



# Oncology Leadership in Pretargeted Radioimmunotherapy Platform and Antibody-based Therapies

November 2024



# Disclaimer

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This presentation contains forward-looking statements within the meaning of the US Private Securities Litigation Reform Act of 1995. The forward-looking statements involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this presentation, including statements regarding our strategy, future operations, future financial position, future revenue, projected costs, prospects, plans and objectives of management and expected market growth are forward-looking statements. The words “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “intend,” “may,” “might,” “plan,” “potential,” “predict,” “project,” “should,” “target,” “would,” “goal,” “objective,” “guidance,” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Such statements include, but are not limited to, statements about early clinical data, regulatory matters, clinical trial timing and plans, the achievement of clinical and commercial milestones, future financial and operating results, including 2024 financial guidance and beyond and anticipated future cash and cash equivalents, business strategies, market opportunities, financing, and other statements that are not historical facts. Our product candidates and related technologies are novel approaches to cancer treatment that present significant challenges. Actual results may differ materially from those indicated by such forward-looking statements as a result of various factors, including but not limited to: risks associated with our financial condition and need for additional capital; risks associated with the Company's development work, including any delays or changes to the timing, cost and success of our product development activities and clinical trials including if we encounter difficulties enrolling patients in our clinical trials; the risks of delays in FDA and/or EU approval of our drug candidates or failure to receive approval; the risks related to commercializing any approved new pharmaceutical product including the rate and degree of market acceptance of our product candidates; development of our sales and marketing capabilities and risks associated with failure to obtain sufficient reimbursement for our products; risks related to our dependence on third parties including for conduct of clinical testing and product manufacture; our ability to enter into collaboration or other arrangements with partners; risks associated with protection of our intellectual property rights; and other risks and uncertainties affecting the Company including those described in the "Risk Factors" section included in our Annual Report on Form 10-K for the fiscal year ended December 31, 2023, our Quarterly Reports on Form 10-Q for the quarters ended March 31, 2024, June 30, 2024 and September 30, 2024, in addition to other reports the Company files from time to time with the Securities and Exchange Commission. Any forward-looking statements contained in this presentation speak only as of the date hereof, and the Company undertakes no obligation to update any forward-looking statement, whether as a result of new information, future events or otherwise.

# Strongly Positioned to Drive Future Value

## Novel Platforms in Development



Self-Assembly  
DisAssembly Pretargeted  
Radioimmunotherapy  
("SADA PRIT")  
Platform

Monoclonal Antibodies

## Commercial Leverage



**DANYELZA**  
(naxitamab-gqgk)

Anti-GD2 Antibody  
Marketed for R/R High-  
Risk Neuroblastoma

FY2024 Total Net  
Revenue Guidance of  
\$87-95 million

## Anticipated 2024 Milestones



GD2-SADA Phase 1  
Part A Completion

CD38-SADA Phase 1  
Study Initiation

MSK Data Readout of  
Phase 2 Osteosarcoma  
Trial Anticipated

## Capital Efficiency



















Independent  
Commercial-stage  
Biotech Company with  
Cash of \$68.1 million\*

Financial Runway  
into 2027

Strategic Capital  
Deployment

\* As of September 30, 2024

# Advancing Focused Pipeline with Multiple Value-Added Catalysts Ahead

	Study	Therapeutic Area	Preclinical	Phase 1	Phase 2/Pivotal	Approved	Trial Sponsor	Status	
<b>Lead Programs</b>									
<b>Naxitamab-gqgk</b> (Anti-GD2)	201	Relapsed/Refractory High-Risk Neuroblastoma (Pediatric)	DANYELZA (naxitamab-gqgk) Confirmatory Trial				✓		U.S. FDA approved
	12-230	Relapsed/Refractory High-Risk Neuroblastoma (Pediatric)	DANYELZA (naxitamab-gqgk)				✓	 Memorial Sloan Kettering Cancer Center	U.S. FDA approved
	BCC018	Front-Line Induction in High-Risk Neuroblastoma (Pediatric)						 <b>Beat Childhood Cancer</b> RESEARCH CONSORTIUM	Randomized trial expected 2H 2024
	15-096	Relapsed Second-Line Osteosarcoma						 Memorial Sloan Kettering Cancer Center	Expected MSK data readout in Q4 2024
	17-251	Chemoimmunotherapy for Relapsed/Refractory High-Risk Neuroblastoma						 Memorial Sloan Kettering Cancer Center	Study completed
	Butterfly	Refractory Ewing's Sarcoma						 <b>Institute of Mother and Child</b>	Initiated in Q4 2023
		Metastatic Breast Cancer						 <b>THE OHIO STATE UNIVERSITY</b> COMPREHENSIVE CANCER CENTER	Expect FPI in 2H 2024
<b>SADA PRIT</b> (Radioimmunotherapy)	1001	GD2-SADA: Solid Tumors (SCLC, Malignant Melanoma, Sarcoma)							Expect to complete Part A in 2024
	1201	CD38-SADA: Non-Hodgkin Lymphoma							Expect FPI in Q4 2024
<b>Early Programs</b>									
<b>SADA PRIT</b> (Radioimmunotherapy)	1002	GD2-SADA: Neuroblastoma							Expected IND filing in 2025
		HER2-SADA							Expected IND filing in 2025
		B7H3-SADA							Expected IND filing in 2025



**Novel SADA Pretargeted  
Radioimmunotherapy  
Platform**



# Current Radiopharma Challenges Negatively Impact Patient Care



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**Infrastructure and  
Manufacturing**



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**Physician  
Participation**



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**Administration  
Sites**



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**Continuing Drug  
Shortages**

