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# BioXcel Therapeutics, Inc. (BTAI-NASDAQ)

***BTAI: On Track for Multiple Data Readouts in 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 (03/17/20) | $14.79 |
| **Valuation** | **$92.00** |

## OUTLOOK

On March 9, 2020, BioXcel Therapeutics, Inc. (BTAI) announced financial results for the fourth quarter of 2019 and provided a business update. The company has a number of key inflection points coming up over the following year, including 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 mid-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 2Q20, the initiation of a Phase 1b/2 clinical trial of BXCL501 in patients suffering from opioid withdrawal, and data for BXCL701 in combination trials in patients with tNEPC and other solid tumors. We've recently raised our valuation to $92 per share, and with the coronavirus-induced drop in the stock we believe now is the time for investors to enter a position ahead of those data readouts.

## SUMMARY DATA

| | |
|---|---|
| **52-Week High** | **$41.14** |
| **52-Week Low** | **$4.05** |
| **One-Year Return (%)** | **43.17** |
| **Beta** | **0.58** |
| **Average Daily Volume (sh)** | **1,012,226** |
| **Shares Outstanding (mil)** | **20** |
| **Market Capitalization ($mil)** | **$298** |
| **Short Interest Ratio (days)** | **N/A** |
| **Institutional Ownership (%)** | **24** |
| **Insider Ownership (%)** | **65** |
| **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** | **-11.6** |
| **P/E using 2020 Estimate** | **-17.1** |

| | |
|---|---|
| **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) |
|---|---|---|---|---|---|
| 2019 | 0 A | 0 A | 0 A | 0 A | 0 A |
| 2020 | 0 E | 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 | -$0.42 E | -$0.45 E | -$0.46 E | -$0.49 E | -$1.80 E |
| 2021 | | | | | -$1.17 E |
| 2022 | | | | | $0.67 E |

## 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:

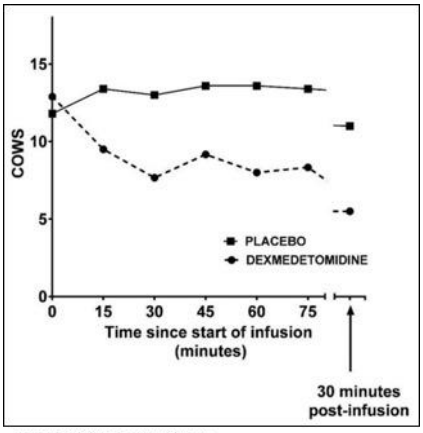
- Phase 3 data for BXCL501 in treating agitation in schizophrenia and bipolar disorder in mid-2020
- Phase 1b/2 data for BXCL501 in treating agitation in dementia in mid-2020
- Phase 2 biomarker data for BXCL501 in 2Q20
- Phase 1b/2 data for BXCL501 in treating opioid withdrawal in 2H20
- NDA filing for BXCL501 in treating agitation in schizophrenia and bipolar disorder in 2H20
- Phase 1b data for BXCL701 in combination with pembrolizumab in patients with neuroendocrine prostate cancer in 2H20
- 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 in 2H20

**Business Update**

*IND for Opioid Withdrawal Cleared by FDA*

On February 6, 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.

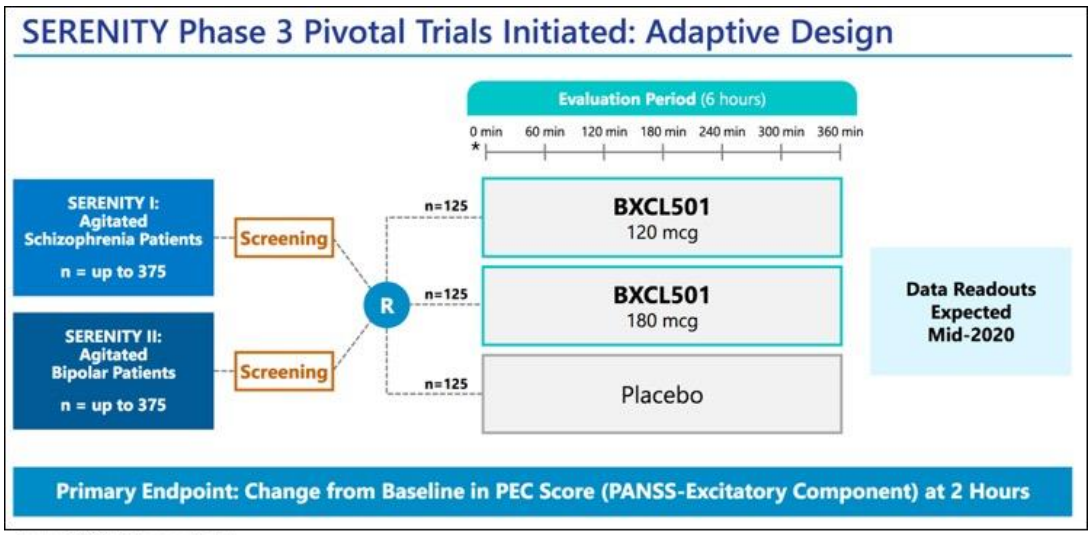
The company had previously tested intravenous (IV) dexmedetomidine in patients suffering from opioid withdrawal symptoms and announced results in February 2019. A total of 15 patients (10 treated with dexmedetomidine and five administered placebo) with opioid dependence were enrolled and opioid withdrawal 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.

