



Oncology Leadership in Pretargeted Radioimmunotherapy Platform and Antibody-based Therapies

September 2024



Disclaimer

Statements in this presentation about future expectations, plans and prospects, as well as any other statements regarding matters that are not historical facts, constitute “forward-looking statements” within the meaning of Section 27A of the Securities Act of 1933 and Section 21E of the Securities Exchange Act of 1934. Such statements include, but are not limited to, statements about the Company’s growth prospects and expectations related thereto; expectations with respect to the Company’s financial expectations, including the Company’s 2023 operating expense, cash burn and DANYELZA net product revenue guidance, and the Company’s estimated cash runway and sufficiency of cash resources and related assumptions; the Company’s ability to deliver value; expectations with respect to the achievement of milestones and the timing thereof; implied and express statements regarding the future of the Company’s business, including with respect to expansion and its goals; the Company’s plans and strategies, development, commercialization and product distribution plans, including potential partnerships; expectations with respect to the Company’s products and product candidates, including potential territory and label expansion of DANYELZA and the potential market opportunity related thereto and potential benefits thereof, and the potential of the SADA Technology and potential benefits and applications and the timing thereof; SADA’s potential to be an industry game-changer; expectations with respect to current and future clinical and pre-clinical studies and the Company’s and its partners’ research and development programs, including with respect to timing and results; expectations related to the timing of the initiation and completion of regulatory submissions; additional product candidates and technologies; expectations regarding collaborations or strategic partnerships and the potential benefits thereof; expectations related to the use of cash and cash equivalents, and the need for, timing and amount of any future financing transaction; expectations with respect to the Company’s future financial performance; and other statements that are not historical facts. Words such as “anticipate,” “believe,” “contemplate,” “continue,” “could,” “estimate,” “expect,” “hope,” “intend,” “may,” “might,” “plan,” “potential,” “predict,” “project,” “should,” “target,” “will,” “would”, “guidance,” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. The Company’s 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 the Company’s financial condition and need for additional capital; the risks that actual results of the Company’s restructuring plan and revised business plan will not be as expected; risks associated with the Company’s development work; cost and success of the Company’s product development activities and clinical trials; the risks of delay in the timing of the Company’s regulatory submissions or failure to receive approval of its drug candidates; the risks related to commercializing any approved pharmaceutical product including the rate and degree of market acceptance of product candidates; development of sales and marketing capabilities and risks associated with failure to obtain sufficient reimbursement for products; the risks related to the Company’s dependence on third parties including for conduct of clinical testing and product manufacture; the Company’s ability to enter into new partnerships and to maintain existing partnerships; the risks related to government regulation; risks related to market size and approval, risks associated with protection of the Company’s intellectual property rights; risks related to employee matters and managing growth; risks related to the Company’s common stock; risks associated with macroeconomic conditions, including the conflict between Russia and Ukraine and sanctions related thereto, the state of war between Israel and Hamas and the related risk of a larger regional conflict, inflation, increased interest rates, uncertain global credit and capital markets and disruptions in banking systems; the completion of financial closing procedures, final audit adjustments and other developments that may arise that would cause the Company’s expectations with respect to the Company’s 2024 guidance to differ, perhaps materially, from the financial results that will be reflected in the Company’s audited consolidated financial statements for the fiscal year ended December 31, 2024; and other risks and uncertainties affecting the Company including those described in the “Risk Factors” section included in the Company’s Annual Report on Form 10-K for the fiscal year ended December 31, 2023, and future filings and reports by the Company. 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.

This presentation includes statistical and other industry and market data that we obtained from industry publications and research, surveys and studies conducted by third parties or us. Industry publications and third-party research, surveys and studies generally indicate that their information has been obtained from sources believed to be reliable, although they do not guarantee the accuracy or completeness of such information. All of the market data used in this presentation involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such estimates. While we believe these industry publications and third-party research, surveys and studies are reliable, we have not independently verified such data. The industry in which we operate is subject to a high degree of uncertainty, change and risk due to a variety of factors, which could cause results to differ materially from those expressed in the estimates made by the independent parties and by us.

