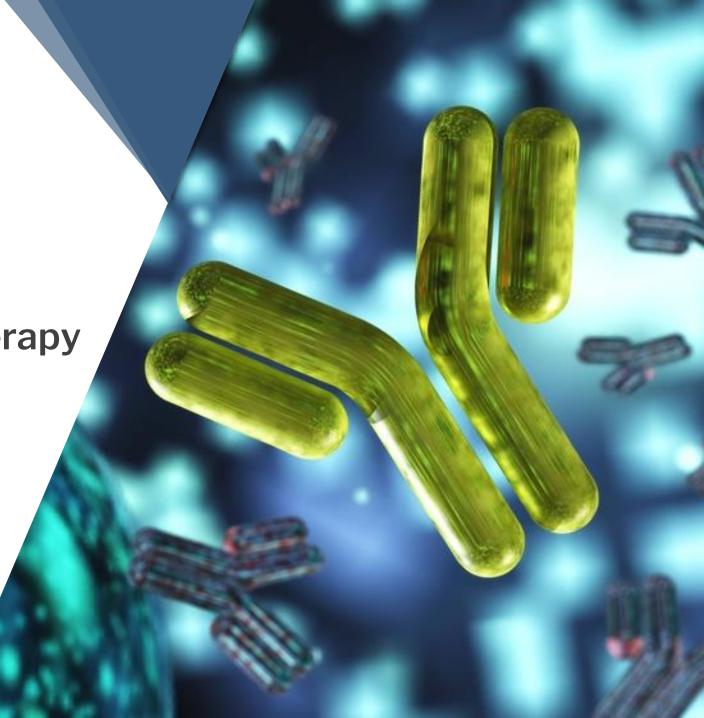


Oncology Leadership in Pretargeted Radioimmunotherapy Platform and Antibody-based Therapies

Michael Rossi, President and CEO

J.P. Morgan Healthcare Conference | January 10, 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," "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 2023 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, 2023; 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, 2022, the Company's Quarterly Report on Form 10-Q for the quarter ended September 30, 2023 and future filings and reports by the Company. Any forward-looking statements contained in this press release 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



<u>S</u>elf-<u>A</u>ssembly <u>D</u>is<u>A</u>ssembly ("SADA") Pretargeted Radioimmunotherapy Platform ("PRIT")

Monoclonal Antibodies

Commercial Leverage

DANYELZA (naxitamab-gqgk)

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





GD2-SADA Phase I Part A Data Readout

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 ~\$87 million^{*}

> Financial Runway into 2027

> Maintain FY2023 Guidance

* As of September 30, 2023



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 (naxitama	ab-gqgk) Confirm	atory Trial	Ø		U.S. FDA approved
	12-230	Relapsed/Refractory High-Risk Neuroblastoma (Pediatric)	DANYELZA (naxitam	ab-gqgk)		<u></u>	Memorial Sloan Kettering Cancer Center	U.S. FDA approved
	BCC018	Front-Line Induction in High-Risk Neuroblastoma (Pediatric)					Beat Childhood Cancer	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
SADA (Radioimmunotherapy)	1001	GD2-SADA: Solid Tumors (SCLC, Malignant Melanoma, Sarcoma)						First cohorts treated
	1201	CD38-SADA: Non-Hodgkin Lymphoma						Expected Phase I initiation in 2024

