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BioXcel Therapeutics, Inc. (BTAI-NASDAQ)

BTAI: Phase 3 Trials of BXCL501 in Agitation to Initiate Before Year End…

Based on our probability adjusted DCF model that takes into account potential future revenues of the cellular oncology and stem cell therapies, BTAI is valued at $21.00/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 (11/21/19)	$6.25
Valuation	**$21.00**

OUTLOOK

BioXcel Therapeutics, Inc. (BTAI) is preparing to initiate two Phase 3 clinical trials of BXCL501, a sublingual formulation of the α2a adrenergic receptor agonist dexmedetomidine, for the treatment of agitation in schizophrenia and bipolar disorder. We anticipate results in the first half of 2020. A Phase 1b clinical trial of BXCL501 will initiate for the treatment of agitation in Alzheimer's disease soon with results also anticipated in the first half of 2020. Lastly, we expect additional data from the Phase 1/2 clinical trial of BXCL701 for the treatment of tNEPC before the end of 2019. Following a recent financing, the company has sufficient capital to fund operations through those expected data readouts.

SUMMARY DATA

52-Week High	**$11.79**
52-Week Low	**$2.41**
One-Year Return (%)	**19.73**
Beta	**2.80**
Average Daily Volume (sh)	**137,536**
Shares Outstanding (mil)	**18**
Market Capitalization ($mil)	**$113**
Short Interest Ratio (days)	**N/A**
Institutional Ownership (%)	**21**
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	**-2.8**
P/E using 2020 Estimate	**-2.5**

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)
2018	0 A	0 A	0 A	0 A	0 A
2019	0 A	0 A	0 A	0 E	0 E
2020					0 E
2021					0 E

Earnings per Share

	Q1 (Mar)	Q2 (Jun)	Q3 (Sep)	Q4 (Dec)	Year (Dec)
2018	-$0.37 A	-$0.19 A	-$0.31 A	-$0.47 A	-$1.32 A
2019	-$0.46 A	-$0.54 E	-$0.57 A	-$0.50 E	-$2.12 E
2020					-$2.00 E
2021					-$2.10 E

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Business Update

BioXcel Therapeutics, Inc. (BTAI) is a biopharmaceutical company developing novel therapeutics using EvolverAI, the company's proprietary research and development engine designed to utilize artificial intelligence (AI) and machine learning to evaluate millions of data points to identify new therapeutic indices for clinically validated and/or approved drugs. By using an AI-driven approach, the company targets areas of high unmet medical need with limited competition and is able to design more efficient clinical trials to speed up development timelines.

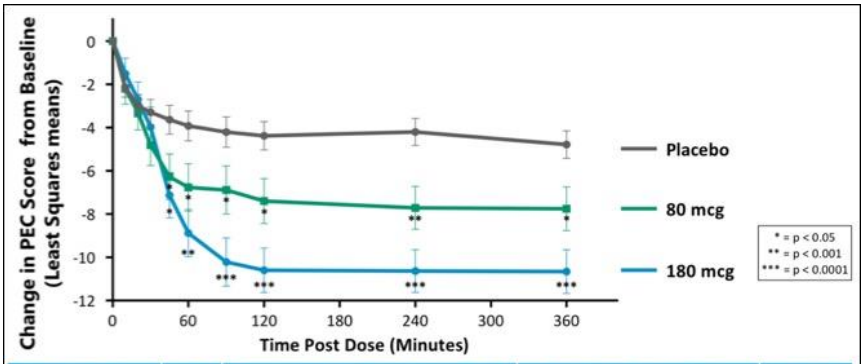
The company has two lead development programs: BXCL501 – a sublingual formulation of the α2a adrenergic receptor agonist dexmedetomidine (Dex) for the treatment of neurological and psychiatric disorders; and BXCL701 – an immuno-oncology agent for treatment of a rare form of prostate cancer and pancreatic cancer.