**Simpler, more user-friendly solutions greatly needed for physicians and patients**

# Potential Improved Capabilities of Novel SADA PRIT 2-Step Approach

## Traditional Radioimmunotherapy



Limited dose-to-tumor due to off-target radiation



Prone to drug shortages / supply issues with single-isotope only capabilities



Limited administration sites with licensed nuclear medicine radiologists



High investment needed for specific infrastructure and manufacturing



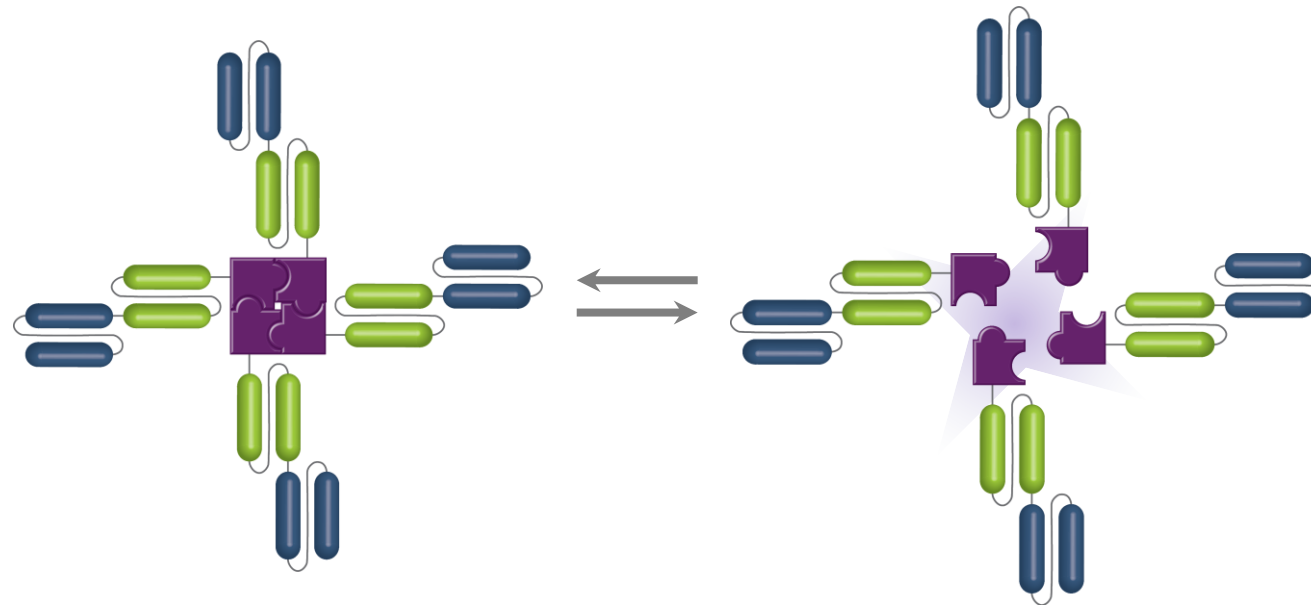
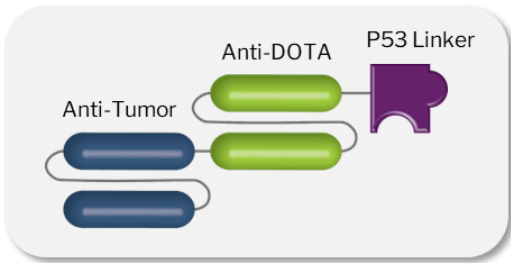
## Novel SADA PRIT Platform Potential Capabilities

- ✓ Pretargeting tumor potentially minimizes toxicity and potentially enhances rapid clearing of unbound protein
- ✓ Potential to work with short  $T_{1/2}$  isotopes
- ✓ Potentially broader site options with protein doses administered by Medical Oncologist at large infusion centers
- ✓ Potential COGS improvements

\* Pending successful development and approval.

# Self-Assembly DisAssembly (SADA) Technology: High Affinity for Tumor Targets and Rapid Clearance from Blood Stream

SADA domains uniquely selected to allow proteins to change size based on concentration