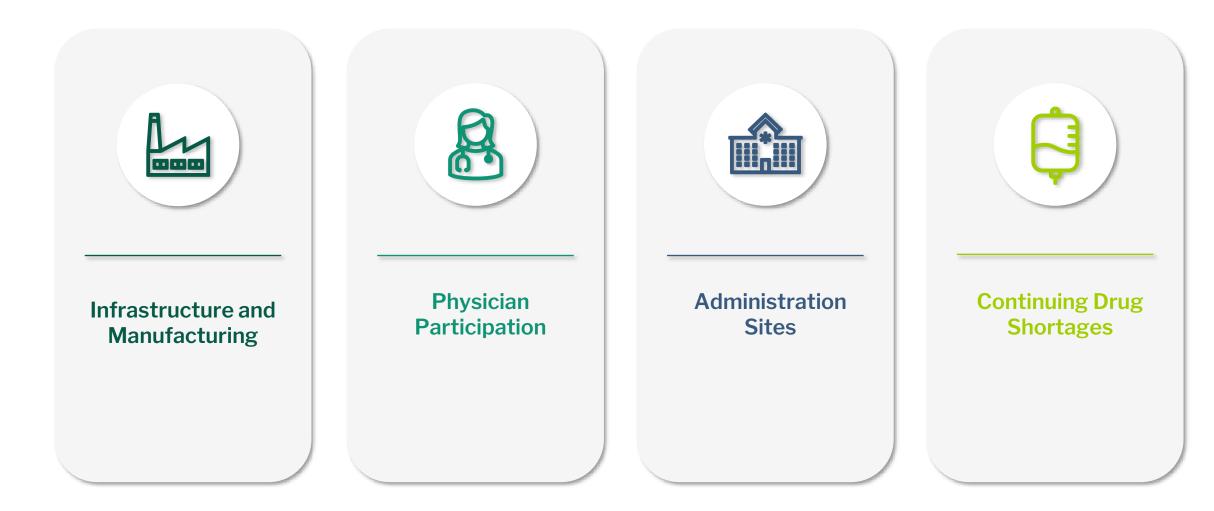
Early Programs			
SADA (Radioimmunotherapy)	GD2-SADA: Neuroblastoma		Expected IND filing in 2024
	HER2-SADA		Expected IND filing in 2025
	B7H3-SADA		Expected IND filing in 2025



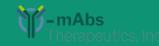


Novel SADA Pretargeted Radioimmunotherapy Technology Platform

Current Radiopharma Challenges Negatively Impact Patient Care



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



SADA's Novel Pretargeted 2-Step Approach a Potential Industry Game-Changer*

Traditional Radioimmunotherapy



Risk of systemic high toxicity



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



Limited administration sites with licensed nuclear medicine radiologists

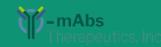


High investment needed for specific infrastructure and manufacturing

Novel SADA Platform Potential Capabilities

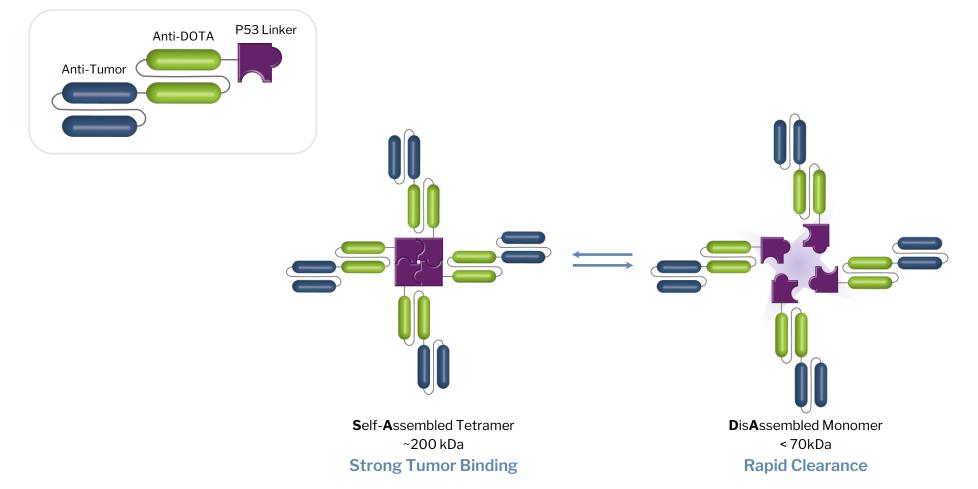
- Pretargeting tumor potentially minimizes toxicity and potentially enhances rapid clearing of unbound protein
- Potential to work with short $T_{1/2}$ isotopes
- Potentially broader site options with 1st protein dose administered by Medical Oncologist at large infusion centers
- Potential COGS improvements

* Pending successful development and approval



Self-**A**ssembly **D**is**A**ssembly (SADA) Technology: High Affinity for Tumor Targets and Rapid Clearance from Blood Stream