BXCL501

BXCL501 is a sublingual formulation of dexmedetomidine (Dex) that is currently being tested as a treatment for agitation in neurodegenerative and psychiatric disorders. Dex was initially approved in 1999 as an injectable for sedation in the intensive care setting and sold under the name Precedex®. It has been prescribed to millions of patients and has an excellent safety record. The sublingual formulation of Dex is designed to be administered in a non-invasive way and have a rapid onset of action. The company has completed a Phase 1b trial of BXCL501 in schizophrenia patients for which positive results were announced in July 2019. BioXcel will be initiating two Phase 3 trials in patients with schizophrenia and bipolar disorder before the end of 2019.

Data from Phase 1b Trial in Schizophrenia Patients

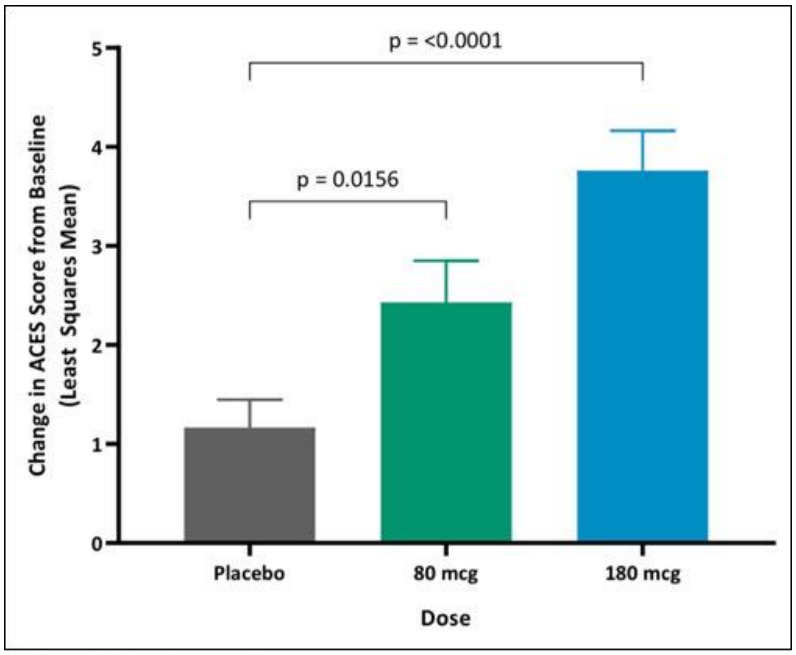
In July 2019, BioXcel announced positive top line results from a Phase 1b, randomized, double blind, placebo controlled, multi-center U.S. trial evaluating multiple doses of BXCL501 for the treatment of agitation in 135 schizophrenia patients (NCT04010305). The primary endpoint was the reduction in PEC score. Results showed rapid calming without excessive sedation at two hours, with the 80 μg, 120 μg, and 180 μg doses showing reductions in PEC scores of -7.1, -9.2, and -10.8, respectively, compared to -4.5 for placebo. These results were all statistically significant. The following slide shows a rapid and durable response with statistically significant separation from placebo <60 minutes following dosing. The reduction in PEC scores attained by BXCL501 was comparable to levels achieved by IM antipsychotics in other trials (Breier *et al*., 2002).



Drug/Dose	#	% Responders (Reduction in PEC of ≥ 40%)	Mean Change in PEC Score	P-Value
Placebo	N=36	28%	-4.5	
BXCL501 (180 mcg)	N=18	89%	-10.8	< 0.0001
BXCL501 (120 mcg)	N=18	67%	-9.2	0.0003
BXCL501 (80 mcg)	N=18	56%	-7.1	0.0152
BXCL501 (60 mcg)	N=18	39%	-6.0	0.1227

Source: BioXcel Therapeutics, Inc.