**Self-Assembled Tetramer**  
~200 kDa

**DisAssembled Monomer**  
< 70kDa

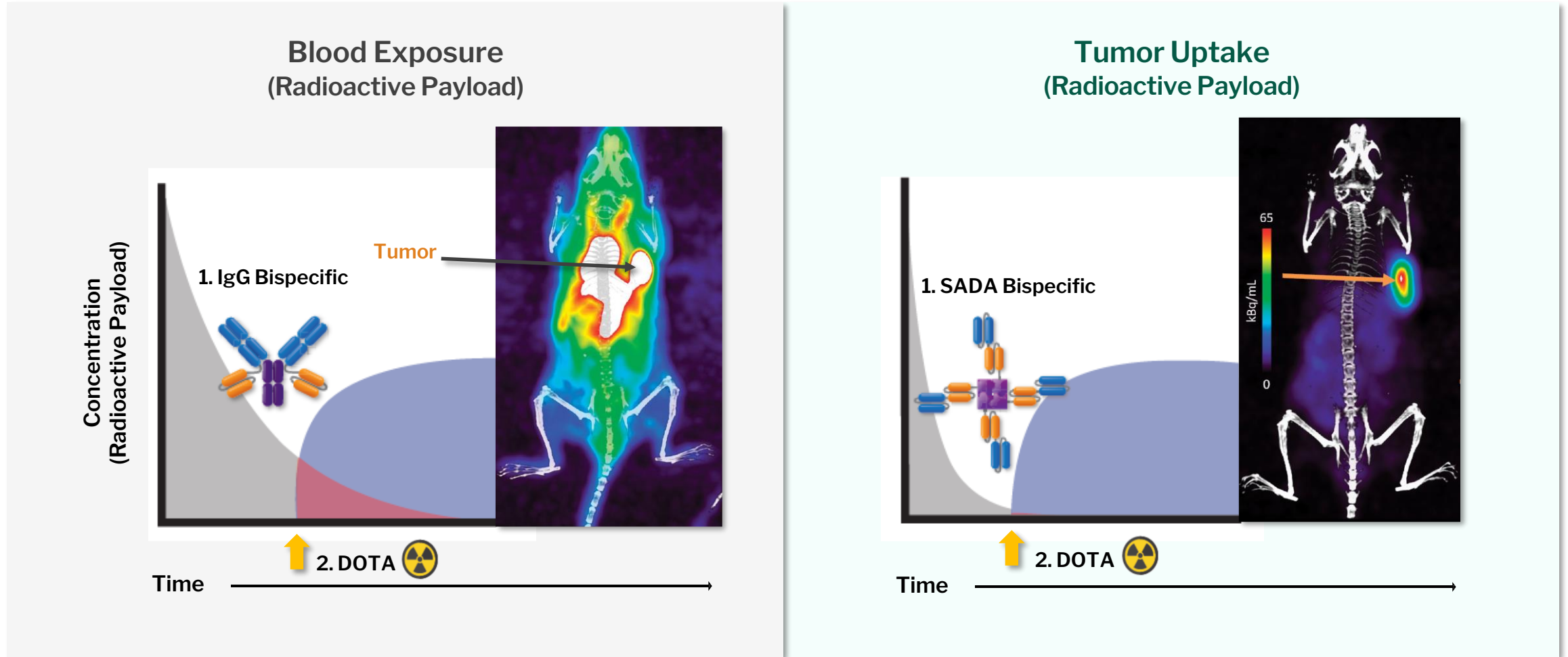
**Strong Tumor Binding**

**Rapid Clearance**

Adapted from Santich et al. Clin Canc Res 2020



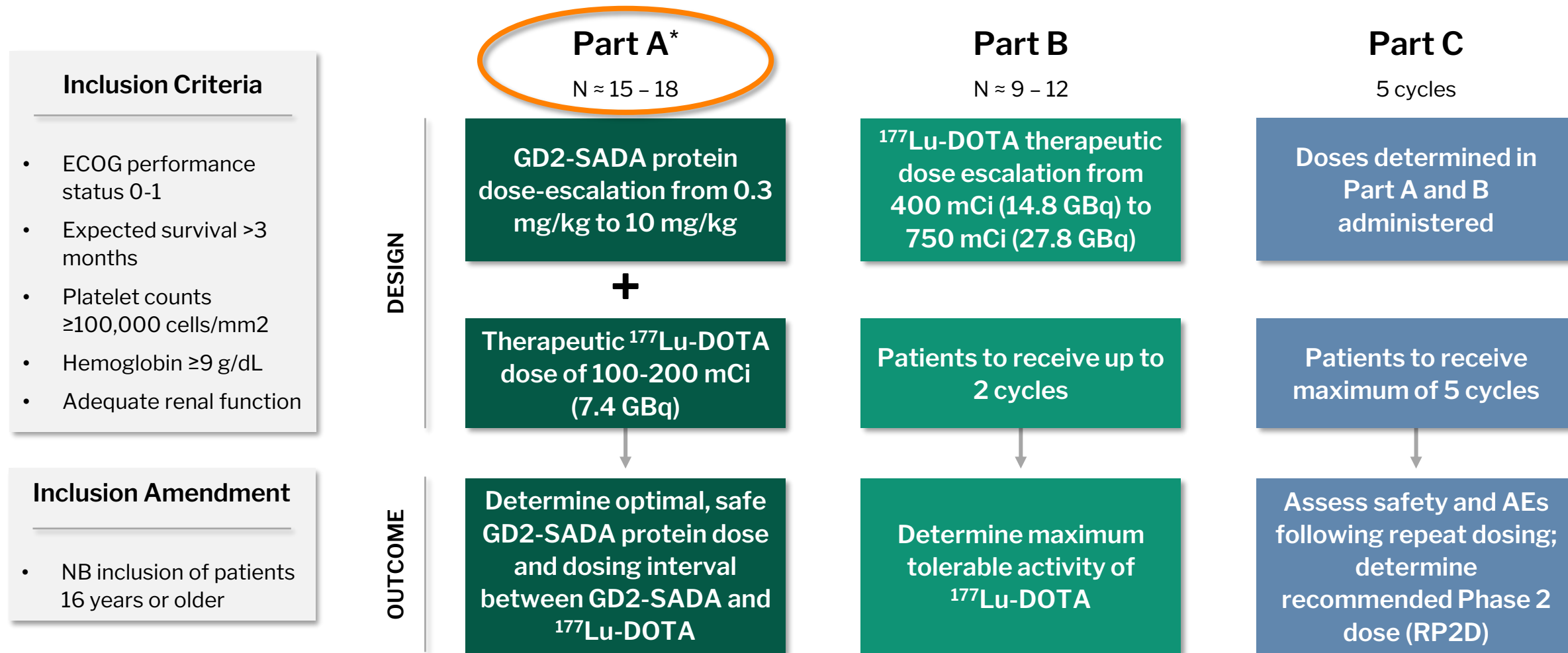
# GD2-SADA Achieves High Tumor Uptake with Minimal Exposure to All Other Tissues



Adapted from Santich et al. Clin Canc Res 2021  
These early results are not complete and are not necessarily indicative of the full results or ultimate success of the trials or SADA development program.