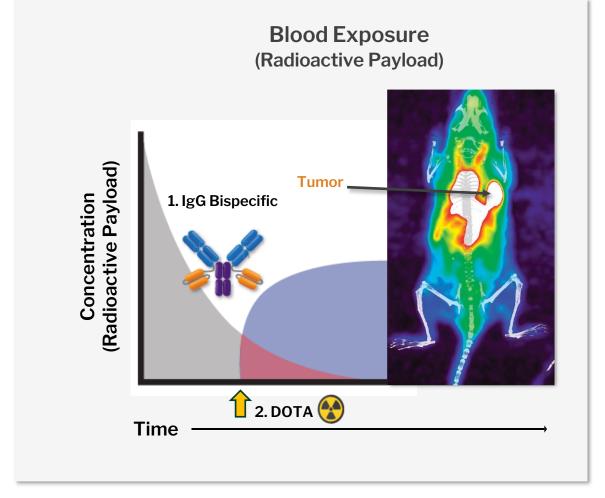
SADA domains uniquely selected to allow proteins to change size based on concentration



Adapted from Santich et al. Clin Canc Res 2020



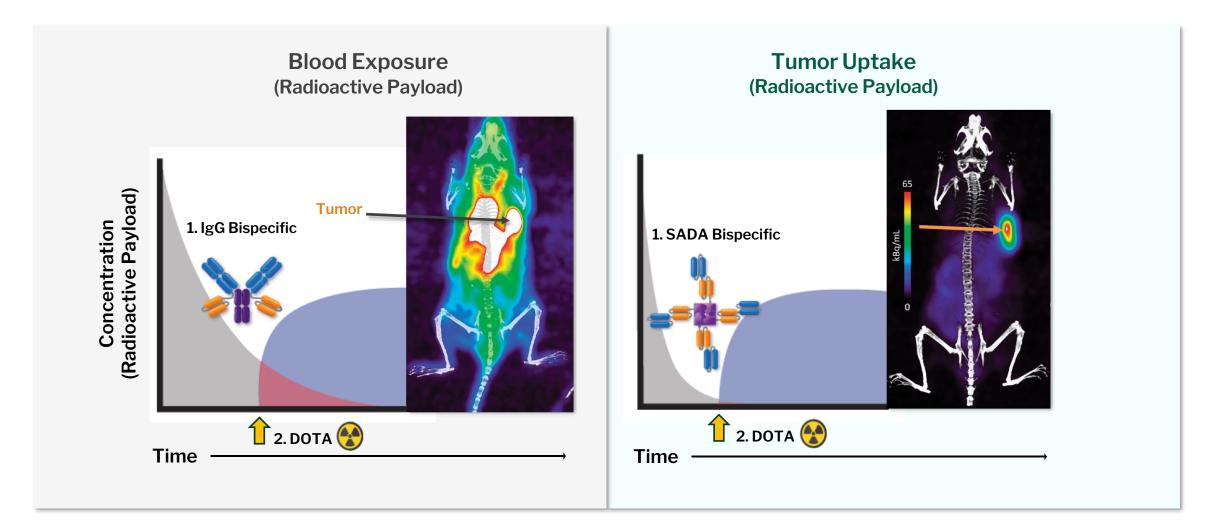
Conventional GD2 Antibody's Persistence in Blood Stream Leads to Substantial Unwanted Exposure and Increased Toxicity



