Zacks Small-Cap Research
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David Bautz, PhD
312-265-9471
dbautz@zacks.com

scr.zacks.com
10 S. Riverside Plaza, Chicago, IL 60606

BioXcel Therapeutics, Inc. (BTAI-NASDAQ)

BTAI: PDUFA Date of Jan. 5, 2022 for BXCL501…

Based on our probability adjusted DCF model that takes into account potential future revenues of BXCL501 and BXCL701, BTAI is valued at $110.00/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 (09/03/21)	$30.75
Valuation	**$110.00**

OUTLOOK

On August 10, 2021, BioXcel Therapeutics, Inc. (BTAI) announced financial results for the second quarter of 2021 and provided a business update. The FDA accepted the New Drug Application (NDA) for BXCL501 for the treatment of schizophrenia and bipolar disorder related agitation and assigned a PDUFA date of January 5, 2022. The Phase 3 study of BXCL501 for the acute treatment of agitation in patients with dementia is expected to begin in the fourth quarter of 2021. We also anticipate topline data from the 40 μg cohort in the Phase 2 trial of BXCL501 for agitation in dementia patients to be reported in the fourth quarter of 2021. The company also recently initiated a pediatric study of BXCL501 for the acute treatment of agitation associated with schizophrenia and bipolar disorder. Data from the adenocarcinoma cohort of the Phase 1b/2 trial of BXCL701 and pembrolizumab in prostate cancer is expected in the third quarter of 2021.

SUMMARY DATA

52-Week High	**$64.60**
52-Week Low	**$23.84**
One-Year Return (%)	**-27.68**
Beta	**1.08**
Average Daily Volume (sh)	**265,072**
Shares Outstanding (mil)	**28**
Market Capitalization ($mil)	**$860**
Short Interest Ratio (days)	**N/A**
Institutional Ownership (%)	**47**
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	**-6.7**
P/E using 2020 Estimate	**-8.0**

Risk Level	**Above Avg.**
Type of Stock	**Mid-Blend**
Industry	**Med-Biomed/Gene**

ZACKS ESTIMATES

Revenue
(in millions of $)

	Q1 (Mar)	Q2 (Jun)	Q3 (Sep)	Q4 (Dec)	Year (Dec)
2020	0 A	0 A	0 A	0 A	0 A
2021	0 A	0 A	0 E	0 E	0 E
2022					126 E
2023					297 E

Earnings per Share

	Q1 (Mar)	Q2 (Jun)	Q3 (Sep)	Q4 (Dec)	Year (Dec)
2020	-$0.79 A	-$1.06 A	-$1.07 A	-$0.87 A	-$3.79 A
2021	-$1.07 A	-$1.11 A	-$0.93 E	-$0.95 E	-$4.05 E
2022					-$1.10 E
2023					$2.81 E

WHAT'S NEW

Business Update

PDUFA Date for BXCL501 of January 5, 2022; MAA Submission Expected 2H21

In May 2021, BioXcel Therapeutics, Inc. (BTAI) announced that the New Drug Application (NDA) for BXCL501 for the treatment of schizophrenia and bipolar disorder related agitation was accepted by the FDA with a PDUFA action date of January 5, 2022.

The NDA filing for BXCL501 is based in part on the positive results from the Phase 3 SERENITY I and SERENITY II clinical trials, which the company disclosed in July 2020. The SERENITY I trial was a randomized, double blind, placebo controlled parallel group adaptive trial in patients with schizophrenia or schizoaffective disorder (n=381) that were randomized to receive 120 μg BXCL501, 180 μg BXCL501, or placebo. The SERENITY II trial was a randomized, double blind, placebo controlled parallel group adaptive trial in patients with bipolar disorders (n=378) that were randomized to receive 120 μg BXCL501, 180 μg BXCL501, or placebo. Results showed a robust response in decreasing PEC scores in both trials and a favorable tolerability profile.

In addition to the NDA filing, we anticipate the company filing a Marketing Authorization Application (MAA) with the European Medicines Agency (EMA) for BXCL501 for the treatment of schizophrenia and bipolar disorder related agitation in the second half of 2021.

In regards to commercialization plans, the company has begun hiring for both the commercial and medical teams. The medical science liaison and medical managed care teams were deployed in March and the company continues to define the design of the sales force and market access strategy while planning for a U.S. commercial launch in the first half of 2022.

Phase 3 Program for BXCL501 in Dementia Related Agitation to Initiate 4Q21

In March 2021, BioXcel announced that the FDA had granted Breakthrough Therapy designation to BXCL501 for the treatment of agitation in dementia patients. Breakthrough Therapy designation allows for additional guidance from the FDA on drug development, the involvement of senior FDA managers, and a rolling review of the NDA.