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 I
Part A Completion

CD38-SADA Phase I
Study Initiation

MSK Data Readout of
Phase II Osteosarcoma
Trial Anticipated

Capital Efficiency



















Independent
Commercial-stage
Biotech Company with
Cash of \$77.8 million*

Financial Runway
into 2027

Strategic Capital
Deployment

* As of June 30, 2024

Advancing Focused Pipeline with Multiple Value-Added Catalysts Ahead

	Study	Therapeutic Area	Preclinical	Phase I	Phase II/Pivotal	Approved	Trial Sponsor	Status
Lead Programs								
Naxitamab-gqgk (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)						Randomized trial expected Q2 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						Initiated in Q4 2023; Enrollment ongoing
SADA PRIT (Radioimmunotherapy)	1001	GD2-SADA: Solid Tumors (SCLC, Malignant Melanoma, Sarcoma)						Expect to complete Part A in 2024
	1201	CD38-SADA: Non-Hodgkin Lymphoma						Activating first sites in Q2 2024
Early Programs								
SADA PRIT (Radioimmunotherapy)	1002	GD2-SADA: Neuroblastoma						Expected IND filing in 2024
		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



**Infrastructure and
Manufacturing**



**Physician
Participation**



**Administration
Sites**



**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



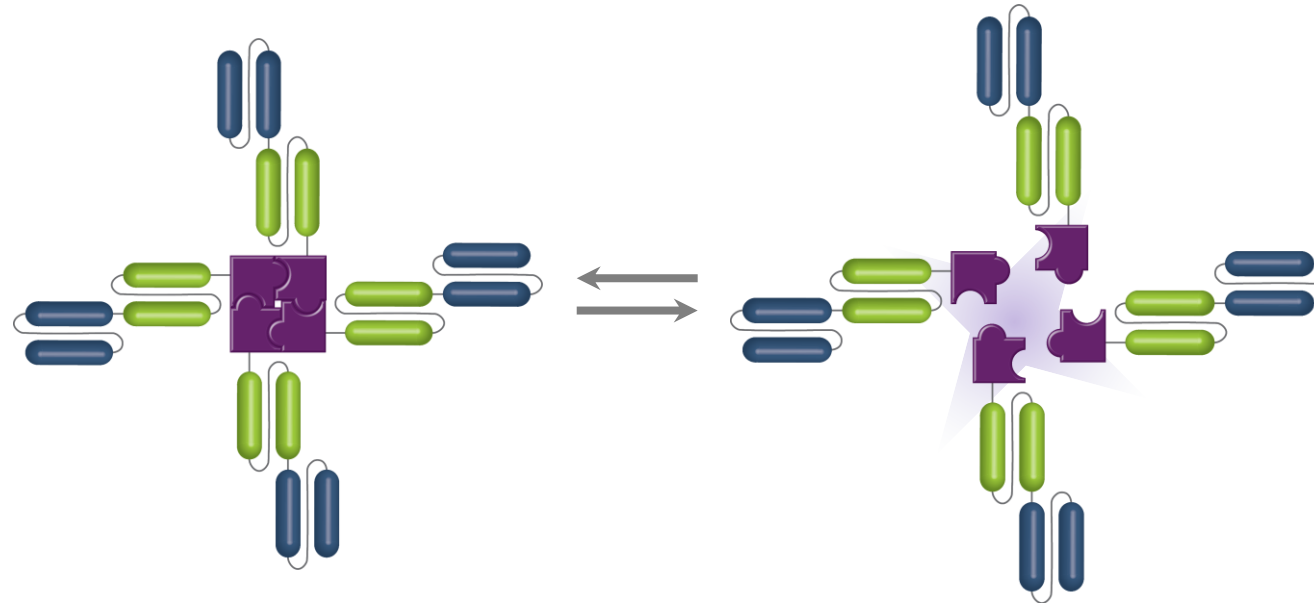
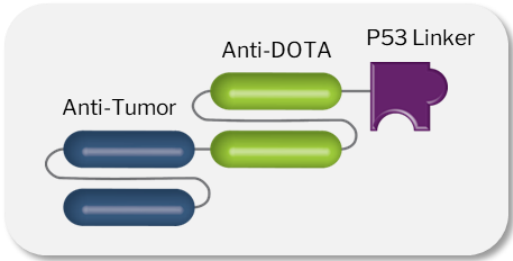
Novel SADA PRIT Platform Potential Capabilities

