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BioXcel Therapeutics, Inc. (BTAI-NASDAQ)

BTAI: Submitting MAA for BXCL501 in Schizophrenia and Bipolar Disorder in 2H21…

Based on our probability adjusted DCF model that takes into account potential future revenues of BXCL501 and BXCL701, BTAI is valued at $120.00/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 (05/12/21)	$33.00
Valuation	**$120.00**

OUTLOOK

On May 10, 2021, BioXcel Therapeutics, Inc. (BTAI) announced financial results for the first quarter of 2021 and provided a business update. The company is awaiting word from the FDA regarding the New Drug Application (NDA) the company submitted in March 2021 for BXCL501 for the treatment of schizophrenia and bipolar disorder related agitation. We anticipate the company hearing whether the FDA accepted the NDA, if the application qualifies for priority review, and what the PDUFA date will be in the next few weeks. BioXcel is also planning to submit a Marketing Authorization Application (MAA) with the EMA for BXCL501 in the second half of 2021. Following the report of positive topline results from the Phase 1b/2 TRANQUILITY trial, the company will be holding an 'End-of-Phase 2' meeting with the FDA in the second quarter of 2021 to discuss the registrational trial for BXCL501 for the treatment of agitation associated with dementia.

SUMMARY DATA

52-Week High	**$64.63**
52-Week Low	**$30.40**
One-Year Return (%)	**-18.88**
Beta	**1.09**
Average Daily Volume (sh)	**333,344**
Shares Outstanding (mil)	**25**
Market Capitalization ($mil)	**$813**
Short Interest Ratio (days)	**N/A**
Institutional Ownership (%)	**49**
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	**-7.0**
P/E using 2020 Estimate	**-12.4**

Risk Level	**Above Avg.**
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)
2020	0 A	0 A	0 A	0 A	0 A
2021	0 A	0 E	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	-$1.02 E	-$1.05 E	-$1.08 E	-$4.22 E
2022					-$1.32 E
2023					$3.46 E

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WHAT'S NEW

Business Update

Awaiting Word from FDA Regarding BXCL501 NDA; MAA Submission Expected 2H21

In March 2021, BioXcel Therapeutics, Inc. (BTAI) announced that it submitted the New Drug Application (NDA) for BXCL501 for the treatment of schizophrenia and bipolar disorder related agitation. The FDA typically takes approximately 60 days to decide whether to accept the NDA, and following acceptance of the NDA the review time will be 10 months (or 6 months if the application is granted priority review). Given the NDA filing in early March, we anticipate news on the acceptance of the NDA in the near future.

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, 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 final decisions regarding the design and size of the sales force will be made soon, with approximately 75 sales reps expected. The company continues to plan for a U.S. commercial launch in the first half of 2022.

'End-of-Phase 2' Meeting for BXCL501 for Dementia Related Agitation

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 mg 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 before the end of 2021, but we don't expect it to delay the initiation of the Phase 3 program in dementia related agitation.

The company will be meeting with the FDA for an 'End-of-Phase 2' meeting in the second quarter of 2021 to receive feedback on the dose, trial design, and primary endpoint for a registrational trial. We anticipate the Phase 3 program in dementia related agitation initiating in the second half of 2021.

Results for BXCL501 in Opioid Withdrawal

In March, 2021, BioXcel announced results from the Phase 1b/2 RELEASE trial, which was a multicenter, randomized, double blind, placebo controlled study of BXCL501 in patients with opioid use disorder who are physically dependent on opioids and experiencing symptoms of opioid withdrawal. The study met its primary endpoint of safety for multiple doses given twice-daily for seven days. The key secondary endpoint of retention showed that the 120 μg and 180 μg dose groups showed 42% and 52% retention rates at Day 6 compared to 24% for placebo. While those data showed numerical superiority for BXCL501 the results were not statistically significant.

A confounding factor disclosed by the company could have been the fact that 50% of patients who had fentanyl in their system upon entering the trial remained fentanyl positive following the morphine stabilization phase of five days. Thus, withdrawal symptoms were not normalized across various dosing cohorts. The company is continuing to analyze the results to determine next steps in opioid withdrawal.

Financial Update

On May 10, 2021, BioXcel announced financial results for the first quarter of 2021. As expected, the company did not report any revenues in the first quarter of 2021. Net loss in the first quarter of 2021 was $26.4 million, compared to a net loss of $14.9 million in the first quarter of 2020. R&D expenses in the first quarter of 2021 were $14.7 million, compared to $12.4 million for the first quarter of 2020. The increase was primarily due to increased personnel, clinical trial, and professional research costs. G&A expenses in the first quarter of 2021 were $11.6 million, compared to $2.6 million for the first quarter of 2020. The increase was primarily due to an increase in personnel related costs along with costs related to the company's expansion in preparation for the commercial launch of BXCL501.

As of March 31, 2021, BioXcel had approximately $194.0 million in cash and cash equivalents. As of May 5, 2021, the company had approximately 24.6 million shares outstanding and when factoring in stock options a fully diluted share count of approximately 28.7 million.

Conclusion

We look forward to hearing about the NDA filing for BXCL501 and whether the application will qualify for priority review. With a plan to also file an MAA for BXCL501 we anticipate learning more about ex-U.S. commercialization plans in the upcoming months, which we anticipate will include a commercialization partner. Following the 'End-of-Phase 2' meeting with the FDA in the second quarter of 2021 for BXCL501 in the treatment of agitation associated with dementia we anticipate learning additional details about the Phase 3 program with a likely start in the second half of 2021.

