

Oncology Leadership in Pretargeted Radioimmunotherapy Platform and Antibody-based Therapies



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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 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 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 Pretargeted Radioimmunotherapy ("SADA PRIT") Platform

**Monoclonal Antibodies** 

Commercial Leverage



DANYELZA (naxitamab-gqgk)

Anti-GD2 Antibody
Marketed for R/R HighRisk Neuroblastoma

FY2024 Net Product Revenue Guidance of \$95-100 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 \$78.6 million\*

Financial Runway into 2027

Responsible Capital Management

\* As of December 31, 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								
<b>Naxitamab-gqgk</b> (Anti-GD2)	201	Relapsed/Refractory High-Risk Neuroblastoma (Pediatric)	DANYELZA (naxitam	nab-gqgk) Confirm	natory Trial	$\checkmark$		U.S. FDA approved
	12-230	Relapsed/Refractory High-Risk Neuroblastoma (Pediatric)	DANYELZA (naxitan	nab-gqgk)			Memorial Sloan Kettering Cancer Center	U.S. FDA approved
	BCC018	Front-Line Induction in High-Risk Neuroblastoma (Pediatric)					Beat Childhood Cancer RESEARCH CONSORTIUM	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					Institute of Mother and Child	Initiated in Q4 2023; Enrollment ongoing
SADA PRIT (Radioimmunotherapy)	1001	GD2-SADA: Solid Tumors (SCLC, Malignant Melanoma, Sarcoma)						Expect to complete Part A in 2H 2024
	1201	CD38-SADA: Non-Hodgkin Lymphoma						Activating first sites in Q2 2024
			•					
Early Programs								
<b>SADA PRIT</b> (Radioimmunotherapy)	1002	GD2-SADA: Neuroblastoma						Expected IND filing in 2024
		HER2-SADA						Expected IND filing in 2025
		B7H3-SADA						Expected IND filing in 2025

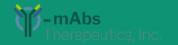




### Current Radiopharma Challenges Negatively Impact Patient Care



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



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

#### **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

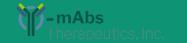


High investment needed for specific infrastructure and manufacturing

## Novel SADA PRIT Platform Potential Capabilities

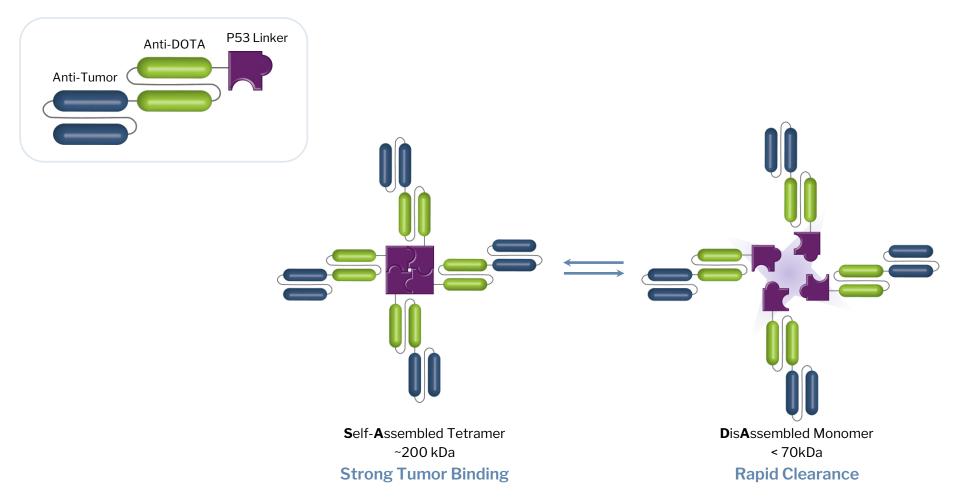
- Pretargeting tumor potentially minimizes toxicity and potentially enhances rapid clearing of unbound protein
- $\checkmark$  Potential to work with short  $T_{1/2}$  isotopes
- ✓ Potentially broader site options with 1<sup>st</sup> protein dose administered by Medical Oncologist at large infusion centers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

<sup>\*</sup> Pending successful development and approval.