- ✓ 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

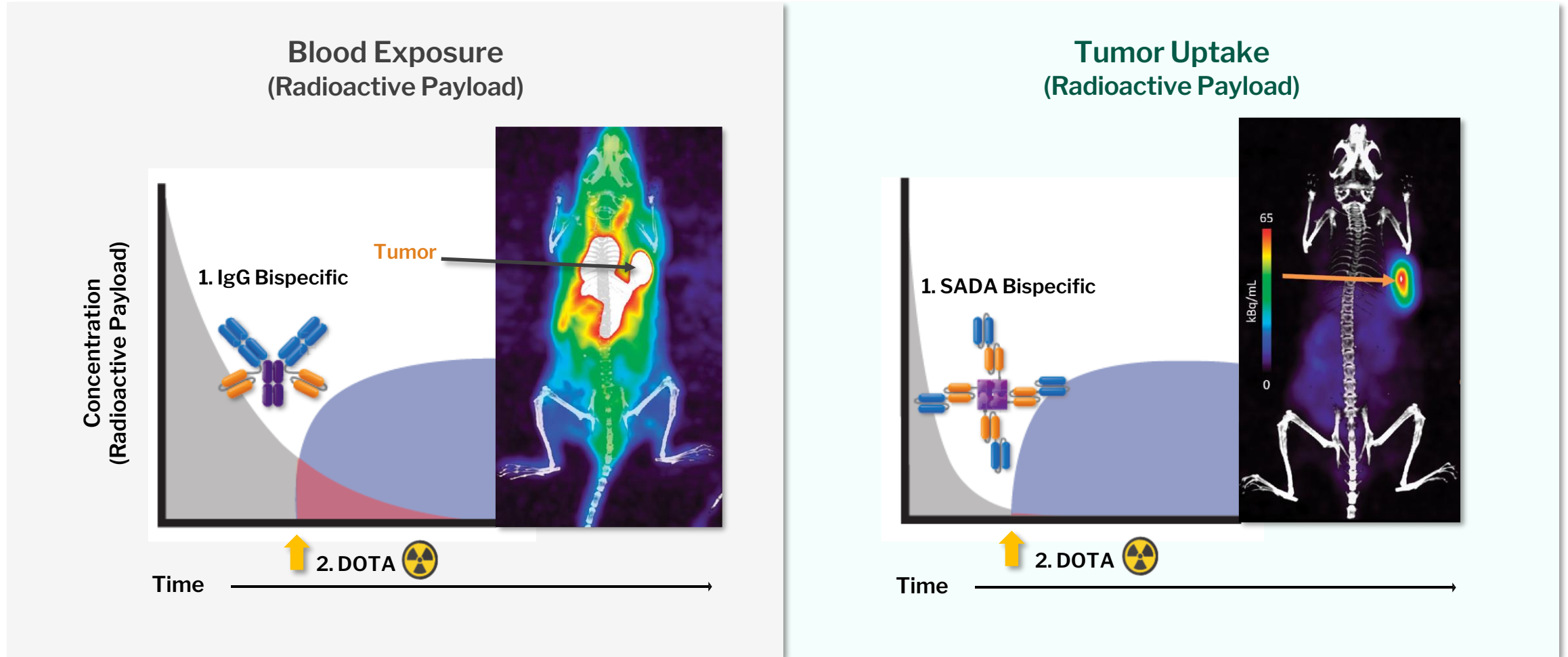
DisAssembled Monomer
< 70kDa

Strong Tumor Binding

Rapid Clearance

Adapted from Santich et al. Clin Canc Res 2020

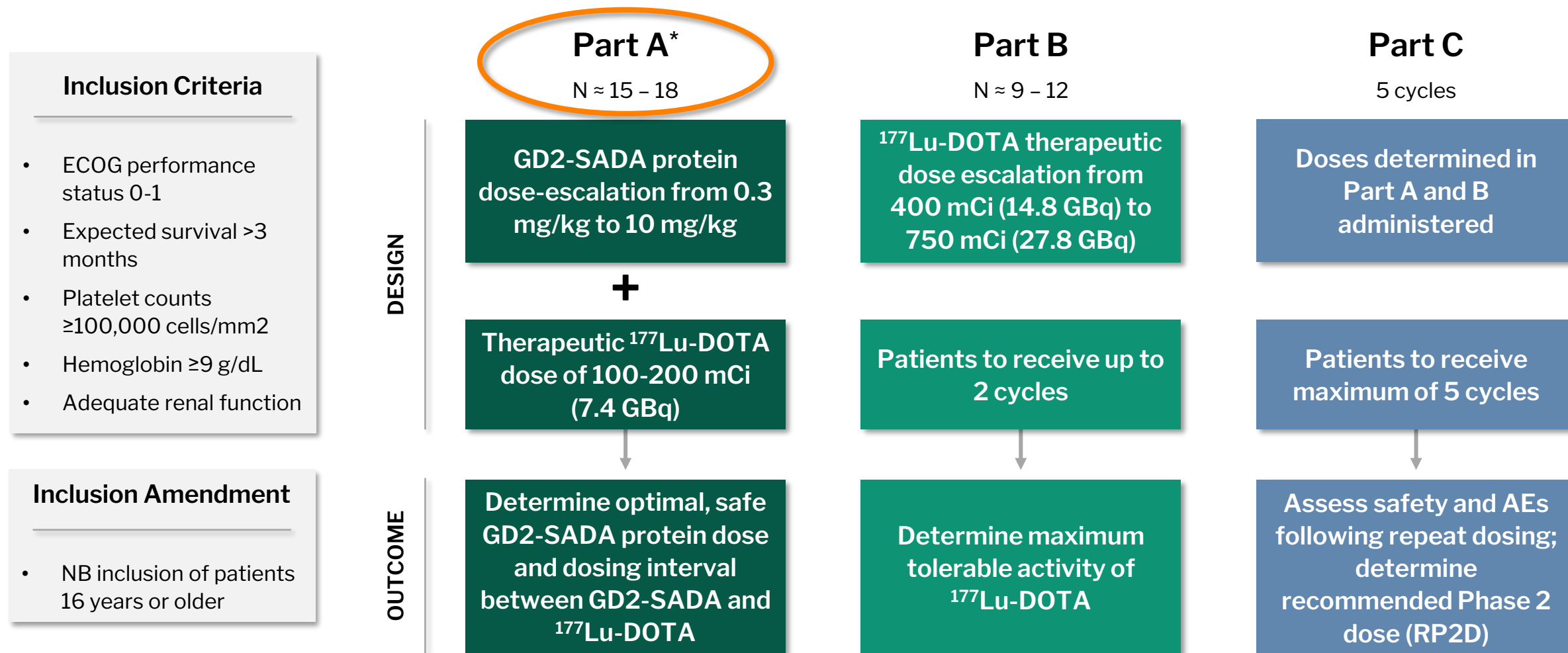
GD2-SADA Achieves High Tumor Uptake with Minimal Exposure to All Other Tissues



