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BioXcel Therapeutics, Inc. (BTAI-NASDAQ)

BTAI: Phase 3 Data from SERENITY Trials in July 2020…

Based on our probability adjusted DCF model that takes into account potential future revenues of BXCL501 and BXCL701, BTAI is valued at $92.00/share. This model is highly dependent upon continued clinical success of the company's pipeline and will be adjusted accordingly based on future clinical results.

Current Price (05/14/20)	$43.07
Valuation	**$92.00**

OUTLOOK

On May 12, 2020, BioXcel Therapeutics, Inc. (BTAI) announced financial results for the first quarter of 2020 and provided a business update. The company has a number of key inflection points coming up over the following year, and thus far the company has been minimally impacted by the coronavirus epidemic. We anticipate topline data for the SERENITY I and SERENITY II Phase 3 trials of BXCL501 for the treatment of agitation in patients with schizophrenia and bipolar disorder in July 2020, topline data for the TRANQUILITY Phase 1b/2 of BXCL501 for the treatment of agitation in patients with dementia in mid-2020, topline data from a Phase 2 biomarker trial of BXCL501 for the treatment of agitation in schizophrenia patients in 2H20, the initiation of the RELEASE Phase 1b/2 clinical trial of BXCL501 in patients suffering from opioid withdrawal, and data for BXCL701 in a combination trial with Keytruda in patients with tNEPC in 4Q20.

SUMMARY DATA

52-Week High	**$43.07**
52-Week Low	**$4.05**
One-Year Return (%)	**301.02**
Beta	**2.10**
Average Daily Volume (sh)	**476,923**
Shares Outstanding (mil)	**20**
Market Capitalization ($mil)	**$871**
Short Interest Ratio (days)	**N/A**
Institutional Ownership (%)	**22**
Insider Ownership (%)	**9**
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 2019 Estimate	**-17.3**
P/E using 2020 Estimate	**-18.2**

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

ZACKS ESTIMATES

Revenue
(in millions of $)

	Q1 (Mar)	Q2 (Jun)	Q3 (Sep)	Q4 (Dec)	Year (Dec)
2019	0 A	0 A	0 A	0 A	0 A
2020	0 A	0 E	0 E	0 E	0 E
2021					0 E
2022					0 E

Earnings per Share

	Q1 (Mar)	Q2 (Jun)	Q3 (Sep)	Q4 (Dec)	Year (Dec)
2019	-$0.46 A	-$0.54 A	-$0.57 A	-$0.42 A	-$2.02 A
2020	-$0.79 A	-$0.63 E	-$0.64 E	-$0.67 E	-$2.71 E
2021					-$1.83 E
2022					$0.07 E

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WHAT'S NEW

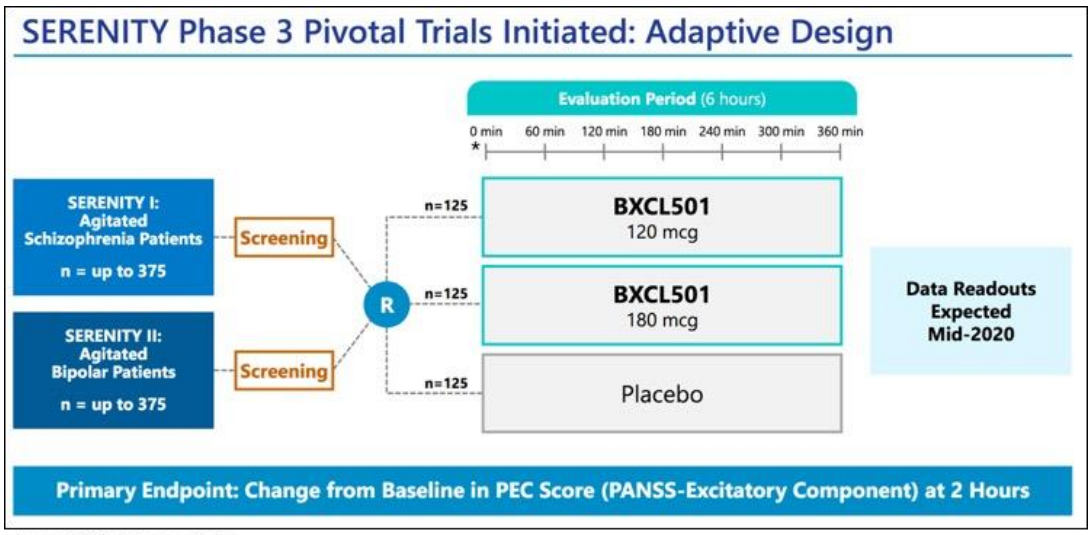
BioXcel Therapeutics, Inc. (BTAI) currently has two lead development programs: BXCL501 – a sublingual formulation of the α2a adrenergic receptor agonist dexmedetomidine (Dex) for the treatment of neurological and psychiatric disorders; and BXCL701 – an immuno-oncology agent for treatment of a rare form of prostate cancer and pancreatic cancer. The company has a number of data readouts and catalysts expected over the next 6-12 months, including:

- Phase 3 data for BXCL501 in treating agitation in schizophrenia and bipolar disorder in July 2020
- Phase 1b/2 data for BXCL501 in treating agitation in dementia in mid-2020
- Phase 2 biomarker data for BXCL501 in 2H2020
- Phase 1b/2 trial for BXCL501 in treating opioid withdrawal to initiate shortly
- NDA filing for BXCL501 in treating agitation in schizophrenia and bipolar disorder in 2021
- Phase 2 data for BXCL701 in combination with pembrolizumab in patients with neuroendocrine prostate cancer in 4Q20
- Initial data from investigator-initiated Phase 2 trial of BXCL701 in combination with pembrolizumab in multiple solid tumors in 2H20
- Initiation of a Phase 2 trial of BXCL701 in combination with bempegaldesleukin and avelumab in pancreatic cancer pending the outcome of the bempegaldesleukin/avelumab combo trial

Business Update

Topline Data from Phase 3 Trials in Schizophrenia and Bipolar Disorder in Mid-2020

BioXcel is currently conducting the pivotal Phase 3 SERENITY (Sub-Lingual DExmedetomidine in Agitation Associated With SchizophRENIa and Bipolar Disorder STudY) trials of BXCL501 for acute treatment of agitation in patients with schizophrenia and bipolar disorder. The primary endpoint of both trials will be a reduction of symptoms of acute agitation using the Positive and Negative Syndrome Scale – Excitatory Component (PEC), a validated regulatory endpoint for quantifying agitation comprised of five elements associated with agitation scored from 1 (minimum) to 7 (maximum) (Montoya *et al*., 2011), as measured from baseline compared to placebo. A key secondary endpoint will be determining the earliest time where an effect on agitation is apparent by measuring the change in PEC score from baseline. An outline of the SERENITY program is shown below.

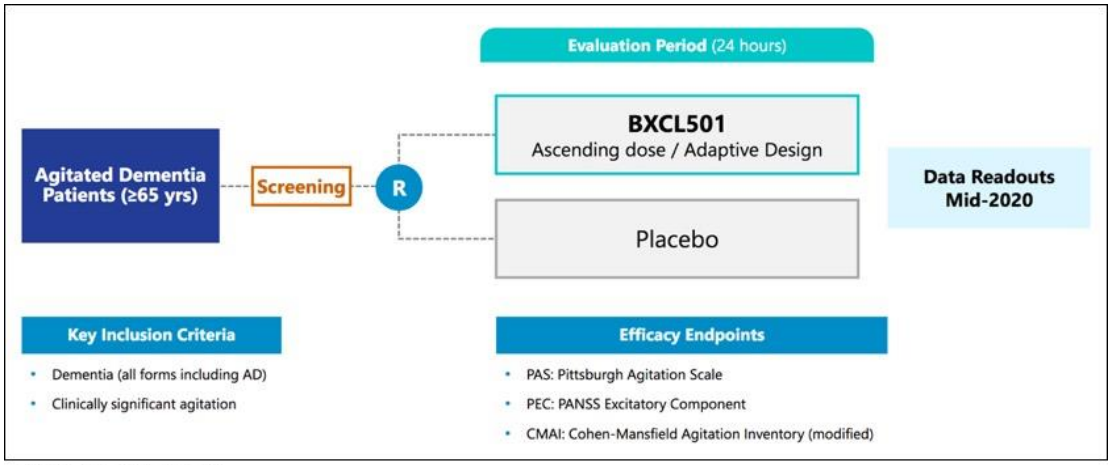


Source: BioXcel Therapeutics, Inc.

In March 2020, the company announced that approximately one-third of patients had been enrolled in the SERENITY trials and since that time enrollment has continued as expected, with no change in enrollment rates due to the COVID-19 pandemic. Thus far, all patients have been able to successfully self-administer the BXCL501 treatment. On May 14, 2020, the company announced completion of enrollment for the SERENITY trials and we anticipate topline data in July 2020.