In January 2021, BioXcel announced positive topline results for the Phase 1b/2 TRANQUILITY trial of BXCL501 for the acute treatment of agitation in dementia patients, including those with Alzheimer's disease. Treatment with BXCL501 resulted in a rapid, dose dependent decrease in PEC score from baseline that separated from placebo numerically by 30 minutes post-treatment and was statistically significantly different by 60 minutes. A full recap of the results can be found in our previous report.

Subsequent to the data announcement, the company initiated a supplemental dosing cohort investigating a 40 μg dose of BXCL501 in 46 patients (randomized 1:1) to help build a comprehensive clinical development strategy with the long-term goal of targeting dementia care from long-term care centers to at-home care. We anticipate a data readout in the fourth quarter of 2021.

We anticipate the Phase 3 program in dementia related agitation initiating in the fourth quarter of 2021, with the results of the 40 μg cohort helping to inform what doses the company takes forward into Phase 3 testing.

BXCL701 Data in 3Q21

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). BioXcel is currently conducting a Phase 1b/2 clinical trial of BXCL701 used in combination with pembrolizumab in

patients with metastatic castration resistant prostate cancer (mCRPC) with either therapy-induced neuroendocrine (t-NEPC) or adenocarcinoma phenotype. In February 2021, the company reported data on seven patients from the Phase 1b portion and five patients from the Phase 2 portion, with a disease control rate (DCR) of 100% in the Phase 1b cohort and 80% in the Phase 2 cohort (please see our previous report for a full discussion of the data). We anticipate an update from the adenocarcinoma cohort in the third quarter of 2021.

Financial Update

On August 10, 2021, BioXcel announced financial results for the second quarter of 2021. As expected, the company did not report any revenues in the second quarter of 2021. Net loss in the second quarter of 2021 was $27.6 million, compared to a net loss of $21.4 million in the second quarter of 2020. R&D expenses in the second quarter of 2021 were $13.5 million, compared to $17.9 million for the second quarter of 2020. The decrease was primarily due to decreased clinical trial costs related to the SERENITY I and II trials and decreased manufacturing costs, partially offset by increased personnel costs and non-cash, stock-based compensation. G&A expenses in the second quarter of 2021 were $14.1 million, compared to $3.5 million for the second quarter of 2020. The increase was primarily due to an increased personnel related costs, non-cash stock-based compensation, professional and consulting fees, marketing research for BXCL501, commercial fees for BXCL501, and insurance costs.

As of June 30, 2021, BioXcel had approximately $273.1 million in cash and cash equivalents, due in part to a public offering in June 2021 that raised gross proceeds of approximately $100 million. As of August 5, 2021, the company had approximately 28.0 million shares outstanding and when factoring in stock options a fully diluted share count of approximately 32.0 million.