The results for the RELEASE trial were disappointing regarding opioid withdrawal, but the trial did show the safety of different dosing regimens for BXCL501. The company has also paused enrollment in the PLACIDITY trial of BXCL501 in delirium based on the challenges of opening clinical sites during the COVID-19 pandemic. Based on that, we have removed opioid withdrawal from our model and decreased the probability of approval for delirium, which has decreased our valuation to $120. However, we remain very positive on the prospects for BXCL501 in treating agitation caused by schizophrenia, bipolar disorder, and dementia.

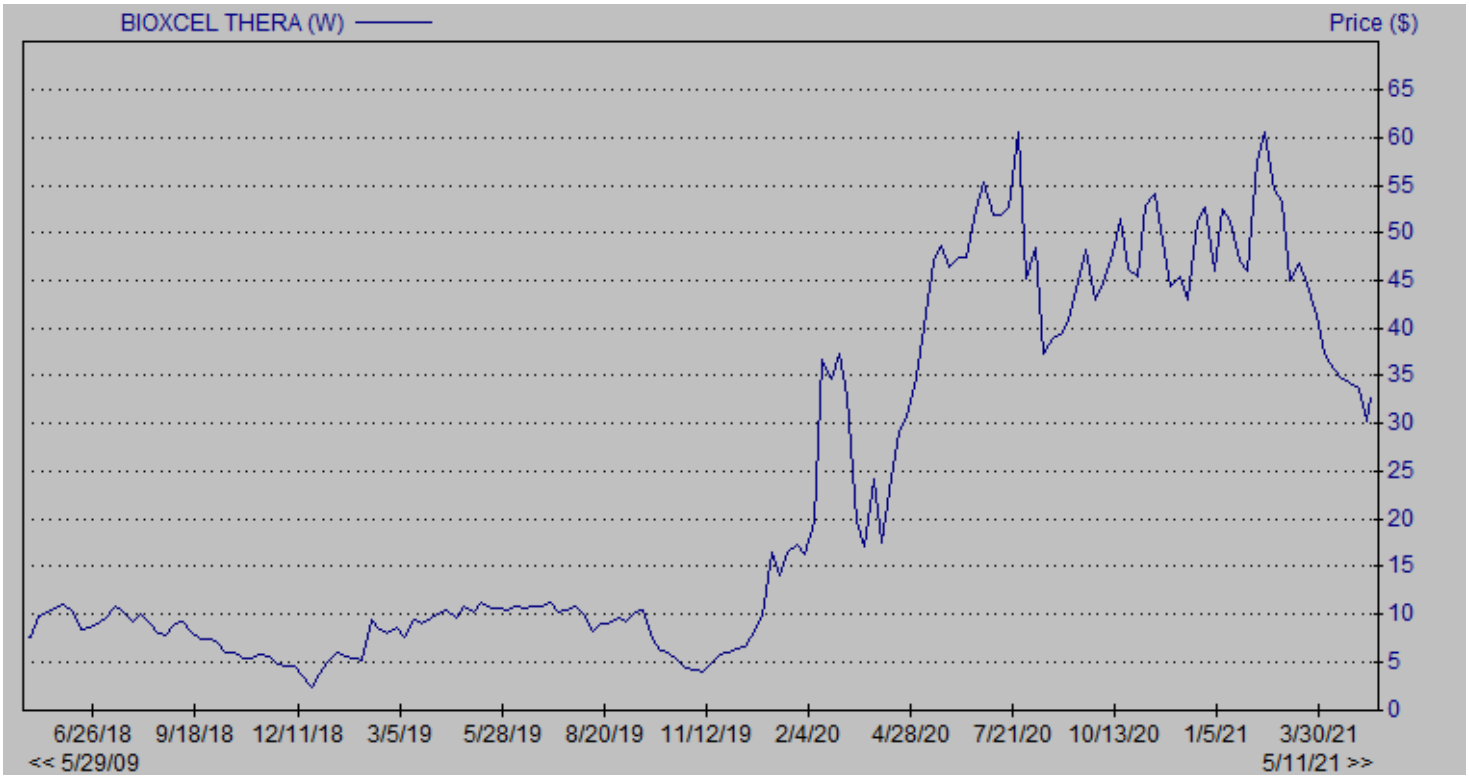
PROJECTED FINANCIALS

BioXcel Therapeutics, Inc.	2020 A	Q1 A	Q2 E	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	$14.0	$14.5	$15.0	$58.2	$65.0	$75.0
General & Administrative	$24.3	$11.6	$11.0	$11.5	$11.8	$45.9	$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)	($25.0)	($26.0)	($26.8)	($104.2)	($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)	($25.0)	($26.0)	($26.7)	($104.0)	($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)**	**($25.0)**	**($26.0)**	**($26.7)**	**($104.0)**	**($33.0)**	**$90.0**
Net Margin	-	-	-	-	-	-	-	-
Reported EPS	**($3.79)**	**($1.07)**	**($1.02)**	**($1.05)**	**($1.08)**	**($4.22)**	**($1.32)**	**$3.46**
YOY Growth	-	-	-	-	-	-	-	-
Basic Shares Outstanding	21.7	24.5	24.6	24.7	24.8	24.7	25.0	26.0

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

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