Adapted from Santich et al. Clin Canc Res 2021



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



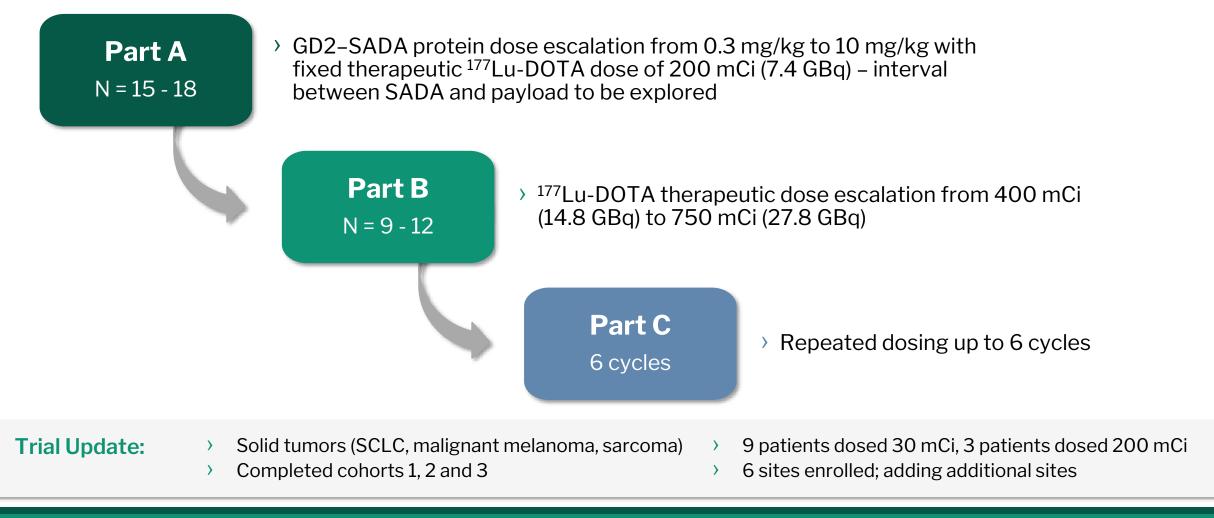
Adapted from Santich et al. Clin Canc Res 2021

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.



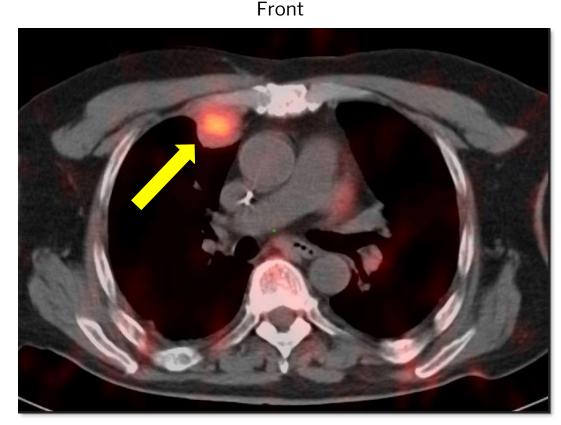
Study 1001: GD2-SADA Ongoing Phase I Clinical Trial – Dosing Patients in Part A

Theragnostic approach using a 30 mCi¹⁷⁷Lu-DOTA imaging dose before exposing to therapeutic dose



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

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



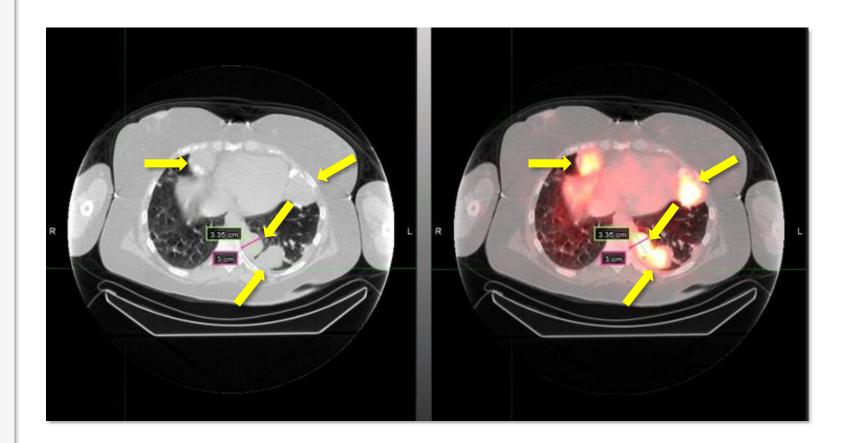


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Study 1001: SPECT/CT Scan on Osteosarcoma Patient Demonstrating Positive Tumor Uptake After Exposure^{*}

- Patient treated with 0.3 mg/kg GD2-SADA, followed by 200 mCi ¹⁷⁷Lu-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 ¹⁷⁷Lu-DOTA SADA (right image)