Conclusion

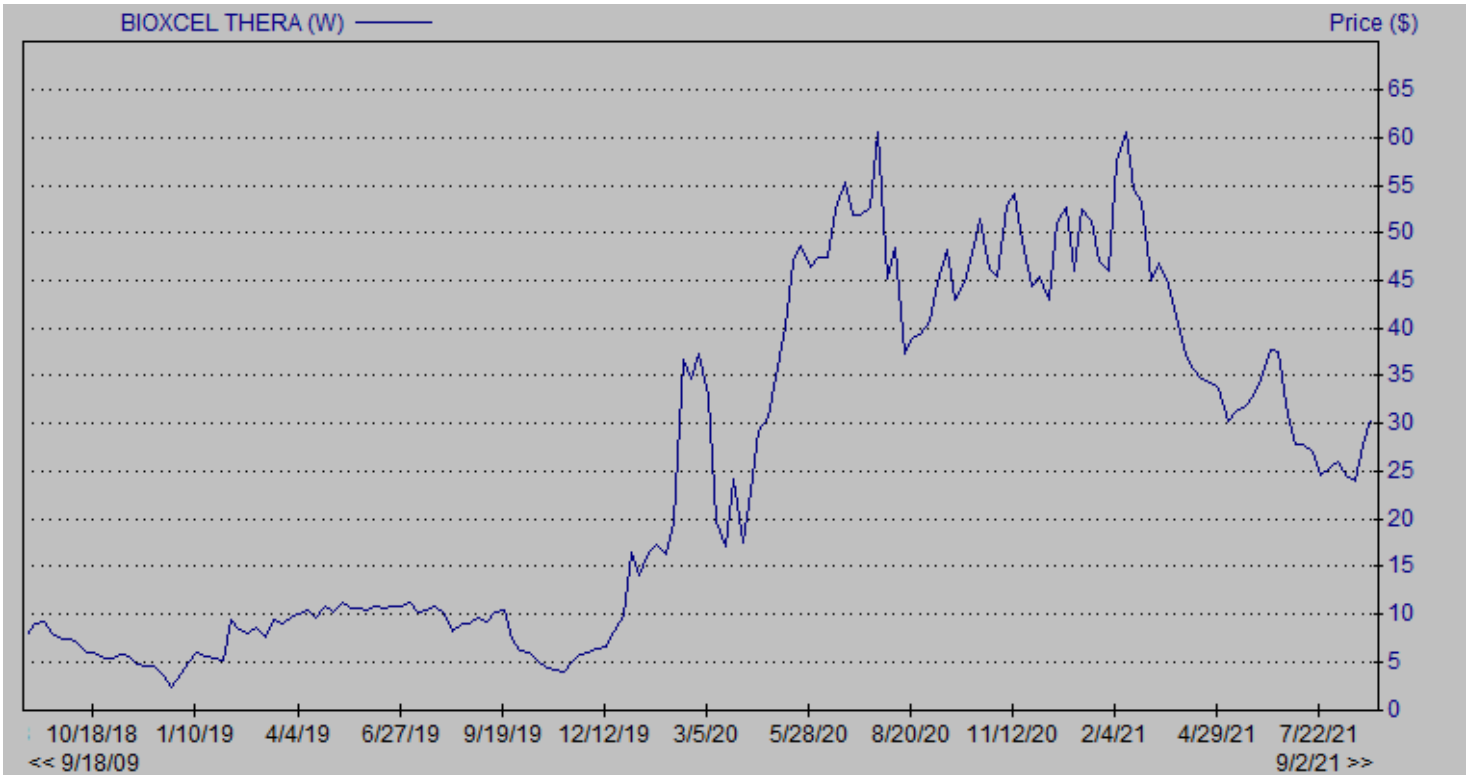
The countdown to the PDUFA date of January 5, 2022 for BXCL501 has begun, with management indicating that it is prepared for any pre-approval CMC inspection, whether it is in person or virtual. In addition, in preparation for commercialization the company has recently hired the VP of Sales and will be working with a recruiting firm to fill out the sales force later in the year. The stock has been under a lot of pressure over the past few months, which we ascribe more to the overall weakness in the biotech sector as a whole as opposed to anything company specific. After incorporating the recent financing into our model our valuation is now at $110 and we view the recent weakness in the stock as an excellent buying opportunity in the lead up to the PDUFA date.

PROJECTED FINANCIALS

BioXcel Therapeutics, Inc.	2020 A	Q1 A	Q2 A	Q3 E	Q4 E	2021 E	2022 E	2023 E
BXCL501	$0	$0	$0	$0	$0	$0	$126	$297
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**	**$126**	**$297**
Cost of Sales	$0	$0	$0	$0	$0	$0	$15	$33
Product Gross Margin	-	-	-	-	-	-	*88%*	*89%*
Research & Development	$58.0	$14.7	$13.5	$14.5	$15.0	$57.8	$65.0	$75.0
General & Administrative	$24.3	$11.6	$14.1	$11.5	$11.8	$49.0	$80.0	$100.0
Other (Income) Expense	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0
Operating Income	($82.3)	($26.3)	($27.6)	($26.0)	($26.8)	($106.8)	($34.0)	$89.0
Operating Margin	-	-	-	-	-	-	-	-
Non-Operating Expenses (Net)	$0.1	$0.0	($0.0)	$0.0	$0.1	$0.1	$1.0	$1.0
Pre-Tax Income	($82.2)	($26.3)	($27.6)	($26.0)	($26.7)	($106.7)	($33.0)	$90.0
Income Taxes	$0	$0.0	$0.0	$0.0	$0.0	$0	$0	$0
Tax Rate	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*
Net Income	**($82.2)**	**($26.3)**	**($27.6)**	**($26.0)**	**($26.7)**	**($106.7)**	**($33.0)**	**$90.0**
Net Margin	-	-	-	-	-	-	-	-
Reported EPS	**($3.79)**	**($1.07)**	**($1.11)**	**($0.93)**	**($0.95)**	**($4.05)**	**($1.10)**	**$2.81**
YOY Growth	-	-	-	-	-	-	-	-
Basic Shares Outstanding	21.7	24.5	25.0	28.0	28.0	26.4	30.0	32.0

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

HISTORICAL STOCK PRICE



Source: Zacks SCR

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