



Company Presentation

R&D Day December 11, 2019



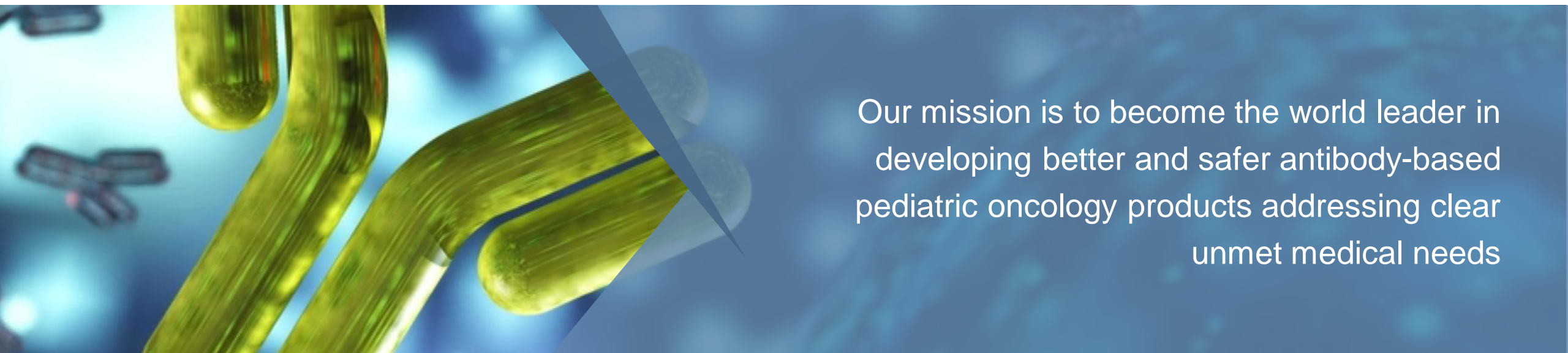
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MISSION



Our mission is to become the world leader in developing better and safer antibody-based pediatric oncology products addressing clear unmet medical needs

Investment Highlights

Two pivotal-stage candidates – naxitamab and omburtamab – with BT¹

Rolling BLA submission for naxitamab initiated Nov 2019, to be completed Q1 2020
Complete BLA for omburtamab expected by end of Q1 2020

Potential to expand into other indications and lines of therapy – studies ongoing

First BsAb product candidate in Phase 1/2

Financial strength – secured financing through the end of 2022

¹BT¹ – Breakthrough Therapy Designation

²BLA – Biologics License Application

Strong Clinical Pipeline

Programs	Phase 1	Phase 2/Pivotal Study	Next Anticipated Milestones
Lead Development Candidates	Naxitamab (GD2)		Rolling BLA submission initiated November 2019
	Omburtamab (B7-H3)		BLA submission to be completed end Q1 2020
Vaccine	GD2-GD3 Vaccine		Ongoing Phase 2 study at MSK
Bispecific/ Early Stage	GD2xCD3 - BsAb		In Phase 1/2 study since Q1 2019
	Omburtamab-DTPA		Expect to file IND by the end of 2019

Lead Development Programs Approaching Registration and Commercialization

Compound	Indication	Total Incidence per Year (US)	Addressable Patient Population per Year (US)
GD2 naxitamab	Neuroblastoma – 2 nd Line	300	300
	Neuroblastoma – Front Line	800	450
	Osteosarcoma – 2 nd Line	450	200
B7-H3 omburtamab	Neuroblastoma Metastatic to the Central Nervous System (CNS/LM from NB)	80	80
	Diffuse Intrinsic Pontine Glioma (DIPG)	300	300
	Desmoplastic Small Round Cell Tumors (DSRCT)	100	100



