UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 8-K

CURRENT REPORT
Pursuant to Section 13 or 15(d) of the
Securities Exchange Act of 1934

Date of Report (Date of earliest event reported) **October 10, 2023**

BioXcel Therapeutics, Inc.
(Exact name of registrant as specified in its charter)

Delaware	**001-38410**	**82-1386754**
(State or other jurisdiction of incorporation)	(Commission File Number)	(IRS Employer Identification No.)

555 Long Wharf Drive
New Haven, CT 06511
(Address of principal executive offices, including Zip Code)

(475) 238-6837
(Registrant's telephone number, including area code)

N/A
(Former name or former address, if changed since last report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	**Trading Symbol(s)**	**Name of each exchange on which registered**
Common Stock, par value $0.001	BTAI	The Nasdaq Capital Market

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company ☒

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☒

Item 7.01 Regulation FD Disclosure.

On October 10, 2023, BioXcel Therapeutics, Inc. (the "Company") issued a press release and provided an update on its Phase 2 trial of BXCL701 in combination with KEYTRUDA® (pembrolizumab). In addition, as announced in the foregoing press release, the Company will be hosting a webcast on October 10, 2023 at 8:00 a.m. Eastern Time to discuss the updates. A copy of the press release and the presentation materials that will be used at this webcast are furnished hereto as Exhibits 99.1 and 99.2, respectively, and are incorporated herein by reference, and will also be available through the "Investors & Media" page of the Company's website at http://www.bioxceltherapeutics.com.

The information in this Current Report on Form 8-K, including Exhibits 99.1 and 99.2 hereto, shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that Section, nor shall it be deemed to be incorporated by reference into any filing of the Company under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such filing.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits.

Exhibit No.	**Description**
99.1	Press Release, dated October 10, 2023
99.2	BioXcel Therapeutics, Inc. Presentation, dated October 10, 2023
104	Cover Page Interactive Data File - the cover page XBRL tags are embedded within the Inline XBRL document

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Date: October 10, 2023

BIOXCEL THERAPEUTICS, INC.

/s/ Richard Steinhart
Richard Steinhart
Chief Financial Officer

Exhibit 99.1

BioXcel Therapeutics Reports Positive Overall Survival Results from Single-Arm, Open-Label Phase 2 Trial of BXCL701 in Patients with Small Cell Neuroendocrine Prostate Cancer

Median overall survival of 13.6 months with BXCL701 + KEYTRUDA® (pembrolizumab), compared to 7.6 months with checkpoint inhibitor monotherapy (CPI) in late-line refractory patients in separate, Phase 2 trial[1]

56% of studied patients alive at one year following treatment with BXCL701 + KEYTRUDA®, compared to ~33% with checkpoint inhibitor monotherapy (CPI) in late-line refractory patients in separate, Phase 2 trial[1]

Results support potential development in small cell lung cancer (SCLC) and other neuroendocrine tumors

Company to host conference call and webcast today at 8:00 a.m. ET

NEW HAVEN, Conn., Oct. 10, 2023 — BioXcel Therapeutics, Inc. (Nasdaq: BTAI), a biopharmaceutical company utilizing artificial intelligence to develop transformative medicines in neuroscience and immuno-oncology, today announced positive overall survival (OS) data from its Phase 2 trial of BXCL701, the Company's investigational oral innate immune activator, in combination with KEYTRUDA® (pembrolizumab) in patients with small cell neuroendocrine prostate cancer (SCNC). As of a data cutoff of September 6, 2023, evaluable patients with SCNC (n=28) showed a median OS of 13.6 months (95% CI 10.9–NR), and a 12-month survival rate of 56.5%.

“OS is the most meaningful measure by which the effectiveness of an oncology treatment is evaluated. Though these results are based on a non-randomized cohort of patients, observing a median OS of this duration including patients with long-term survival at 12 months and beyond shows exceptional promise, bearing in mind historic data with checkpoint inhibitor monotherapy in this high-risk subset of prostate cancer,” said Rahul Aggarwal, M.D., Principal Investigator, Associate Director for Clinical Sciences, Helen Diller Family Comprehensive Cancer Center, and Professor of Medicine at the University of California San Francisco (UCSF). “SCNC represents a major unmet medical need, with the majority of patients unfortunately succumbing to their disease in less than one year following chemotherapy. The results of this trial suggest that BXCL701 has the potential to extend the lives of patients, and I look forward to its continued clinical development.”

SCNC, classified as a “cold” tumor, represents an underserved, growing patient population, with cases increasing due to earlier and more widespread use of androgen receptor inhibitors. In 2023, there will be an estimated 288,300[2] new patients with prostate cancer in the United States, with approximately 11,532 patients progressing to SCNC.

“We believe our trial results are highly encouraging for patients with this disease and have potential implications for our evaluation of BXCL701 for the treatment of other high-grade neuroendocrine tumors, such as small cell lung cancer, where effective therapies are lacking,” said Vincent J. O’Neill, M.D., Chief R&D Officer, OnkosXcel Therapeutics, a wholly owned subsidiary of BioXcel Therapeutics. “We intend to discuss these data with the FDA to help determine our next steps with clinical development.”

The Phase 2 trial is an open-label, multicenter study to evaluate the safety and efficacy of BXCL701 in combination with pembrolizumab in men with SCNC. Twenty-eight (28) evaluable SCNC patients received 0.3 mg of BXCL701 twice daily (BID) on days 1 through 14 of a 21-day cycle (0.2 mg BID the first week of cycle 1) plus 200 mg of pembrolizumab administered intravenously on day 1 and every subsequent 21 days. The primary objective of the trial is a composite response rate defined as either objective response by RECIST 1.1 criteria and/or PSA_{50} and/or CTC count conversion. Secondary objectives include duration of response, progression-free survival, overall survival, and biomarker evaluation as measured by changes in circulating cytokines and correlation of outcome with baseline tumor characteristics.

The Company is continuing to actively evaluate strategic options for OnkosXcel Therapeutics, including potential financing, strategic partnership, or M&A.

Conference Call

BioXcel Therapeutics will host a conference call and webcast on October 10, 2023 at 8:00 a.m. ET to discuss the data results from the Phase 2 trial of BXCL701. To access the call, please dial 877-407-5795 (domestic) or 201-689-8722 (international). A live webcast and presentation materials will be available on the Investors section of the corporate website, bioxceltherapeutics.com, and a webcast replay will be available through January 10, 2024.

BioXcel Therapeutics may use its website as a distribution channel of material information about the Company. Financial and other important information is routinely posted on and accessible through the Investors sections of its website at www.bioxceltherapeutics.com. In addition, you may sign up to automatically receive email alerts and other information about the Company by visiting the “Email Alerts” option under the News/Events section of the Investors & Media website section and submitting your email address.

About Metastatic Castration-Resistant Prostate Cancer (mCRPC) and Treatment-Emergent Small Cell Neuroendocrine Prostate Cancer (SCNC)

mCRPC is a form of advanced prostate cancer that is no longer responding to testosterone-lowering hormone treatments and has spread to other areas of the body such as the lymph nodes, bones, bladder, rectum, liver, or lungs. Treatment-emergent SCNC is a particularly difficult-to-treat histologic subtype of mCRPC that emerges in approximately 20% of mCRPC patients, though this number is increasing due to earlier and more widespread use of androgen blockers.

About OnkosXcel Therapeutics, LLC and BXCL701

OnkosXcel Therapeutics, LLC is a wholly owned subsidiary of BioXcel Therapeutics, Inc., focused on developing transformative medicines in oncology utilizing artificial intelligence. The subsidiary was formed in 2022 to develop BXCL701.

