate capital to fund development of NurOwn®. We believe investors were concerned about how the company was going to fund the Phase 3 trial, which resulted in a drag on the stock price. We estimate the company has sufficient capital to fund operations through 2018, and can now choose to raise additional funds on its terms, which could easily be accomplished through an at-the-market agreement or a warrant exchange, like the company did in 2014. Either way, investors concern with funding the Phase 3 trial should be alleviated by the CIRM grant.

In addition to allaying the fears associated with financing the trial, the CIRM grant should also serve to alleviate any trepidation investors had regarding the integrity of the data BrainStorm has accumulated thus far for NurOwn® in ALS. The CIRM review process includes a highly rigorous evaluation of the clinical data accumulated thus far by a panel of leading scientists, thus awarding a grant is a validation of the technology and clinical data. While this does not guarantee success in the Phase 3 trial, it is highly supportive of the approach BrainStorm is taking and the data up to this point.

The approval of edaravone is exciting news for ALS patients, as it is the first medicine approved for the condition in 22 years. However, it is also good news for BrainStorm and its shareholders, as the company now has some idea of the type of data that the FDA would like to see in order to approve an ALS treatment. As shown in the graph above, one treatment of NurOwn® results in comparable, if not better, efficacy as edaravone as measured by change in ALSFRS-R score.

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. We continue to believe that upon approval NurOwn® could generate peak revenues of over $1 billion. With additional non-dilutive funding in place we have increased our valuation to $15 per share.

## PROJECTED FINANCIALS

**BrainStorm Cell Therapeutics, Inc.**
Income Statement

| Brainstorm Cell Therapeutics | 2016 A | Q1 A | Q2 A | 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 | $0.4 | $1.0 | $1.0 | $3.4 | $5.0 | $6.0 |
| *% R&D* | - | - | - | - | - | - | - | - |
| SG&A | $2.8 | $0.8 | $0.6 | $0.8 | $0.8 | $3.1 | $4.0 | $4.5 |
| *% SG&A* | - | - | - | - | - | - | - | - |
| Operating Income | ($5.1) | ($1.8) | ($1.1) | ($1.8) | ($1.8) | ($6.4) | ($9.0) | ($10.5) |
| Net Other Income | $0.1 | ($0.0) | $0.0 | $0.0 | $0.0 | $0.1 | $0.1 | $0.1 |
| Pre-Tax Income | ($5.0) | ($1.8) | ($1.0) | ($1.8) | ($1.8) | ($6.4) | ($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) | ($1.0) | ($1.8) | ($1.8) | ($6.4) | ($8.9) | ($10.4) |
| *Net Margin* | - | - | - | - | - | - | - | - |
| **Reported EPS** | **($0.27)** | **($0.10)** | **($0.06)** | **($0.09)** | **($0.09)** | **($0.34)** | **($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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## HISTORICAL STOCK PRICE




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