BXCL701 / KEYTRUDA®
in Small Cell Neuroendocrine Prostate Cancer

Phase 2 Overall Survival Results

October 10, 2023

Forward-Looking Statements

This presentation 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”). Forward-looking statements in this presentation include but are not limited to: statements regarding BioXcel Therapeutics’ expected timing of, and data results from, trials and clinical studies involving its product candidates, in particular BXCL701; potential benefits from treatment with BXCL701, planned discussions with the FDA; strategic options for OnkosXcel; 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 BioXcel Therapeutics’ current expectations and various assumptions. BioXcel Therapeutics believes there is a reasonable basis for its expectations and beliefs, but they are inherently uncertain.

BioXcel Therapeutics 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 presentation. Any such forward-looking statements represent management's estimates as of the date of this presentation. While BioXcel Therapeutics 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 BioXcel Therapeutics’ views as of any date subsequent to the date of this presentation.

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 Transformative Medicine in Hard-to-Treat Tumors

Lead Oncology Drug Candidate: BXCL701

- Unique oral innate immune activator, designed to turn cold tumors hot via DPP8/9 inhibition
- Combination approach, BXCL701+ KEYTRUDA® (pembrolizumab)
- Potential to extend the value of immuno-oncology in large underserved patient populations
- Focusing on cold tumor types
- Positive Phase 2 data in SCNC presented at ASCO GU 2023

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

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 SCNC Presented at ASCO GU 2023

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

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 Immunity
DPP8/9 Biology

FortySeven — *Acquired for ~$5B by* GILEAD

Scarcity of assets in innate immunity

TRILLIUM THERAPEUTICS INC. — *Acquired for ~$2.3B by* Pfizer

OnkosXcel Therapeutics — A subsidiary of BioXcel Therapeutics, Inc.

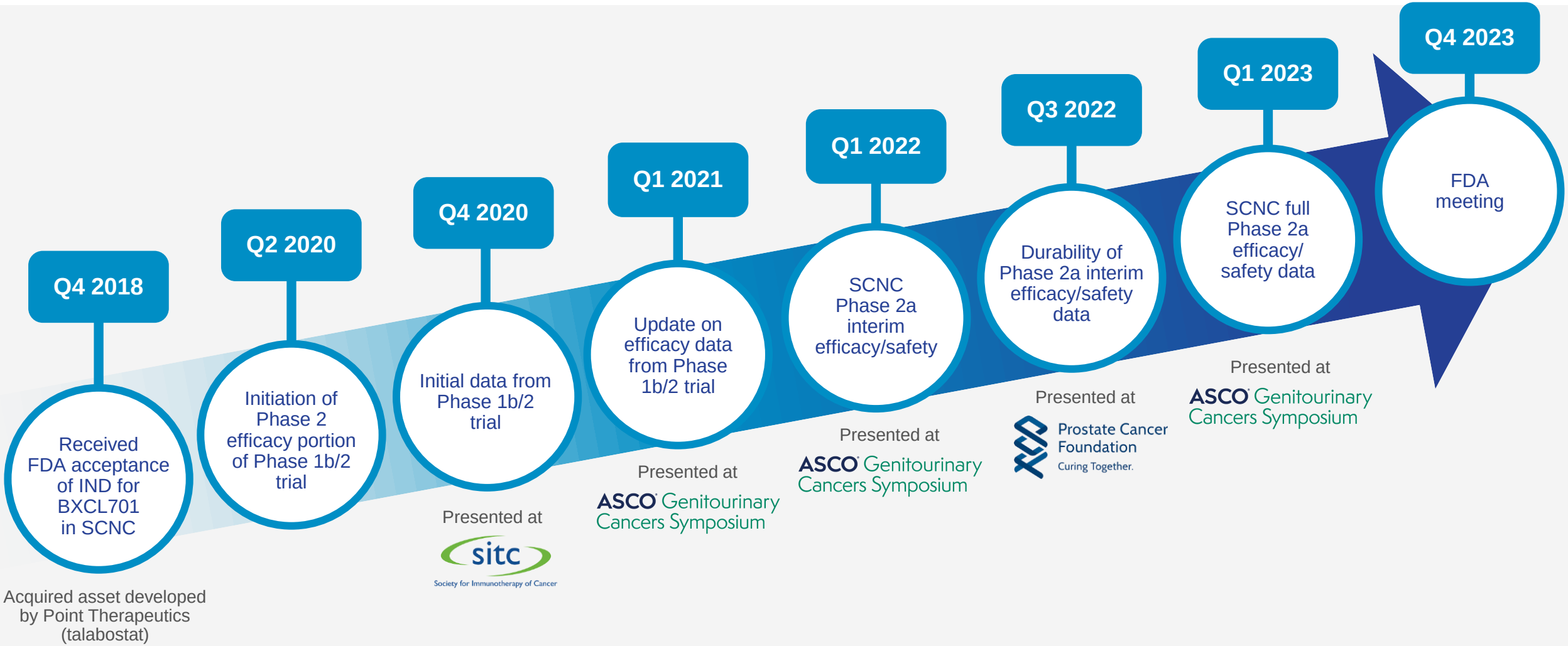
Exploring Strategic Options

* Source: www.clinicaltrials.gov

bioxcel therapeutics®

First AI-Derived Human POC for Oral Innate Immune Activator

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



bioxcel therapeutics®

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 SCNC phenotype**, characterized by poor prognosis and low survival rate
- Current treatment protocols that are sub-optimal include platinum-based cytotoxic chemotherapies despite short duration of response and considerable toxicities
- Current CPIs[2] targeting PD-1 and CTLA-4 have not demonstrated meaningful single-agent therapeutic 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 prostate cancer

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 low patients with BXCL701
  - **Overall Response Rate (RECIST) 20%** *vs*. **6.7%***
- **High rates of stable disease and disease control** (stable disease + partial response) with BXCL701
  - **Disease Control Rate 48%** *vs*. **27%***

