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BioXcel Therapeutics, Inc. (BTAI-NASDAQ)

BTAI: Promising Results for Part 1 of SERENITY III Trial…

Based on our probability adjusted DCF model that takes into account potential future revenues of BXCL501 and BXCL701, BTAI is valued at $75.00/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 (06/02/23)	$18.15
Valuation	**$75.00**

OUTLOOK

On May 25 2023, BioXcel Therapeutics, Inc. (BTAI) announced topline results from Part 1 of the Phase 3 SERENITY III trial of 60 μg BXCL501 for the treatment of agitation in bipolar or schizophrenia patients in institutional settings. The results are expected to enable the initiation of Part 2 of the trial in which BXCL501 will be tested in the at-home setting. Clinically meaningful results were seen using half (60 μg) of the lowest approved dose of IGALMI. In addition, there were no serious adverse events reported and safety results were comparable to placebo. Part 2 of the study will have an adaptive trial design with 60 μg and 80 μg BXCL501 along with the potential for repeat dosing.

SUMMARY DATA

52-Week High	**$33.24**
52-Week Low	**$9.03**
One-Year Return (%)	**70.26**
Beta	**1.09**
Average Daily Volume (sh)	**611,977**
Shares Outstanding (mil)	**29**
Market Capitalization ($mil)	**$529**
Short Interest Ratio (days)	**N/A**
Institutional Ownership (%)	**42**
Insider Ownership (%)	**36**
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	**-2.6**
P/E using 2020 Estimate	**-3.3**

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)
2022	0 A	0 A	0 A	0 A	0 A
2023	0 A	0 E	1 E	3 E	5 E
2024					12 E
2025					35 E

Earnings per Share

	Q1 (Mar)	Q2 (Jun)	Q3 (Sep)	Q4 (Dec)	Year (Dec)
2022	-$1.12 A	-$1.35 A	-$1.49 A	-$1.95 A	-$5.92 A
2023	-$1.84 A	-$1.66 E	-$1.74 E	-$1.68 E	-$6.60 E
2024					-$6.07 E
2025					-$5.59 E

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Business Update

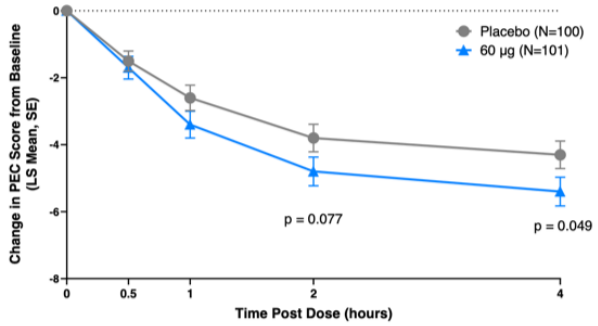
Promising Topline Results for SERENITY III

On May 25, 2023, BioXcel Therapeutics, Inc. (BTAI) announced topline results from Part 1 of the Phase 3 SERENITY III trial of 60 μg BXCL501 for the acute treatment of bipolar disorders- or schizophrenia-associated agitation in the institutional setting. An overview of the trial results can be found here. The SERENITY III trial is a two-part, double blind, placebo controlled study evaluating BXCL501 for at-home use; Part 1 was similar in design to the SERENITY I and II trials with Part 2 of the trial designed to evaluate BXCL501 in the at-home setting.

The results from Part 1 of the study showed that at the 2-hour time point post-dose, the change in PEC did not reach statistical significance (P=0.077), however at 4 hours post-dose the p-value was 0.049, as shown in the following image. In addition, 52% of BXCL501-treated patients responded compared to 36% of placebo patients (P=0.019). A responder was defined as someone who had ≥40% reduction from baseline PEC total score by 2 hours.

SERENITY III Part 1: Results Over Time

Change From Baseline PANSS Excitatory Component (PEC) Total Score Over Time



Endpoint	Placebo N = 100	BXCL501 60mcg N = 101
Change from Baseline (LS Mean (SE))	-3.8 (0.4) 2 hours -4.3 (0.4) 4 hours	-4.8 (0.4) (p = 0.077) -5.4 (0.4) (p = 0.049)
Response Rate[1]	36%	52% (p = 0.019)

[1]Responder: patients who had a ≥ 40% reduction from baseline PEC total score by 2 hours

Source: BioXcel Therapeutics, Inc.

