



Magenta Therapeutics Announces Positive Preliminary Results from Phase 2 Clinical Trial of MGTA-145 in Multiple Myeloma

May 12, 2021

(NASDAQ:MGTA)



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Agenda & Introductions

INTRODUCTIONS

PIPELINE OVERVIEW

MGTA-145 MULTIPLE MYELOMA PHASE 2: BACKGROUND, RESULTS AND NEXT STEPS

Q&A SESSION

CLOSING REMARKS



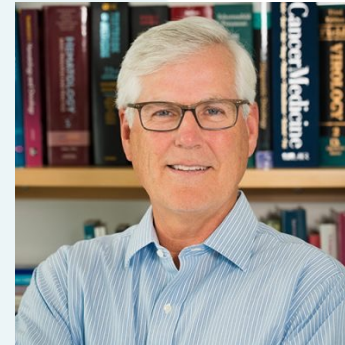
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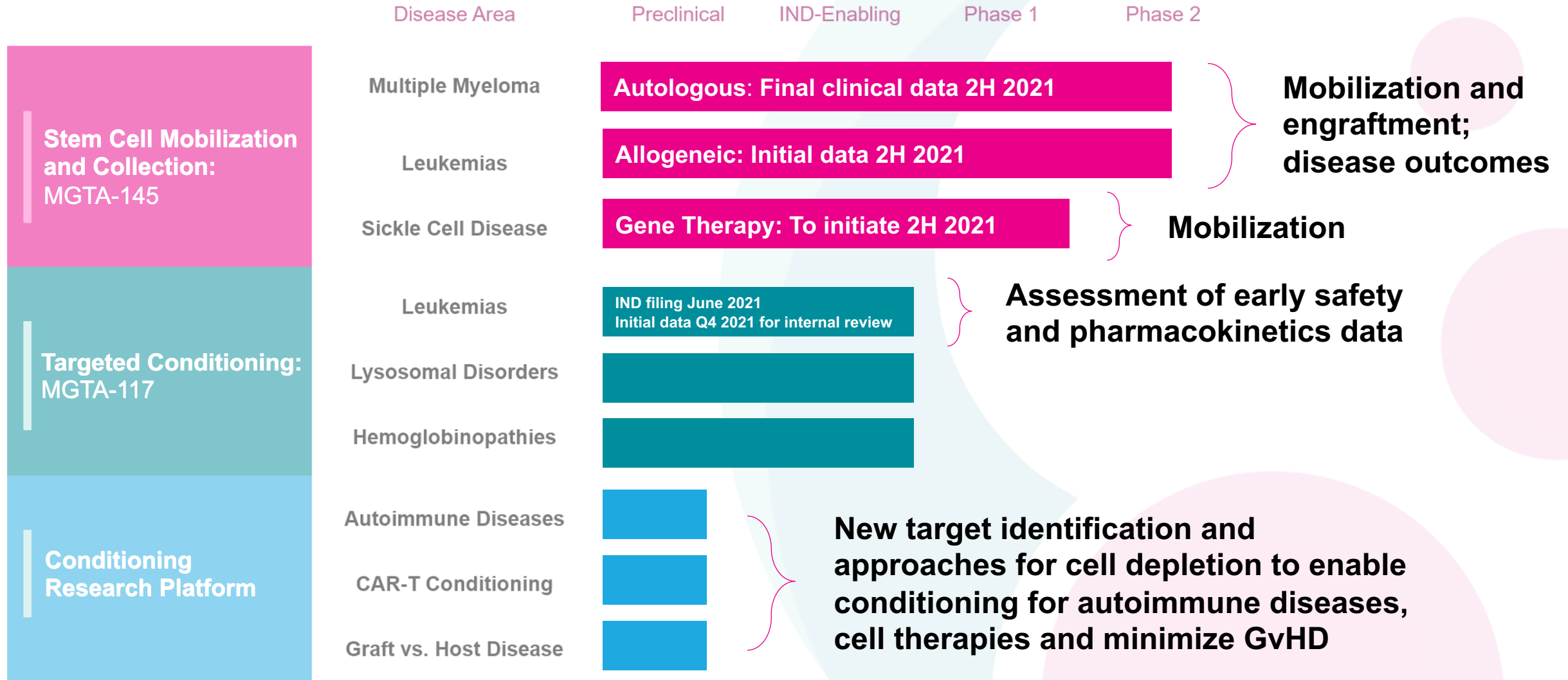