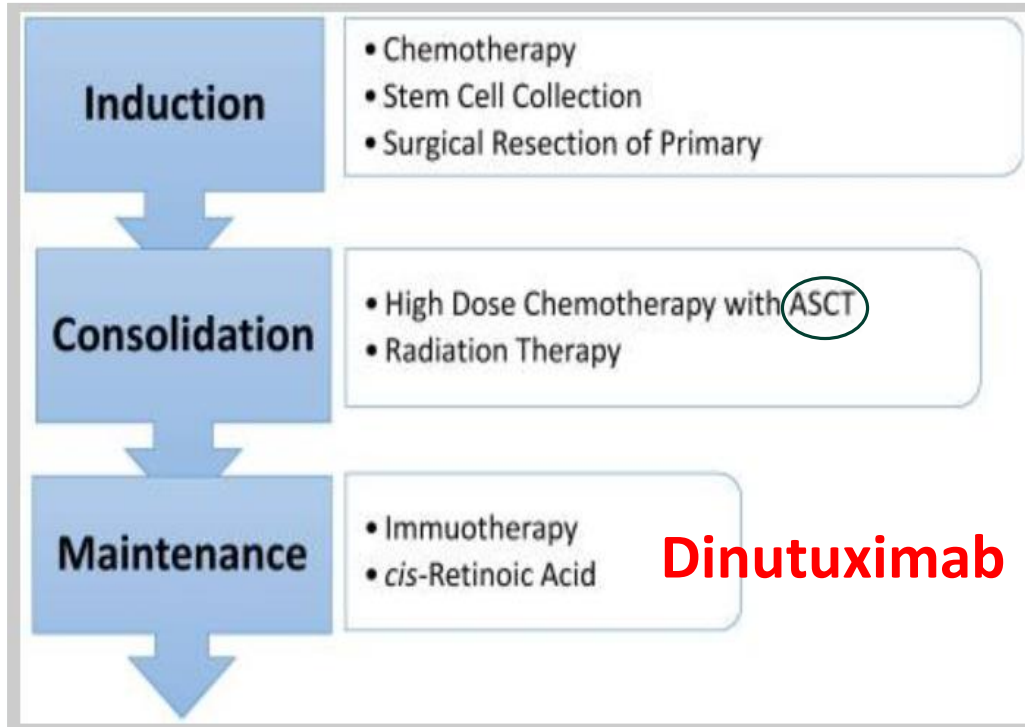
Naxitamab: Anti-GD2 Antibody
Neuroblastoma and Osteosarcoma

Naxitamab Targets GD2 with Expanding Clinical Program

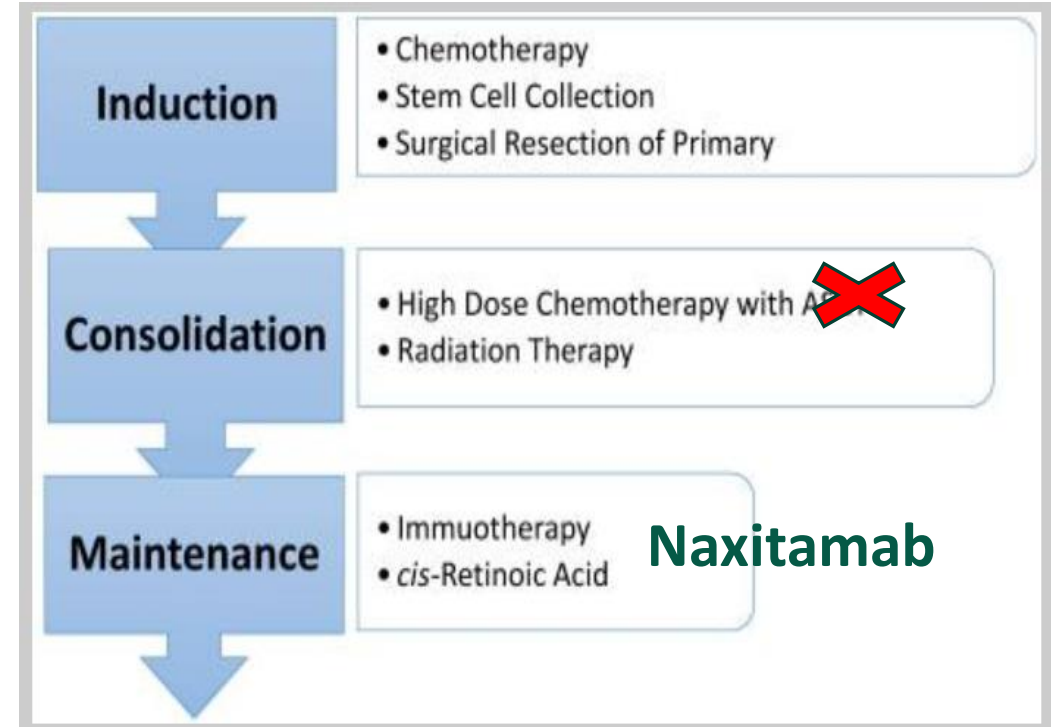
Naxitamab (GD2)	Phase 1	Phase 2/Pivotal Study	Highlights
Accelerated Pathway		Phase 2: Primary R/R High-Risk NB (Pediatric) – Study 201	Multi-center pivotal study per FDA; rolling BLA submission commenced November 2019
			Single-center study – part of rolling BLA pivotal data package
Expanding to Frontline		Phase 2: Frontline High-Risk NB (Pediatric) – Study 16-1643	Ongoing Phase 2 study
		Phase 2: Front-line naxitamab - Study 202	Frontline Phase 2 study to initiate in 2020
Label Expansion		Phase 2: Chemoimmunotherapy for R/R High-Risk NB – Study 17-251	Heavily pre-treated, high-risk NB patients
		Phase 2: Relapsed Second-line Osteosarcoma – Study 15-096	If successful, may form part of support for future sBLA in Osteosarcoma
		Phase 2: Combo naxitamab plus chemo – Study 203	Combo Phase 2 to initiate in 2020