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 ¹⁷⁷Lu-DOTA imaging dose before exposing to therapeutic dose



- Inclusion Criteria**
- ECOG performance status 0-1
 - Expected survival >3 months
 - Platelet counts ≥100,000 cells/mm²
 - 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)
- › Completed Cohorts 1 through 4; currently in Cohort 5*
- › 17 patients dosed to date*
- › 6 sites open*; planning to add additional sites
- › No DLTs or instances of treatment-related AEs reported*

UPCOMING CATALYSTS

- › Expect to complete Part A in Q4 2024
- › Anticipate Part A data readout in late-2024/2025

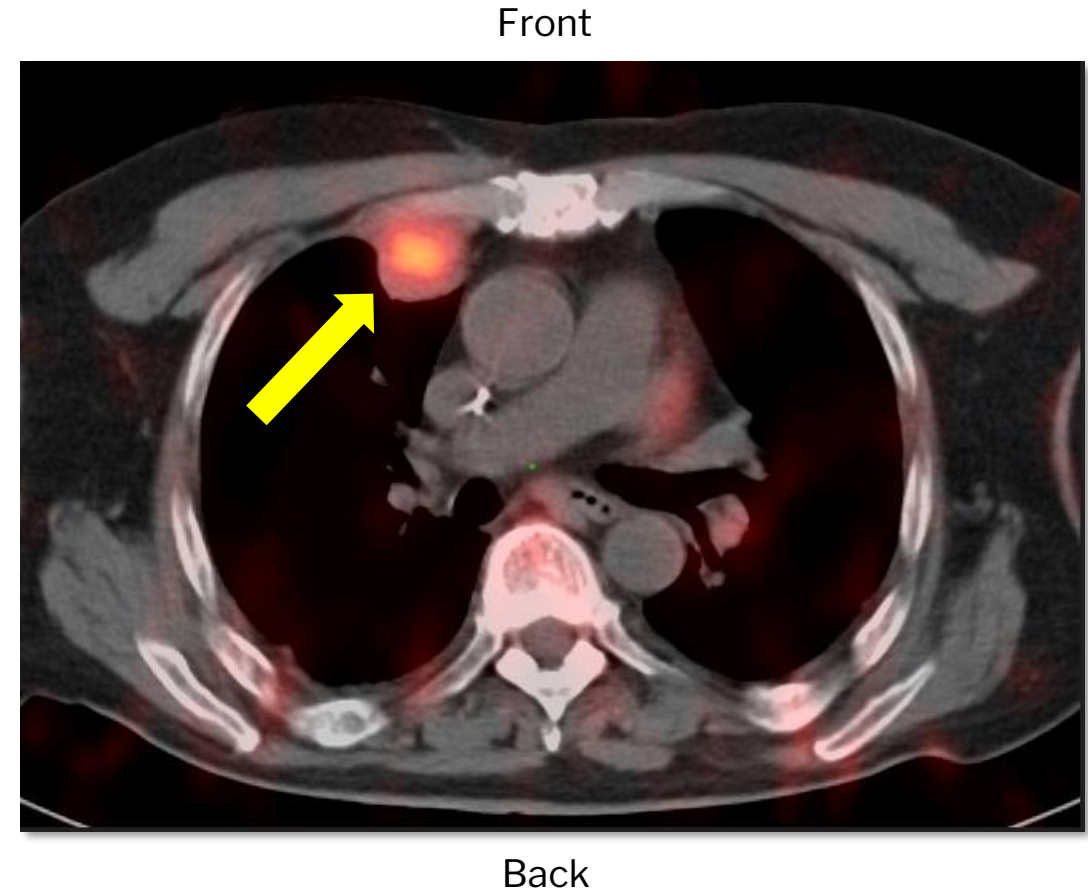
PART A DATA ELEMENTS

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

*As of June 30, 2024

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

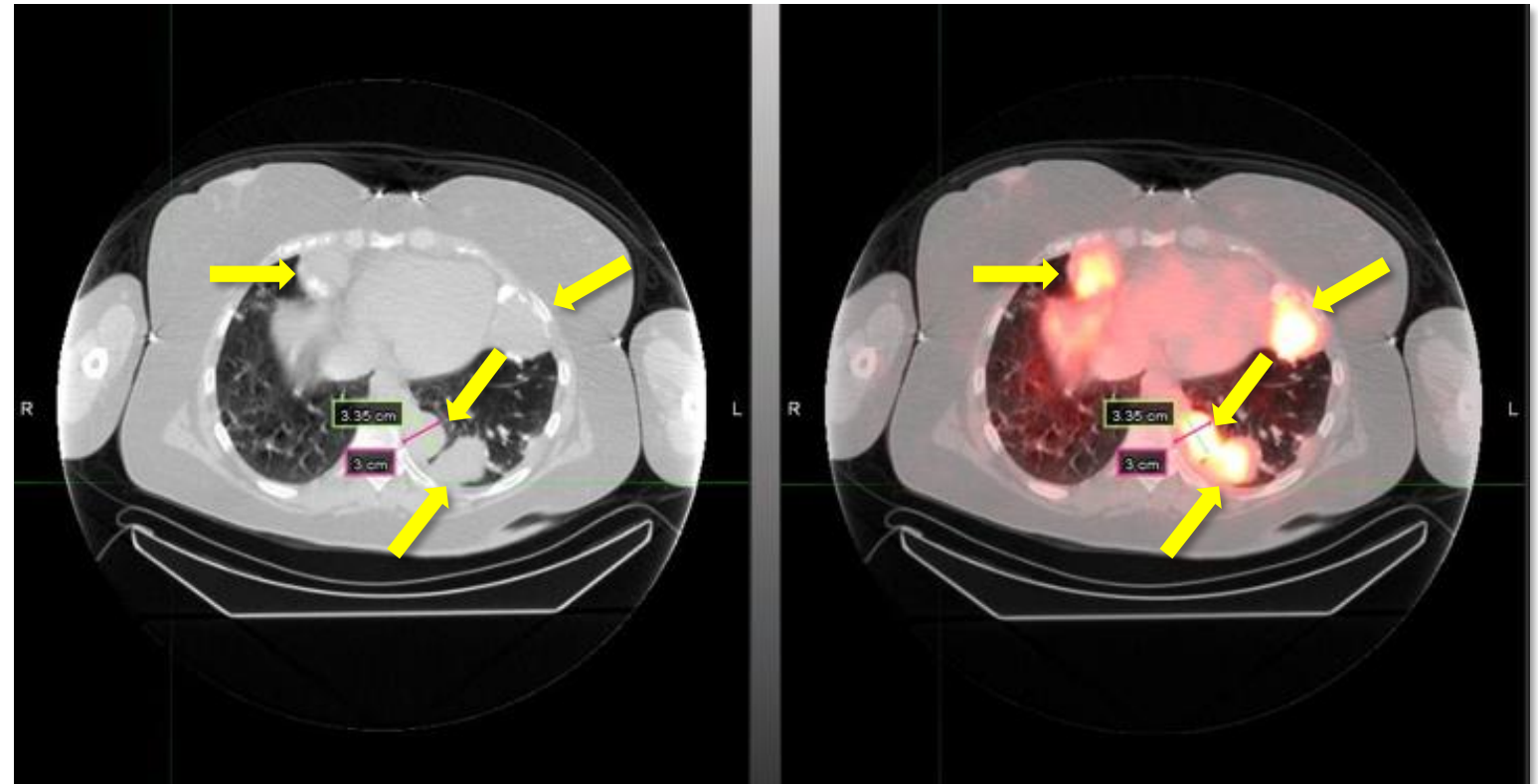
- Example of tumor targeting in Synovial Sarcoma using ^{177}Lu -DOTA dose of **30 mCi** (imaging dose) in patient
- Arrow indicates tumor metastasis located in the Thoracic cavity – with ^{177}Lu -DOTA uptake
- Scan performed 24 hours after radionuclide administration