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The Magenta Pipeline

	Disease Area	Preclinical	IND-Enabling	Phase 1	Phase 2	Clinical Trial Partners	Product Rights
Stem Cell Mobilization and Collection: MGTA-145	Multiple Myeloma	Autologous Transplant				Stanford University	magenta THERAPEUTICS
	Leukemias	Allogeneic Transplant				BE THE MATCH®	
	Sickle Cell Disease	Gene Therapy				bluebirdbio®	
Targeted Conditioning: MGTA-117	Leukemias	Allogeneic Transplant					
	Lysosomal Disorders	Gene Therapy				AVROBIO	
	Hemoglobinopathies	Gene Therapy				Beam THERAPEUTICS	
Conditioning Research Platform	Autoimmune Diseases						
	CAR-T Conditioning						
	Graft vs. Host Disease						

Magenta 2021: Deliver Clinical Data and Advance Platform



MGTA-145 with Plerixafor: Complementary Mechanisms of Action for Mobilization & Collection of Stem Cells for Transplant

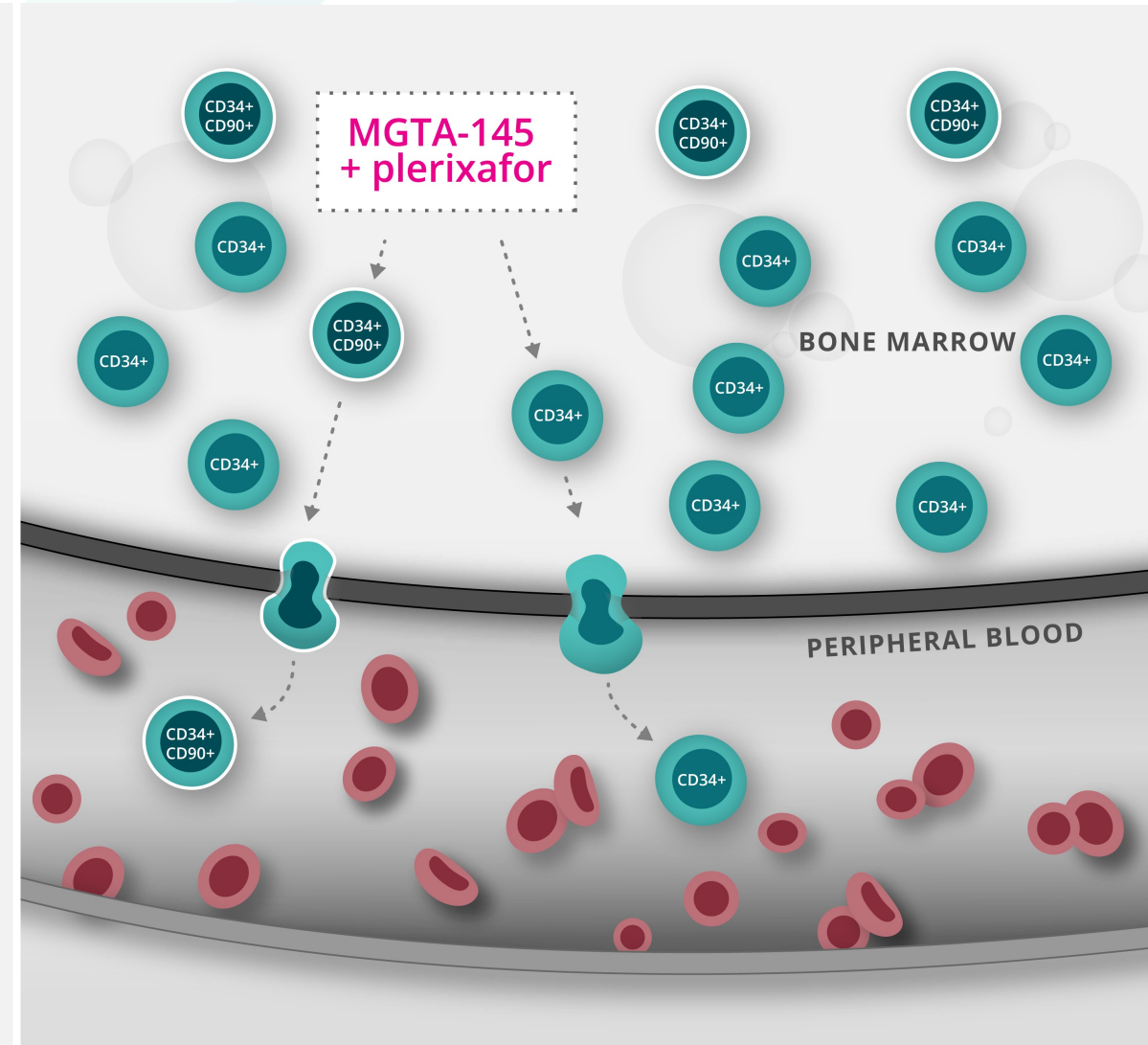
Two agents mobilize hematopoietic stem cells (HSCs) through complementary mechanisms of action