Topline Data for Phase 1b/2 TRANQUILITY Trial in Mid-2020

BioXcel is currently conducting the Phase 1b/2 TRANQUILITY clinical trial of BXCL501 for the acute treatment of agitation in patients with dementia, including Alzheimer's disease (AD). The multicenter, randomized, double blind, placebo controlled, ascending dose trial is designed to evaluate the safety, efficacy, tolerability, and pharmacokinetics of BXCL501 in patients age 65 and older who exhibit acute agitation associated with all forms of dementia. It is an adaptive trial design and will evaluate multiple doses of BXCL501 or matching placebos. Following the completion of each dosing cohort, a safety and tolerability review will be conducted to determine the next tested dose. All of the BXCL501 doses have been manufactured and are at the long-term care facility conducting the TRANQUILITY trial, thus limiting the risk of exposure for the patients. There have been no issues with enrollment and we continue to anticipate topline data is expected in mid-2020. An outline of the trial is below.

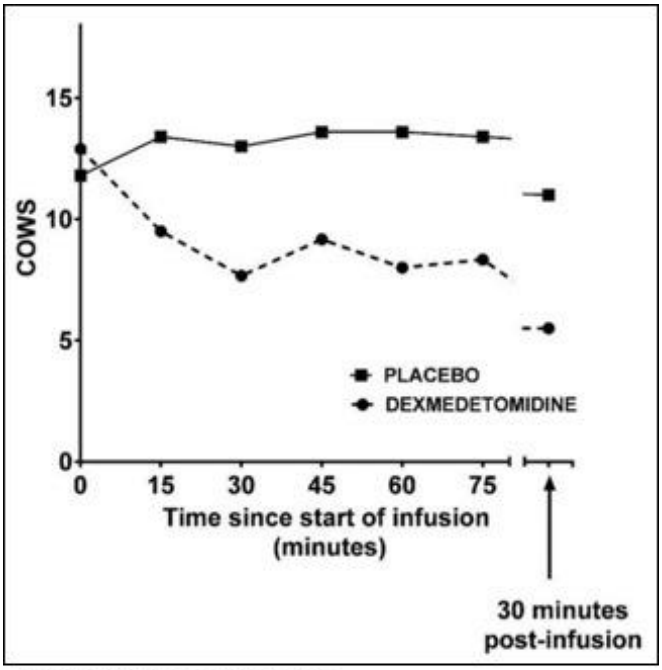


Source: BioXcel Therapeutics, Inc.

Phase 1b/2 RELEASE Trial in Opioid Withdrawal to Initiate Soon

In February 2020, BioXcel announced that the FDA has cleared the company's IND for BXCL501 for the treatment of opioid withdrawal symptoms. The company will soon be initiating the Phase 1b/2 RELEASE trial, which will be a multicenter, randomized, double blind, placebo controlled study in patients with opioid use disorder who are physically dependent on opioids and experiencing symptoms of opioid withdrawal. Multiple dose cohorts of BXCL501 or placebo will be administered twice daily for five days. Patients will be assessed using both the Clinical Opiate Withdrawal Scale (COWS), an 11-item scale that measures a range of withdrawal symptoms experienced after quitting opioids (Wesson *et al*., 2003). and Short Opiate Withdrawal Scale (Gossop, 1990) over a 10-day period.

The company had previously tested intravenous (IV) dexmedetomidine in patients suffering from opioid withdrawal symptoms and announced results in February 2019. A total of 15 patients (10 treated with dexmedetomidine and five administered placebo) with opioid dependence were enrolled and opioid withdrawal symptoms evaluated using the COWS. All 10 patients treated with dexmedetomidine responded to treatment, with the following graph showing the average decrease for treated patients, while no patients treated with placebo responded.



Source: BioXcel Therapeutics, Inc.

Biomarker Study Ongoing in Schizophrenia Patients

In February 2020, BioXcel announced the initiation of a Phase 2 study at Yale University that is examining biomarkers associated with agitation in schizophrenia patients and the response to treatment with BXCL501 (NCT03708315). The company hopes to utilize various biomarkers (change in heart rate, electrodermal activity, electroencephalogram [EEG]) that may help it to identify other indications that show the same physiological signs of hyperarousal. The hope is that some or all of these bodily signals could be utilized to indicate an agitated state prior to visible symptoms becoming apparent. This may allow doctors to intervene before an agitated person becomes a danger to themselves or others. We anticipate data from this study in the second half of 2020.