# GD2-SADA Phase 1 Clinical Trial (Study 1001): Dosing Patients in Part A

Theranostic approach using a 30 mCi <sup>177</sup>Lu-DOTA imaging dose before exposing to therapeutic dose



- Inclusion Criteria**
- ECOG performance status 0-1
  - Expected survival >3 months
  - Platelet counts ≥100,000 cells/mm<sup>2</sup>
  - Hemoglobin ≥9 g/dL
  - Adequate renal function

- Inclusion Amendment**
- NB inclusion of patients 16 years or older

\*Currently in Part A

# Ongoing GD2-SADA Phase 1 Clinical Trial (Study 1001): Part A Overview

## TRIAL UPDATE

- › Solid tumors (SCLC, malignant melanoma, sarcomas, adult neuroblastoma)
- › Completed Cohorts 1 through 5; currently in Cohort 6\*
- › 20 patients dosed\*
- › 6 sites open\*
- › No DLTs or instances of treatment-related AEs reported\*

## UPCOMING CATALYSTS

- › On track to complete Part A in Q4 2024
- › Anticipate Part A data readout in Q1 2025

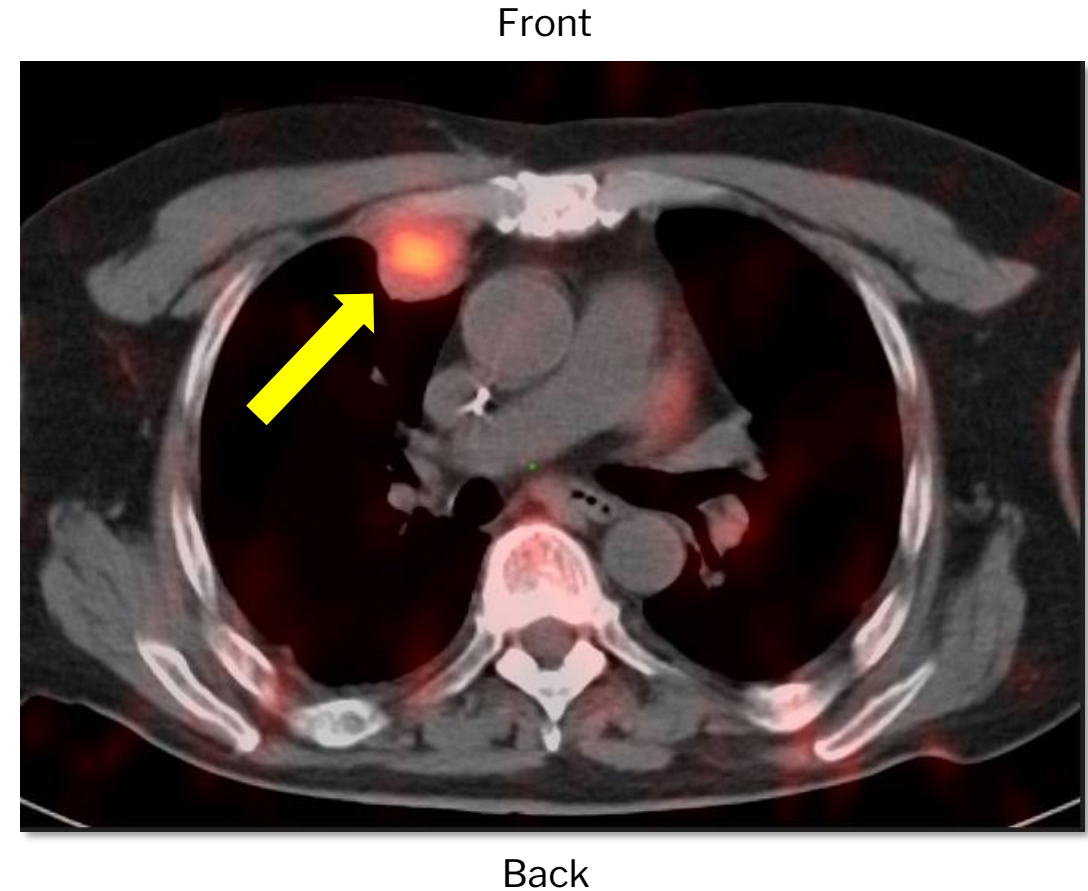
## PART A DATA ELEMENTS

- Optimal and safe GD2-SADA protein dose
- Dosing interval between GD2-SADA and <sup>177</sup>Lu-DOTA
- PK dosimetry
- Rate of excretion
- Aggregation in tissue
- Tumor burden
- Scan images