MGTA-145 (GroßT)
CXCR2 agonist
Protein

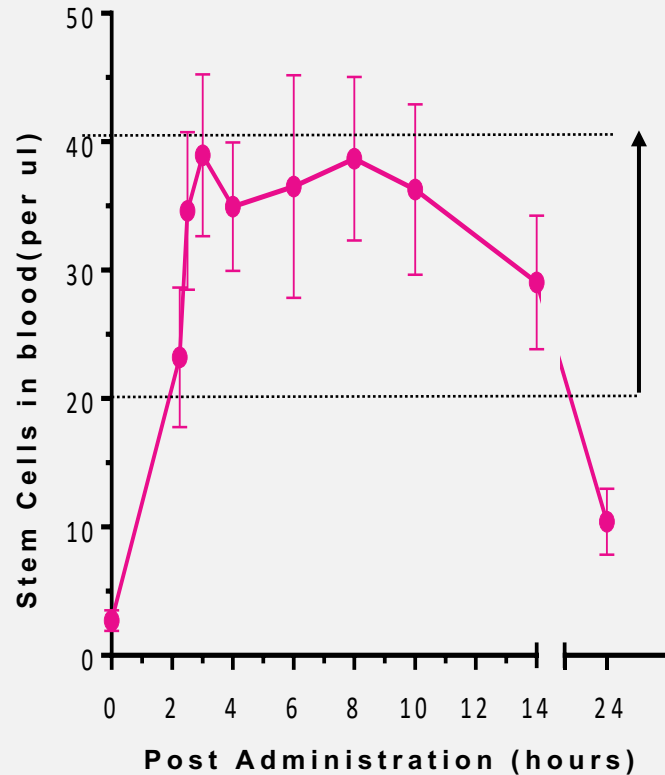
+

plerixafor
CXCR4 antagonist
Small molecule

- MGTA-145 binds to CXCR2 on neutrophils which triggers release of factors that stimulate HSCs to easily migrate out of their niche in the bone marrow
- Plerixafor disrupts CXCR4, which anchors HSCs to bone marrow stromal cells
- Together, these actions release large numbers of functional HSCs (CD34+CD90+ cells) from the bone marrow niche into circulation, where they can be collected for HSC transplant



MGTA-145 Phase 1 Clinical Results Demonstrated Rapid, Reliable, Predictable and Well-Tolerated Mobilization of Functional Stem Cells



Double the target number of stem cells mobilized in hours (compared to 5 days minimum with G-CSF or G-CSF + plerixafor)

Mobilized large numbers of stem cells in hours¹

Grade 2-4 side effects

MGTA-145

1%

VS.

G-CSF²

38%

Well-tolerated¹

Speed and predictability

MGTA-145 +
plerixafor¹

88%

(7 of 8 patients)

VS.

G-CSF or
G-CSF + plerixafor

0%

**Reliable, same-day dosing,
mobilization and collection**

MGTA-145 Phase 2 Clinical Trials: Ongoing and Planned Proof-of-Concept Across Multiple Disease Areas

2021 Anticipated Milestones

Key Endpoints

Safety & Tolerability

Collection Yield

Engraftment (Rate and Durability)

Disease Outcomes

Multiple Myeloma (autologous)



May 2021: Preliminary results

2H 2021: Final clinical data expected



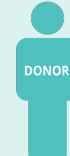
Remission and survival

Leukemias (allogeneic – healthy donors)



Q1 2021: Trial started

2H 2021: Initial data expected



*De-risked from Phase I**



*De-risked from Phase I**



Remission and survival; GvHD

Sickle Cell Disease (gene therapy)



2H 2021: Trial initiation



MGTA-145 Multiple Myeloma Clinical Trial Design

Purpose

Evaluate the safety and efficacy of MGTA-145 in combination with plerixafor, for the mobilization of hematopoietic stem cells for autologous transplantation in patients with multiple myeloma

Design

25 patients, open label, single arm study, Stanford University

Eligibility criteria

Patients aged 18-70 with multiple myeloma (MM), including patients with multiple risk factors, eligible for transplant per institutional guidelines and within one year of start of myeloma therapy