BXCL701 is an investigational, oral innate immune activator designed to initiate inflammation in the tumor microenvironment. Approved and experimental immunotherapies often fail to address cancers that appear “cold.” Therefore, BXCL701 is being evaluated to determine if it can render “cold” tumors “hot,” making them more detectable by the adaptive immune system and thereby facilitating the development of a strong anti-cancer immune response. OnkosXcel Therapeutics’ preclinical data support BXCL701’s potential synergy with both current checkpoint inhibitors and emerging immunotherapies directed to activate T-cells. BXCL701 is currently being developed as a potential therapy for the treatment of aggressive forms of prostate cancer and advanced solid tumors that are refractory or treatment naïve to checkpoint inhibitors. BXCL701 has received Orphan Drug Designation from the U.S. Food & Drug Administration (FDA) in four indications: acute myelogenous leukemia, pancreatic cancer, stage IIb to IV melanoma, and soft tissue sarcoma. An approximately 800-subject clinical database, with data collected by the Company and others, supports the ongoing development of BXCL701.

About BioXcel Therapeutics, Inc.

BioXcel Therapeutics, Inc. (Nasdaq: BTAI) is a biopharmaceutical company utilizing artificial intelligence to develop transformative medicines in neuroscience and immuno-oncology. The Company's drug re-innovation approach leverages existing approved drugs and/or clinically validated product candidates together with big data and proprietary machine learning algorithms to identify new therapeutic indications. For more information, please visit bioxceltherapeutics.com.

Forward-Looking Statements

This press release includes "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. We intend such forward-looking statements to be covered by the safe harbor provisions for forward-looking statements contained in Section 27A of the Securities Act of 1933, as amended (the "Securities Act") and Section 21E of the Securities Exchange Act of 1934, as amended (the "Exchange Act"). All statements contained in this press release other than statements of historical fact should be considered forward-looking statements, including, without limitation, statements regarding: the Company's expected timing of, trial design and data results from, future clinical trials of BXCL701 with pembrolizumab, potential benefits from treatment with BXCL701, the Company's planned discussions with FDA, the Company's plans to evaluate strategic options for OnkosXcel Therapeutics and potential market size and opportunity for product candidates. The words "anticipate," "believe," "can," "continue," "could," "designed," "estimate," "expect," "forecast," "goal," "intend," "may," "might," "plan," "possible," "potential," "predict," "project," "should," "target," "will," "would" and similar expressions are intended to identify forward-looking statements, though not all forward-looking statements use these words or expressions. In addition, any statements or information that refer to expectations, beliefs, plans, projections, objectives, performance or other characterizations of future events or circumstances, including any underlying assumptions, are forward- looking. All forward-looking statements are based upon the Company's current expectations and various assumptions. The Company believes there is a reasonable basis for its expectations and beliefs, but they are inherently uncertain. The Company may not realize its expectations, and its beliefs may not prove correct. Actual results could differ materially from those described or implied by such forward-looking statements as a result of various important factors, including, without limitation, its limited operating history; its incurrence of significant losses; its need for substantial additional funding and ability to raise capital when needed; its ability to successfully negotiate amended terms under the financing agreements to be able to access funding and to obtain relief under financial covenants; its significant indebtedness and potential payment obligations related to such indebtedness and other contractual obligations; risks associated with the strategic reprioritization; its limited experience in drug discovery and drug development; risks related to the TRANQUILITY II Phase 3 trial and related audit; its dependence on the success and commercialization of IGALMI™, BXCL501, BXCL502 BXCL701 and BXCL702 and other product candidates; its lack of experience in marketing and selling drug products; the risk that IGALMI or the Company's product candidates may not be accepted by physicians or the medical community in general; the failure of preliminary data from its clinical studies to predict final study results; failure of its early clinical studies or preclinical studies to predict future clinical studies; its ability to receive regulatory approval for its product candidates; its ability to enroll patients in its clinical trials; undesirable side effects caused by the Company's product candidates; its novel approach to the discovery and development of product candidates based on EvolverAI; the significant influence of and dependence on BioXcel LLC; its exposure to patent infringement lawsuits; its reliance on third parties; its ability to comply with the extensive regulations applicable to it; impacts from data breaches or cyber-attacks, if any; impacts from the COVID-19 pandemic; risks associated with the increased scrutiny relating to environmental, social and governance (ESG) matters; its ability to commercialize its product candidates; and the other important factors discussed under the caption "Risk Factors" in its Quarterly Report on Form 10-Q for the quarterly period ended June 30, 2023, which are accessible on the SEC's website at www.sec.gov. These and other important factors could cause actual results to differ materially from those indicated by the forward-looking statements made in this press release. Any such forward-looking statements represent management's estimates as of the date of this press release. While the Company may elect to update such forward-looking statements at some point in the future, except as required by law, it disclaims any obligation to do so, even if subsequent events cause our views to change. These forward-looking statements should not be relied upon as representing the Company's views as of any date subsequent to the date of this press release.

Contact Information

Corporate

BioXcel Therapeutics
Erik Kopp
1.203.494.7062
ekopp@bioxceltherapeutics.com

Investor Relations

BioXcel Therapeutics
Brennan Doyle
1.475.355.8462
bdoyle@bioxceltherapeutics.com

Media

Russo Partners
Scott Stachowiak
1-646-942-5630
Scott.stachowiak@russopartnersllc.com

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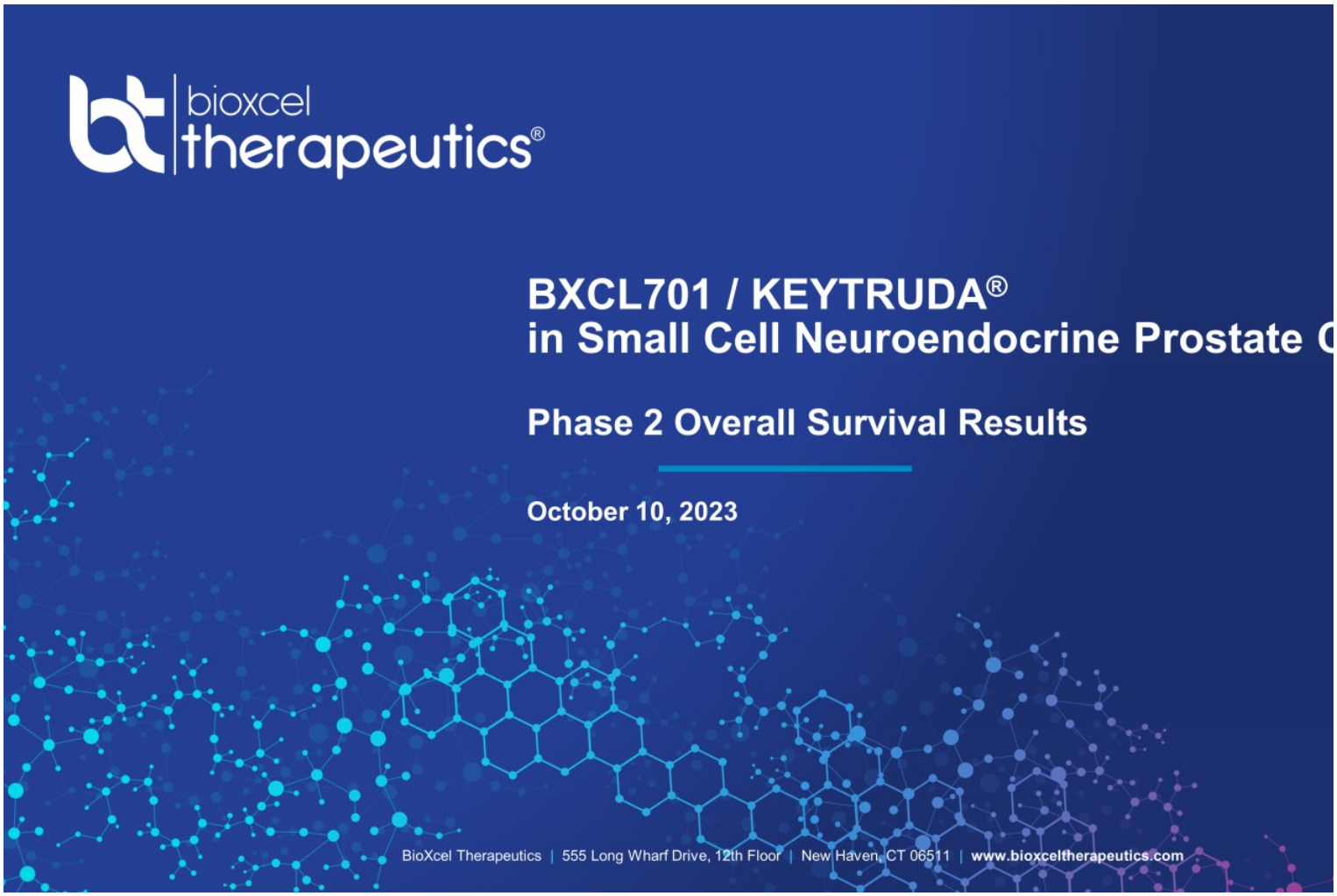
[1] FOR ILLUSTRATIVE PURPOSES ONLY: Analysis based on results from avelumab PICK-NEPC study. Landon C. Brown et al. Prostate Cancer and Prostatic Diseases; https://doi.org/10.1038/s41391-022-00524-7. No head-to-head clinical trial has been conducted evaluating BXCL701 against avelumab or other candidates or products. Notable differences exist between the Company's trial designs, conditions under study and subject characteristics as compared to the evaluated third-party Phase 2 results discussed above and caution should be exercised when comparing data across these studies. One year survival rate extrapolated by the Company using Kaplan-Meier estimates of overall survival curve from the PICK-NEPC study. Please see the “Appendix” of the presentation materials for the conference call being held today, October 9, 2023, for information regarding the differences between the clinical trials.