A secondary endpoint in the trial included assessment using the Agitation-Calmness Evaluation Scale (ACES), which is a single item that rates the overall agitation and sedation at the time of evaluation, ranging from 1-marked agitation, 2-moderate agitation, 3-mild agitation, 4-normal behavior, 5-mild calmness, 6-moderate calmness, 7-marked calmness, 8-deep sleep, and 9-unarousable. The following graph shows a statistically significant change in ACES from baseline at both 180 μg and 80 μg BXCL501 compared to placebo, consistent with what was seen in the primary endpoint.



Source: BioXcel Therapeutics, Inc.

BXCL501 was well tolerated with no serious adverse events reported across the entire dose range tested. The most common treatment emergent adverse event was mild somnolence and dry mouth. In addition, all study subjects were able to self-administer the film and complete the study.

Phase 3 Trials to Initiate in Schizophrenia and Bipolar Disorder

BioXcel is planning to conduct two pivotal Phase 3 trials for BXCL501 in the treatment of agitation associated with schizophrenia and bipolar disorder. We anticipate a total of approximately 500 patients (250 each for schizophrenia and bipolar disorder patients) randomized 1:1:1 to one of two doses of BXCL501 or placebo. The primary endpoint will be the change in PEC score at two hours with a 24-hour evaluation period. We anticipate the trials initiating in the fourth quarter of 2019 with top line data available in the first half of 2020. Positive results could lead to a New Drug Application (NDA) in the second half of 2020.

Phase 1b Trial to Initiate in Alzheimer's/Dementia

In addition to schizophrenia and bipolar disorder, we anticipate BioXcel initiating a Phase 1b trial in agitated Alzheimer's/dementia patients in the fourth quarter of 2019. Data from that trial should be available in the first half of 2020.

Karuna Therapeutics KarXT Shows Potential for Successful CNS Treatment

Karuna Therapeutics (KTRX) is a biopharmaceutical company developing KarXT, which is a proprietary formulation of two compounds: xanomeline, a muscarinic receptor agonist, and trospium, an FDA approved muscarinic antagonist. This formulation is designed to allow stimulation of muscarinic receptors in the central nervous system (CNS) while preventing the stimulation of muscarinic receptors in peripheral tissues. The company recently announced positive results for a Phase 2 clinical trial of KarXT for the treatment of acute psychosis in schizophrenia patients, with the stock increasing in value >500% following release of the data! KarXT has a number of similarities with BXCL501, including the fact it is a reformulation of an already FDA

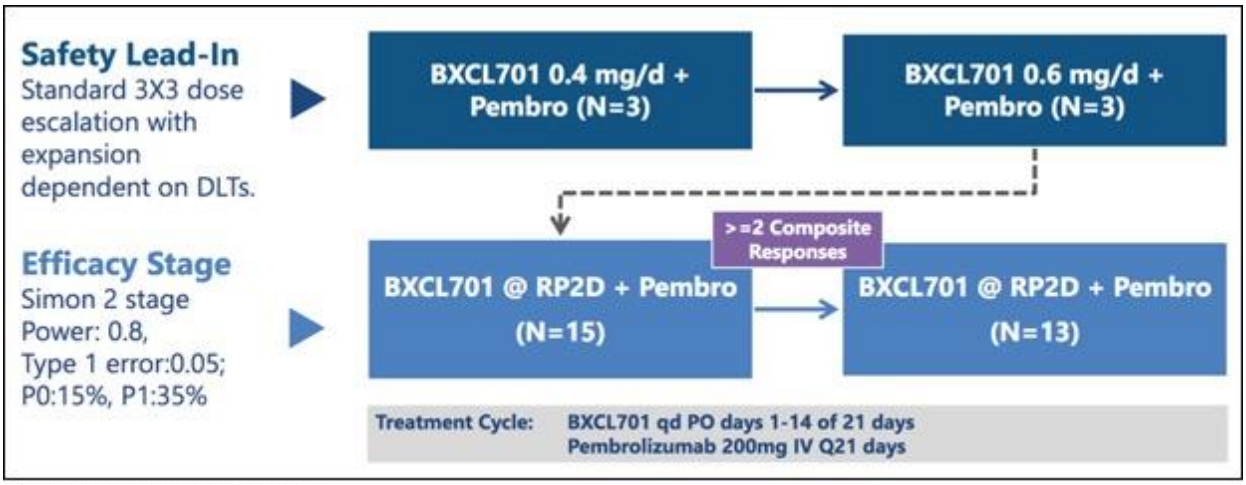
approved compound, it is targeting the same patient population, and it is being evaluated through a rapid development pathway, thus we believe it provides an excellent case study for how a company's valuation can dramatically change following positive results for a CNS-targeted therapy.