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

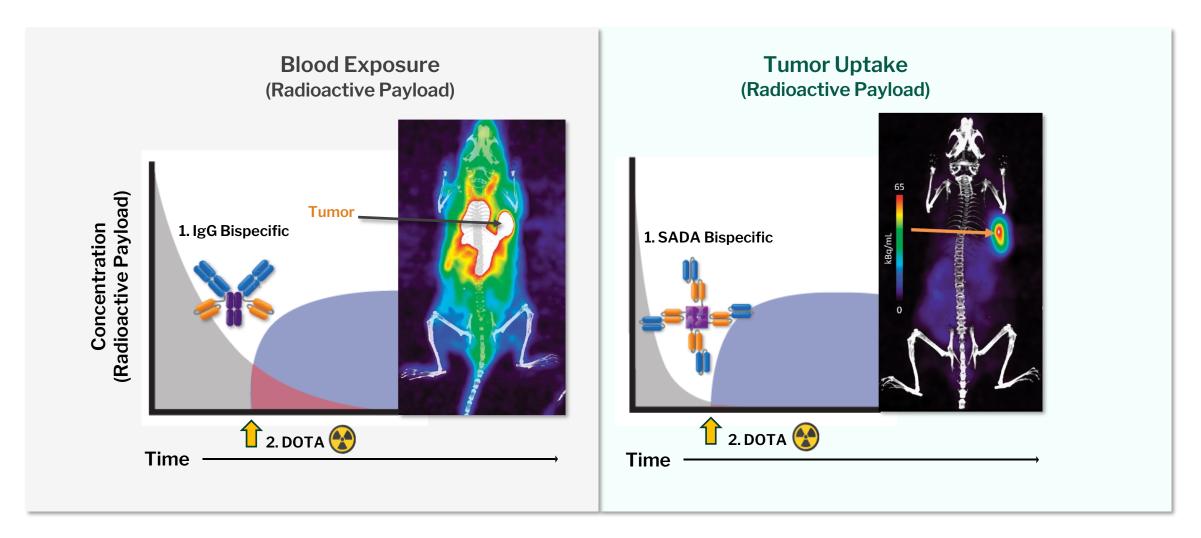
SADA domains uniquely selected to allow proteins to change size based on concentration



Adapted from Santich et al. Clin Canc Res 2020



## 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 Phase I Clinical Trial – Dosing Patients in Part A

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

**Trial Update:** 

- Solid tumors (SCLC, malignant melanoma, sarcomas)
- > Completed Cohorts 1, 2 and 3; currently in Cohort 4
- > 13 patients dosed\*
- > 6 sites open\*; planning to add additional sites

**Part A** N = 15 - 18

GD2–SADA protein dose-escalation from 0.3 mg/kg to 10 mg/kg with a fixed therapeutic <sup>177</sup>Lu-DOTA dose of 200 mCi (7.4 GBq) – the interval between SADA and payload to be explored

**Part B** N = 9 - 12

<sup>177</sup>Lu-DOTA therapeutic dose escalation from 400 mCi (14.8 GBq) to 750 mCi (27.8 GBq). Patients to receive up to 2 cycles