High Risk Neuroblastoma Treatment Recommendation – COG and MSK/Y-mAbs

COG – 8-20 h infusion (x4 per week)



MSK/Y-mAbs – app 30 min infusion (x3 per week)



Naxitamab: Key Takeaways

Addresses Significant Unmet Needs in R/R High-Risk NB; Potential to Expand to Broader Populations

Multiple potential advantages over other GD2 targeting antibody-based therapies: modest toxicity, shorter infusion time, ability to be administered in outpatient setting

Naxitamab has been granted ODD, BTD, and RPDD¹

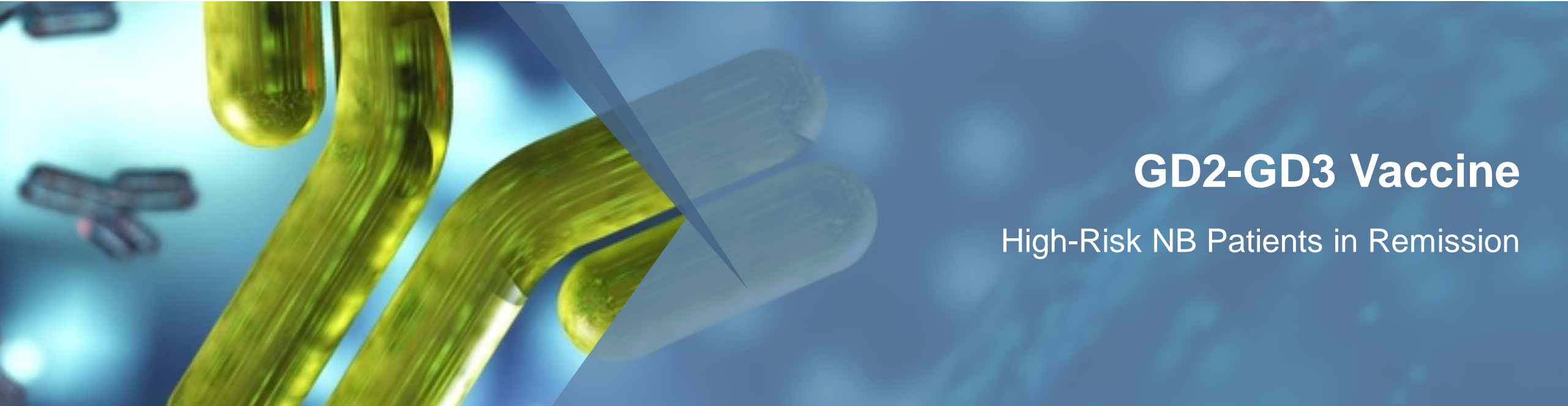
Study 12-230 and Study 201 forming primary basis of rolling BLA submission including the first 24 patients from study 201