2. American Cancer Society. Key Statistics for Prostate Cancer. Retrieved October 9, 2023. https://www.cancer.org/cancer/types/prostate-cancer/about/key-statistics.html#:~:text=The%20American%20Cancer%20Society's%20estimates,34%2C700%20deaths%20from%20prostate%20cancer

Exhibit 99.2


bioxcel therapeutics®
BXCL701 / KEYTRUDA®
in Small Cell Neuroendocrine Prostate C
Phase 2 Overall Survival Results
October 10, 2023
BioXcel Therapeutics | 555 Long Wharf Drive, 12th Floor | New Haven, CT 06511 | www.bioxceltherapeutics.com

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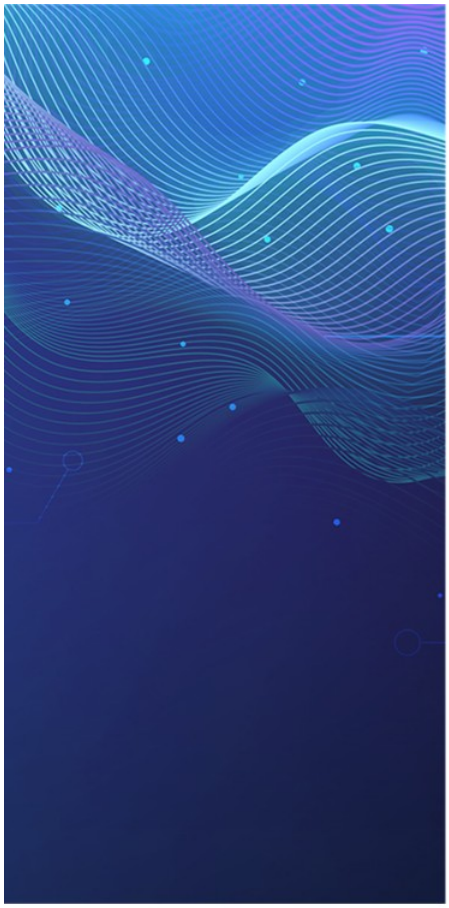


Agenda

Overview and Summary

- Vimal Mehta, Ph.D., Founder and CEO, BioXcel Therapeutics

Phase 2 Overall Survival Results

- Vincent J. O'Neill, M.D., Chief R&D Officer, OnkosXcel Therapeutics

Q&A Session

Transformative Drug Re-innovation Approach Using AI

bioxcel therapeutics®

Targeting High Unmet Needs in Neuroscience

Neuroscience (BXCL501): First-in-human trials to FDA approval and launch in under 4 years

- IGALMI™ (dexmedetomidine) sublingual film, for the acute treatment of agitation in schizophrenia or bipolar I and II disorder in adults
- Multiple potential additional indications for BXCL501, if approved

AI Engine

OnkosXcel Therapeutics

A subsidiary of BioXcel Therapeutics, Inc.

Developing Trans Medicine in Hard-

Lead Oncology Drug Candidate: BXC

- Unique oral innate immune activator, turn cold tumors hot via DPP8/9 inhil
- Combination approach, BXCL701+ K (pembrolizumab)
- Potential to extend the value of immu in large underserved patient populati
- Focusing on cold tumor types
- Positive Phase 2 data in SCNC pres GU 2023

BXCL701: Strong Value Proposition in Hard-to-Treat Tumo

Mechanism of Action
Data Published in JITC

One of the most clinically advanced oral innate immune activators, designed to activate inflammasome via DPP8/9 inhibition*

Full Phase 2 Data for SCN
Presented at ASCO GU 20

- Composite response rate: 25%
- Median duration of response: 6+ month
- Generally well tolerated in combination

Clinical Proof of Concept
Cold Tumors

- Demonstrated positive efficacy results in two cancer types: small cell neuroendocrine prostate cancer (SCNC) and mCRPC adenocarcinoma
- ~800-subject clinical safety database

