

## **Inspired to Cure**

January 2021



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## We are Inspired to Cure: 2021 Outlook and Priorities

## Making an impact with two promising <u>advanced cell therapy</u> programs that leverage our proprietary cell expansion platform

#### Omidubicel — Nearing commercialization to address a major unmet need in hematopoietic stem cell transplant

- Potential to be first FDA-approved cell therapy for bone marrow transplantation
- Compelling Phase 3 clinical profile to date
- Preparing for BLA submission in 2H21
- Pre-commercial activities underway for potential launch

#### GDA-201 — Harnessing natural killer cells to fight Non-Hodgkin lymphoma

- Promising Phase 1 clinical data with an overall response rate of 74 percent
- Initiating a Phase 1/2 clinical study in NHL in 2021
- Plan to submit an investigational new drug application in 2021

#### Strong financial position to execute goals

- Cash position of \$127M supports capital needs into 2H22\*
- Approximately 110 employees

\*Unaudited cash position, which Includes cash, cash equivalents, marketed securities and short-term deposits. Cash runway guidance is based on our current operational plans, including the assumption that we will continue to advance both our commercial readiness and all our clinical programs and excludes any additional funding that may be received or business development activities that may be



## **Our Advanced Cell Therapy Programs**

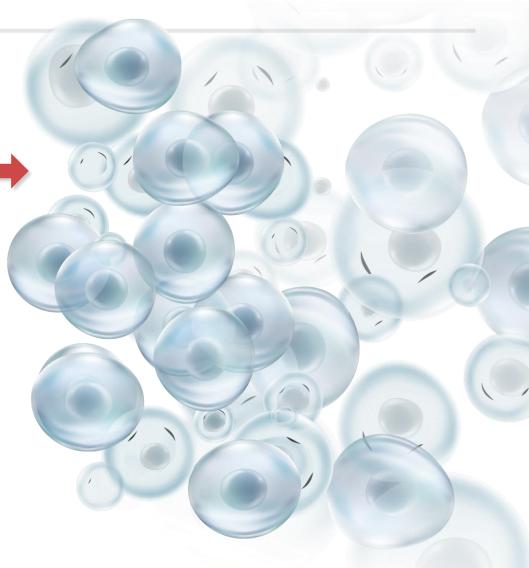
CANDIDATE	PRECLINICAL	PHASE 1	PHASE 2	PHASE 3	MILESTONES
OMIDUBICEL					
High-Risk					✓ Topline data 2Q20
Hematologic	FDA Breakthrough De	esignation			<ul> <li>Detailed data presentation 4Q20</li> </ul>
Malignancies					BLA submission 2H21
Severe Aplastic Anemia*					✓ Additional data 4Q20
004					
GDA-201					
Non-Hodgkin					
Lymphoma, Multiple					<ul> <li>Additional data 4Q20</li> <li>IND submission 2021</li> </ul>
Myeloma					

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## Pipeline Built on Proprietary NAM Platform Technology



- Enhances the **number** of allogeneic donor cells
- Preserves cellular functionality and phenotype
- Potential to expand any cell type





# Omidubicel

A potentially curative treatment for patients in need of a bone marrow transplant





## Meet Stacey

Stacey participated in the first clinical study of omidubicel at Duke University Medical Center after being diagnosed with AML. She has been cancer-free since her bone marrow transplant in 2011.

"My ultimate goal was I wanted to live. We were ever so thankful to hear that there was a possible opportunity for me in a trial going on at Duke University."

This is one patient and results may not be indicative. Omidubicel is investigational and safety and efficacy have not been established by any agency.

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Substantial Market Opportunity to Both Improve Known Issues with Existing Donor Source as well as Expand the Market to Treat Untransplanted Patients

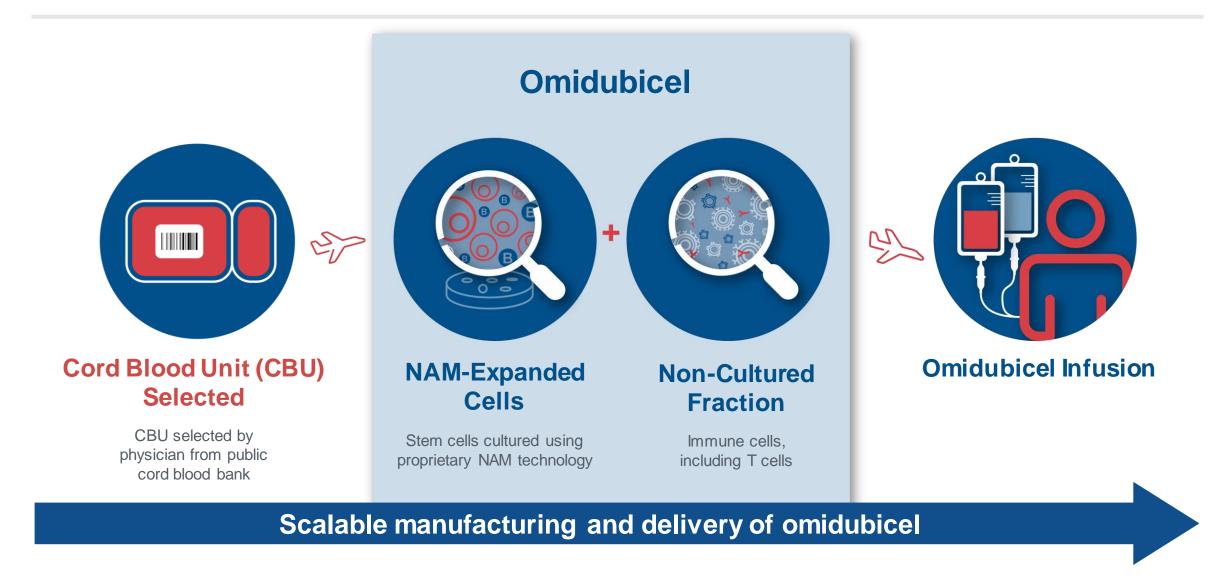
~13,000 patients with hematologic malignancies are eligible for transplant annually in the U.S.