Endpoints

Primary Endpoint: Collect $\geq 2\text{M}$ CD34+ cells/kg in up to two days of apheresis
[threshold number of cells for transplant]

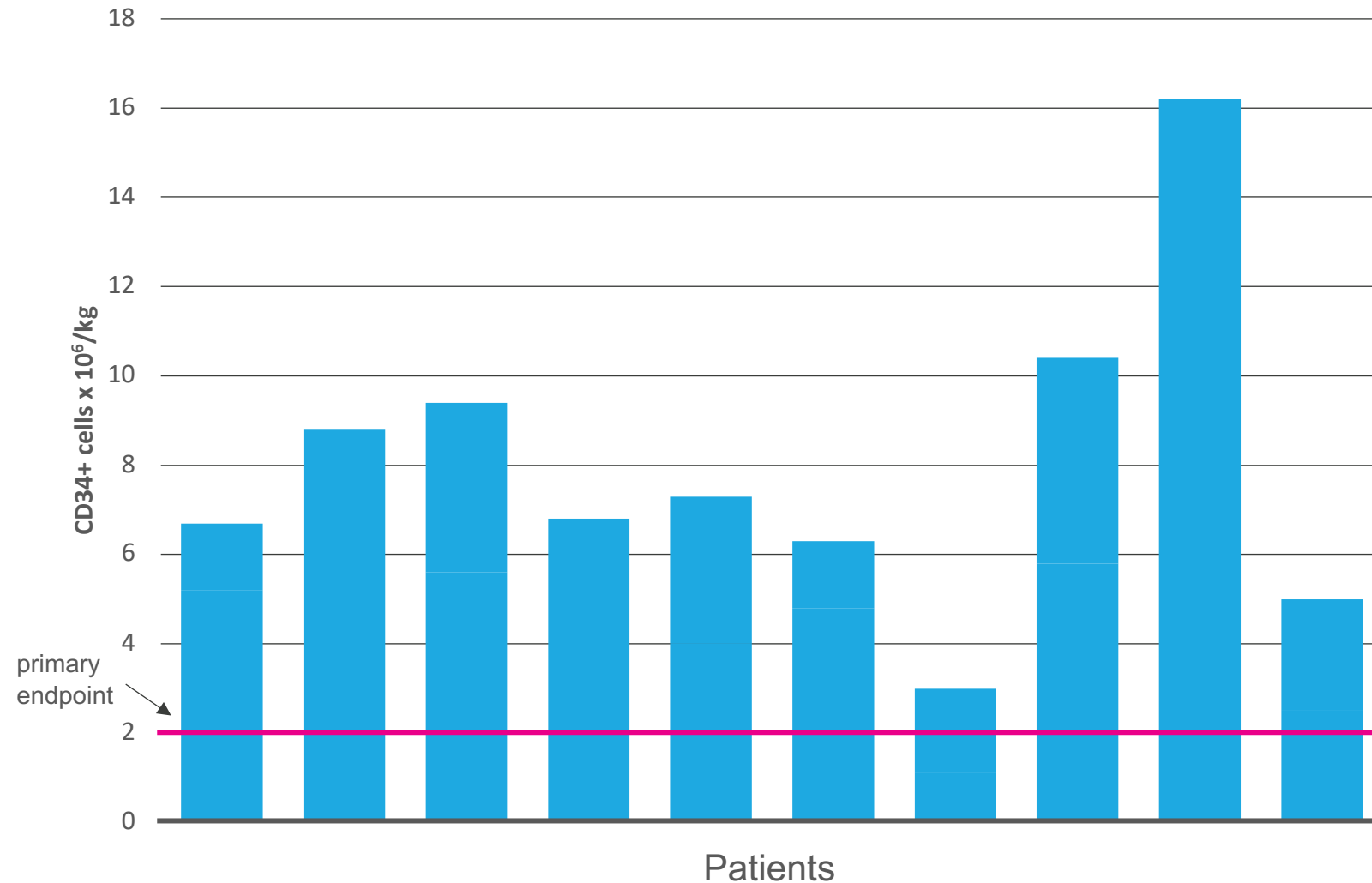
Key Secondary Endpoints:

- (a) Collect $\geq 4\text{M}$ CD34+ cells/kg in up to two apheresis sessions [target number of cells for transplant]
- (b) Collect $\geq 6\text{M}$ CD34+ cells/kg in up to two apheresis sessions
- (c) Time to neutrophil and platelet engraftment
- (d) Assess engraftment at 30 and 100 days