*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*

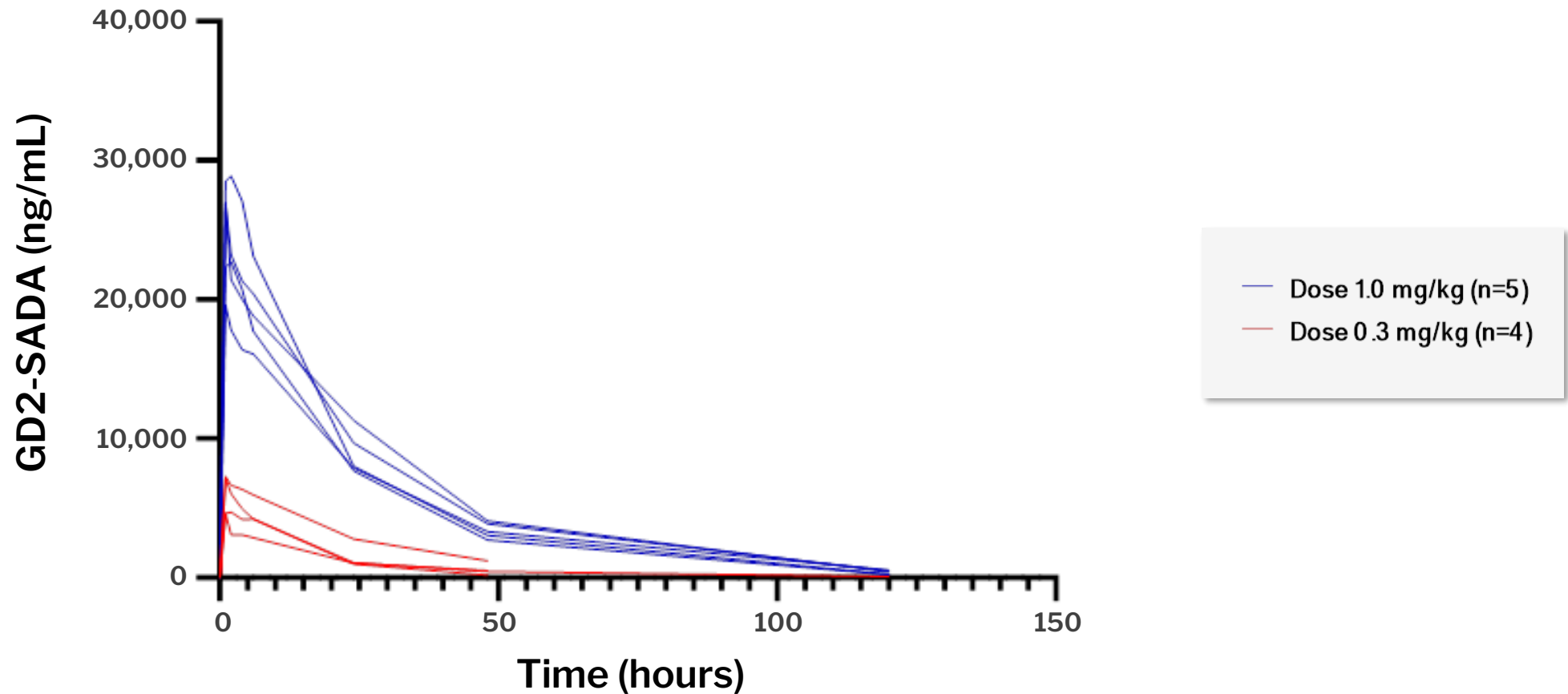
- Patient treated with 0.3 mg/kg GD2-SADA, followed by 200 mCi ^{177}Lu -DOTA (lowest therapeutic radionuclide dose) 48-hours later
- Scan performed 24 hours after radionuclide administration
- 4 target lesions marked on CT scan (left image) – all targeted by ^{177}Lu -DOTA SADA (right image)



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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*