Leadership Position in Innate I
DPP8/9 Biology

Forty Seven

Acquired for ~$5B by

GILEAD

Scarcity of assets in innate immunity

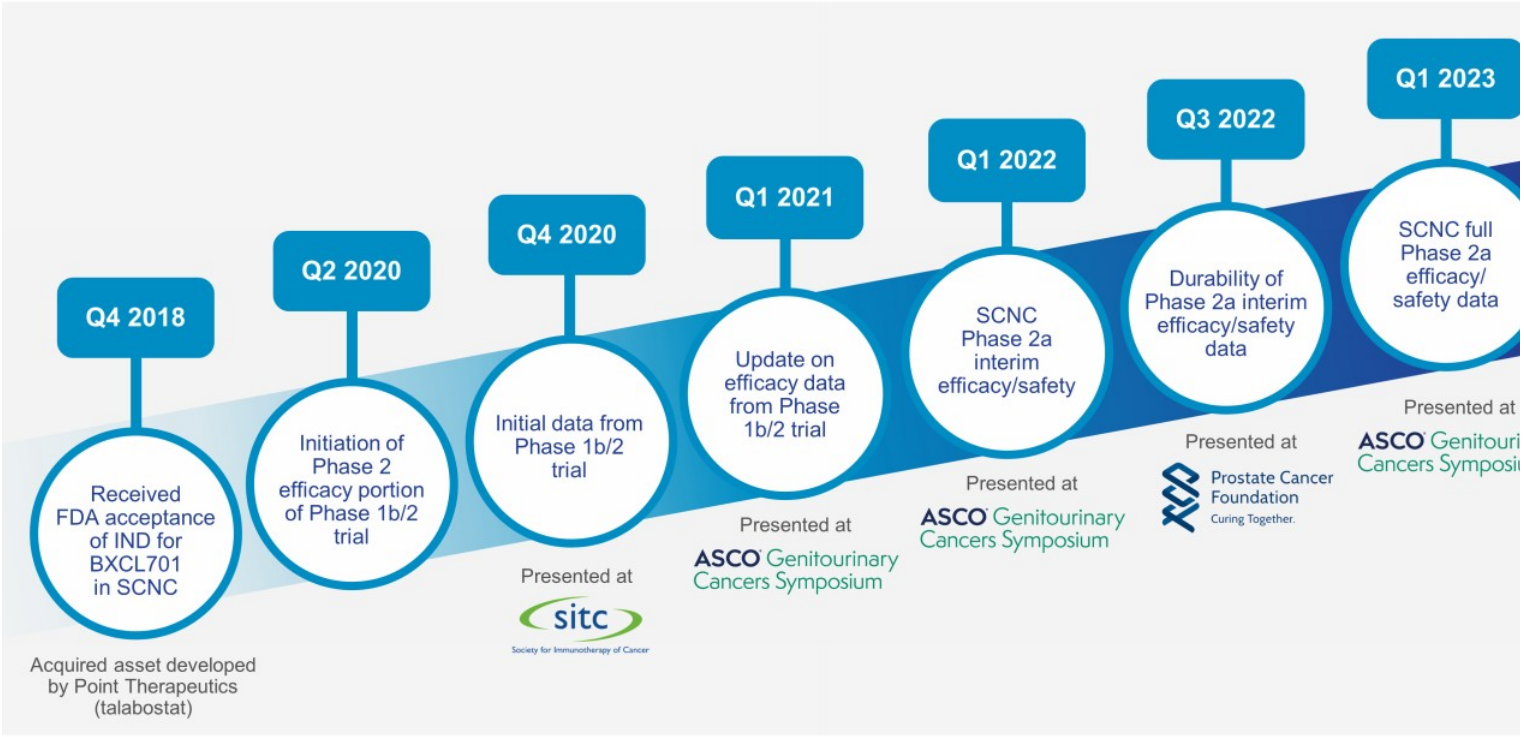
OnkosXcel Therapeutics

Exploring Strategic Options

* Source: www.clinicaltrials.gov

First AI-Derived Human POC for Oral Innate Immune Activa

Utilizing extensive data from 11 prior clinical trials and ~700 subjects



Phase 2 Overall Survival Results

High Unmet Need in SCNC
No FDA-Approved Therapy and Increasing Incidence

288,300 men diagnosed with prostate cancer in U.S. in 2023[1]
~20% expected to progress to more aggressive mCRPC

- **~20% of these mCRPC patients will develop S phenotype**, characterized by poor prognosis and survival rate
- Current treatment protocols that are sub-optimal platinum-based cytotoxic chemotherapies despite duration of response and considerable toxicities
- Current CPIs[2] targeting PD-1 and CTLA-4 have n demonstrated meaningful single-agent therapeuti benefit in SCNC

American Cancer Society. Key Statistics for Prostate Cancer. Retrieved October 9, 2023. https://www.cancer.org/cancer/types/prostate-cancer/about/key-statistics.html#:~:text=The%20American%20Cancer%20Society's%20estimates,34%2C700%20deaths%20from%20prostate%20cancer

[2] Checkpoint Inhibitors

BXCL701 Survival Benefit and Antitumor Activity in SCNC

In the context of historical data with checkpoint inhibitor monotherapy in this high-risk subset of pr

Survival Results

- **Median overall survival** with BXCL701 almost double — **13.6 months** *vs.* **7.4***
- **12-month survival rate** almost double with BXCL701 — **56.5%** *vs.* **~33%*** ****

Antitumor Activity

- **Compelling tumor shrinkage**: high response rate (partial response) in MSS/TMB lov with BXCL701
  - **Overall Response Rate (RECIST) 20%** *vs.* **6.7%***
- **High rates of stable disease and disease control** (stable disease + partial response BXCL701
  - **Disease Control Rate 48%** *vs.* **27%***

* Avelumab PICK-NEPC study. Brown, L.C., Halabi, S., Somarelli, J.A. et al. A phase 2 trial of avelumab in men with aggressive-variant or neuroendocrine prostate ca Prostate Cancer Prostatic Dis 25, 762–769 (2022). https://doi.org/10.1038/s41391-022-00524-7 ** Extrapolated by the Company using Kaplan-Meier estimates of over survival curve from avelumab PICK-NEPC study

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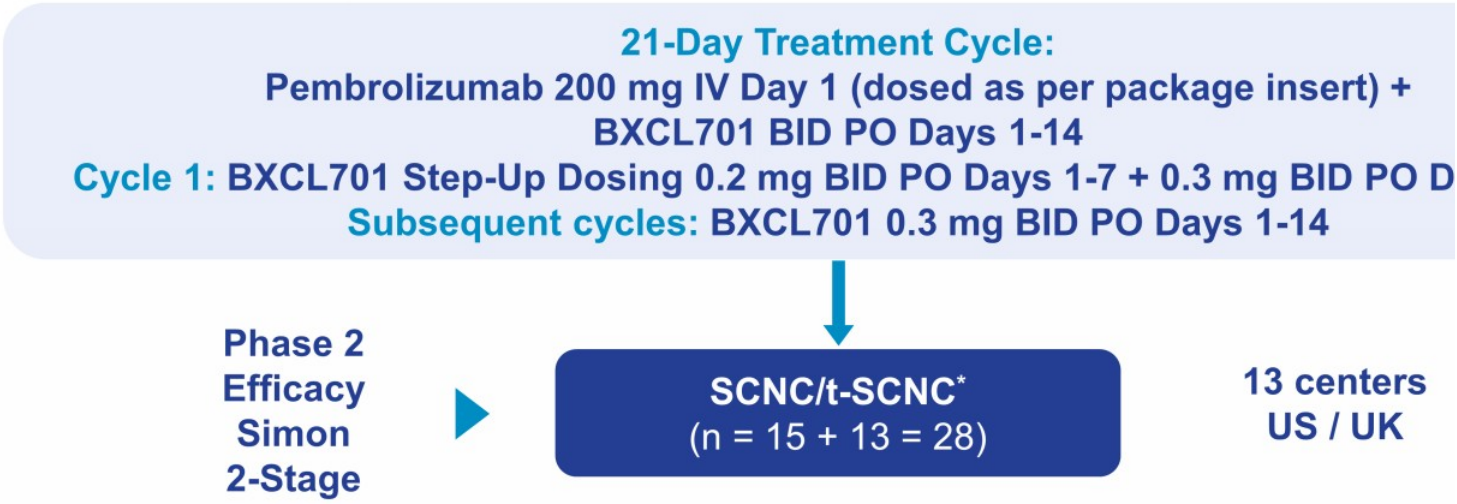
BXCL701 SCNC Phase 2 Trial Design



Primary objective: Composite Response Rate, either objective response by RECIST 1
and/or CTC Conversion from ≥5/7.5 mL to <5/7.5 mL, and/or ≥-50% PSA decline from I

Additional objectives: DoR, OS, PFS, changes in circulating cytokines and predictive biomarker identification