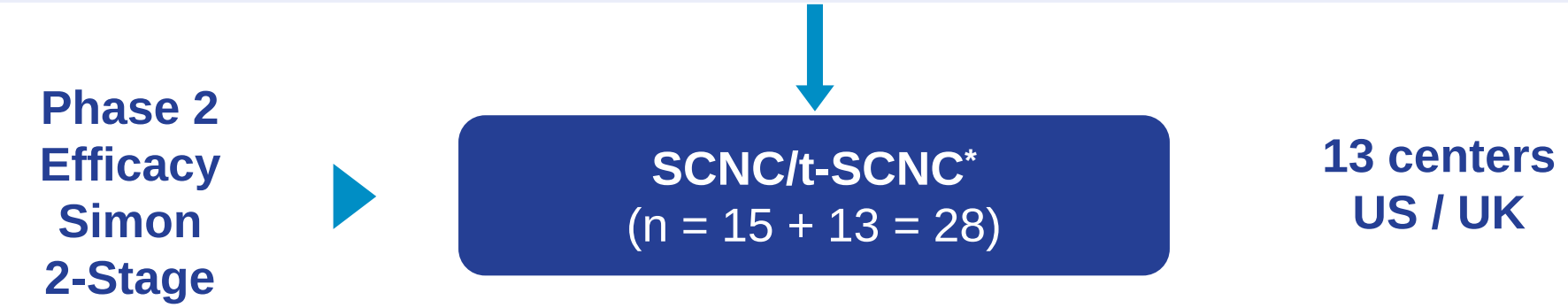
* Avelumab PICK-NEPC study. Brown, L.C., Halabi, S., Somarelli, J.A. et al. A phase 2 trial of avelumab in men with aggressive-variant or neuroendocrine prostate cancer. Prostate Cancer Prostatic Dis 25, 762–769 (2022). https://doi.org/10.1038/s41391-022-00524-7 ** Extrapolated by the Company using Kaplan-Meier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

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.

bioxcel therapeutics®

BXCL701 SCNC Phase 2 Trial Design

21-Day Treatment Cycle:
Pembrolizumab 200 mg IV Day 1 (dosed as per package insert) +
BXCL701 BID PO Days 1-14
Cycle 1: BXCL701 Step-Up Dosing 0.2 mg BID PO Days 1-7 + 0.3 mg BID PO Days 8-14
Subsequent cycles: BXCL701 0.3 mg BID PO Days 1-14



Primary objective: Composite Response Rate, either objective response by RECIST 1.1 criteria, and/or CTC Conversion from ≥5/7.5 mL to <5/7.5 mL, and/or ≥-50% PSA decline from baseline