Delirium Trial in Planning Stages; Potential to Treat COVID-19 Patients with Delirium

A potential fifth indication for BXCL501 is in the treatment of delirium and the company is currently in the planning stages for a Phase 1b/2 trial that we anticipate getting underway in the second half of 2020. Treating delirium is taking on additional urgency during the COVID-19 epidemic as there are numerous reports of severely COVID-19 patients developing delirium, an acute brain condition in which a patient is confused, inattentive, and has a decreased ability to understand their surroundings. The condition is particularly common in patients that are sedated and placed on ventilators, which is how a number of severely ill COVID-19 patients are treated. During the first quarter of 2020 conference call, the company indicated that it was evaluating how it may be able to help COVID-19 patients with delirium and management was aware of reports of dexmedetomidine (not BXCL501) being used in the ICU to treat patients with delirium.

Phase 2 Study of BXCL701 in Combination with Keytruda® Underway

In April 2020, BioXcel announced the initiation of the Phase 2 portion of the Phase 1b/2 clinical trial of BXCL701 in combination with Keytruda® for the treatment of treatment emergent neuroendocrine prostate cancer (tNEPC) (NCT03910660). Results from the Phase 1b safety portion of the study indicated that a 0.6 mg total daily dose showed on-target effects and that splitting the dose improved the safety profile.

The Phase 2 portion of the study will enroll approximately 30 tNEPC patients who will receive 0.3 mg of BXCL701 twice daily for two weeks of a 21-day cycle in combination with 200 mg pembrolizumab dosed on Day 1 and every 21 days thereafter. The primary endpoint of the trial is the composite response rate, with a goal of achieving a 15% composite response rate. Secondary endpoints of the trial include overall survival, duration of response, and progression-free survival.

Financial Update

On May 12, 2020, BioXcel announced financial results for the first quarter of 2020. As expected, the company did not report any revenues. Net loss for the first quarter of 2020 was $14.9 million, compared to a net loss of $7.2 million for the first quarter of 2019. R&D expenses for the first quarter of 2020 were $12.4 million, compared to $5.7 million for the first quarter of 2019. The increase was primarily due to an increase in clinical trial expenses, salaries, and manufacturing costs. G&A expenses in the first quarter of 2020 were $2.6 million, compared to $1.7 million for the first quarter of 2019. The increase was primarily due to increased professional fees.

As of March 31, 2019, BioXcel had cash and cash equivalents of approximately $80.1 million, which was due in part to a public offering in February 2020 that raised gross proceeds of approximately $64 million. We estimate that the company has sufficient capital to fund operations into mid-2021. As of May 4, 2020, the company had approximately 20.2 million shares outstanding and when factoring in stock options there is a fully diluted share count of approximately 23.7 million shares.

Conclusion

We are glad to see that the ongoing COVID-19 epidemic has not affected the company's operations or the ongoing clinical trials and we are looking forward to results from the SERENTIY trials in July 2020 and the TRANQUILITY trial in mid-2020. Since bottoming out near $15 in March 2020, the stock has been on a great run, however we think there is significant additional upside if the company reports positive results from the SERENITY and/or TRANQUILITY trials. Our current valuation for BioXcel Therapeutics is $92 per share.

PROJECTED FINANCIALS

BioXcel Therapeutics, Inc.	2019 A	Q1 A	Q2 E	Q3 E	Q4 E	2020 E	2021 E	2022 E
BXCL501	$0	$0	$0	$0	$0	$0	$15	$94
BXCL701	$0	$0	$0	$0	$0	$0	$0	$0
Other Income	$0	$0	$0	$0	$0	$0	$0	$0
Total Revenues	**$0**	**$0**	**$0**	**$0**	**$0**	**$0**	**$15**	**$94**
Cost of Sales	$0	$0	$0	$0	$0	$0	$1	$7
Product Gross Margin	-	-	-	-	-	-	-	-
Research & Development	$25.8	$12.4	$10.0	$10.5	$11.0	$43.9	$45.0	$47.0
General & Administrative	$7.8	$2.6	$2.7	$2.7	$2.8	$10.8	$12.0	$40.0
Other (Income) Expense	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0
Operating Income	($33.6)	($15.0)	($12.7)	($13.2)	($13.8)	($54.7)	($43.0)	$0.5
Operating Margin	-	-	-	-	-	-	-	-
Non-Operating Expenses (Net)	$0.6	$0.1	$0.1	$0.1	$0.1	$0.4	$1.0	$1.0
Pre-Tax Income	($33.0)	($15.0)	($12.8)	($13.1)	($13.7)	($54.3)	($42.0)	$1.5
Income Taxes	$0.0	$0.0	$0.0	$0.0	$0.0	$0	$0	$0
Tax Rate	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*
Net Income	**($33.0)**	**($15.0)**	**($12.8)**	**($13.1)**	**($13.7)**	**($54.3)**	**($42.0)**	**$1.5**
Net Margin	-	-	-	-	-	-	-	-
Reported EPS	**($2.02)**	**($0.79)**	**($0.63)**	**($0.64)**	**($0.67)**	**($2.71)**	**($1.83)**	**$0.07**
YOY Growth	-	-	-	-	-	-	-	-
Basic Shares Outstanding	16.3	19.0	20.3	20.4	20.5	20.0	23.0	23.0