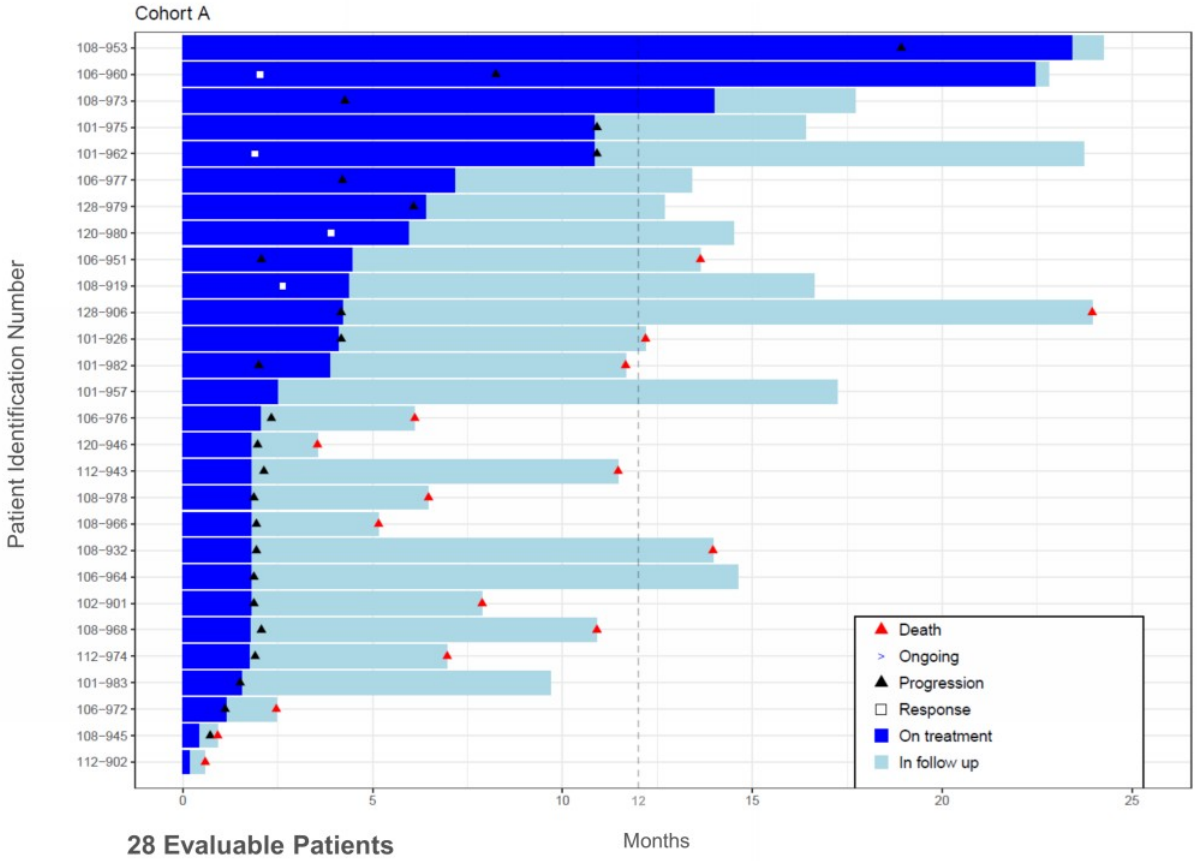
* Small Cell Neuroendocrine Prostate Cancer/Treatment-emergent Small Cell Neuroendocrine Prostate Cancer

SCNC Patients Baseline Characteristics

ASCO Genitouri
Cancers Symposi

Patients Enrolled n = 34	
Median Age (range)	67.5 ye
ECOG Performance Status	
0	16
1	16
2	2
Visceral Metastases	
Any site	21
Liver	11
Median lines of prior systemic therapy (range)	3
Prior Systemic Treatment	
Androgen signaling inhibitor(s)	25
Platinum-based Chemotherapy	19
Taxane Chemotherapy	17

SCNC Patients Swimmer's Plot



28 Evaluable Patients

Median durat
follow- up = 2
(11.3 – 38.2)

6 patients (21
beyond prog

Data cut-off for survival 0

SCNC Patients Efficacy Results

ASCO Genitouri
Cancers Symposi

Best Response	SCNC Evaluable Patients n = 28 (%) [95% Exact CI]
Composite Response *(includes unconfirmed PR)*	**7 (25%)** [8.3–41]
Best RECIST 1.1 Response by Investigator Assessment	
RECIST Evaluable[a]	**25 (89%)**
Partial Response	**5 (20%)** [6.8–40.7]
Confirmed PR	*4 (16%)*
Unconfirmed PR	*1 (4%)*
Stable Disease (any duration)	7 (28%)
Progressive Disease	13 (52%)
Disease Control Rate (PR + SD)	**12 (48%)**
CTC[b]	
CTC Evaluable[c]	**4**
CTC Response[d]	**1 (25%) [0.6–80.6]**
PSA	
PSA Evaluable[e]	**27 (96%)**
PSA_{50} Response	**3 (11%) [2.4–29]**

Objective response ra

4 confirmed partial re
1 unconfirmed partial

Median duration of re

6+ months
(range: 1.8 - 9.8 mont

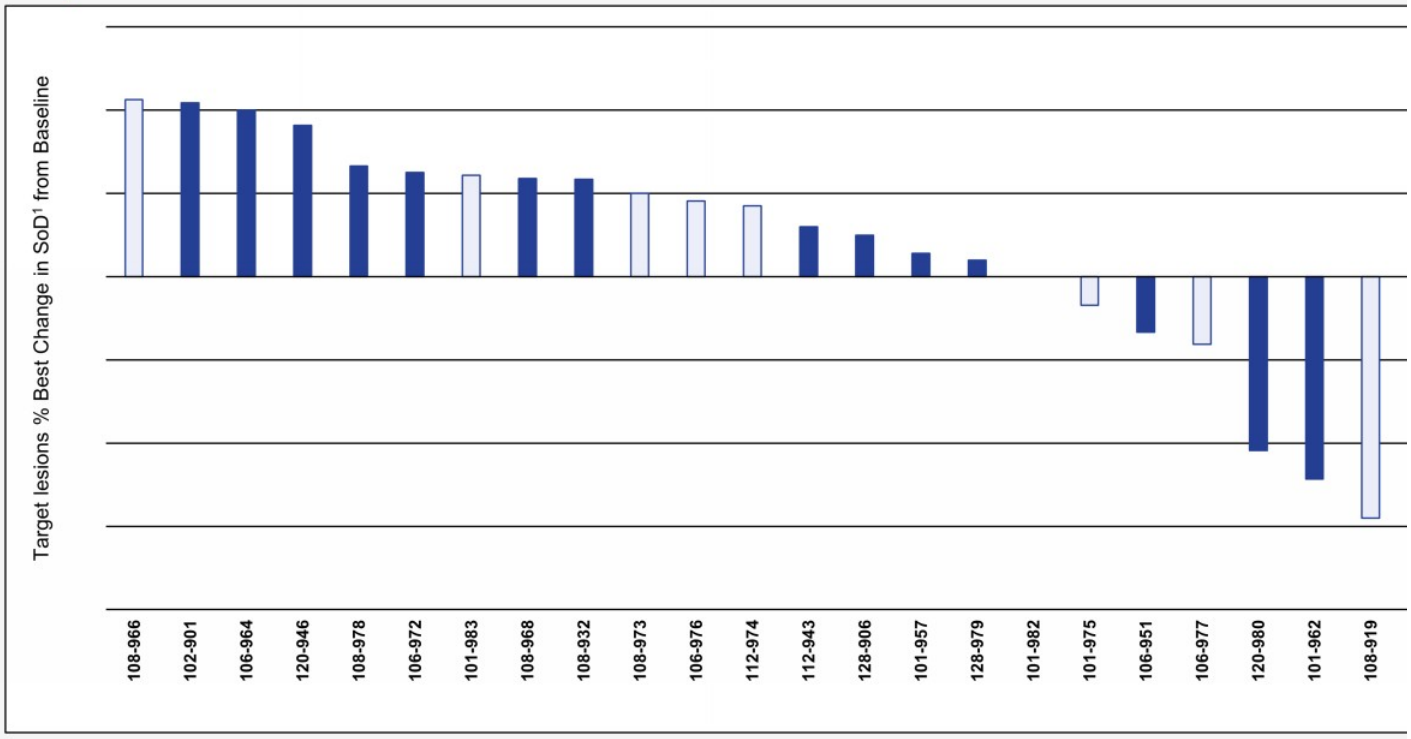
Data cut-off 19-DEC-22

[a] Patients who received ≥2 cycles of study therapy and had 1 on-treatment tumor assessment [b] Circulating tumor cell [c] Baseline CTC value ≥5/7.5 mL and 1 measurable on-treatment assessment [d] CTC conversion from ≥5/7.5 mL to <5/7.5 mL; [e] Baseline PSA >4 ng/mL and 1 on-treatment PSA assessment