Part C 5 cycles

Repeated dosing up to 5 cycles

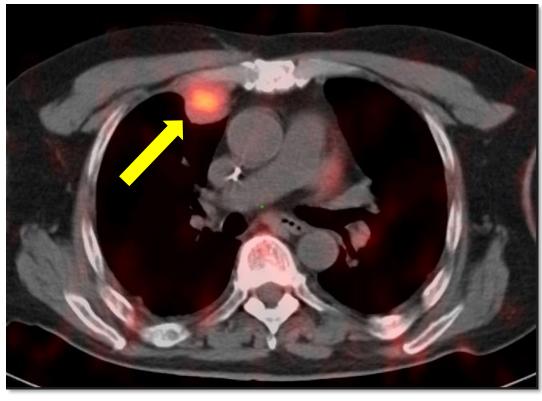
\* As of March 1, 2024



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

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

Front



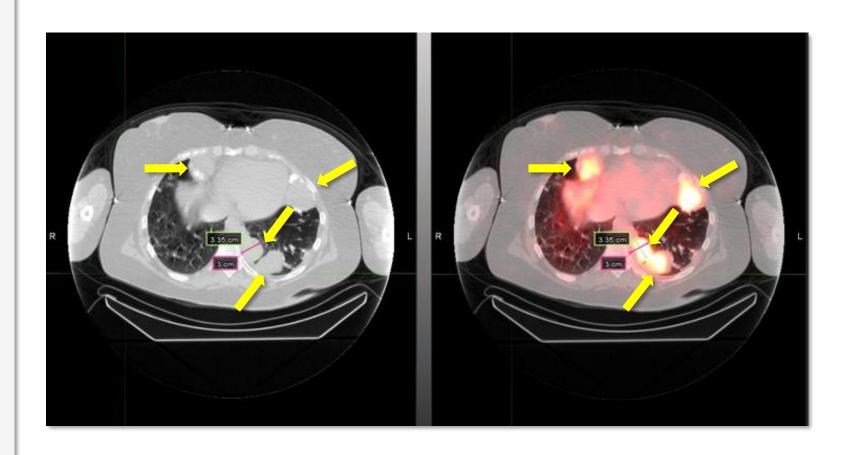
Back

\*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 <sup>177</sup>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 <sup>177</sup>Lu-DOTA SADA (right image)

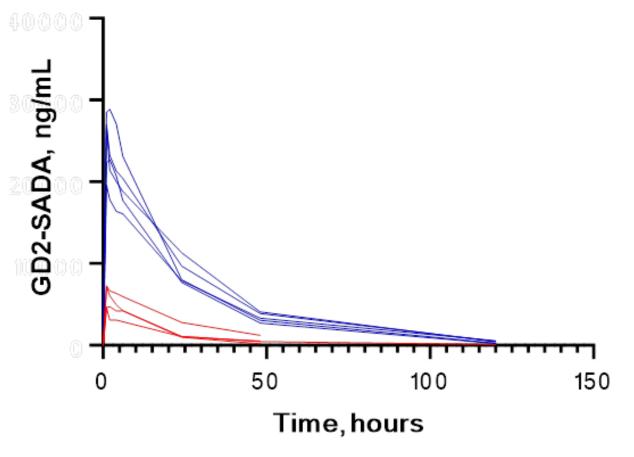


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## Study 1001: Ongoing GD2-SADA Phase I Trial – Initial PK Data\*

#### **GD2-SADA** Concentration vs. Time Profiles



— Dose 1.0 mg/kg (n=5)

\_\_\_ Dose 0.3 mg/kg (n=4)

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

**Trial Update:** 

> IND approved by U.S. FDA in Q4 2023

Anticipate activating first sites in Q2 2024

**Part A** N = 12 - 15

CD38-SADA dose-escalation with fixed imaging and therapeutic <sup>177</sup>Lu-DOTA doses



<sup>177</sup>Lu-DOTA therapeutic dose escalation with the CD38-SADA dose determined in Part A



# 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 update
- 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 activating first sites in Q2 2024



- GD2-SADA-Neuroblastoma IND filing anticipated in 2024
- Additional IND filings anticipated in 2025:
  - HER2-SADA
  - > B7H3-SADA