BXCL701

BXCL701 (talabostat) is an oral small molecule immunomodulator designed to activate the innate immune system through inhibition of dipeptidyl peptidase (DPP) 8/9 and fibroblast activation protein (FAP). The drug has been tested in over 700 patients through multiple clinical trials, thus there exists a large amount of data on its safety, tolerability, proof of mechanism, and single-agent anti-tumor activity. BioXcel is developing BXCL701 as a treatment for treatment-emergent neuroendocrine prostate cancer (tNEPC) and pancreatic cancer.

BXCL701 in tNEPC

BioXcel is currently conducting a Phase 1/2 clinical trial of BXCL701 in combination with Keytruda® (anti-PD-1 mAb) in patients with tNEPC (NCT03910660). It is a single arm, open label trial to examine the safety, pharmacokinetics, and anti-tumor activity of the combination of BXCL701 and Keytruda®. An outline of the trial is shown below.



Source: BioXcel Therapeutics, Inc.

The company presented interim safety and tolerability data from the Phase 1b portion of the study at the 26th Annual Prostate Cancer Foundation Scientific Retreat in Oct. 2019. A total of five patients were evaluable at the time of the presentation (three at 0.4 mg/day BXCL701 + Keytruda® and two at 0.6 mg/day BXCL701 + Keytruda®). Results showed that the combination of BXCL701 and Keytruda® was safe with no serious adverse events (SAEs) or dose limiting toxicities (DLTs). We anticipate additional safety data being reported before the end of 2019, and assuming no SAEs or DLTs are seen the company anticipates advancing to the Phase 2 efficacy portion of the study and initial efficacy data are likely to be reported in the first half of 2020.

BXCL701 in Pancreatic Cancer

BioXcel is conducting a Phase 1/2 clinical trial of BXCL701 in combination with NKTR-214 and avelumab (anti-PD-L1 mAb). The company has entered into a collaboration agreement with both Nektar Therapeutics, in which Nektar will supply NKTR-214 and share in the costs of the combination trial, and Merck KGaA, which will supply avelumab.

A safety run-in study of NKTR-214 and avelumab is currently being conducted, as those two agents had not previously been studied together. Once the safety of those two agents in combination is established, then additional patients can be treated with all three agents. We anticipate the safety run-in study of NKTR-214 and avelumab to complete in the first half of 2020 and safety data for the triplet combination to be available in the second half of 2020.

Financials and Capital Structure

On November 14, 2019, BioXcel Therapeutics announced financial results for the third quarter of 2019. The company reported a net loss of $9.0 million for the third quarter of 2019 compared to a net loss of $4.9 million for the third quarter of 2018. R&D expenses were $7.1 million for the third quarter of 2019 compared to $3.8 million for the third quarter of 2018. The increase was primarily due to increased clinical trial expenses, personnel costs, and professional fees. G&A expenses for the third quarter of 2019 were $2.0 million compared to $1.3 million for the third quarter of 2018. The increase was primarily due to increased payroll expenses and professional fees.

As of September 30, 2019, BioXcel had cash and cash equivalents of approximately $40.3 million. In September, 2019, the company announced the pricing of a public stock offering of approximately 2.3 million shares of common stock at an offering price of $8.25 per share for net proceeds of approximately $17.4 million. We estimate the company has sufficient capital to fund operations through the end of 2020.