SCNC Patients Best Response

All responders are MSS and/or TMB low

ASCO Genitourin
Cancers Symposiu



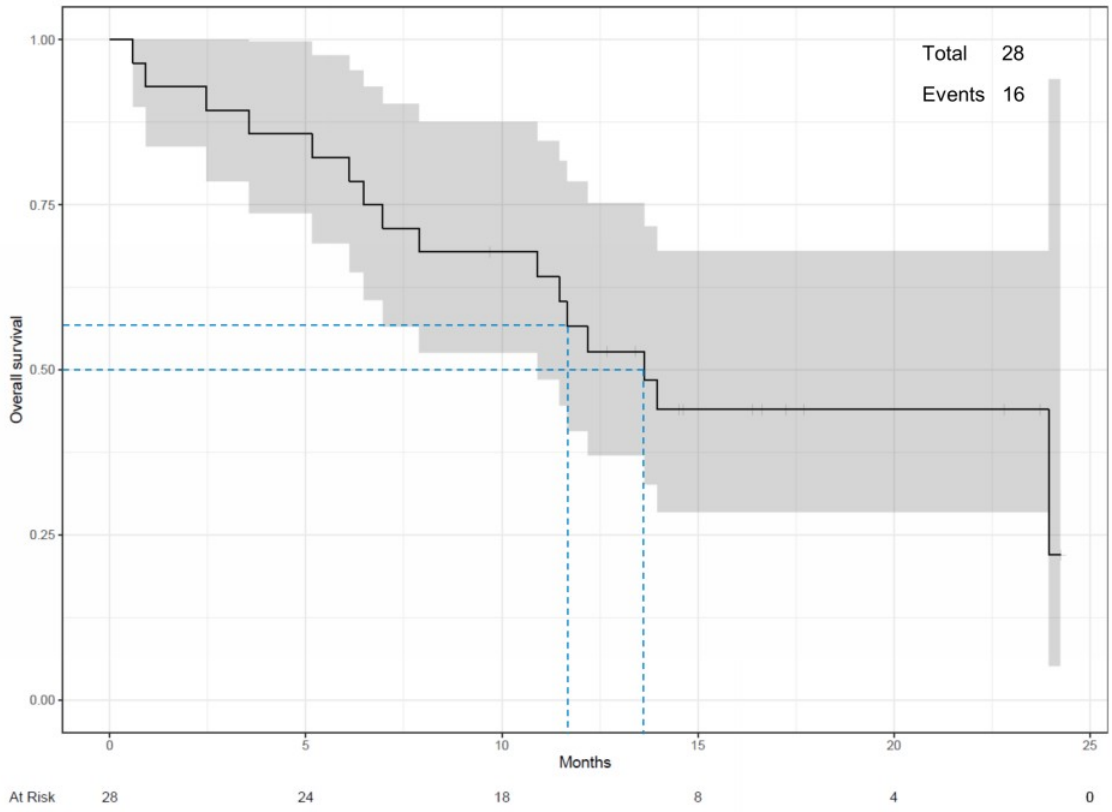
[1] SoD = Sum of Diameters

No Prior Platinum Chemotherapy

Prior Platinum Chemotherapy

Data cut-

SCNC Patients Overall Survival Kaplan-Meier Curve

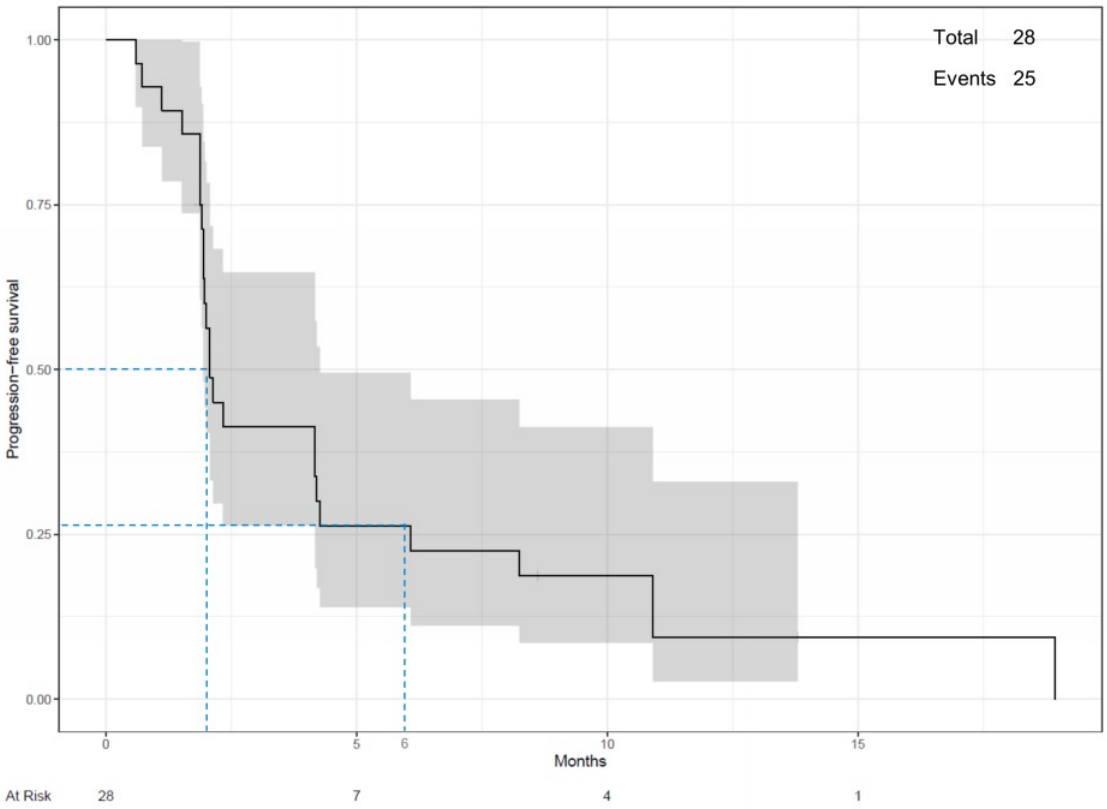


Median OS 13
(95% CI 10

12-month su
56.5

Data cut-off 06-SEP-23

SCNC Patients Radiographic Progression-Free Survival Kaplan-Meier Curve



mPFS 2.07
(95% CI 1.9

6-month PF
(95% CI 9.

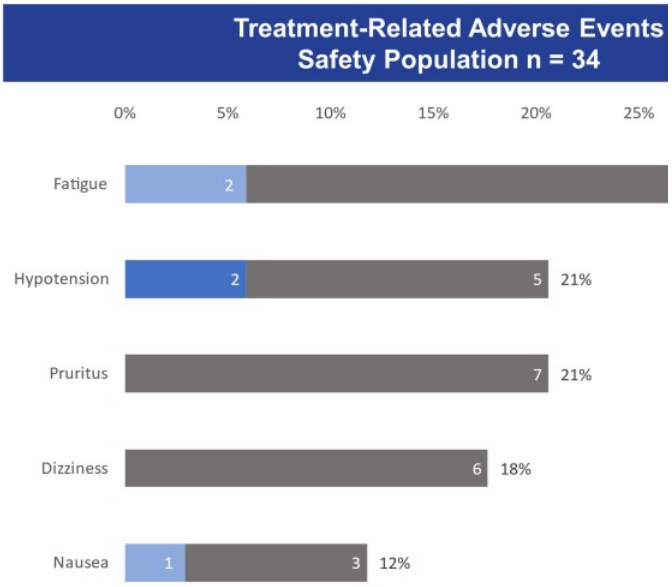
Data cut-off 06-SEP-23

BXCL701 / KEYTRUDA® Combination Has Demonstrated Manageable Safety

ASCO Genitouri Cancers Symposi

Treatment-Emergent Adverse Events n = 34	n (%)
Any Grade	**33** (97%)
Attributed to BXCL701	**29** (85%)
Attributed to Pembrolizumab	**23** (68%)
Grade 3	**16** (47%)
Grade 4	**0**
Grade 5	**1*** (3%)
AE Leading to Treatment Discontinuation	**6** (18%)
BXCL701 Discontinuation	**6** (18%)
Pembrolizumab Discontinuation	**5** (15%)
Immune-Related Adverse Events Any Grade	**14** (41%)
Grade ≥3	**1**** (7%)