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

bioxcel therapeutics®

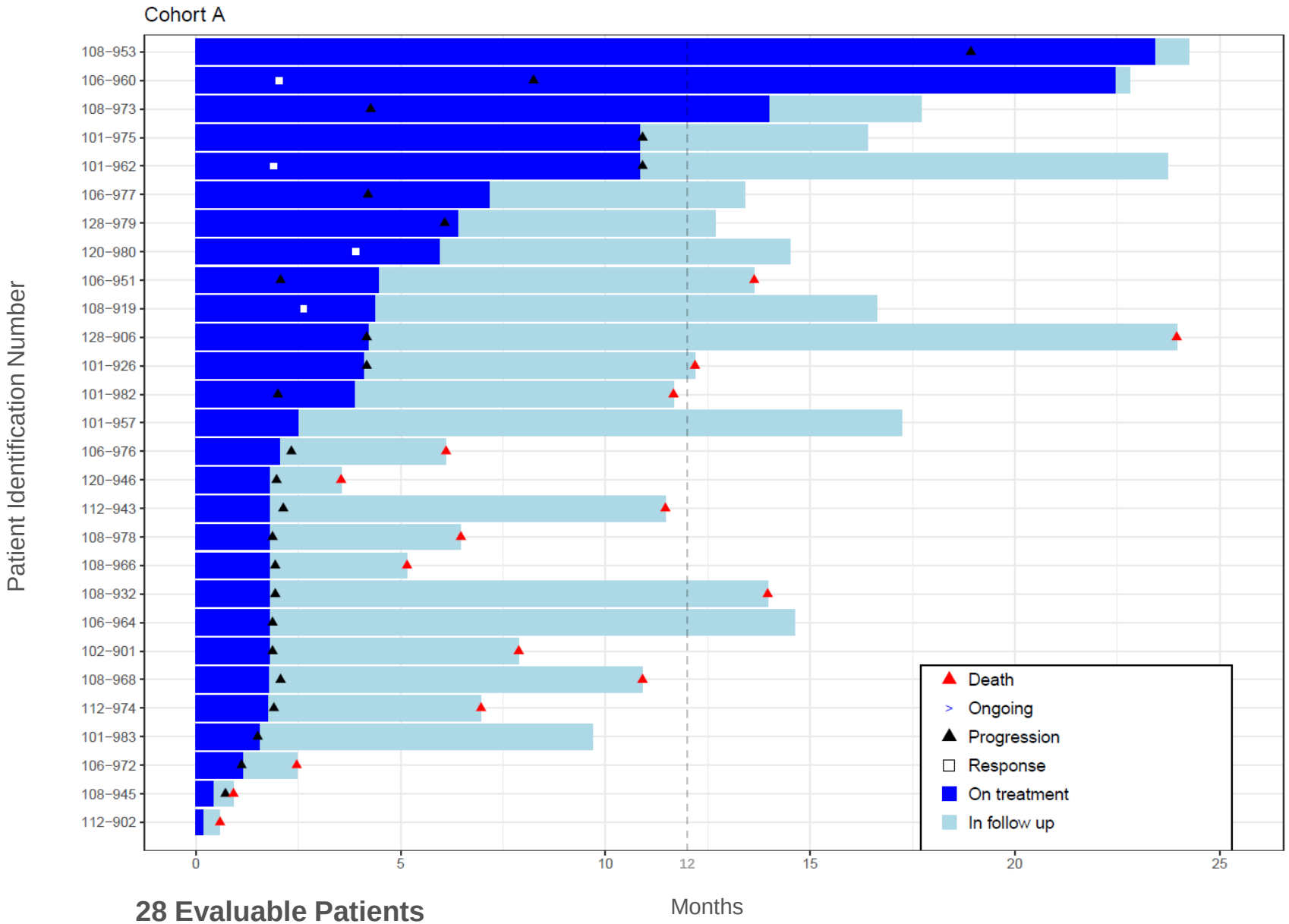
SCNC Patients Baseline Characteristics

ASCO Genitourinary Cancers Symposium 2023

Patients Enrolled n = 34	n (%)
Median Age (range)	**67.5 years** (54 – 80)
ECOG Performance Status	
0	**16** (47%)
1	**16** (47%)
2	**2** (6%)
Visceral Metastases	
Any site	**21** (62%)
Liver	**11** (32%)
Median lines of prior systemic therapy (range)	**3** (1 – 8)
Prior Systemic Treatment	
Androgen signaling inhibitor(s)	**25** (89%)
Platinum-based Chemotherapy	**19** (68%)
Taxane Chemotherapy	**17** (50%)

bioxcel therapeutics®

SCNC Patients Swimmer's Plot



28 Evaluable Patients

Median duration of follow- up = 23.9 months (11.3 – 38.2)