The 60 μg dose of BXCL501 was well tolerated. There were no reported serious adverse events (AEs) and all AEs were either mild or moderate. The most commonly reported AE was somnolence (feeling drowsy, sleepy, fatigued, or sluggish), which occurred in 13% of the BXCL501 group compared to 7% in the placebo group. Additional AEs are indicated in the table below. The safety results are encouraging, as the 60 μg dose was chosen to increase the margin of safety while supporting efficacy for at-home use.

Adverse Events Reported In SERENITY III Part 1 and in SERENITY I and II

Adverse Event	SERENITY III Part 1		SERENITY I and II		
	BXCL501 60mcg N = 101	Placebo N = 100	IGALMI™ 120mcg[3] N = 255	IGALMI™ 180mcg[3] N = 252	Placebo N = 252[3]
Somnolence[1]	13 (13)	7 (7)	56 (22)	57 (23)	16 (6)
Oral paresthesia or oral hypoesthesia	6 (6)	4 (4)	14 (5)	18 (7)	2 (1)
Dizziness	3 (3)	1 (1)	10 (4)	15 (6)	2 (1)
Hypotension	1 (1)	0	14 (5)	13 (5)	0
Orthostatic hypotension	1 (1)	0	7 (3)	13 (5)	1 (0)
Dry mouth	5 (5)	3 (3)	19 (7)	11 (4)	3 (1)
Nausea	2 (2)	1 (1)	6 (2)	7 (3)	4 (2)
Bradycardia	0	0	5 (2)	5 (2)	0
Abdominal discomfort[2]	0	0	0 (0)	6 (2)	1 (0)

No SAEs. All AEs reported of mild to moderate severity, none were severe

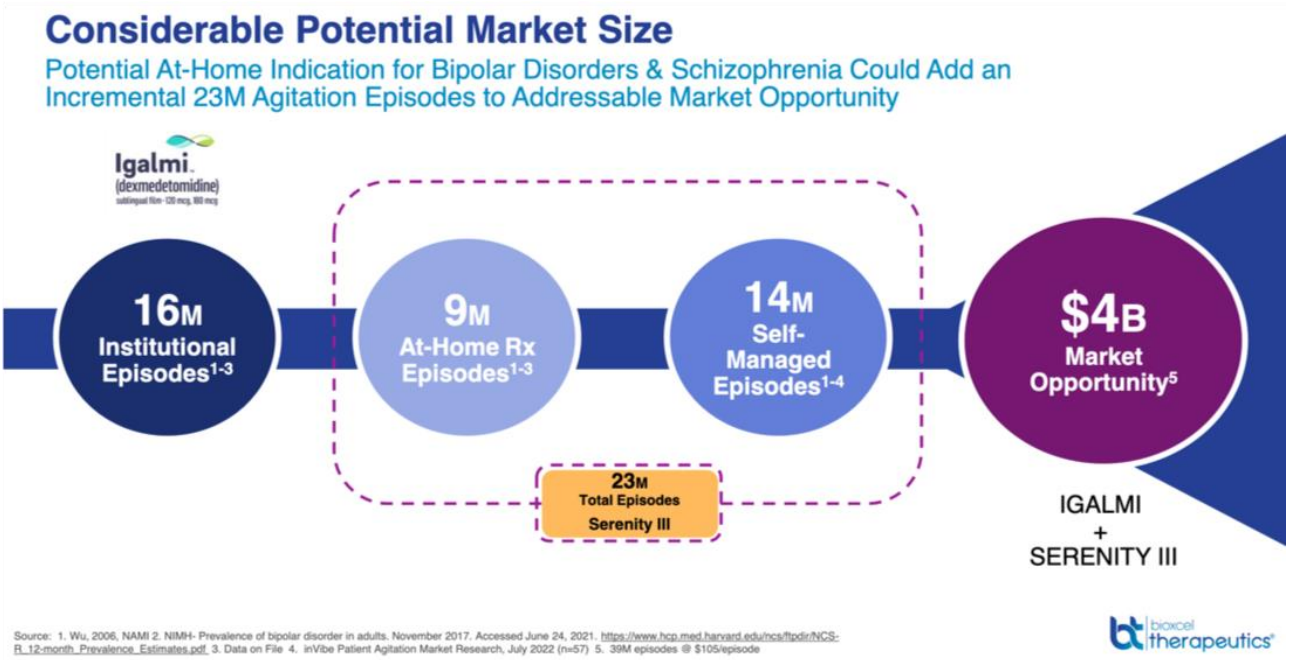
1 Somnolence includes the terms feeling drowsy, feeling sleepy, fatigue and sluggishness
2 Abdominal discomfort includes dyspepsia, gastroesophageal reflux disease
3 IGALMI™ (desmedetomidine) USPI, July 2022
No SAEs observed
The adverse events (AEs) listed correspond to those in the label for IGALMI. No other AEs were observed that would fulfill the criteria for inclusion in the AE table (at least 2% and greater than with placebo).

bioxcel therapeutics

Source: BioXcel Therapeutics, Inc.