\*As of November 8, 2024

# Study 1001: SPECT/CT Scan Demonstrating Tumor Binding of $^{177}\text{Lu}$ -GD2 SADA\*

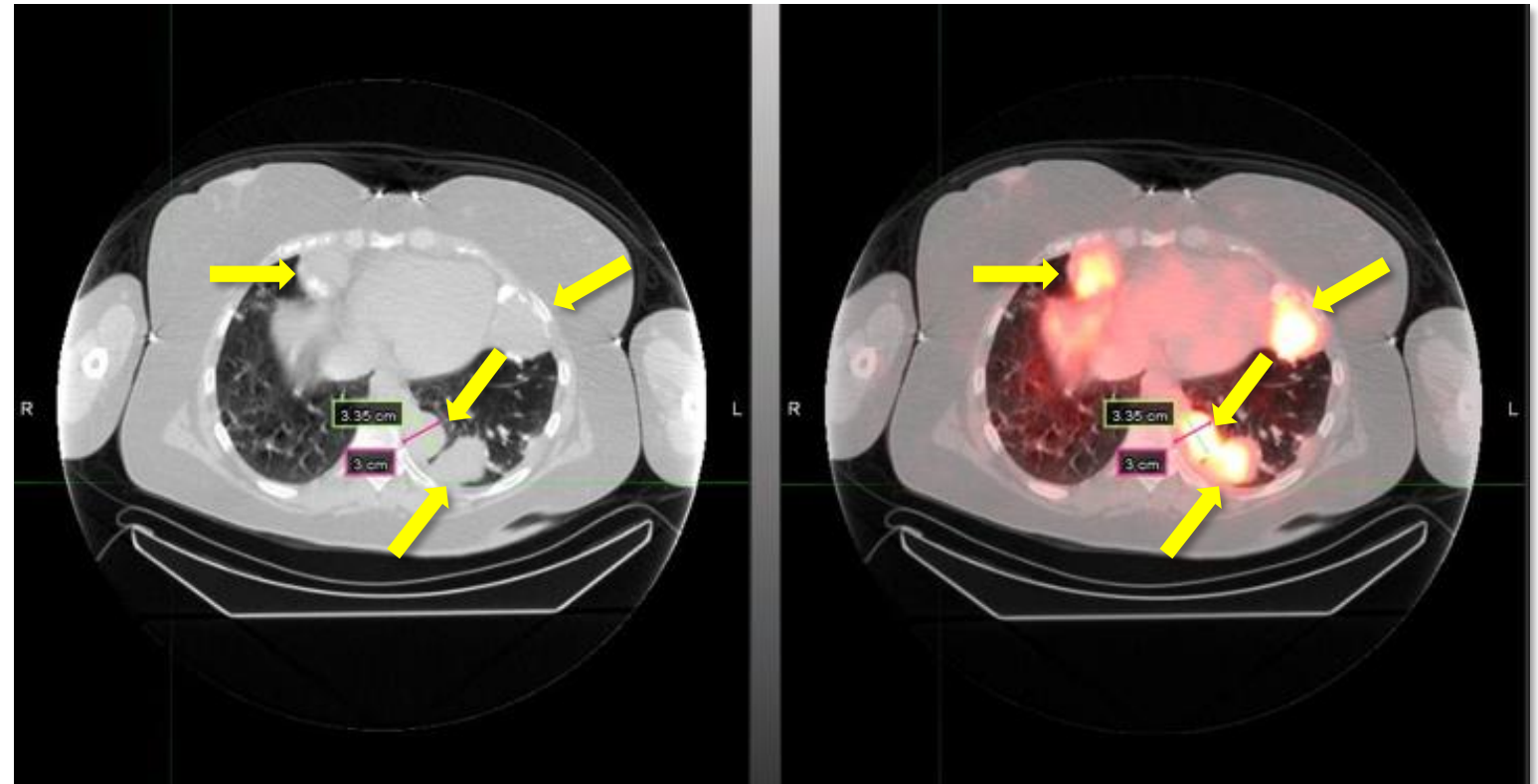
- Example of tumor targeting in Synovial Sarcoma using  $^{177}\text{Lu}$ -DOTA dose of **30 mCi** (imaging dose) in patient
- Arrow indicates tumor metastasis located in the Thoracic cavity – with  $^{177}\text{Lu}$ -DOTA uptake
- Scan performed 24 hours after radionuclide administration



\*These early results are not complete and are not necessarily indicative of the full results or ultimate success of the SADA trials or the SADA development program. Limitation: Patient-level data are for descriptive purposes and should not be considered indicative of typical product efficacy or duration; interpret with caution.

