Zacks Small-Cap Research
Sponsored – Impartial - Comprehensive

February 17, 2021
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: BXCL701 Shows Encouraging Anti-Tumor Activity When Combined with Pembrolizumab in mCRPC…

Based on our probability adjusted DCF model that takes into account potential future revenues of BXCL501 and BXCL701, BTAI is valued at $145.00/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 (02/17/21)	$56.48
Valuation	**$145.00**

OUTLOOK

On February 11, 2021, BioXcel Therapeutics, Inc. (BTAI) presented a poster at the 2021 ASCO GU Cancers Symposium that provided an update on the ongoing Phase 1b/2 trial of BXCL701, the company's oral innate immune system activator, in combination with pembrolizumab in metastatic castration resistant prostate cancer (mCRPC) patients. The results showed that BXCL701/pembrolizumab demonstrated encouraging anti-tumor activity in mCRPC patients with adenocarcinoma phenotype. BXCL701 was administered as 0.3 mg twice a day, which demonstrated an acceptable safety profile with mostly low grade, on target adverse events consistent with cytokine activation. The trial continues to enroll patients and we expect additional updates as the year progresses.

SUMMARY DATA

52-Week High	**$64.63**
52-Week Low	**$14.79**
One-Year Return (%)	**42.66**
Beta	**1.32**
Average Daily Volume (sh)	**541,563**
Shares Outstanding (mil)	**24**
Market Capitalization ($mil)	**$1,376**
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	**-16.8**
P/E using 2020 Estimate	**-30.9**

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)
2019	0 A	0 A	0 A	0 A	0 A
2020	0 A	0 A	0 A	0 E	0 E
2021					0 E
2022					113 E

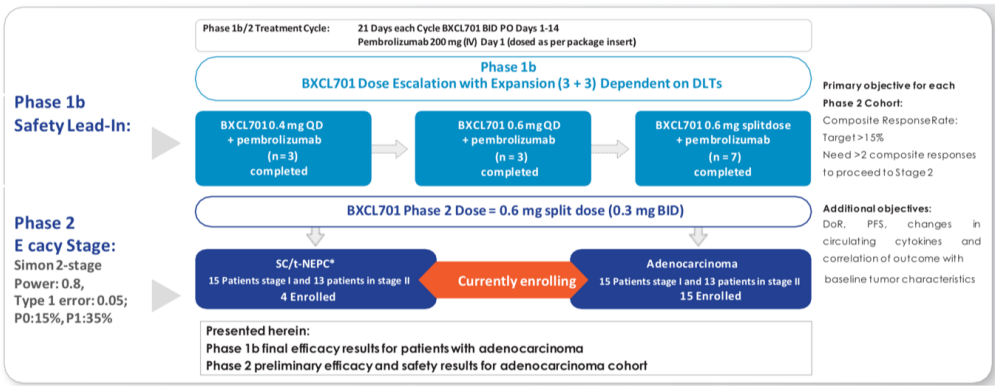
Earnings per Share

	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	-$0.79 A	-$1.06 A	-$1.07 A	-$0.56 E	-$3.45 E
2021					-$2.24 E
2022					$0.88 E

Business Update

Encouraging Anti-Tumor Activity for BXCL701 in mCRPC

On February 11, 2021, BioXcel Therapeutics, Inc. (BTAI) presented updated data for the ongoing Phase 1b/2 clinical trial of BXCL701, the company's oral innate immune system modulator, at the 2021 ASCO GU Cancers Symposium. The multicenter, open label trial is designed to determine the safety and efficacy 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 (NCT03910660). A schematic of the trial is shown below, with final efficacy results presented for patients with adenocarcinoma in the Phase 1b portion of the study and preliminary results presented for patients with adenocarcinoma in the Phase 2 portion of the study.



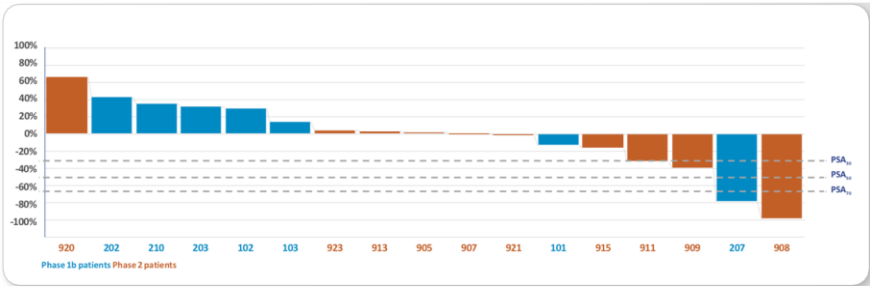
Source: BioXcel Therapeutics, Inc.