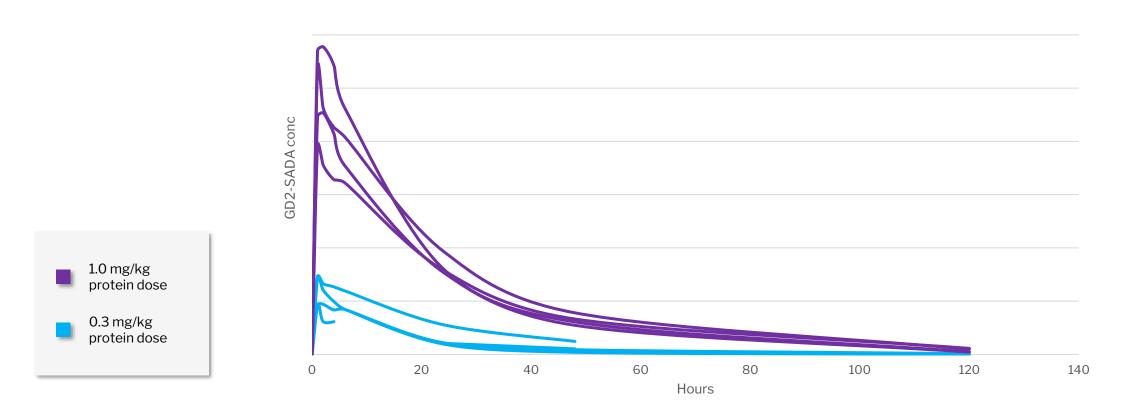


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Study 1001: Ongoing GD2-SADA Phase | Trial – Initial PK Data*

Non-QC data

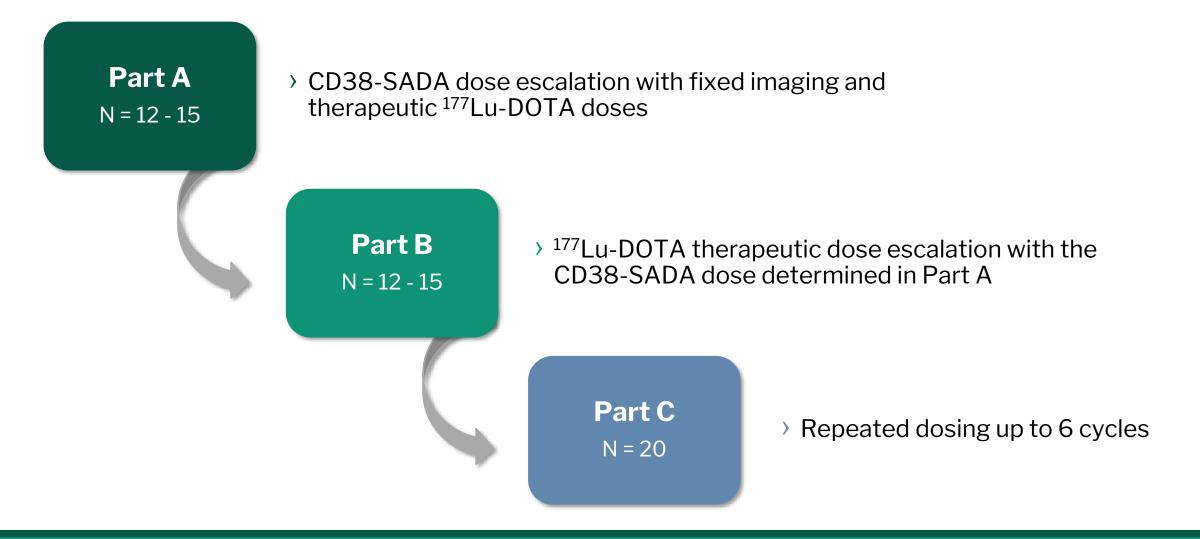


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Study 1201: Planned CD38-SADA Phase I Clinical Trial Design

Theragnostic approach using a ¹⁷⁷Lu-DOTA imaging dose before exposure to a therapeutic ¹⁷⁷Lu-DOTA dose



Novel SADA Platform Potentially Provides Simplicity and Enhanced Precision for Physicians and Patients^{*}



Ongoing GD2-SADA Phase I Trial (Study 1001)

- > Evidence of tumor update
- > No DLTs or pain 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 in patients with R/R non-Hodgkin Lymphoma
- > Trial initiation expected in Q2 2024



- Additional early-stage programs include:
 HER2-SADA
 - > B7H3-SADA



Potential to shift radioimmunotherapy treatment paradigm for patients and physicians with simplicity and enhanced precision of novel SADA platform

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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