6 patients (21%) treated beyond progression

Data cut-off for survival 06-SEP-23

bioxcel therapeutics®

SCNC Patients Efficacy Results

ASCO Genitourinary Cancers Symposium 2023

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 rate: 20%

4 confirmed partial responses + 1 unconfirmed partial response

Median duration of response:

6+ months

(range: 1.8 - 9.8 months)

Data cut-off 19-DEC-22

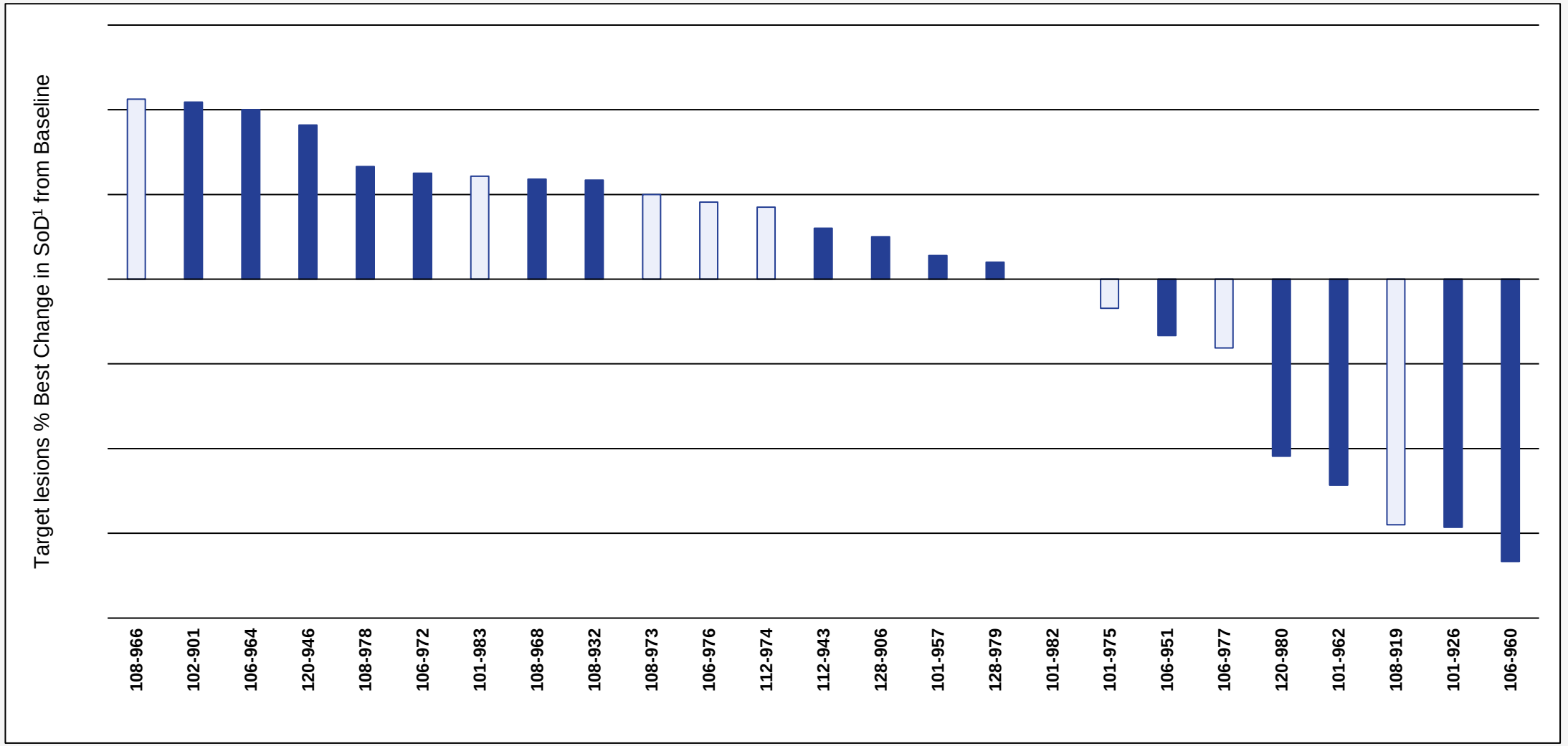
[a] Patients who received ≥2 cycles of study therapy and had 1 on-treatment tumor assessment [b] Circulating tumor cell [c] Baseline CTC value ≥5/7.5 mL and 1 measurable on-treatment assessment [d] CTC conversion from ≥5/7.5 mL to <5/7.5 mL; [e] Baseline PSA >4 ng/mL and 1 on-treatment PSA assessment