Potential to expand application to the treatment of adults with cancers that express GD2

Rolling BLA submission initiated in November 2019

US commercialization being planned

¹Indicates eligibility for a Priority Review Voucher (PRV) on approval



GD2-GD3 Vaccine

High-Risk NB Patients in Remission

GD2-GD3 Vaccine Update – A Naxitamab Add-On

Ongoing Phase 2 Study at MSK; Phase 1 Study Published in 2014; First Phase 2 Study Data Published May 2018 at ANR



More than 230 patients on study drug – ODD granted



84 high-risk NB patients received the GD2-GD3 Vaccine, all of whom were in second or later remission



PFS of approximately 51% and OS of approximately 90% at two years



Study now also enrolling patients in first remission

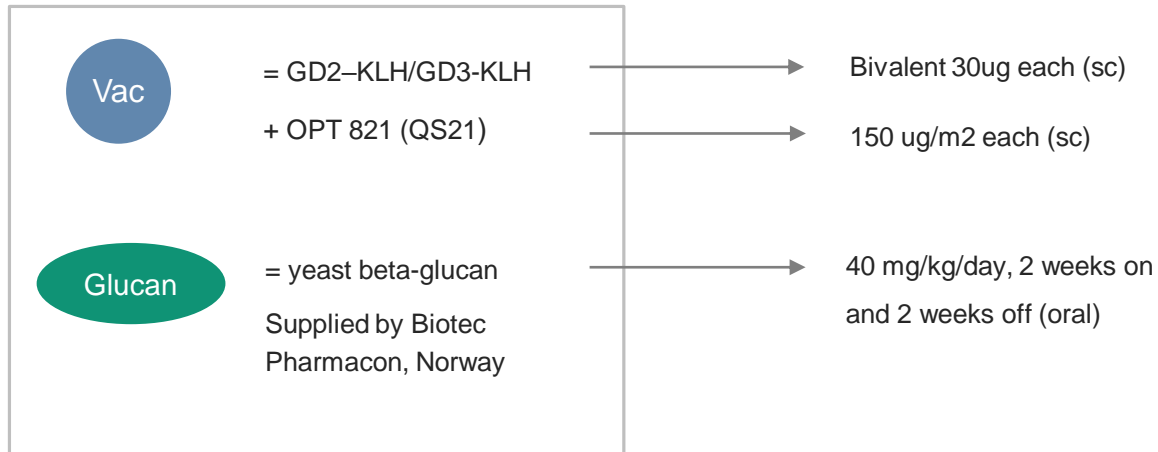
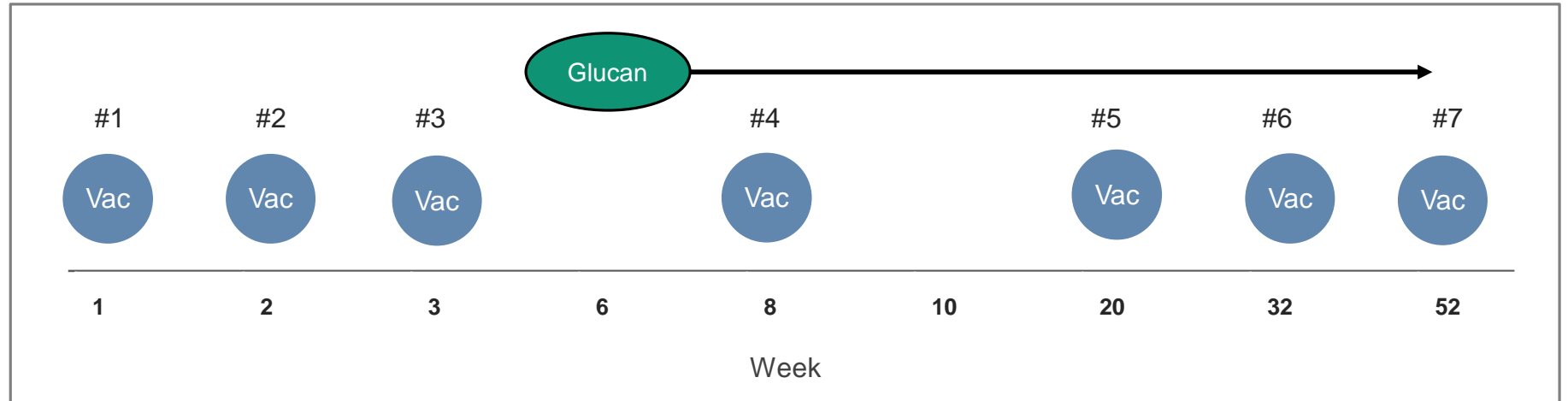