# Study 1001: SPECT/CT Scan on Osteosarcoma Patient Demonstrating Positive Tumor Uptake After Exposure\*

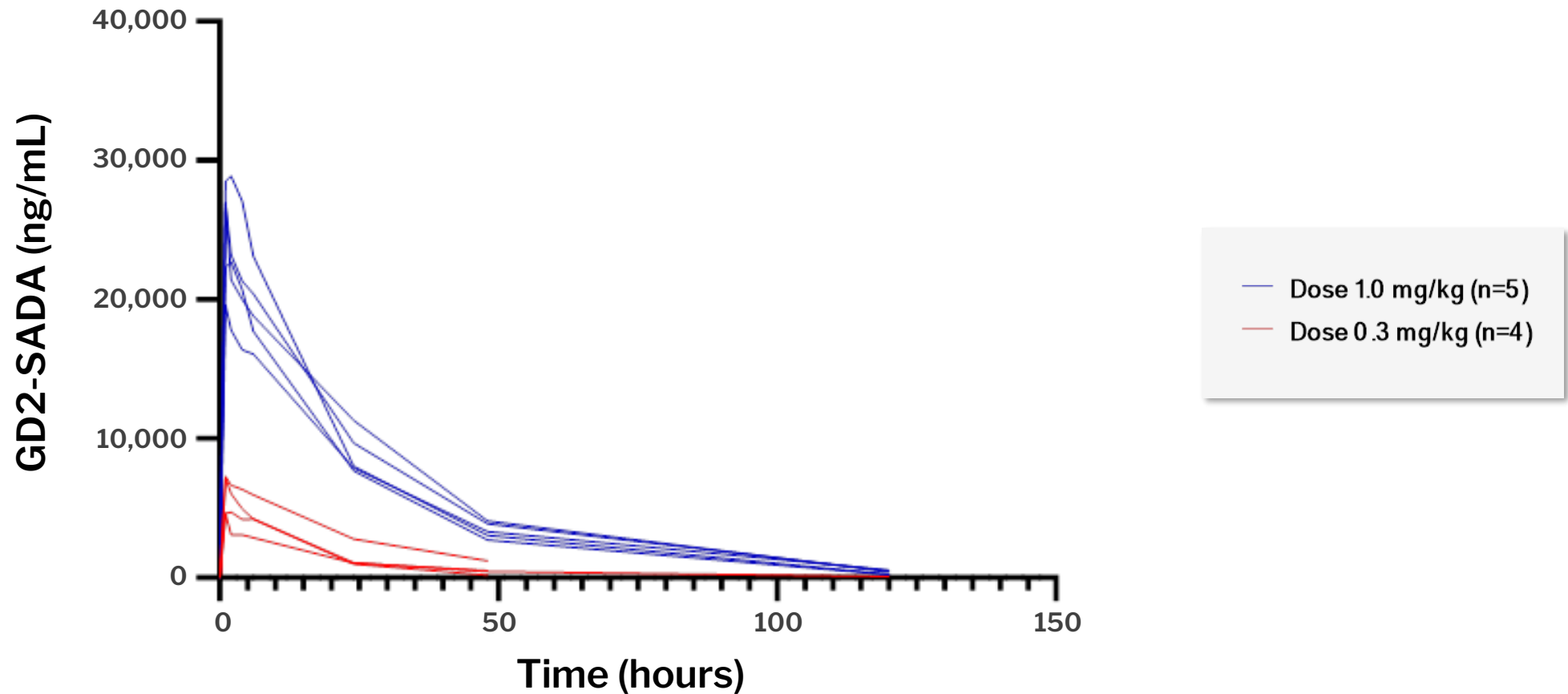
- Patient treated with 0.3 mg/kg GD2-SADA, followed by 200 mCi  $^{177}\text{Lu}$ -DOTA (lowest therapeutic radionuclide dose) 48-hours later
- Scan performed 24 hours after radionuclide administration
- 4 target lesions marked on CT scan (left image) – all targeted by  $^{177}\text{Lu}$ -DOTA SADA (right image)



\*These early results are not complete and are not necessarily indicative of the full results or ultimate success of the SADA trials or the SADA development program.  
Limitation: Patient-level data are for descriptive purposes and should not be considered indicative of typical product efficacy or duration; interpret with caution.

# Study 1001: Ongoing GD2-SADA Phase I Trial – Initial PK Data\*

## GD2-SADA Concentration vs. Time Profiles

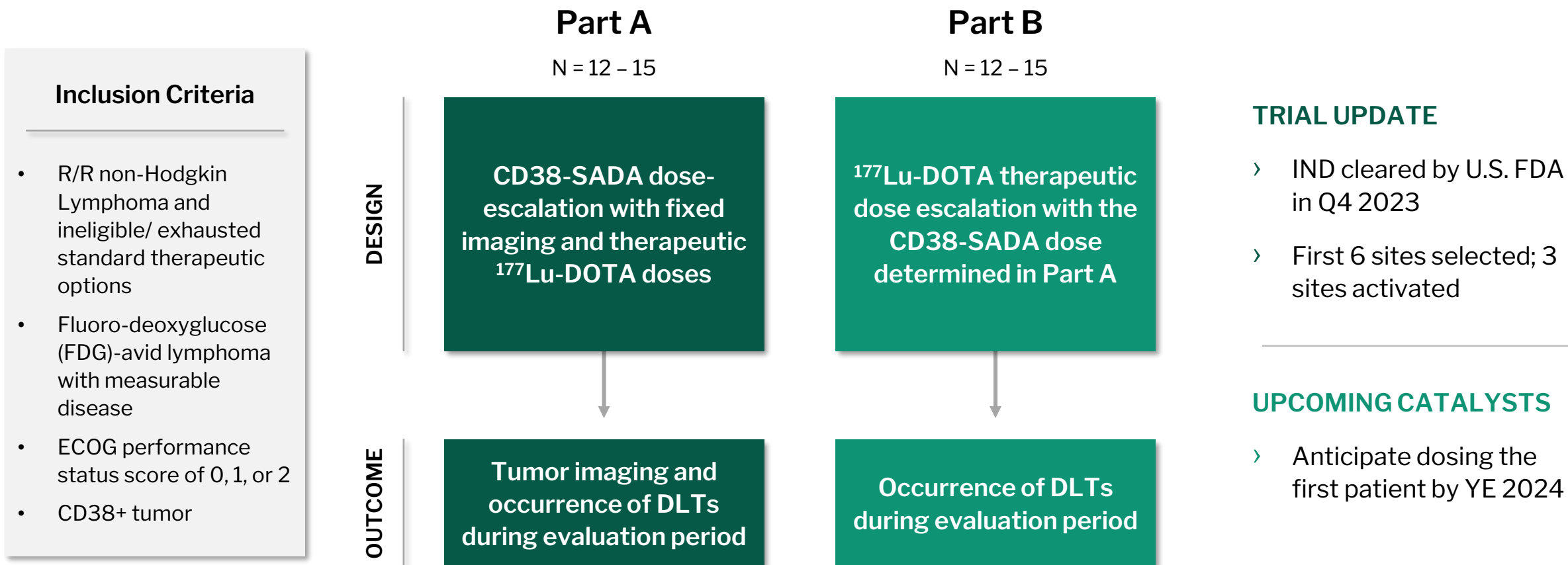


\*These early results are not complete and are not necessarily indicative of the full results or ultimate success of the SADA trials or the SADA development program. Limitation: Patient-level data are for descriptive purposes and should not be considered indicative of typical product efficacy or duration; interpret with caution. Non-QC data cut as of Company's Q3 2023 earnings report.