Part 2 of the trial has an adaptive design with 60 μg BXCL501 or greater (such as 80 μg) to address agitation across a wide spectrum. The 80 μg dose previously showed statistical significance in the prior Phase 1b dose ranging study. Alignment has also been obtained with the FDA on repeat dosing. Patients and caregivers are recording a number of measures to report their response at home, including the CGI improvement scale, and once the company has collected enough data on these various measures the study can be properly powered to demonstrate efficacy at one or more doses.

The at-home agitation market is potentially quite considerable. Approximately 16 million agitation episodes occur in the institutional setting every year, however approximately 23 million episodes occur in the at-home setting. This makes the total agitation market a potential $4 billion opportunity.



Source: BioXcel Therapeutics, Inc.

Positive Topline Results for BXCL501 Repeat Dosing for MDD Program

On May 16, 2023, BioXcel announced positive topline results for the Phase 1b multiple ascending dose trial of BXCL501, which is designed to evaluate the safety and tolerability of repeat dosing of BXCL501 in healthy volunteers as a single agent and in combination with the antidepressant duloxetine. The results of the study will be utilized to select an appropriate dose of BXCL501 for a Phase 2 proof-of-concept trial of BXCL501 in combination with selective serotonin reuptake inhibitors (SSRIs) and serotonin-norepinephrine reuptake inhibitors (SNRIs) in patients with major depressive disorder (MDD).

A total of 125 healthy adult volunteers were enrolled across seven cohorts in a 2:1 randomization to BXCL501 or placebo film dosed for seven days. Four distinct cohorts received 30 μg, 60 μg, 80 μg, or 120 μg doses of BXCL501 or placebo. Two additional cohorts received twice-daily BXCL501 at either 30 μg in the morning and 60 μg at night, or 40 μg in the morning and 80 μg at night. The final escalation cohort received BXCL501 at 60 μg in the morning and 80 μg at night in combination with 30 μg of duloxetine BID.

BXCL501 was well tolerated in all dosing cohorts. The maximum tolerated dose was not reached and all adverse events were reported as mild or moderate. The most common adverse events were orthostatic hypotension, somnolence, headache, dizziness, and hypotension. In addition, there were no serious adverse events. The twice-daily dosing regimen improved daytime tolerability compared to once-daily dosing in the morning. Lastly, BXCL501 showed consistent pharmacokinetics with proportional increases with dose, no accumulation with either once- or twice-daily dosing, and consistency with or without duloxetine co-administration.

Conclusion

While disappointing that the 60 μg dose of BXCL501 did not attain statistical significance at the two-hour time point in Part 1 of the SERENITY III trial, we still believe that the results provide plenty of support for advancing to Part 2 of the study, particularly with the option for higher dosing or multiple doses. The results for the multiple dose trial of BXCL501 as part of the MDD program are very encouraging and it is good to see that no new safety signals were observed with multiple doses or twice-daily dosing. We have made no changes to our model and our valuation remains at $75 per share.

PROJECTED FINANCIALS

BioXcel Therapeutics, Inc.	2022 A	Q1 A	Q2 E	Q3 E	Q4 E	2023 E	2024 E	2025 E
BXCL501	$0	$0	$0	$1	$3	$5	$12	$35
BXCL701	$0	$0	$0	$0	$0	$0	$0	$0
Other Income	$0	$0	$0	$0	$0	$0	$0	$0
Total Revenues	**$0**	**$0**	**$0**	**$1**	**$3**	**$5**	**$18**	**$35**
Cost of Sales	$0	$0	$0	$0	$0	$0	$1	$2
Product Gross Margin	*95%*	*96%*	*96%*	*96%*	*96%*	*96%*	*94%*	*94%*
Research & Development	$91.2	$27.8	$23.0	$26.0	$29.0	$105.8	$105.0	$110.0
General & Administrative	$68.8	$23.6	$24.0	$24.0	$24.0	$95.6	$100.0	$108.0
Other (Income) Expense	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0
Operating Income	($159.6)	($51.1)	($46.6)	($49.0)	($50.1)	($196.9)	($188.0)	($185.0)
Operating Margin	-	-	-	-	-	-	-	-
Non-Operating Expenses (Net)	$6.1	$1.6	$1.5	$1.5	$1.5	$6.1	$6.0	$6.0
Pre-Tax Income	($165.8)	($52.7)	($48.1)	($50.5)	($48.6)	($190.8)	($182.0)	($179.0)
Income Taxes	$0.0	$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	**($165.8)**	**($52.7)**	**($48.1)**	**($50.5)**	**($48.6)**	**($190.8)**	**($182.0)**	**($179.0)**
Net Margin	-	-	-	-	-	-	-	-
Reported EPS	**($5.92)**	**($1.84)**	**($1.66)**	**($1.74)**	**($1.68)**	**($6.60)**	**($6.07)**	**($5.59)**
YOY Growth	-	-	-	-	-	-	-	-
Basic Shares Outstanding	28.0	28.6	29.0	29.0	29.0	28.9	30.0	32.0