bioxcel therapeutics®

SCNC Patients Best Response

All responders are MSS and/or TMB low

ASCO Genitourinary Cancers Symposium 2023



[1] SoD = Sum of Diameters

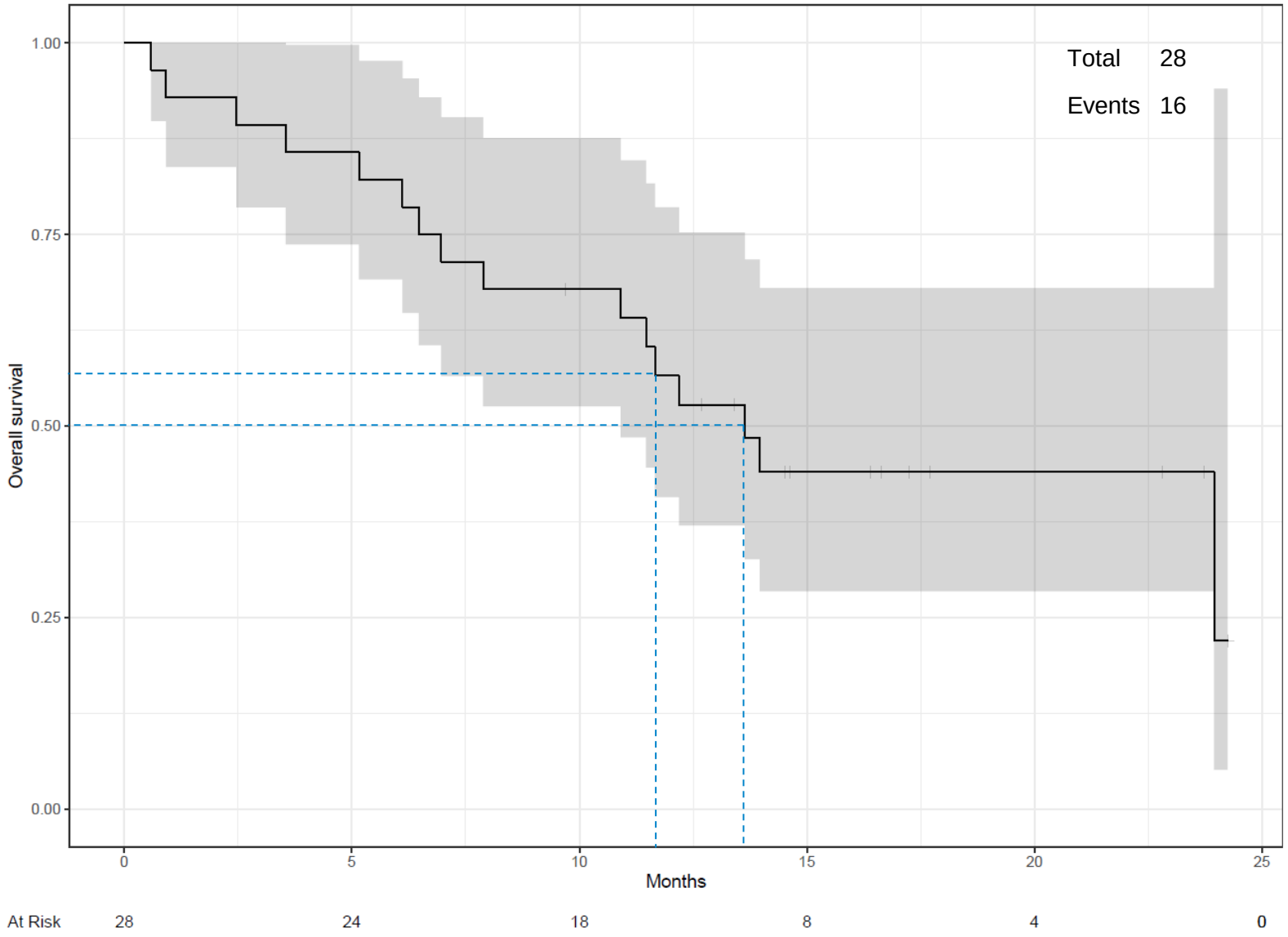
No Prior Platinum Chemotherapy

Prior Platinum Chemotherapy

Data cut-off 19-DEC-22

bioxcel therapeutics®

SCNC Patients Overall Survival Kaplan-Meier Curve



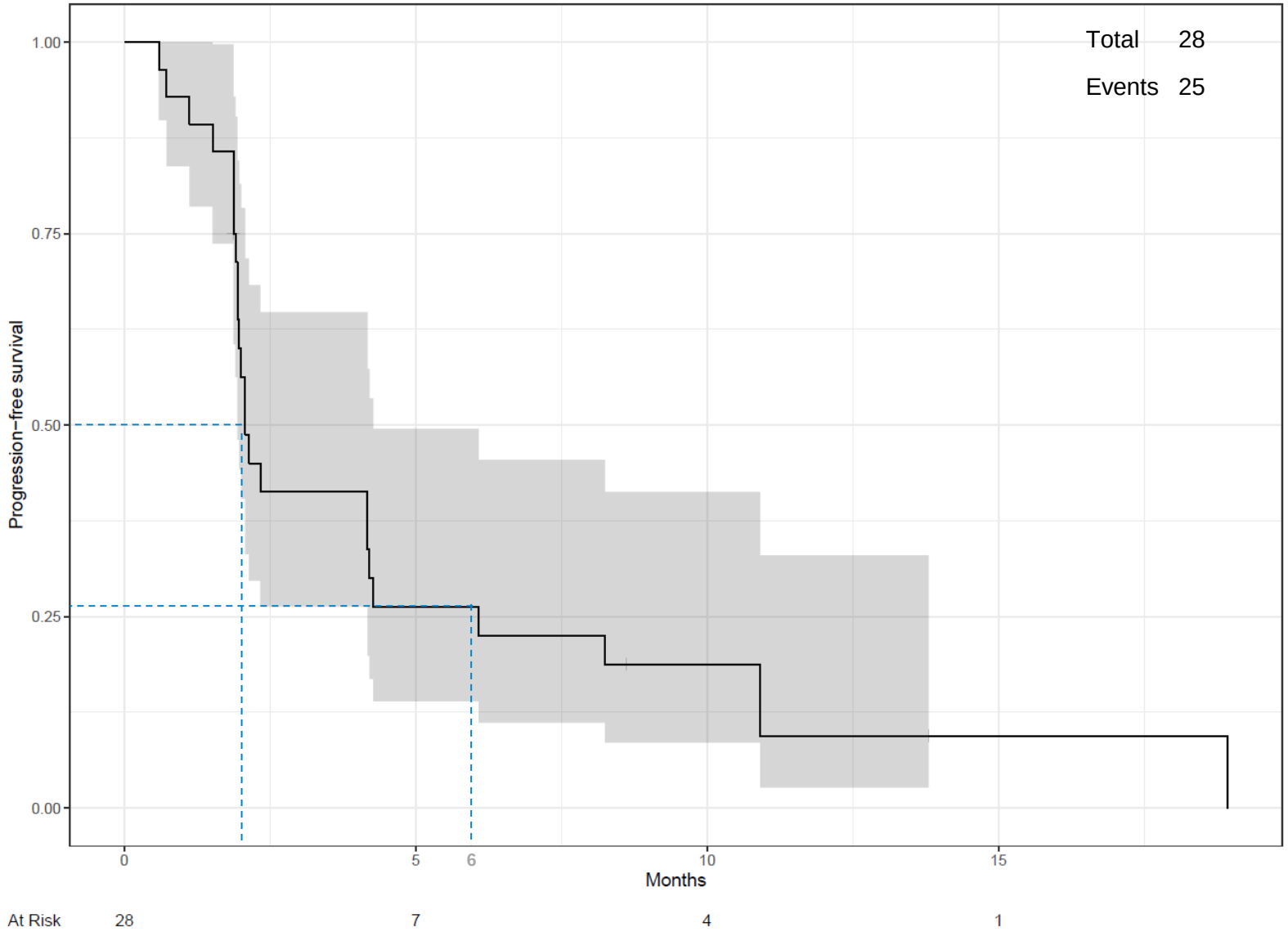
Median OS 13.6 months (95% CI 10.9–NR)

12-month survival rate 56.5%

Data cut-off 06-SEP-23

bioxcel therapeutics®

SCNC Patients Radiographic Progression-Free Survival Kaplan-Meier Curve



mPFS 2.07 months
(95% CI 1.94–4.27)

6-month PFS 26.2%
(95% CI 9.6–42.9)