# CD38-SADA Phase 1 Clinical Trial (Study 1201): Trial Design

Theranostic approach using CD38 positivity on IHC and  $^{177}\text{Lu}$ -DOTA organ dosimetry before repeat dosing in patients with relapsed or refractory non-Hodgkin Lymphoma



# Novel SADA PRIT Platform Potentially Provides Simplicity and Enhanced Precision for Physicians and Patients\*



## Ongoing GD2-SADA Phase 1 Trial (Study 1001)

- › Evidence of tumor uptake
- › No DLTs observed to date
- › Demonstrated PoC that GD2-SADA targets and binds to tumor in humans



## CD38-SADA Phase 1 Trial (Study 1201)

- › IND cleared by U.S. FDA
- › First-in-human trial in patients with R/R non-Hodgkin Lymphoma
- › Anticipate dosing first patient in 2H 2024



- › GD2-SADA-Neuroblastoma IND filing anticipated in 2025
- › Additional IND filings anticipated in 2025:
  - › HER2-SADA
  - › B7H3-SADA



- › Potential to shift radio-immunotherapy treatment paradigm for patients and physicians with simplicity and enhanced precision of novel SADA platform

\*These early results are not complete and are not necessarily indicative of the full results or ultimate success of the SADA trials or the SADA development program which is in early development with no guaranty of approval



## Commercial Progress

**DANYELZA<sup>®</sup> (naxitamab-gqgk):**  
GD2 Antibody for  
R/R High-Risk Neuroblastoma

# DANYELZA: Only FDA-Approved Medicine for R/R NB Patients



## FDA Approval for R/R Neuroblastoma (NB)

- Differentiated therapy:
  - › Humanized antibody
  - › Rapid infusion, modest toxicity
  - › Administered in outpatient treatment setting
- U.S. addressable market:
  - › 2L NB: **300** patients



## Neuroblastoma

- NB forms in certain types of nerve tissue, most frequently starting from adrenal glands; can also develop in the neck, chest, abdomen or spine
- NB is the **most common cancer in infants**



## Global Commercial Launch Performance

- FY 2023 net sales of **\$84.3 million**
- **68 sites** across the U.S. have utilized DANYELZA\*
- Ex-U.S. commercial ramp progressing in China; Strong EU demand through WEP
- Ex-U.S. markets include China, Brazil and Mexico; NPPs+ in Europe, Turkey