The following table shows the best response for patients in both the Phase 1b (n=7) and Phase 2 (n=15) portion of the study. For patients in the Phase 1b portion of the study, there was a disease control rate (DCR) of 100%, with one patient experiencing a partial response (PR) and another three patients experiencing stable disease (SD). In addition, one patient experienced a PSA_{50} response (50% reduction from peak PSA value). For the Phase 2 portion of the study, five of the patients were evaluable by RECIST 1.1 and there was a DCR of 80%. One patient experienced a PSA_{50} response and out of four evaluable patients for circulating tumor cells (CTC), 1 patient had a response (defined as conversion from ≥5/7.5 mL to <5/7.5 mL).

Best Response	Phase 1b Population Total = 7 n (%)	Phase 2 Population Total = 15 n (%)
RECIST 1.1 by Investigator Assessment[a]		
Evaluable	7	5
Measurable	4 (57%)	4 (80%)
Best RECIST Response		
PR	1 (14%)	0
SD (any duration)	3 (43%)	3 (60%)
Including Minor Response		2 (40%)
Disease Control Rate (PR + SD + Non-CR/Non-PD)	7 (100%)	4 (80%)
PD	0	1 (20%)
PSA		
PSA Evaluable[b]	7	10
PSA_{50} Response	1 (14%)	1 (10%)
CTC		
CTC Evaluable[c]	3	4
CTC Response[d]	0	1 (25%)
Composite Response	1 of 7 evaluable	1 of 10 evaluable to date

Source: BioXcel Therapeutics, Inc.

The following graph shows the best PSA response for patients in both the Phase 1b and Phase 2 portion of the trial. A total of 6/17 (35%) had any PSA response while 2/17 (12%) had a PSA_{70} response.



Source: BioXcel Therapeutics, Inc.

The poster presentation also highlighted a few of the best responders from the trial:

- PID 207: This patient had five prior systemic therapies (bicalutamide, abiraterone+prednisone, enzalutamide, sipuleucel, docetaxel+cabazitaxel). Thus far, they have received 16 cycles of BXCL701+pembrolizumab. The patient exhibited a PSA50 response by 6 weeks with a maximum reduction of 84% (baseline PSA = 163.1 ng/mL). The patient also exhibited a RECIST 1.1 PR by 12 weeks with a 35% reduction in target lesions and the disappearance of 2/5 non-target lymph nodes.

- PID 908: This patient had five prior systemic therapies (leuprolide, sipuleucel, enzalutamide, docetaxel, cabazitaxel). Thus far, they have received 5 cycles of BXCL701+pembrolizumab. A PSA50 response occurred at 3 weeks and is ongoing at 15 weeks (99% reduction; 25 ng/mL to 0.2 ng/mL). The patient also experienced a CTC conversion at 3 weeks. Lastly, the patient had a RECIST 1.1 minor response (19% reduction in target lesions) that is ongoing.

- PID 909: This patient had five prior systemic therapies (bicalutamide, leuprolide, enzalutamide, docetaxel, abiraterone+prednisone). Thus far, they have received 4 cycles of BXCL701+pembrolizumab. There was a 40% reduction in PSA (111.4 ng/mL to 66.9 ng/mL) at 12 weeks and a RECIST 1.1 minor response (27% reduction in target lesions) that is ongoing.

Regarding safety, the following table shows the reported adverse events that were judged by the investigator to be related to BXCL701. The majority of events were low grade and a number of them were consistent with cytokine activation. Grade 3 hypotension was seen in a patient during the first week of treatment, thus step up dosing was implemented for all new patients for the first week of treatment with 0.2 mg BXCL701 administered twice a day. Escalation to 0.3 mg twice a day was permitted if no treatment related adverse events Grade >1 or skipped doses due to hypotension or orthostasis occurred during the first week of treatment.

Preferred Term	Grade n = 13			
BXCL701 Related Events#	Grade 1 n	Grade 2 n	Grade 3 n	Total n
Subjects with any event	12	7	4	13
Hypotension*	2	2	1	5
Fatigue	4	1	-	5
Nausea	4	1	-	5
Dizziness	1	2	1	4
Vomiting	4	-	-	4
Rash	3	-	-	3
Decreased appetite	3	-	-	3
Decreased platelet count	3	-	-	3
Blood lactic acid increased	-	-	2	2
Myalgia	1	-	1	2
Chills	2	-	-	2
Fever	2	-	-	2
Constipation	2	-	-	2
Dry mouth	2	-	-	2

Source: BioXcel Therapeutics, Inc.

Conclusion

The Phase 1b/2 results for BXCL701 in combination with pembrolizumab for the treatment of advanced prostate cancer are encouraging, particularly the PSA responses seen in heavily pretreated patients who have few treatment options available. We look forward to additional updates from the trial later in the year. At this point the majority of the company's value is based on the potential for BXCL501, however based on the Phase 1b/2 results we have increased the contribution of BXCL701 to our model, which has slightly increased our valuation to $145.