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

<sup>\*</sup>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







### 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
- 58 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



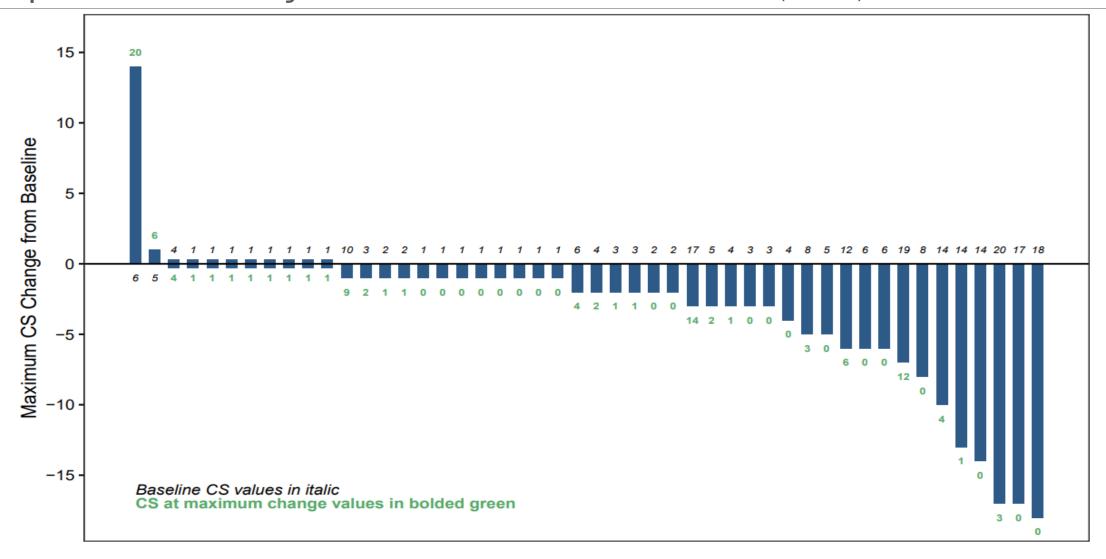
#### Solid Drivers of Market Uptake

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



\* As of December 31, 2023

## Pivotal Study 201 Data: 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 RESEARCH CONSORTIUM

- Phase II BCC multicenter trial evaluating naxitamab + standard induction therapy in patients with newly diagnosed HR NB
- 13 sites initiated to date; target 40-50 sites in U.S. and Canada
- 7 patients dosed to date; target 76 total patients



#### Memorial Sloan Kettering Cancer Center

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



#### THE OHIO STATE UNIVERSITY

COMPREHENSIVE CANCER CENTER

- ISS Phase Ib/II trial investigating TGFβ NKs, gemcitabine + naxitamab in patients with metastatic breast cancer
- Target enrollment of 42 patients
- Anticipate first patient to be dosed in 1H 2024



## Institute of Mother and Child

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



Prepared to initiate pivotal randomized trial in Q2 2025 following data readout from MSK

Consider multi-center Phase II study based on the results from Phase Ib

Anticipated study completion in 2028

Anticipate transitioning to a multicenter randomized trial in Q2 2024

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	33 13373	1 <sup>st</sup> line Induction BCC-018 Phase II BCC study			
<b>Osteosarcoma</b> Relapsed/Recurrent		200	~ 88%	Relapsed Osteosarcoma MSKCC Study 15-096 Pivotal RCT**			
Soft-Tissue Sarcomas Including Ewings		2,900 (1 <sup>st</sup> -line population)	> 90%	ISS – Ongoing Phase II (Ewings)			
<b>Breast Cancer</b> Triple Negative / Advanced		8,900 (2 <sup>nd</sup> line & 3 <sup>rd</sup> line +)	> 50%	ISS – Ongoing Phase Ib/II			
<b>Melanoma</b> Newly Unresectable and Metastatic		11,400 (2 <sup>nd</sup> line & 3 <sup>rd</sup> line +)	> 50%	ISS – Area of Interest			

<sup>\*</sup> 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).

<sup>\*\*</sup> 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

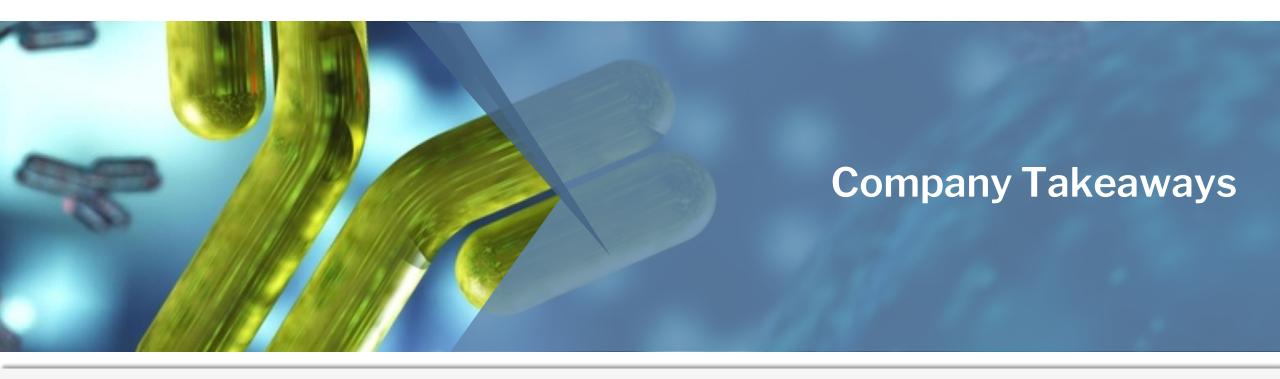


- U.S. commercialization in highrisk NB
- > Expanding ex-U.S. reach
  - Launch in China by
     SciClone; LATAM partner
     Adium; EU access via WEP



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





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<sup>\*</sup> Anticipate presenting data from more mature dataset in Part A of GD2-SADA Phase 1 trial in 2H 2O24



<sup>\*</sup> As of December 31, 2023