The GD2-GD3 Vaccine appears to be well tolerated, with no reported grade 3 or grade 4 toxicities

Phase 2 Vaccine Study at Memorial Sloan Kettering

Clinicaltrials.gov
NCT00911560

7 cycles

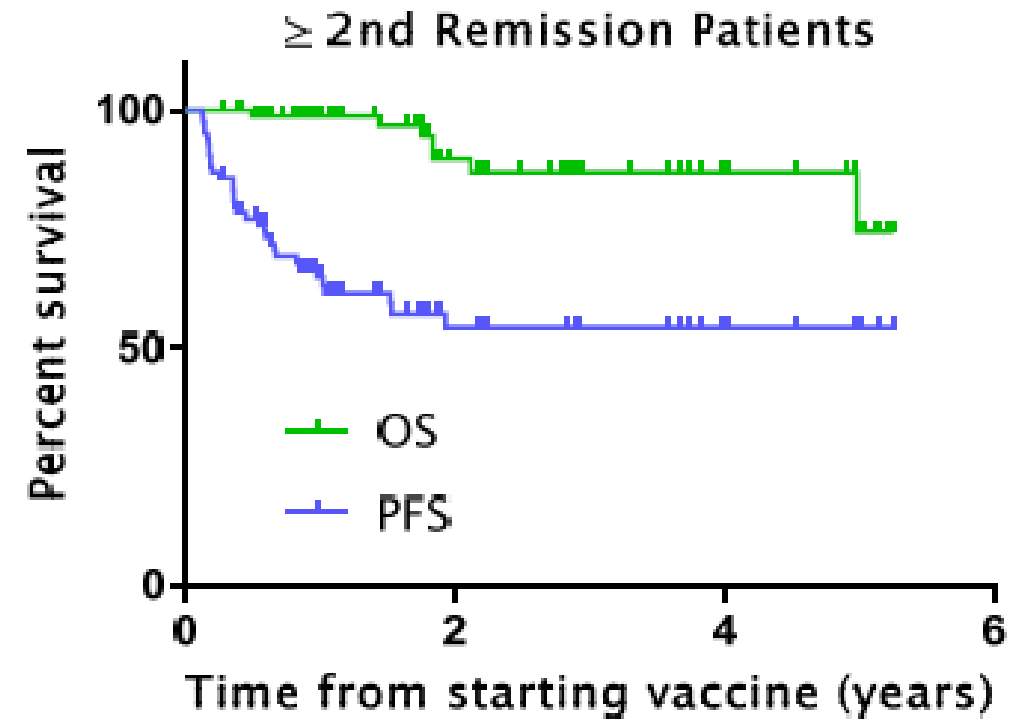


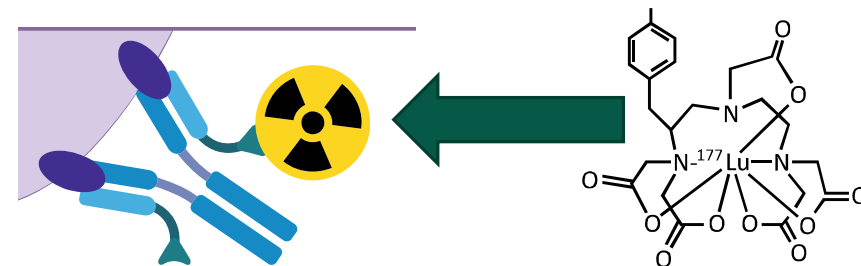
Seroconversion = antibody response		
% patients with positive		
	Anti-GD2 titer	Anti-GD3 titer
Pre-vaccine	13.3%	29.4%
During vaccine/follow-up	82.7%	70.4%