Data cut-off 06-SEP-23

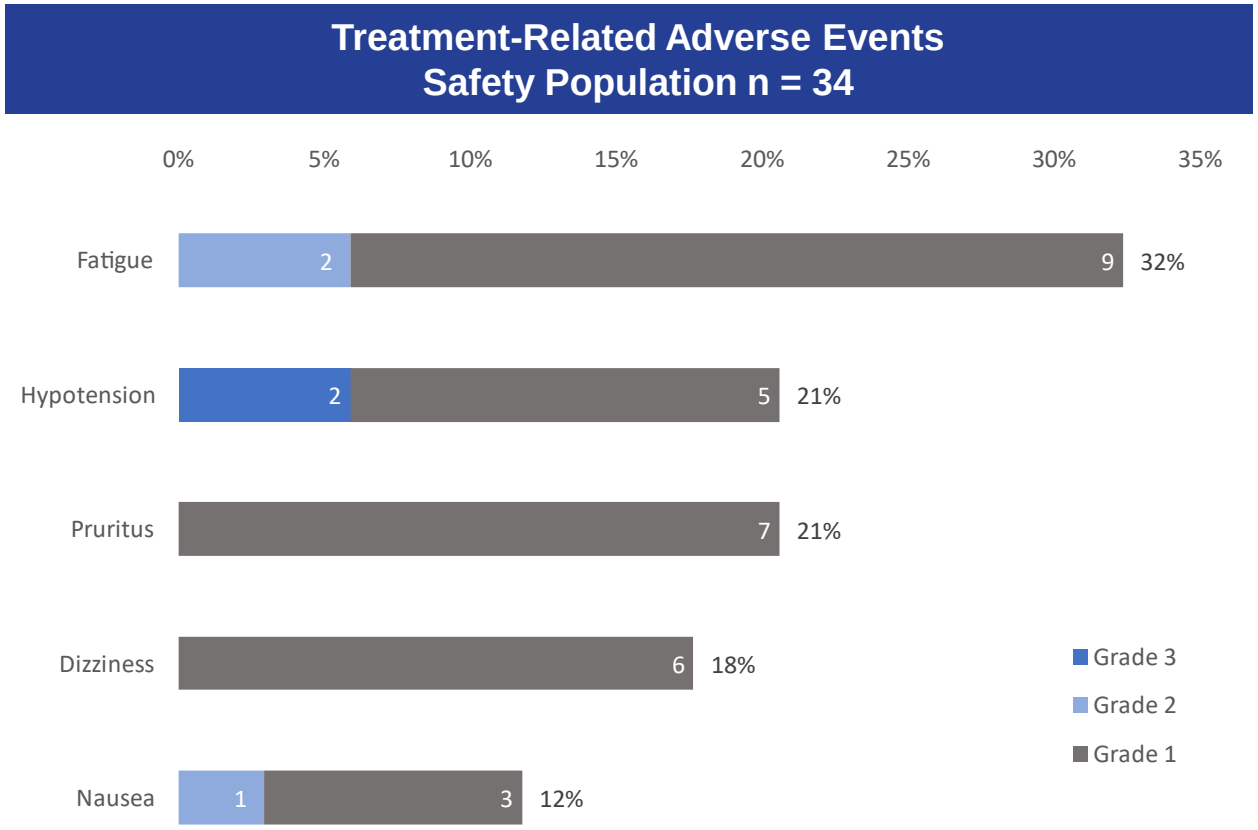
bioxcel therapeutics®

BXCL701 / KEYTRUDA® Combination Has Demonstrated Manageable Safety

ASCO Genitourinary Cancers Symposium 2023

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 in >10% of patients

Data cut-off 19-DEC-22

bioxcel therapeutics®

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

	BXCL701	Avelumab PICK-NEPC
n patients	28 patients evaluable	15 patients evaluable
Histology Confirmed SCNC	100%	33%
Median age	67.5 years (range, 54–80)	71 years (range, 51–85)
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.

bioxcel therapeutics®

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

	BXCL701	Avelumab PICK-NEPC
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 MSI-high**)
Median Duration of Response RECIST	6+ months[1]	24 months (**the responder is MSI-high**)
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% CI 1.6–3.6)
mOS	13.6 months (95% CI 10.9–NR)	7.4 months (95% CI 2.8–12.6)
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

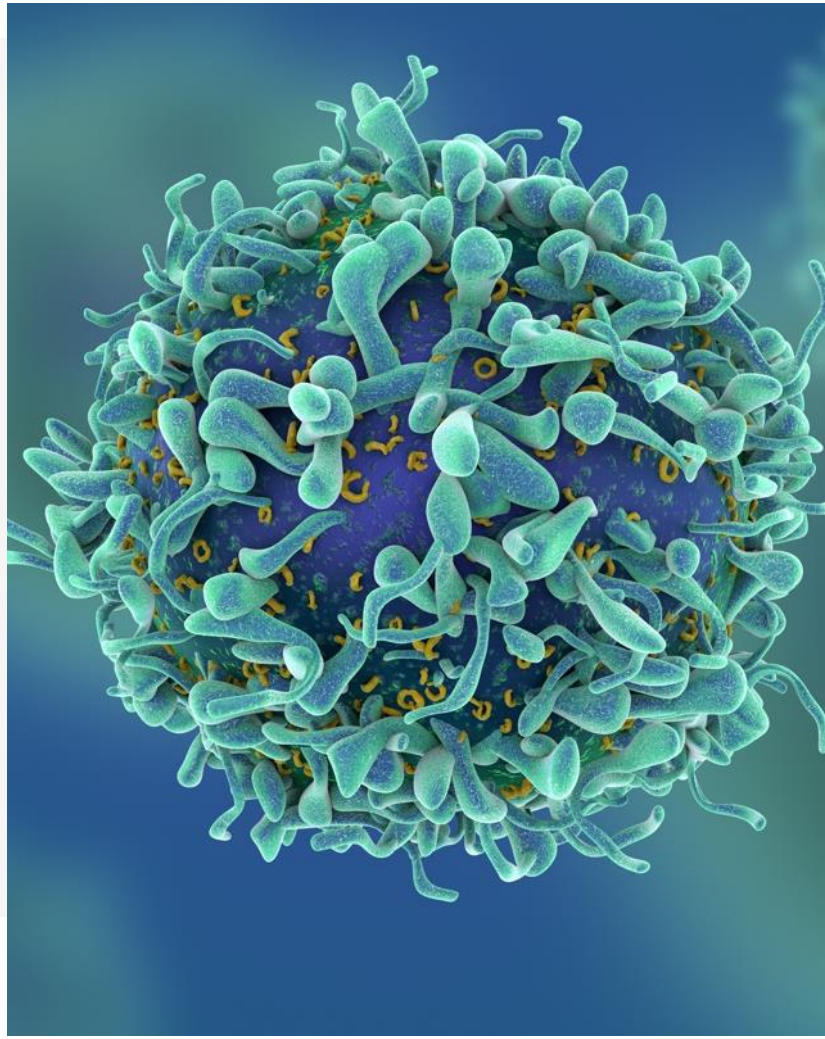
Avelumab PICK-NEPC study. Brown, L.C., Halabi, S., Somarelli, J.A. et al. A phase 2 trial of avelumab in men with aggressive-variant or neuroendocrine prostate cancer. Prostate Cancer Prostatic Dis 25, 762–769 (2022). https://doi.org/10.1038/s41391-022-00524-7

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.