* Grade 5 tumor lysis syndrome
** Grade 3 Colitis



At least possibly related to BXCL701 or pembrolizumab, occurring

Data cut-off 1

BXCL701 Study *vs.* Avelumab PICK-NEPC Study: Comparability of Patient Populations

	BXCL701	Avelumab PICK-N
n patients	28 patients evaluable	15 patients evalua
Histology Confirmed SCNC	100%	33%
Median age	67.5 years (range, 54–80)	71 years (range, 51
Median lines of prior systemic therapy	3 (range, 1 – 8)	2 (range, 1 – 3
Platinum-based Chemotherapy	68%	26.7%
Taxane Chemotherapy	50%	86.6%

Avelumab PICK-NEPC study. Brown, L.C., Halabi, S., Somarelli, J.A. et al. A phase 2 trial of avelumab in men with aggressive-variant or neuroendocrine prostate cancer. Prostate Cancer Prostatic Dis 25, 762–769 (2022). https://doi.org/10.1038/s41391-022-00524-7

FOR ILLUSTRATIVE PURPOSES ONLY: no head-to-head clinical trial has been conducted evaluating BXCL701 against avelumab or other candidates or products. Notable differences exist between the Company's trial designs, conditions under study and subject characteristics as compared to those evaluated third-party Phase 2 results discussed above and caution should be exercised when comparing data across these studies. See Appendix for description of the differences between the trial designs.

BXCL701 Study *vs.* Avelumab PICK-NEPC Study Efficacy / Survival Results

	BXCL701	Avelumab PICK
Composite Response Rate	7 / 28 (25%)	NA
Overall Response Rate RECIST	5 / 25 (20%) (**all responders MSS and/or TMB low**)	1 / 15 (6.7% (**the responder is M**
Median Duration of Response RECIST	6+ months[1]	24 months (**the responder is M**
Disease Control Rate	12 / 25 (48%)	4 / 15 (27%
PSA Response Rate	3 / 27 (11%)	1 / 15 (6.7%
mPFS	2.07 months (95% CI 1.94–4.27)	1.8 months (95% C
mOS	13.6 months (95% CI 10.9–NR)	7.4 months (95% CI
12-month Survival Rate	56.5%	~33%[2]

[1] patients with confirmed response n = 4 [2] Extrapolated by the Company using Kaplan-Meier estimates of overall survival curve from avelumab PICK-NEPC study

Avelumab PICK-NEPC study. Brown, L.C., Halabi, S., Somarelli, J.A. et al. A phase 2 trial of avelumab in men with aggressive-variant or neuroendocrine prostate cancer. Prostate Cancer Prost Dis 25, 762–769 (2022). https://doi.org/10.1038/s41391-022-00524-7

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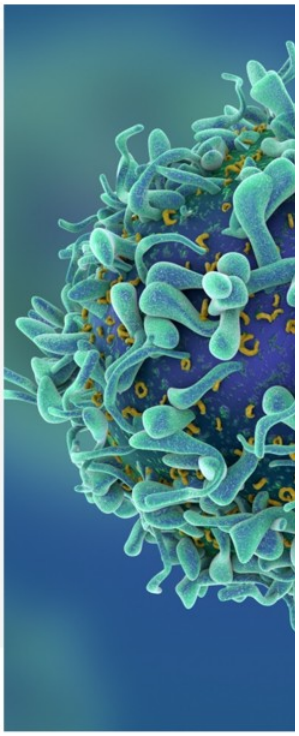
Conclusions

- BXCL701 + pembrolizumab demonstrated **compelling median OS and 12-month survival rate**
  - In excess of historic data from avelumab PICK-NEPC trial
  - OS represents the gold standard for measuring effectiveness
  - No MSI-H/TMB high patients detected in responders
- BXCL701 + pembrolizumab demonstrated **manageable safety**
  - Split and step dosing to mitigate cytokine release
  - No evidence of potentiation of immune-related adverse events
- Next steps including pivotal study design to be discussed with FDA
- Results have **potential implications for treatment of other high-grade neuroendocrine tumors**, such as small cell lung cancer

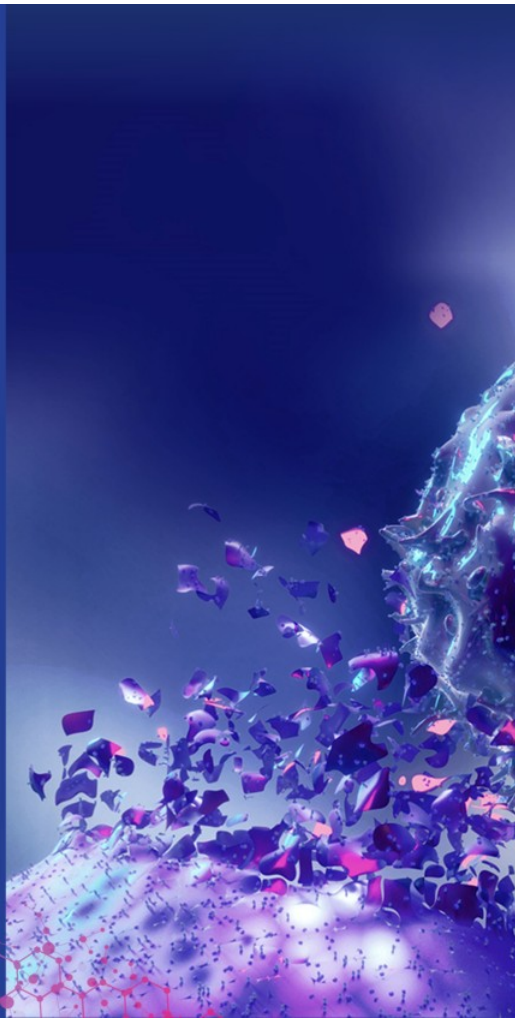


Q&A Period

Thank you

BioXcel Therapeutics | 555 Long Wharf Drive, 12th Floor | New Haven, CT 06511 | www.bioxceltherapeutics.com

Appendix



BXCL701-201 Study *vs.* Avelumab PICK-NEPC Study Desig

	BXCL701	Avelumab PICK-NI
Simon 2-Stage Study Design	Multicenter, prospective, single-arm Phase 2 clinical trial	Single-center, prospective, Phase 2 clinical tri
SCNC Histology	SCNC either *de novo* or treatment-emergent including mixed SCNC	Neuroendocrine or neuroend prostate cancer
Progressive Disease	PCWG3 criteria	iRECIST 1.1 replaces RE
No Prior CPIs	No prior anti–PD-1, anti–PD-L1, anti–PD-L2 or anti-CTLA4	No prior anti-PD(L)1 or an

Avelumab PICK-NEPC study. Brown, L.C., Halabi, S., Somarelli, J.A. et al. A phase 2 trial of avelumab in men with aggressive-variant or neuroendocrine prostate cancer. Prostate Cancer Prostatic Dis 25, 762–769 (2022). https://doi.org/10.1038/s41391-022-00524-7