I. Cheung et al., Phase II Trial of GD2-KLH/GD3-KLH Vaccine for Stage 4 Neuroblastoma in 2nd or later Remission ANR, San Francisco, May 2018

Focus on 2nd and Later Remission Group

Y-mAbs vaccine multi-center
Trial 601 – NB patients 2nd CR





^{177}Lu -omburtamab-DTPA: B7-H3

Targeting B7-H3 Positive Solid Tumors

^{177}Lu -Omburtamab-DTPA Pediatric and Adult Strategy

Pediatric

- First indication: **Medulloblastoma**
- Prior experience from compartmental treatment with ^{131}I -omburtamab – 27 pediatric patients treated

Adult

- First indication: **Basket Trial** of B7-H3 positive CNS/LM tumors
- Prior experience from compartmental treatment of adult patients with ^{131}I -omburtamab

Clinical Testing (Adult)

- Experience using ^{131}I -omburtamab in 41 patients with tumors such as sarcoma, melanoma, and medulloblastoma
- Animal toxicity studies of **omburtamab-DTPA** completed on GLP material
- cGMP production established
- Expect to file an IND for treatment of B7-H3 positive LM from solid tumors by the end of 2019

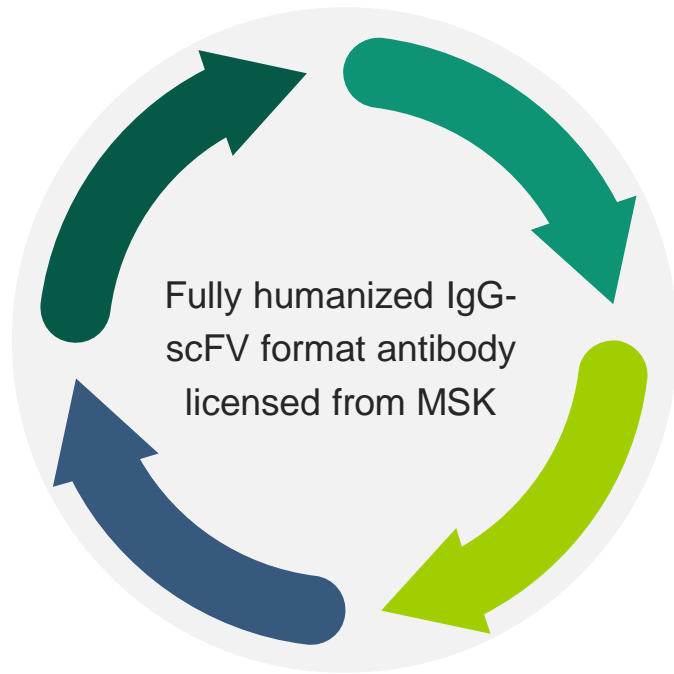


Bispecific Antibodies

First Two Antibodies Targeting GD2 and CD33 Positive
Cancers

Bispecific GD2 Antibody Candidate

Currently in Phase 1/2 Clinical Development



Phase 1/2 clinical study initiated. Recruiting patients with:

R/R NB

High grade osteosarcoma

Other GD2(+) solid tumors, where patients have relapsed or refractory disease that is resistant to standard therapy

30 patients across two cohorts (R/R NB, R/R osteosarcoma)