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

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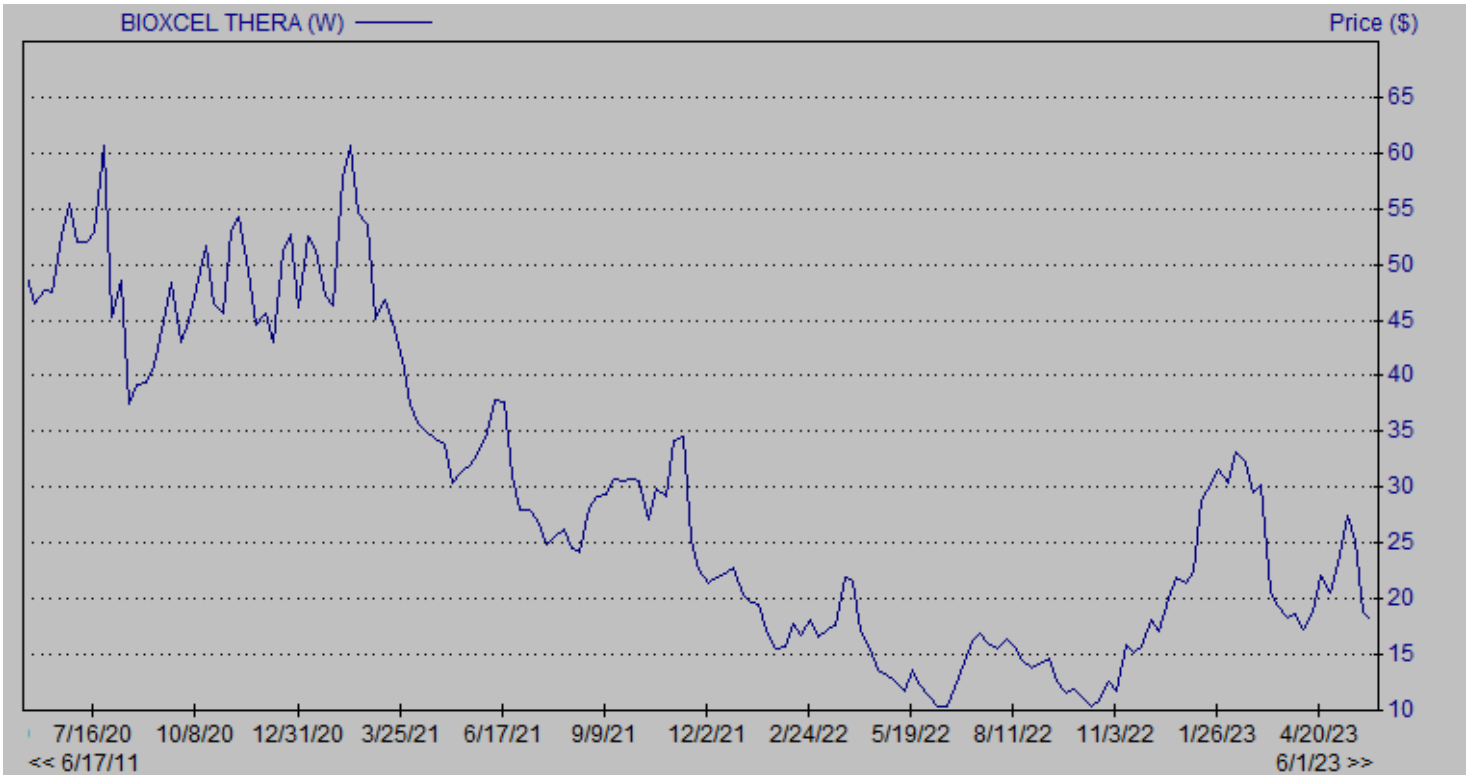
HISTORICAL STOCK PRICE



Source: Zacks SCR

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