Note: Protocol requires second day of mobilization for collection of 6M CD34+ cells/kg for potential second transplant (cells beyond 4M CD34+ cells/kg are frozen)

Preliminary Data Show All Patients (10/10) Met the Primary Endpoint of 2M CD34⁺ Stem Cells/kg in Up to Two Days of Mobilization and Collection

Stem Cell Collection (over 2 days*)



- All patients (10/10) met the primary endpoint of collection of 2M CD34⁺ cells/kg in up to two days of same-day mobilization and apheresis, 90% of patients achieved this endpoint in one day.
- Median number of stem cells collected in one day was 5.4M CD34⁺ cells/kg. Including patients who had two days of apheresis, the median number of stem cells collected was 7.1M CD34⁺ cells/kg.
- Study criteria allowed for a broad clinically representative patient population including patients with multiple risk factors for poor mobilization.
- MGTA-145 + plerixafor regimen was well-tolerated, with transient, drug-related Grade 1 bone or musculoskeletal pain observed in 40% of patients.

All Transplanted Patients Have Successfully Engrafted with Enriched Collection of Long-Term Engrafting Stem Cells and Low Residual Disease

Engraftment Data N=6

















Engraftment, patients	6 (100%)
Neutrophil engraftment, ANC $\geq 500 \times 10^6/L$, median (range)	12 days (11-13)
Platelet engraftment $\geq 20,000 \times 10^6/L$, no transfusion in 7 days, median (range)	17 days (16-19)

- 100% successful engraftment.
- Neutrophil and platelet recovery within transplant expectations in multiple myeloma.
- 31% of collected CD34+ stem cells expressed CD90+CD45RA-, a cell phenotype associated with durable engraftment function, three-fold greater than G-CSF mobilized grafts.

The MGTA-145 Opportunity: Rapid, Reliable, Predictable and Well-Tolerated Mobilization Can Improve Patient, Physician and System Experience

Current Mobilization Regimen: Unpredictable & Inefficient









Autologous (Blood Cancers)

Day	1	2	3	4	5	6	7	8
G-CSF								
plerixafor								
Collection								

5-8 days of mobilization & collection

~40% of patients require multiple collections¹













Allogeneic (Healthy Donors)

Day	1	2	3	4	5	6
G-CSF						
Collection						

5-6 days of mobilization & collection

~15% of healthy donors require multiple collections¹



Sickle Cell Disease (Gene Therapy)

Day	1	2	...	16	17	...	31	32
plerixafor			...			...		
Collection			...			...		

Up to 32 days to collect sufficient cells for gene therapy

~75% of patients require multiple collections²

MGTA-145 + plerixafor Target Profile*

Day	1
MGTA 145 + plerixafor	
Collection	

Preliminary Results Show Potential for MGTA-145 + Plerixafor to Become a First-Line Mobilization Regimen with Rapid, Reliable and Predictable Outcomes

Conclusions based on preliminary data

- This is the first study to evaluate MGTA-145 + plerixafor for stem cell mobilization and collection in a broad multiple myeloma patient population, including patients with multiple risk factors for poor mobilization, undergoing autologous stem cell transplant
- Preliminary results show 100% efficacy in collecting HSCs in up to two days of same-day mobilization and apheresis collection
- MGTA-145 was well-tolerated
- MGTA-145 + plerixafor -mobilized HSCs resulted in timely and durable engraftment in all patients who underwent transplant
- Potential for MGTA-145 + plerixafor to become first-line, G-CSF-free standard-of-care regimen for stem cell mobilization

Phase 2 Clinical Trial of MGTA-145 in Multiple Myeloma Final Clinical Data Expected in the Second Half of 2021

Next steps

- Continue to enroll the MGTA-145 multiple myeloma investigator-initiated trial at Stanford University
- Additional preliminary data to be presented in June at the American Society of Clinical Oncology (ASCO) Annual Meeting and at the European Hematology Association (EHA) Congress
- Final clinical data expected in the second half of 2021
- Driving towards efficient registration path based on final Phase 2 data

The MGTA-145 Opportunity

Rapid & Efficient

Improved experience for donors & patients, operational efficiencies and overall cost savings to healthcare system

Predictable & Reliable

Same-day mobilization and collection of large numbers of HSCs vs. 5- to 8-day process with current options

Well-Tolerated

Well-tolerated and not dependent on G-CSF, allowing for all patients – including those living with sickle cell disease – to potentially benefit

Clinical and Commercial

Driving towards efficient registration path based on Phase 2 data; positioned to leverage significant existing market opportunity

Q&A Session

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