Y-mAbs
Trial 402 – SCLC

Phase II SCLC IND submission Q4-2020

MSK legacy study
18-034 GD2+ tumors
Cohort 6 ready

Phase II 3rd line NB
Phase II refractory Osteosarcoma



Omburtamab - B7-H3

CNS/LM from NB, DIPG and DSRCT

Omburtamab Clinical Platform

Omburtamab B7-H3	Phase 1	Phase 2/Pivotal Study	Highlights
Accelerated Pathway	Phase 2: CNS/LM from NB (Pediatric) – Study 101		Multi-center PK study; BLA submission by Q1 2020
	Phase 1: CNS/LM – Study 03-133		MSK single-center efficacy data
Label Expansion	Phase 2: DIPG multi-center – Study 102		Multi-center study to initiate in 2020
	Phase 1: DIPG – Study 11-011		Study update presented at ASCO 2019
	Phase 2: DSRCT – Study 19-182		Study update from Phase 1 to be presented at CTOS in November 2019

Omburtamab Regulatory Path to BLA Approval

Regulatory

Studies 03-133 and 101 to form basis of BLA submission:
OS data accepted by FDA for accelerated approval
PK and dosimetry comparison required

Data from first 18 patients (Study 101) could support BLA submission

Qualifies for accelerated approval

BLA submission planned to be complete by end of Q1 2020;
PDUFA date expected in October 2020

ODD, BTD, and RPDD¹

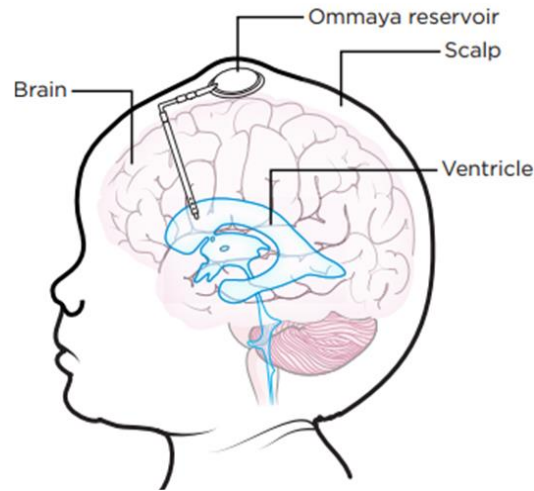


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Omburtamab: Delivered in an Outpatient Setting – 2 doses per Patient

CNS/LM from NB patients

Administration of radiolabeled omburtamab via Ommaya reservoir



Omburtamab being delivered in an outpatient setting



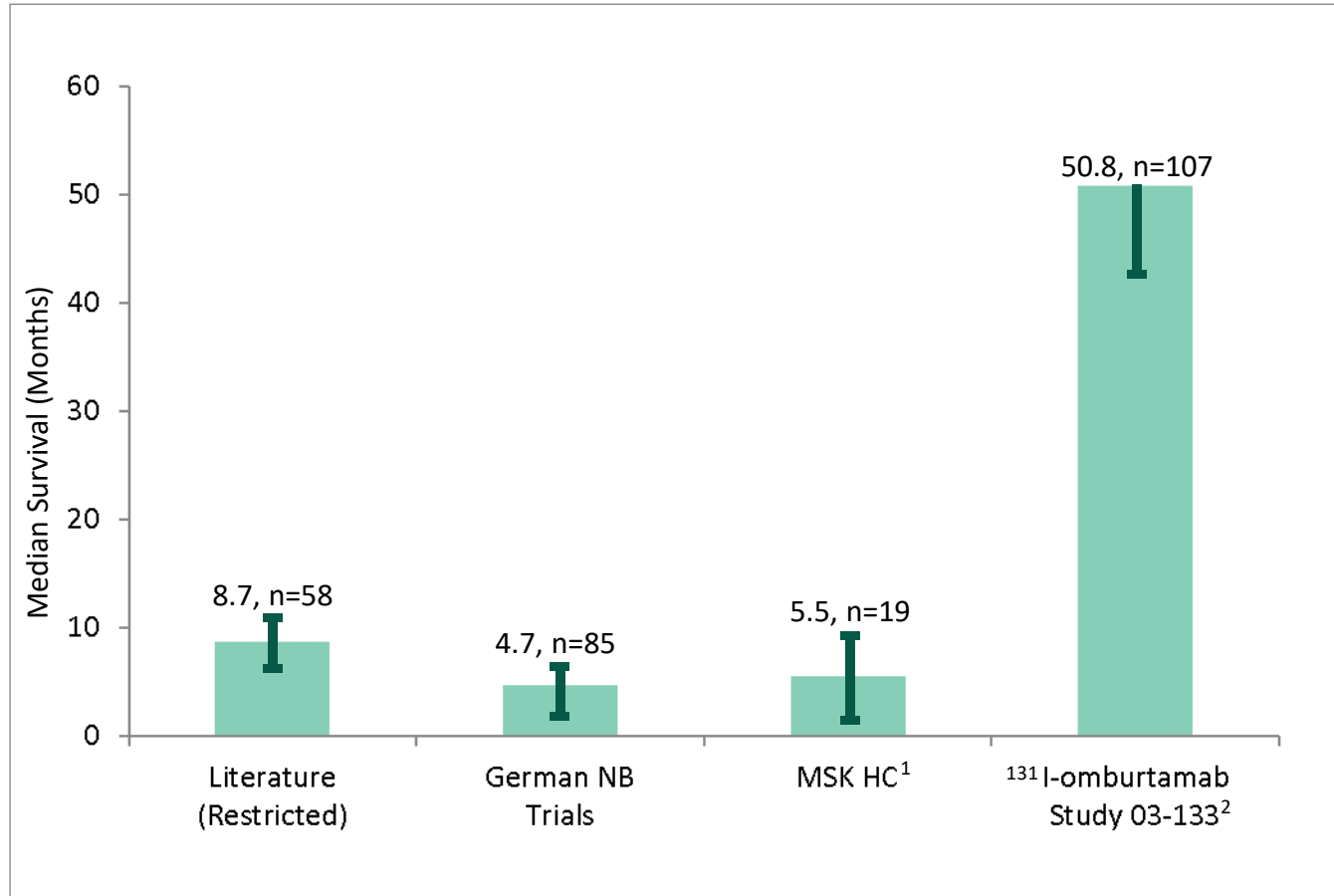
PET scan of distribution of radiolabeled omburtamab two hours after administration