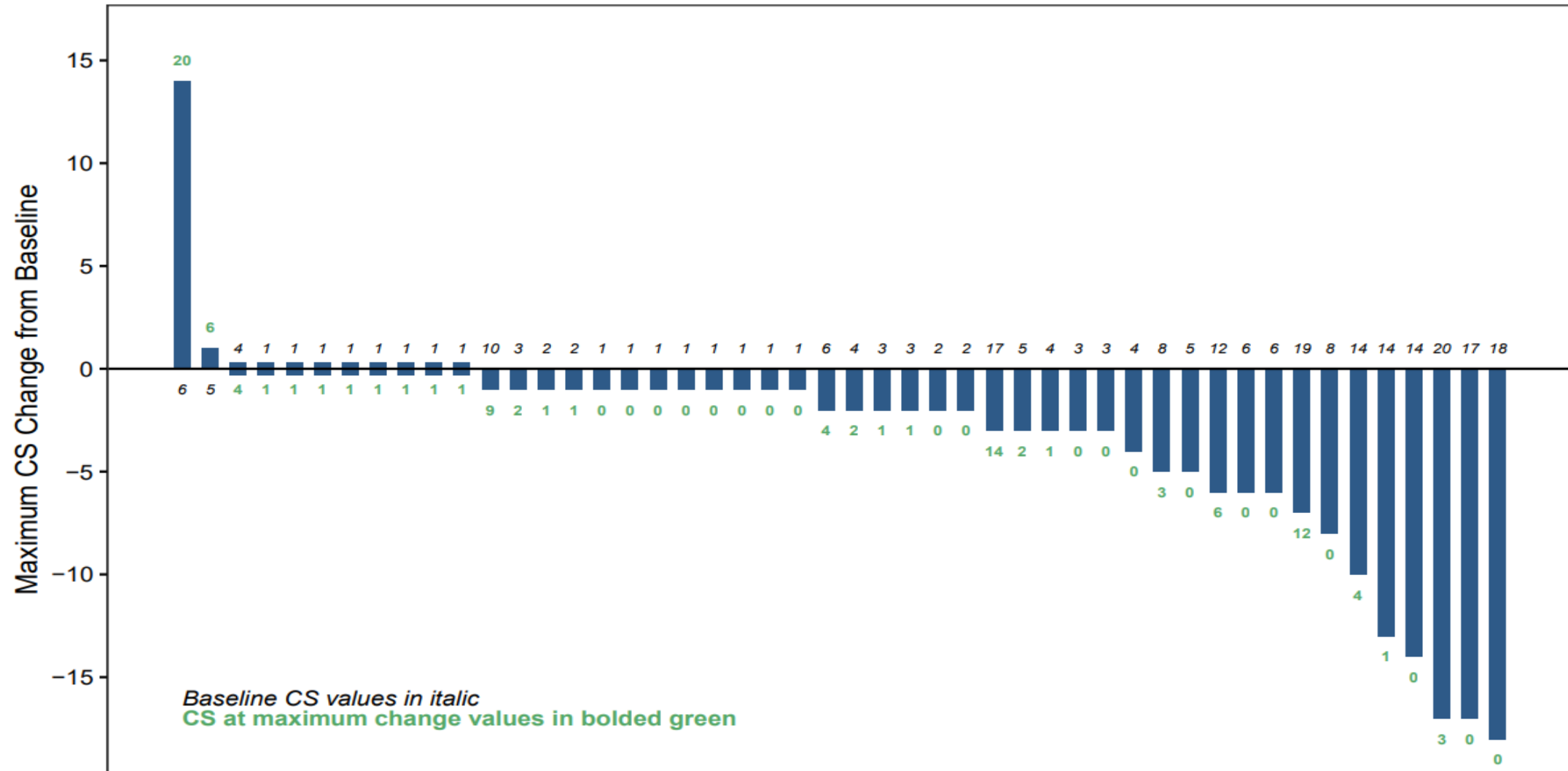
## Solid Drivers of Market Uptake

- DANYELZA added to **48 hospital formularies** since initial launch in 2021\*
- **113 HCPs** prescribed DANYELZA since launch\*
- DANYELZA remains a **leading therapy** in U.S. anti-GD2 market

**DANYELZA**<sup>™</sup>  
(naxitamab-gqgk)  
40mg/10mL Injection

\* As of September 30, 2024  
+ Named Patient Program

# Pivotal Study 201 Data: Waterfall Plot of Change in Curie Score in all Relapsed/Refractory Patients with Bone Disease (n = 48)



# Ongoing Naxitamab Clinical Trials



Memorial Sloan Kettering  
Cancer Center

- Multi-center Phase 2 trial investigating naxitamab in patients with relapsed osteosarcoma
- Anticipated complete data readout from MSK in Q4 2024



Evaluate potential to initiate pivotal randomized trial following data readout from MSK

**Beat Childhood Cancer**  
RESEARCH CONSORTIUM

- Phase 2 BCC multi-center trial evaluating naxitamab + standard induction therapy in patients with newly diagnosed HR NB
- 22 sites initiated to date; target 40-50 sites in U.S. and Canada
- 11 patients dosed to date\*; target 76 total patients



Matched comparison with an external control currently being developed



THE OHIO STATE UNIVERSITY  
COMPREHENSIVE CANCER CENTER

- Phase 1b trial investigating TGF $\beta$  NKs, gemcitabine + naxitamab in patients with advanced breast cancer
- 2 patients treated with combo gemcitabine + NK cells
- Patients in F/U for DLT and persistence of NK cells (following gem+NK cell combo, prior to addition of naxitamab)



Potential multi-center Phase 2 study based on results from Phase 1b trial



Institute  
of Mother and Child

- Randomized Phase 2 trial evaluating efficacy and safety of naxitamab in patients with refractory Ewing sarcoma initiated in Q4 2023
- 3 patients dosed in naxitamab arm to date; target 24 patients total (16 naxitamab, 8 control)



Anticipated study completion in 2028

Clinicaltrials.gov: BCC trial NCT05489887, MSK trial NCT02502786, OSU trial NCT06026657



# Ongoing and Potential New Studies for Naxitamab: Expanding Usage in New Indications

Cancer Indications		Treatable Patient Population (U.S.)	GD2 Expression	2022	2023	2024	2025	2026
<b>High-Risk Neuroblastoma</b>	Relapsed / Refractory	300	~ 99-100%	R/R HRNB Confirmatory Study 201*				
	Front-line Induction	450		1 <sup>st</sup> line Induction BCC-018 Phase 2		1 <sup>st</sup> line Induction RCT BCC study		
<b>Osteosarcoma</b> Relapsed/Recurrent		200	~ 88%	Relapsed Osteosarcoma MSKCC Study 15-096				Pivotal RCT**
<b>Soft-Tissue Sarcomas</b> Including Ewings		2,900 (1 <sup>st</sup> -line population)	> 90%	ISS – Ongoing Phase 2 (Ewings)				
<b>Breast Cancer</b> Triple Negative / Advanced		8,900 (2 <sup>nd</sup> line & 3 <sup>rd</sup> line +)	> 50%	ISS – Ongoing Phase 2				
<b>Melanoma</b> Newly Unresectable and Metastatic		11,400 (2 <sup>nd</sup> line & 3 <sup>rd</sup> line +)	> 50%	ISS – Area of Interest				

\* This indication is approved under accelerated approval. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial(s).

\*\* Subject to data readout of MSKCC study 15-096.

# DANYELZA Addresses Significant Unmet Needs in R/R High-Risk NB with Expansion Potential Across Broader Patient Populations



- › Studies 12-230 and 201 formed primary basis of approval in November 2020
  - › Reached 100 patients in Study 201



- › Granted ODD and BTD
  - › Frontline study ongoing



- › U.S. commercialization in high-risk NB
- › Expanding ex-U.S. reach
  - › Commercially available in China through partner SciClone, LATAM partner Adium in Brazil and Mexico; EU and Turkey access via WEP



- › Multiple potential advantages over other anti-GD2 therapies:
  - › Modest toxicity
  - › Shorter infusion time
  - › Ability to be administered in outpatient setting



Company Takeaways

# Strongly Positioned to Drive Future Value

## Novel Platforms in Development



Self-Assembly  
DisAssembly Pretargeted  
Radioimmunotherapy  
("SADA PRIT")  
Platform

Monoclonal Antibodies

## Commercial Leverage



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## Capital Efficiency



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Cash of \$68.1 million\*

Financial Runway  
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\* As of September 30, 2024



The background is a microscopic view of a cell, likely a bacterium, showing internal structures like flagella and a large, textured organelle. The color palette is dominated by teal and blue. In the foreground, several green, rod-shaped bacteria are visible, some of which are curved. A white rectangular box is centered in the lower half of the image, containing the text 'THANK YOU' in white, bold, uppercase letters.

**THANK YOU**