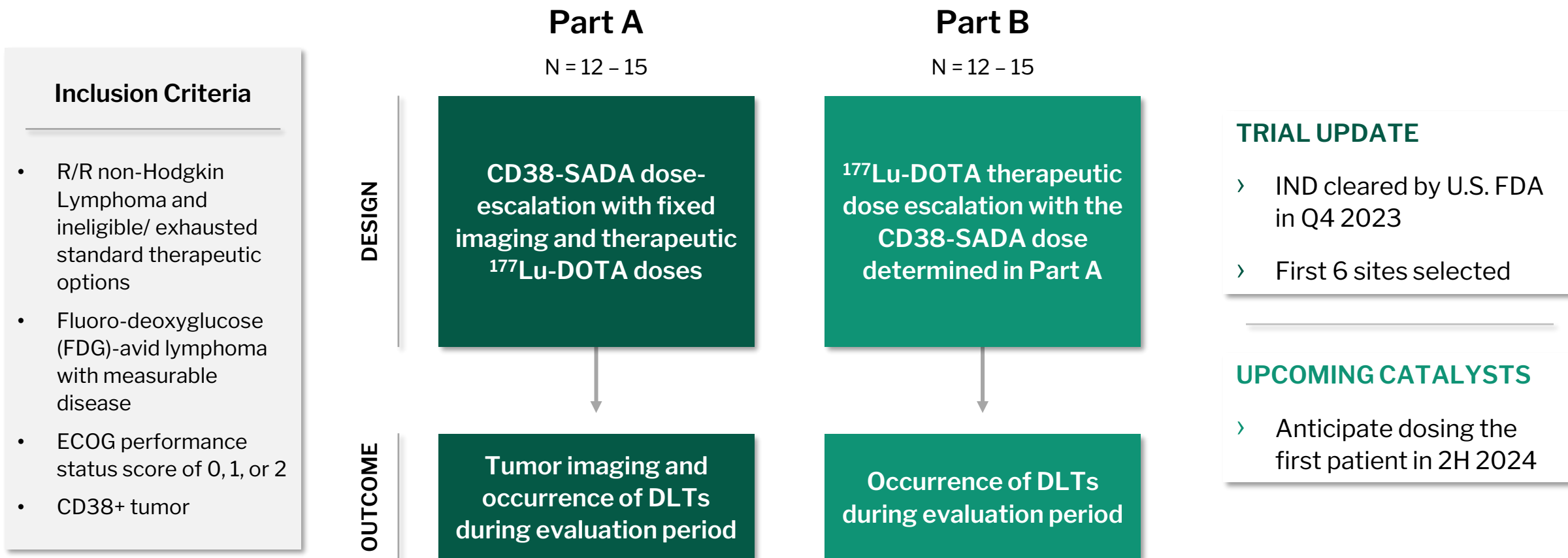
GD2-SADA Concentration vs. Time Profiles



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Non-QC data cut as of Company's Q3 2023 earnings report.

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

Theranostic approach using CD38 positivity on IHC and ¹⁷⁷Lu-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 I 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 I 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 2024
- › 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[®] (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**
- **65 sites** across the U.S. have utilized DANYELZA*
- Ex-U.S. commercial ramp progressing in China; Strong EU demand through WEP
- LATAM launched in Brazil and Mexico; approval in Hong Kong