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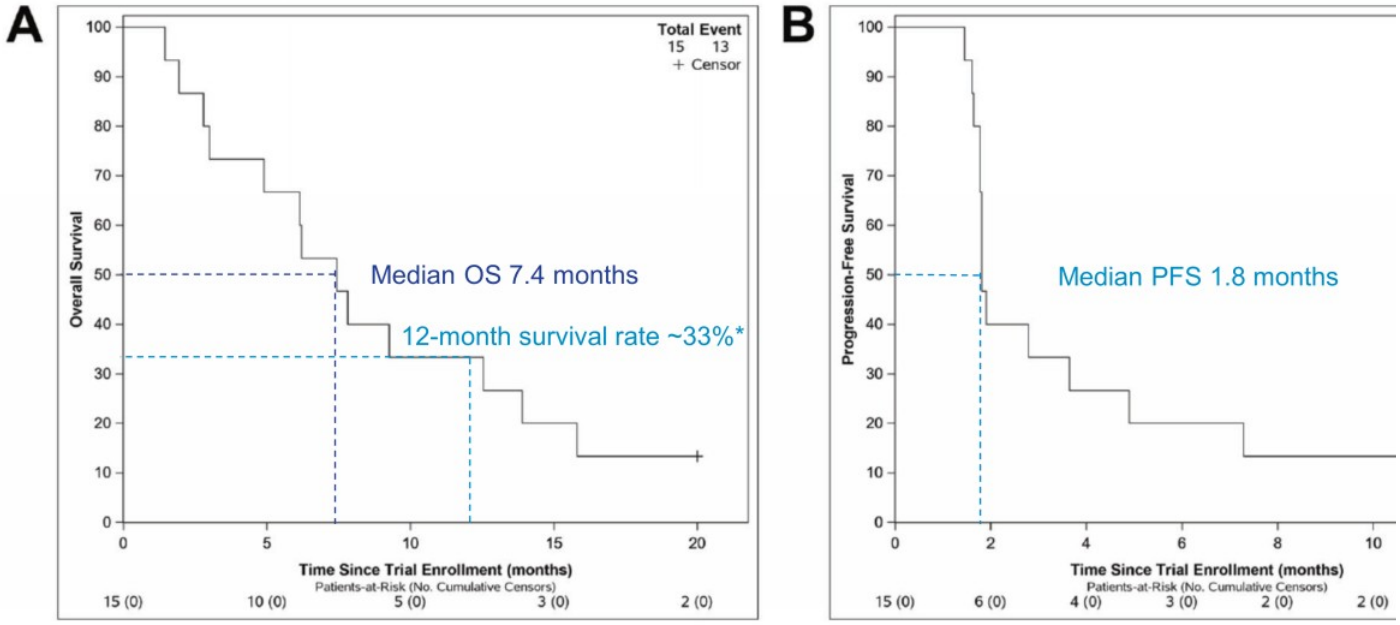
Avelumab PICK-NEPC Study Overall Survival and Progression-Free Survival Kaplan-Meier Curves



* Extrapolated by the Company using Kaplan-Meier estimates of overall survival curve from avelumab PICK-NEPC study

Adapted from: Avelumab PICK-NEPC study. Brown, L.C., Halabi, S., Somarelli, J.A. et al. A phase 2 trial of avelumab in men with aggressive-variant or neuroendocrine prostate cancer. Prostate Cancer Prostatic Dis 25, 762–769 (2022). https://doi.org/10.1038/s41391-022-00524-7

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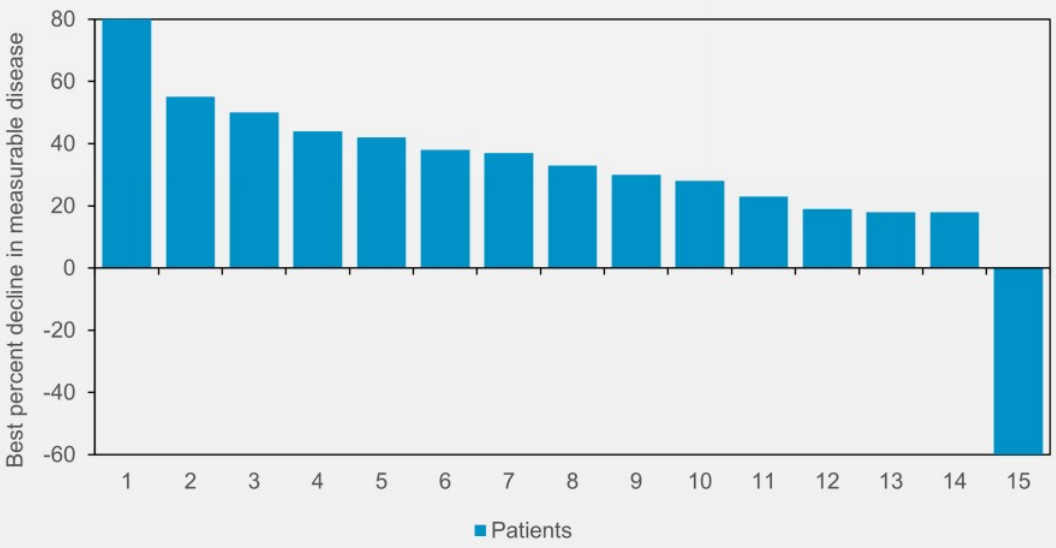
Avelumab PICK-NEPC Study
Did not Show Tumor Shrinkage in MSS SCNC



A best percentage decline in measurable disease from baseline by RECIST 1.1 criteria

- **Objective re 1/15 patient**
- **Responder microsatelli instability-h**
- **No respons in microsat patients**

Adapted from: Avelumab PICK-NEPC study. Brown, L.C., Halabi, S., Somarelli, J.A. et al. A phase 2 trial of avelumab in men with aggressive-variant or neuroendocrine prostate cancer. Prostate Cancer Prostatic Dis 25, 762–769 (2022). https://doi.org/10.1038/s41391-022-00524-7

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IGALMI™ Indication and Important Safety Information

INDICATION
IGALMI™ (dexmedetomidine) sublingual film is a prescription medicine, administered under the supervision of a health care provider, that is placed under the tongue and is used for the acute treatment of agitation associated with schizophrenia and bipolar disorder I or II in adults. The safety and effectiveness of IGALMI has not be hours from the first dose. It is not known if IGALMI is safe and effective in children.

IMPORTANT SAFETY INFORMATION
IGALMI can cause serious side effects, including:

- **Decreased blood pressure, low blood pressure upon standing, and slower than normal heart rate**, which may be more likely in patients with low blood volun high blood pressure, and older patients. IGALMI is taken under the supervision of a healthcare provider who will monitor vital signs (like blood pressure and heart after IGALMI is administered to help prevent falling or fainting. Patients should be adequately hydrated and sit or lie down after taking IGALMI and instructed to tell provider if they feel dizzy, lightheaded, or faint.
- **Heart rhythm changes (QT interval prolongation).** IGALMI should not be given to patients with an abnormal heart rhythm, a history of an irregular heartbeat, slo potassium, low magnesium, or taking other drugs that could affect heart rhythm. Taking IGALMI with a history of abnormal heart rhythm can increase the risk of tor sudden death. Patients should be instructed to tell their healthcare provider immediately if they feel faint or have heart palpitations.
- **Sleepiness/drowsiness.** Patients should not perform activities requiring mental alertness, such as driving or operating hazardous machinery, for at least 8 hours
- **Withdrawal reactions, tolerance, and decreased response/efficacy**. IGALMI was not studied for longer than 24 hours after the first dose. Physical dependence (e.g., nausea, vomiting, agitation), and decreased response to IGALMI may occur if IGALMI is used longer than 24 hours.

The most common side effects of IGALMI in clinical studies were sleepiness or drowsiness, a prickling or tingling sensation or numbness of the mouth, dizziness, c pressure, and low blood pressure upon standing.

These are not all the possible side effects of IGALMI. Patients should speak with their healthcare provider for medical advice about side effects.

Patients should tell their healthcare provider about their medical history, including if they suffer from any known heart problems, low potassium, low magnesium low heart rate, diabetes, high blood pressure, history of fainting, or liver impairment. They should also tell their healthcare provider if they are pregnant or breastfeedi medicines, including prescription and over-the-counter medicines, vitamins, and herbal supplements. Patients should especially tell their healthcare provider if they ta lower blood pressure, change heart rate, or take anesthetics, sedatives, hypnotics, and opioids.

Everyone is encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch or call 1-800-FDA-1088. You can also contact I Inc. at 1-833-201-1088 or medinfo@bioxceltherapeutics.com.

Please see full Prescribing Information.