As of Nov. 8, 2019, BioXcel had approximately 18 million shares of common stock outstanding and when factoring in employee and non-employee stock options a fully diluted share count of approximately 21.6 million.

Conclusion and Valuation

We look forward to the results of the upcoming Phase 3 clinical trials of BXCL501 for agitation associated with schizophrenia and bipolar disorder, which we anticipate will be reported in the first half of 2020. Based upon the Phase 1b results we believe there is a high probability of success in both those trials. We also anticipate results from the company's Phase 1b clinical trial of BXCL501 for agitation in Alzheimer's and dementia in the first half of 2020. Success in that trial could lead to a significantly expanded potential market opportunity for the compound. The initial safety data for BXCL701 in tNEPC looks promising and we look forward to initial efficacy data in the first half of 2020.

For BXCL501, we model for Phase 3 data in schizophrenia and bipolar disorder to be reported in the first half of 2020, with an NDA filing in the second half of 2020 and approval in the first half of 2021. We estimate that the company will receive approval for treatment of agitation in dementia in 2023 and in opioid withdrawal and delirium in 2024. We model for each treatment to cost $130 and we forecast for peak sales of $200 million in schizophrenia, $350 million in dementia, $200 million in opioid withdrawal, and $220 million in delirium. We estimate for a 75% probability of approval in schizophrenia and a 40% probability of approval in each of the other indications. Using a 15% discount rate leads to a net present value for BXCL501 of $274 million.

For BXCL701, we model for BioXcel to partner and to receive a 15% royalty on net sales. Assuming positive results in the Phase 1/2 clinical trials, we model for pivotal trials in both tNEPC and pancreatic cancer to initiate in 2021 with potential approval in 2024. Given the limited treatment options for each of those indications, we believe $1 billion in peak sales is possible for each indication. We estimate for a 33% probability of approval for each indication and using a 15% discount rate leads to a net present value for BXCL701 in tNEPC of $54 million and in pancreatic cancer of $63 million.

Combining the net present values for each of the company's assets along with the current cash balance and potential money from exercised options leads to a net present value for the company of $446 million. Dividing by the fully diluted share count of approximately 21.6 million shares leads to a valuation of approximately $21.00 per share.

PROJECTED FINANCIALS

BioXcel Therapeutics, Inc.	2018 A	Q1 A	Q2 A	Q3 A	Q4 E	2019 E	2020 E	2021 E
BXCL501	$0	$0	$0	$0	$0	$0	$0	$0
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**	**$0**	**$0**
Cost of Sales	$0	$0	$0	$0	$0	$0	$0	$0
Product Gross Margin	-	-	-	-	-	-	-	-
Research & Development	$14.6	$5.7	$6.5	$7.1	$6.7	$26.0	$27.0	$30.0
General & Administrative	$5.4	$1.7	$2.1	$2.0	$2.3	$8.2	$10.0	$12.0
Other (Income) Expense	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0
Operating Income	($19.9)	($7.4)	($8.6)	($9.1)	($9.0)	($34.2)	($37.0)	($42.0)
Operating Margin	-	-	-	-	-	-	-	-
Non-Operating Expenses (Net)	$0.7	($0.2)	($0.2)	$0.1	($0.1)	($0.4)	$0.0	$0.0
Pre-Tax Income	($19.2)	($7.2)	($8.5)	($9.0)	($9.1)	($34.6)	($37.0)	($42.0)
Income Taxes	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0	$0
Tax Rate	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*
Net Income	**($19.2)**	**($7.2)**	**($8.5)**	**($9.0)**	**($9.1)**	**($34.6)**	**($37.0)**	**($42.0)**
Net Margin	-	-	-	-	-	-	-	-
Reported EPS	**($1.32)**	**($0.46)**	**($0.54)**	**($0.57)**	**($0.50)**	**($2.12)**	**($2.00)**	**($2.10)**
YOY Growth	-	-	-	-	-	-	-	-
Basic Shares Outstanding	14.6	15.7	15.7	15.8	18.1	16.3	18.5	20.0