bioxcel therapeutics®

Conclusions

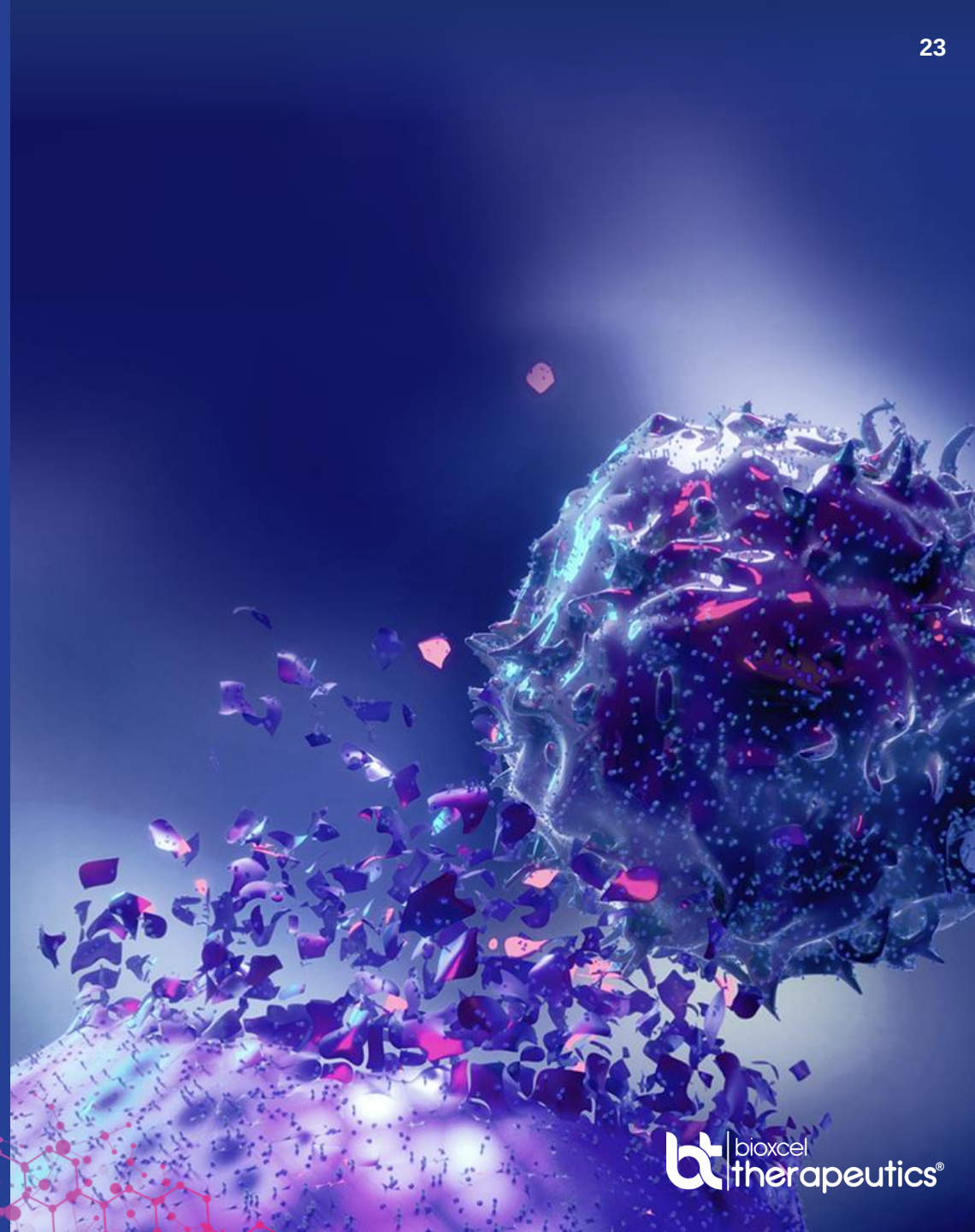
- BXCL701 + pembrolizumab demonstrated **compelling median OS and 12-month survival rate**
  - In excess of historic data from avelumab PICK-NEPC trial
  - OS represents the gold standard for measuring effectiveness
  - No MSI-H/TMB high patients detected in responders
- BXCL701 + pembrolizumab demonstrated **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

Appendix



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

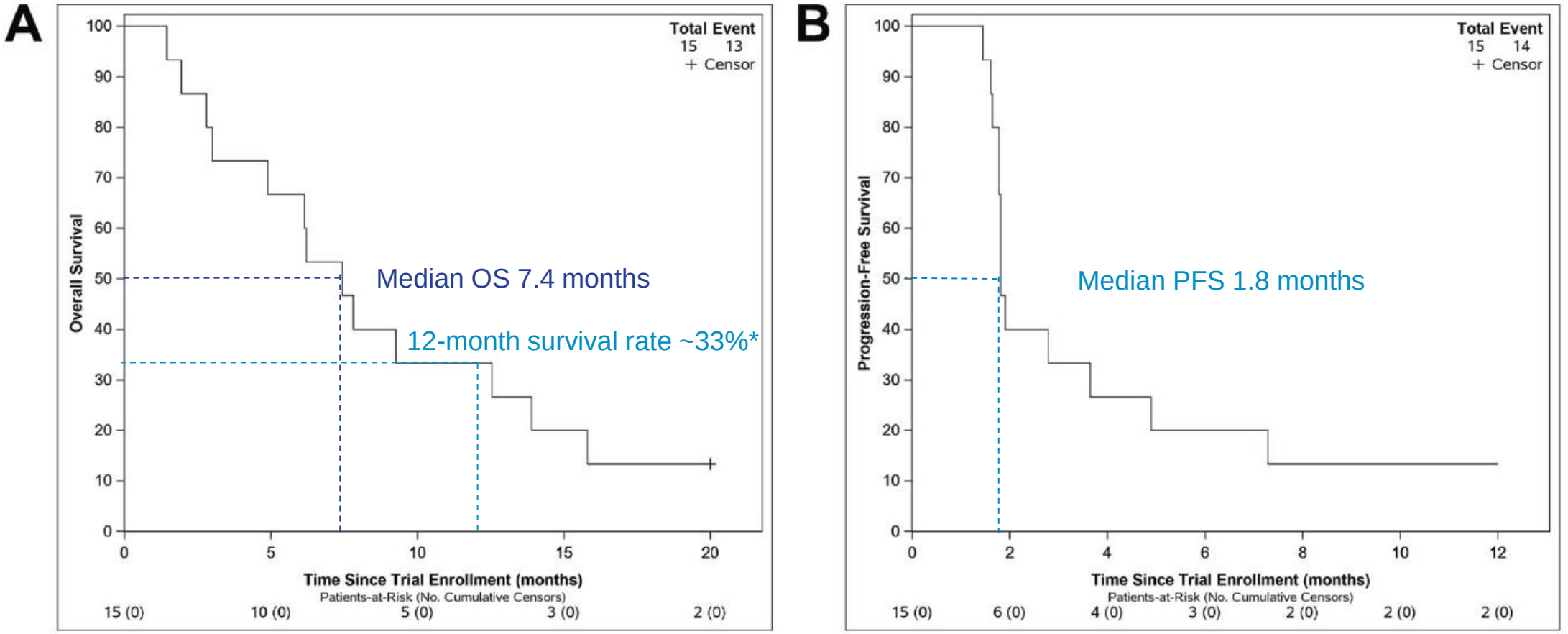
	BXCL701	Avelumab PICK-NEPC
Simon 2-Stage Study Design	Multicenter, prospective, single-arm Phase 2 clinical trial	Single-center, prospective, single-arm Phase 2 clinical trial
SCNC Histology	SCNC either *de novo* or treatment-emergent including mixed SCNC	Neuroendocrine or neuroendocrine-like prostate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