After induction treatment including all or some of the three treatments (chemotherapy, surgery, and radiation) patients will receive radiolabeled omburtamab

Omburtamab: Clinical Overview

Study 03-133: ¹³¹I-omburtamab Improves Survival in CNS/LM from NB Patients



These results demonstrate the opportunity for ¹³¹I-omburtamab to address the lack of an established, effective therapy for patients with CNS/LM from NB

¹MSK HC = neuroblastoma patients with CNS/LM treated at MSK prior to 2003

²¹³¹I-omburtamab = Patients with CNS/LM treated under Study 03-133

Omburtamab: Key Takeaways

Addresses Significant Unmet Needs and has the Potential to Expand its Application to Broader Populations

No approved products for patients with R/R NB who have CNS/LM from NB or, as widely accepted; no effective treatment regimens; goal of treatment is generally palliative

Demonstrated median OS of 51 months (including an estimated five-year OS of ~44%), as compared to historical median OS of ~six months and no expected five-year survival

Granted ODD, BTD, and RPDD¹

We believe there is a large market opportunity for the treatment of LM from tumors that express B7-H3

Study 03-133 together with Study 101 to form primary basis for BLA submission

BLA for CNS/LM from NB expected to be submitted in Q1 2020. May qualify for a sBLA for DIPG and DSRCT assuming positive pivotal data

¹Indicates eligibility for a Priority Review Voucher (PRV) on approval



Financial Summary

Strong Financial Position with Blue Chip Investors

Y-mAbs Has Completed a Series of Successful Financing Rounds, with \$374 Million Raised to Date



IPO – September 2018

\$110 Million

Follow on: November 2019

\$144 Million



\$374 Million

Raised to Date

\$233 Million

of cash and cash equivalents pro forma (cash balance as of September 30, 2019 and net proceeds from follow-on offering)

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The background is a microscopic scene. On the left, a large, textured, blue-green spherical structure, possibly a cell or virus, is partially visible. Scattered throughout the scene are various rod-shaped bacteria. Some are bright green and appear to be in motion, while others are darker, reddish-brown. The overall lighting is a mix of cool blues and greens, creating a scientific and dynamic atmosphere.

THANK YOU