- Q3 2023 net sales of **\$20.0** million
- **59 sites** across the U.S. have utilized DANYELZA*
- Ex-U.S. commercial ramp progressing in China; Strong EU demand through WEP
- Additional regulatory approvals in LATAM

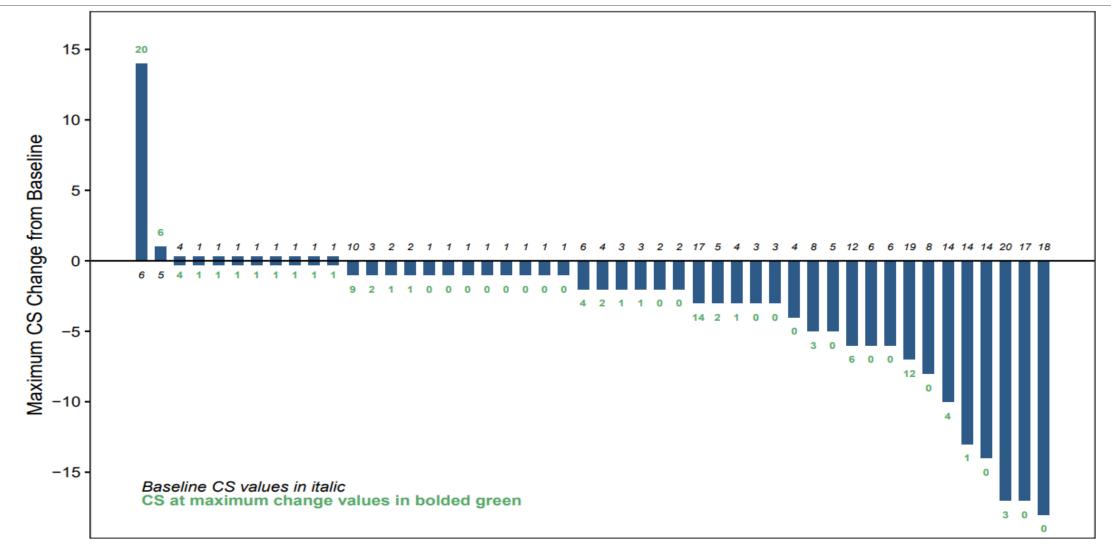


- New DANYELZA campaign rolled out in Q4 2023
- **167 new patient starts** to date since 2021 launch
- Increasing share of U.S. anti-GD2 market with **17%****



* As of January 4, 2024 ** YTD through September 30, 2023

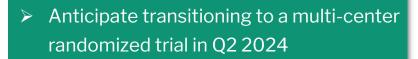
Study 201 Prespecified Interim Analysis: Waterfall Plot of Change in Curie Score in <u>all</u> Relapsed/Refractory Patients with Bone Disease (n = 48)



Ongoing Naxitamab Clinical Trials

Beat Childhood Cancer

- Phase II BCC multi-center trial evaluating naxitamab + standard induction therapy in patients with newly diagnosed High-Risk Neuroblastoma
- 13 sites initiated; target 40-50 sites in U.S. and Canada
- 6 patients dosed; target 76 total patients





- Multi-center Phase II trial investigating naxitamab in patients with relapsed osteosarcoma
- Anticipated data readout from MSKCC in Q4 2024

THE OHIO STATE UNIVERSITY

- ISS Phase Ib/II trial investigating TGFβ NKs, gemcitabile + naxitamab in patients with metastatic breast cancer
- Target enrollment of 42 patients
- Anticipate first patient to be dosed in Q1 2024

- Following data readout from MSKCC, Y-mAbs prepared to initiate pivotal randomized Phase II trial
- Consider multi-center Phase II study based on the results from Phase Ib

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	Relapsed / Refractory	300	~ 99-100%	R/R HRNB Confirmatory Study 201*	
Neuroblastoma	Front-line Induction	450		1st line Induction1st line Induction RCTBCC-018 Phase IIBCC 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 highrisk NB. Launch in China by SciClone; LATAM partner Adium; EU access via WEP



Multiple potential advantages over other GD2 targeting antibody-based 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



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Anti-GD2 Antibody Marketed for R/R High-Risk Neuroblastoma Anticipated 2024 Milestones



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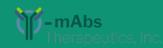


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THANK YOU