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.

bioxcel therapeutics®

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



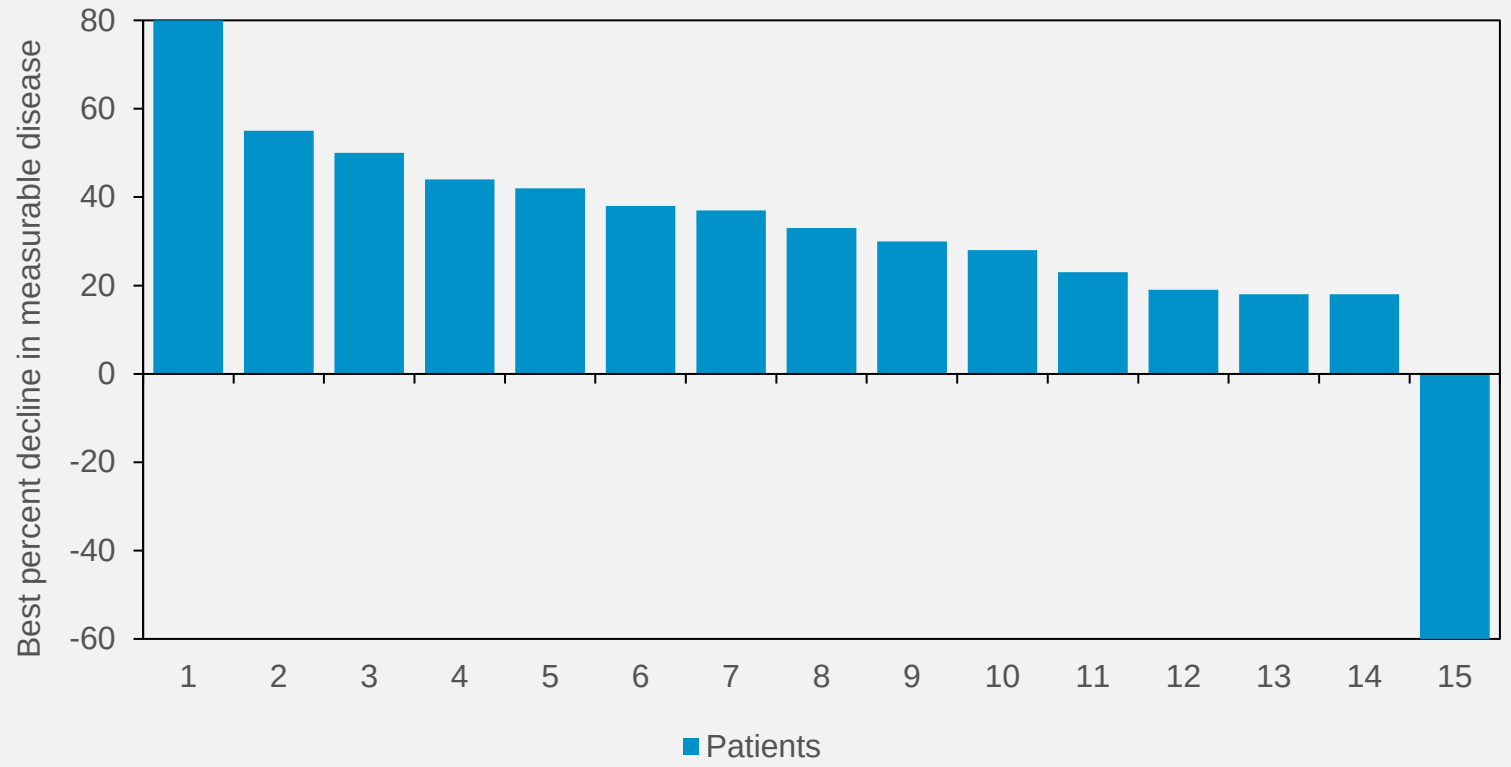
* Extrapolated by the Company using Kaplan-Meier estimates of overall survival curve from avelumab PICK-NEPC study

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

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.

bioxcel therapeutics®

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 response rate 1/15 patients (6.7%)[1]**
- **Responder was microsatellite instability-high**
- **No response observed in microsatellite stable 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

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.

bioxcel therapeutics®

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 or behind the lower lip 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 been studied beyond 24 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 volume, diabetes, chronic 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 rate) and alertness 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 their healthcare 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, slow heart rate, low 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 torsades de pointes and 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 after taking IGALMI.
- **Withdrawal reactions, tolerance, and decreased response/efficacy**. IGALMI was not studied for longer than 24 hours after the first dose. Physical dependence, withdrawal symptoms (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, dry mouth, low blood 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 blood pressure, 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 breastfeeding or take any medicines, including prescription and over-the-counter medicines, vitamins, and herbal supplements. Patients should especially tell their healthcare provider if they take any drugs that 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 BioXcel Therapeutics, Inc. at 1-833-201-1088 or medinfo@bioxceltherapeutics.com.

Please see full Prescribing Information.

bioxcel therapeutics®