*Phase 3 Trials in Schizophrenia and Bipolar Disorder Underway*

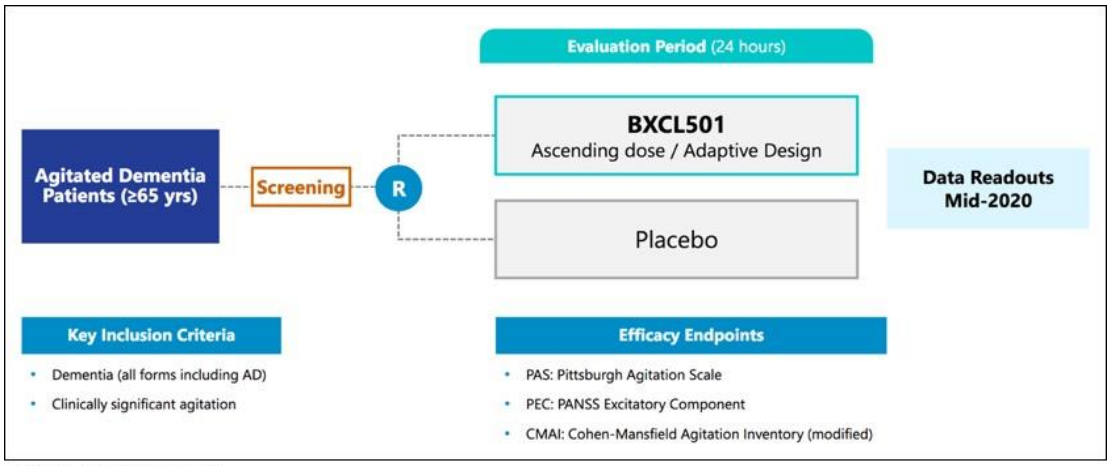
The company recently initiated the pivotal Phase 3 SERENITY (Sub-Lingual DExmedetomidine in Agitation Associated With SchizophRENIa and Bipolar Disorder STudY) trials of BXCL501 for acute treatment of agitation in patients with schizophrenia and bipolar disorder. The primary endpoint of both trials will 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. Topline data is expected in mid-2020.




*Source*: BioXcel Therapeutics, Inc.

*Phase 1b/2 Trial in Dementia Initiated*

The company recently announced the initiation of a Phase 1b/2 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. An outline of the trial is below. Topline data is expected in mid-2020.




*Source*: BioXcel Therapeutics, Inc.

*Biomarker Study Initiated in Schizophrenia Patients*

On February 18, 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 quarter of 2020.

## Financial Update

On March 9, 2020, BioXcel announced financial results for the fourth quarter and full year 2019. As expected, the company did not report any revenues in 2019. Net loss in the fourth quarter of 2019 was $8.3 million compared to a net loss of $7.1 million in the fourth quarter of 2018. R&D expenses in the fourth quarter of 2019 were $6.5 million compared to $6.0 million in the fourth quarter of 2018. The increase was primarily due to increased research costs, salaries, and manufacturing costs partially offset by a decrease in clinical trial expenses. G&A expenses in the fourth quarter of 2019 were $1.9 million compared to $1.3 million in the fourth quarter of 2018. The increase was primarily due to an increase in salary, payroll costs, and professional fees.

For 2019, BioXcel reported a net loss of $33.0 million compared to a net loss of $19.3 million for 2018. R&D expenses in 2019 were $25.8 million compared to $14.6 million in 2018. The increase was primarily due to clinical trial costs, increased salaries, professional research costs, and increased manufacturing costs. G&A expenses in 2019 were $7.8 million compared to $5.4 million in 2018. The increase was primarily due to increased salaries, payroll costs, and professional fees.

As of Dec. 31, 2019, BioXcel had cash and cash equivalents of approximately $32.4 million. In February 2020, the company announced a public offering that raised gross proceeds of approximately $64 million. We estimate that the company has sufficient capital to fund operations into mid-2021. As of March 9, 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 look forward to the upcoming data readouts for the company's lead assets and believe that positive data will cause a significant revaluation of the company's shares, with Karuna Therapeutics (KRTX) being a good comparator for what can occur following release of positive data in a CNS indication. Karuna released positive results for a Phase 2 clinical trial of KarXT for the treatment of acute psychosis in schizophrenia patients in the Fall of 2019 and the company's stock increased in value >500%! KarXT has a number of similarities with BXCL501, including the fact it is a reformulation of an already FDA approved compound, it is targeting the same patient population, and it is being evaluated through a rapid development pathway, thus we believe it provides an excellent case study for how a company's valuation can dramatically change following positive results for a CNS-targeted therapy. Our current valuation for BioXcel Therapeutics is $92 per share.

## PROJECTED FINANCIALS

| BioXcel Therapeutics, Inc. | 2019 A | Q1 E | 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 | $6.0 | $6.5 | $7.0 | $7.5 | $27.0 | $30.0 | $33.0 |
| General & Administrative | $7.8 | $2.4 | $2.5 | $2.5 | $2.6 | $10.0 | $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) | ($8.4) | ($9.0) | ($9.5) | ($10.1) | ($37.0) | ($28.0) | $14.5 |
| *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) | ($8.5) | ($9.1) | ($9.4) | ($10.0) | ($36.6) | ($27.0) | $15.5 |
| 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)** | **($8.5)** | **($9.1)** | **($9.4)** | **($10.0)** | **($36.6)** | **($27.0)** | **$15.5** |
| *Net Margin* | - | - | - | - | - | - | - | - |
| **Reported EPS** | **($2.02)** | **($0.42)** | **($0.45)** | **($0.46)** | **($0.49)** | **($1.80)** | **($1.17)** | **$0.67** |
| *YOY Growth* | - | - | - | - | - | - | - | - |
| Basic Shares Outstanding | 16.3 | 20.2 | 20.3 | 20.4 | 20.5 | 20.4 | 23.0 | 23.0 |

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

## HISTORICAL STOCK PRICE


Source: Zacks SCR

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