PROJECTED FINANCIALS

BioXcel Therapeutics, Inc.	2019 A	Q1 A	Q2 A	Q3 A	Q4 E	2020 E	2021 E	2022 E
BXCL501	$0	$0	$0	$0	$0	$0	$0	$113
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**	**$113**
Cost of Sales	$0	$0	$0	$0	$0	$0	$0	$4
Product Gross Margin	-	-	-	-	-	-	-	-
Research & Development	$25.8	$12.4	$17.9	$16.3	$11.0	$57.6	$45.0	$47.0
General & Administrative	$7.8	$2.6	$3.5	$8.5	$2.8	$17.4	$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)	($15.0)	($21.4)	($24.8)	($13.8)	($75.0)	($57.0)	$22.0
Operating Margin	-	-	-	-	-	-	-	-
Non-Operating Expenses (Net)	$0.6	$0.1	$0.0	$0.0	$0.1	$0.2	$1.0	$1.0
Pre-Tax Income	($33.0)	($15.0)	($21.5)	($24.8)	($13.7)	($74.8)	($56.0)	$23.0
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)**	**($15.0)**	**($21.5)**	**($24.8)**	**($13.7)**	**($74.8)**	**($56.0)**	**$23.0**
Net Margin	-	-	-	-	-	-	-	-
Reported EPS	**($2.02)**	**($0.79)**	**($1.06)**	**($1.07)**	**($0.56)**	**($3.45)**	**($2.24)**	**$0.88**
YOY Growth	-	-	-	-	-	-	-	-
Basic Shares Outstanding	16.3	19.0	20.3	23.1	24.4	21.7	25.0	26.0

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

HISTORICAL STOCK PRICE

Source: Zacks SCR

DISCLOSURES

The following disclosures relate to relationships between Zacks Small-Cap Research ("Zacks SCR"), a division of Zacks Investment Research ("ZIR"), and the issuers covered by the Zacks SCR Analysts in the Small-Cap Universe.

ANALYST DISCLOSURES

I, David Bautz, PhD, hereby certify that the view expressed in this research report accurately reflect my personal views about the subject securities and issuers. I also certify that no part of my compensation was, is, or will be, directly or indirectly, related to the recommendations or views expressed in this research report. I believe the information used for the creation of this report has been obtained from sources I considered to be reliable, but I can neither guarantee nor represent the completeness or accuracy of the information herewith. Such information and the opinions expressed are subject to change without notice.

INVESTMENT BANKING AND FEES FOR SERVICES

Zacks SCR does not provide investment banking services nor has it received compensation for investment banking services from the issuers of the securities covered in this report or article.
Zacks SCR has received compensation from the issuer directly, from an investment manager, or from an investor relations consulting firm engaged by the issuer for providing non-investment banking services to this issuer and expects to receive additional compensation for such non-investment banking services provided to this issuer. The non-investment banking services provided to the issuer includes the preparation of this report, investor relations services, investment software, financial database analysis, organization of non-deal road shows, and attendance fees for conferences sponsored or co-sponsored by Zacks SCR. The fees for these services vary on a per-client basis and are subject to the number and types of services contracted. Fees typically range between ten thousand and fifty thousand dollars per annum. Details of fees paid by this issuer are available upon request.

POLICY DISCLOSURES

This report provides an objective valuation of the issuer today and expected valuations of the issuer at various future dates based on applying standard investment valuation methodologies to the revenue and EPS forecasts made by the SCR Analyst of the issuer's business.
SCR Analysts are restricted from holding or trading securities in the issuers that they cover. ZIR and Zacks SCR do not make a market in any security followed by SCR nor do they act as dealers in these securities. Each Zacks SCR Analyst has full discretion over the valuation of the issuer included in this report based on his or her own due diligence. SCR Analysts are paid based on the number of companies they cover. SCR Analyst compensation is not, was not, nor will be, directly or indirectly, related to the specific valuations or views expressed in any report or article.

ADDITIONAL INFORMATION

Additional information is available upon request. Zacks SCR reports and articles are based on data obtained from sources that it believes to be reliable, but are not guaranteed to be accurate nor do they purport to be complete. Because of individual financial or investment objectives and/or financial circumstances, this report or article should not be construed as advice designed to meet the particular investment needs of any investor. Investing involves risk. Any opinions expressed by Zacks SCR Analysts are subject to change without notice. Reports or articles or tweets are not to be construed as an offer or solicitation of an offer to buy or sell the securities herein mentioned.

CANADIAN COVERAGE

This research report is a product of Zacks SCR and prepared by a research analyst who is employed by or is a consultant to Zacks SCR. The research analyst preparing the research report is resident outside of Canada, and is not an associated person of any Canadian registered adviser and/or dealer. Therefore, the analyst is not subject to supervision by a Canadian registered adviser and/or dealer, and is not required to satisfy the regulatory licensing requirements of any Canadian provincial securities regulators, the Investment Industry Regulatory Organization of Canada and is not required to otherwise comply with Canadian rules or regulations.