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

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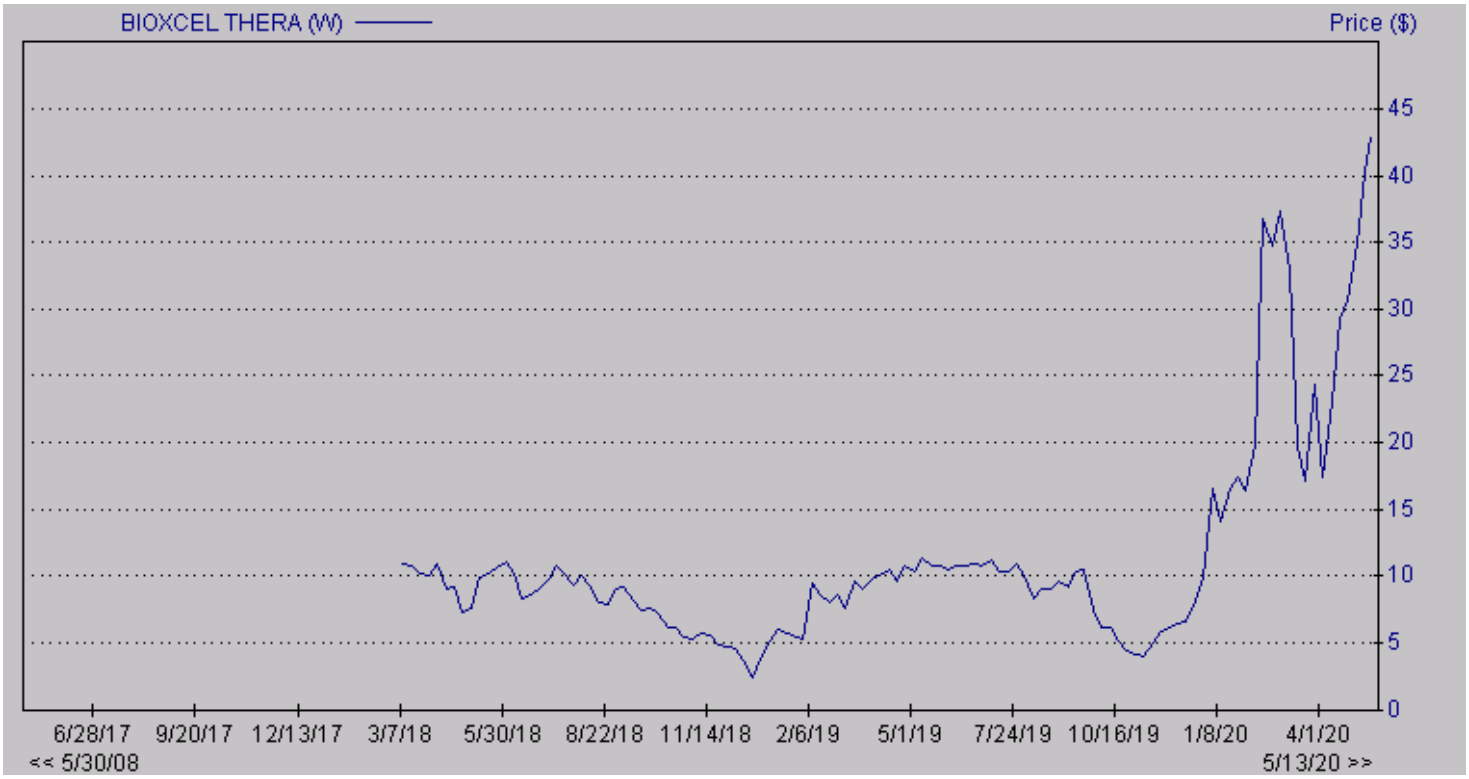
HISTORICAL STOCK PRICE



Source: Zacks SCR

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