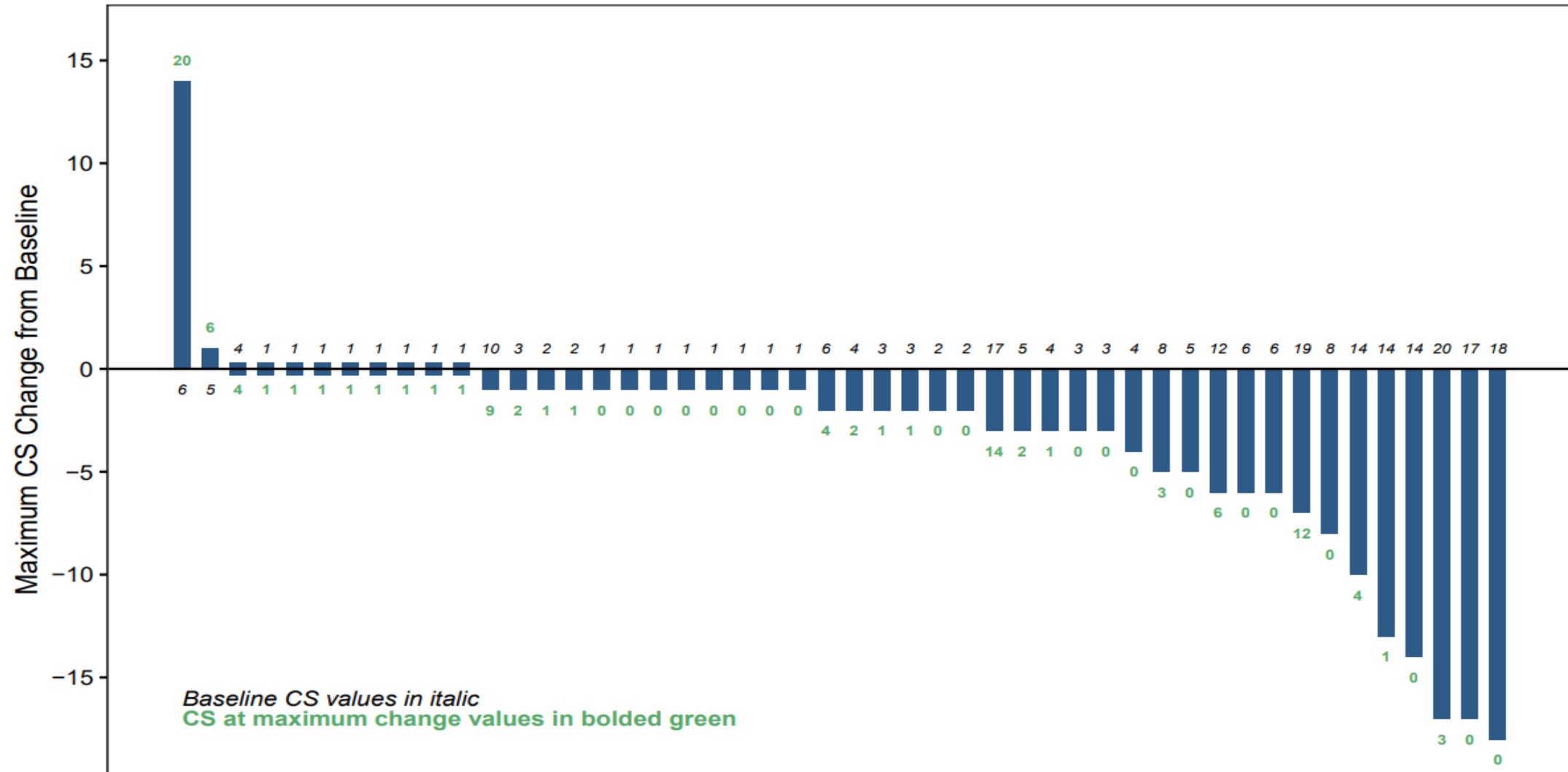
Solid Drivers of Market Uptake

- New DANYELZA campaign rolled out in Q4 2023
- **108 HCPs** prescribed DANYELZA since initial 2021 launch*
- DANYELZA remains a **leading therapy** in U.S. anti-GD2 market

DANYELZA[™]
(naxitamab-gqgk)
40mg/10mL Injection

* As of June 30, 2024

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



Prepared to initiate pivotal randomized trial in Q2 2025 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
- 17 sites initiated to date; target 40-50 sites in U.S. and Canada
- 10 patients dosed to date*; target 76 total patients



Anticipate transitioning to a multi-center randomized trial in 2H 2024



THE OHIO STATE UNIVERSITY
COMPREHENSIVE CANCER CENTER

- ISS Phase 1b/2 trial investigating TGF β NKs, gemcitabine + naxitamab in patients with metastatic breast cancer
- Two patients enrolled and treated with combo gemcitabine + NK cells
- Anticipate first patient to be dosed with naxitamab in 2H 2024



Potential multi-center Phase 2 study based on results from the 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
High-Risk Neuroblastoma	Relapsed / Refractory	300	~ 99-100%	R/R HRNB Confirmatory Study 201*				
	Front-line Induction	450		1 st line Induction BCC-018 Phase II		1 st line Induction RCT BCC study		
Osteosarcoma Relapsed/Recurrent		200	~ 88%	Relapsed Osteosarcoma MSKCC Study 15-096				Pivotal RCT**
Soft-Tissue Sarcomas Including Ewings		2,900 (1 st -line population)	> 90%	ISS – Ongoing Phase II (Ewings)				
Breast Cancer Triple Negative / Advanced		8,900 (2 nd line & 3 rd line +)	> 50%	ISS – Ongoing Phase Ib/II				
Melanoma Newly Unresectable and Metastatic		11,400 (2 nd line & 3 rd 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
 - › Launch in China by SciClone; LATAM partner Adium in Brazil and Mexico; EU 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

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* As of June 30, 2024

The background is a microscopic scene. On the left, a large, textured, blue-green spherical structure is visible. Scattered throughout are various rod-shaped bacteria. Some are green and appear to be in motion or interacting. Others are blue and have a more complex, multi-segmented structure. The overall lighting is cool, with a mix of teal, blue, and green tones.

THANK YOU