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

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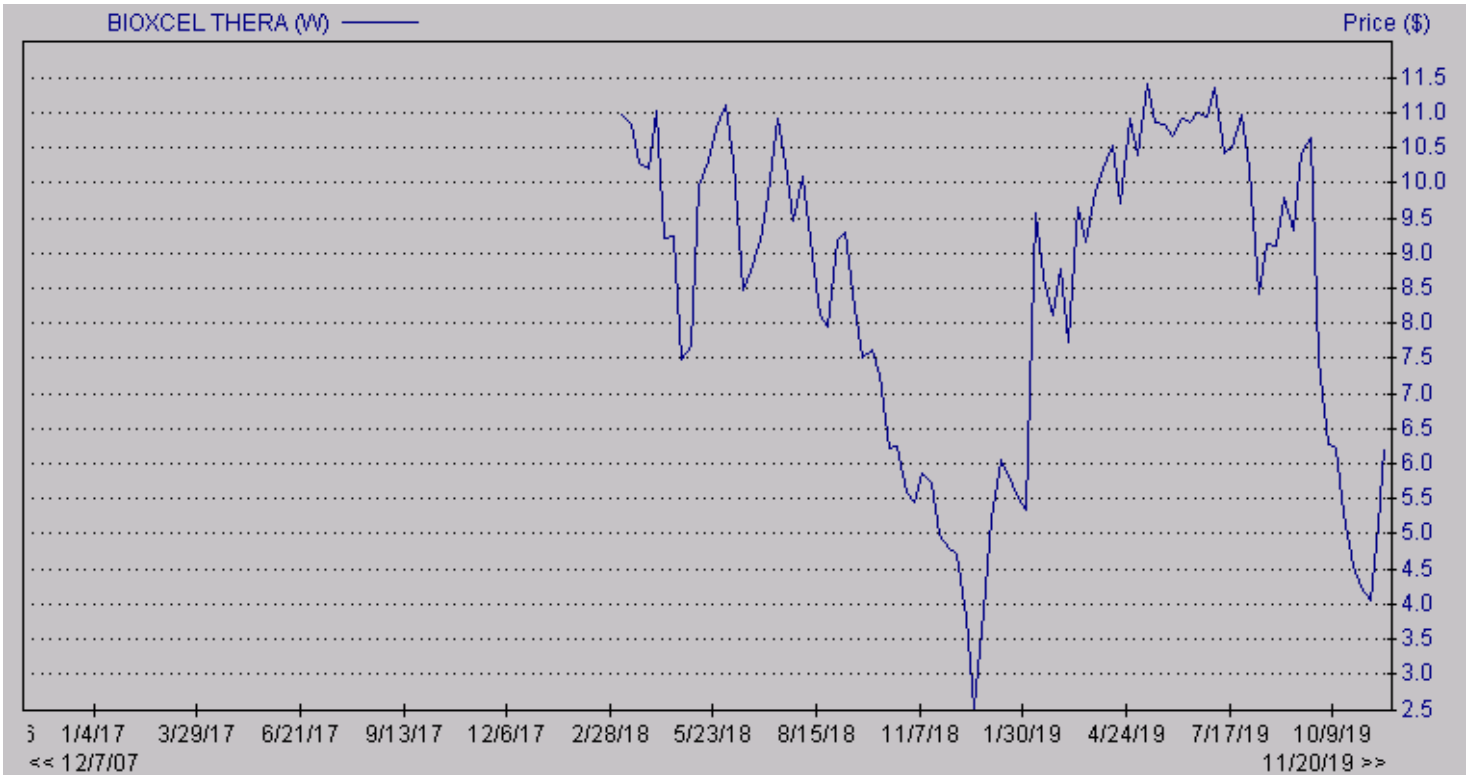
HISTORICAL STOCK PRICE



Source: Zacks SCR

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