		Patients	Challenges
Omidubicel opportunity	Not Matched / Not Referred	5,200	<ul><li>Access to care and graft source</li><li>Limited therapy options</li></ul>
	Matched Unrelated (MUD)		Availability of graft
	Mismatched Unrelated (mMUD)	E 200	<ul> <li>Quality of graft source</li> <li>Time to approximant</li> </ul>
	Haploidentical	5,200	<ul> <li>Time to engraftment</li> <li>Infection</li> </ul>
	Cord Blood		<ul><li>Risk of GvHD</li><li>Potency of GvL effect</li></ul>
	Matched Related (MRD)	2,600	<ul> <li>Availability of sibling donor</li> </ul>

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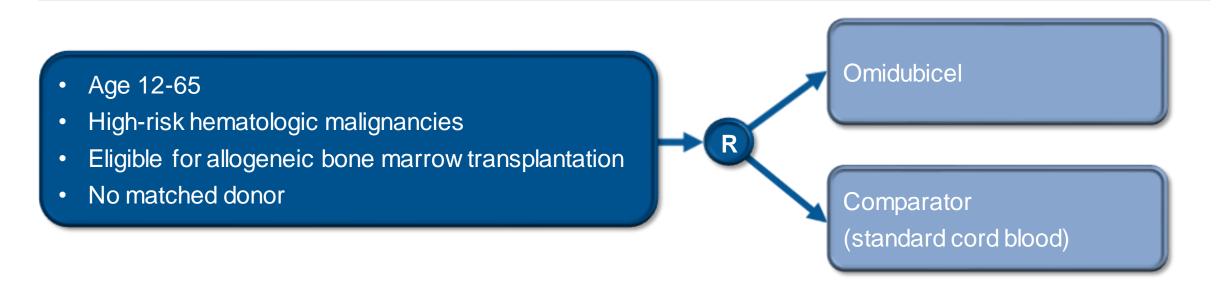


## Omidubicel Is a Cell Therapy Option for Patients in Need of a Transplant





## Phase 3 Global, Randomized Study



Primary endpoint: Time to neutrophil engraftment

Secondary endpoints: Platelet engraftment, infections, hospitalizations

Additional endpoints: Acute GvHD, chronic GvHD, adverse events, non-relapse mortality, disease-free survival, overall survival

Clinicaltrials.gov identifier NCT01221857.

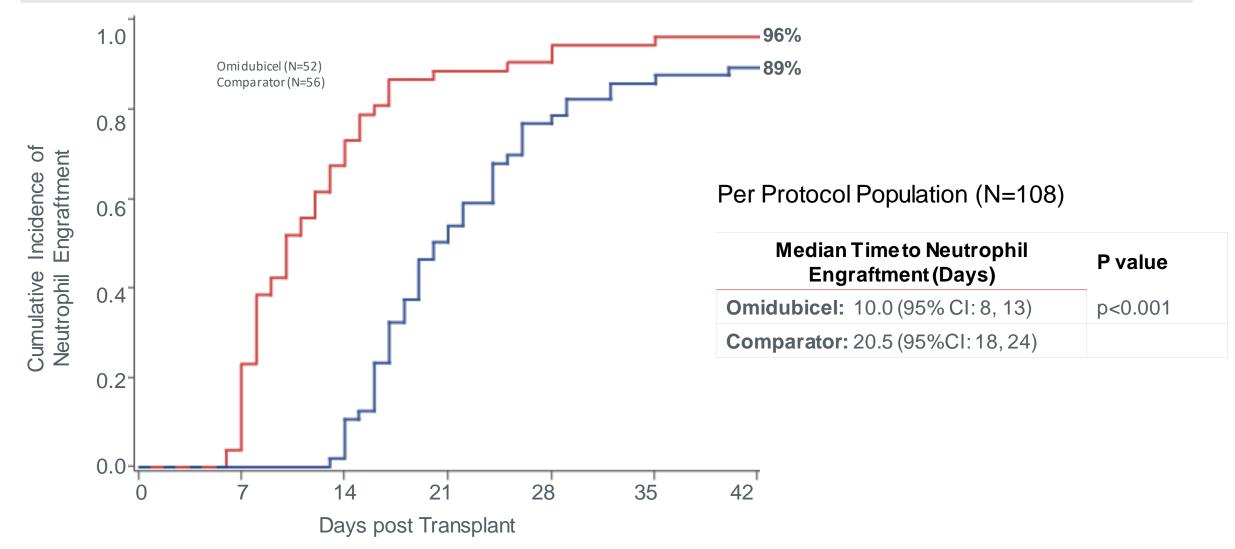


- 125 patients were randomized at 33 sites
- Demographics and baseline characteristics were well-balanced in the two arms
- Omidubicel was generally well-tolerated

INTENT-TO-TREAT	MEDIAN TIME TO NEUTROPHIL ENGRAFTMENT (DAYS)	95% CI	p-VALUE
Omidubicel (N = $62$ )	12.0	(10.0, 15.0)	p<0.001
Comparator ( $N = 63$ )	22.0	(19.0, 25.0)	



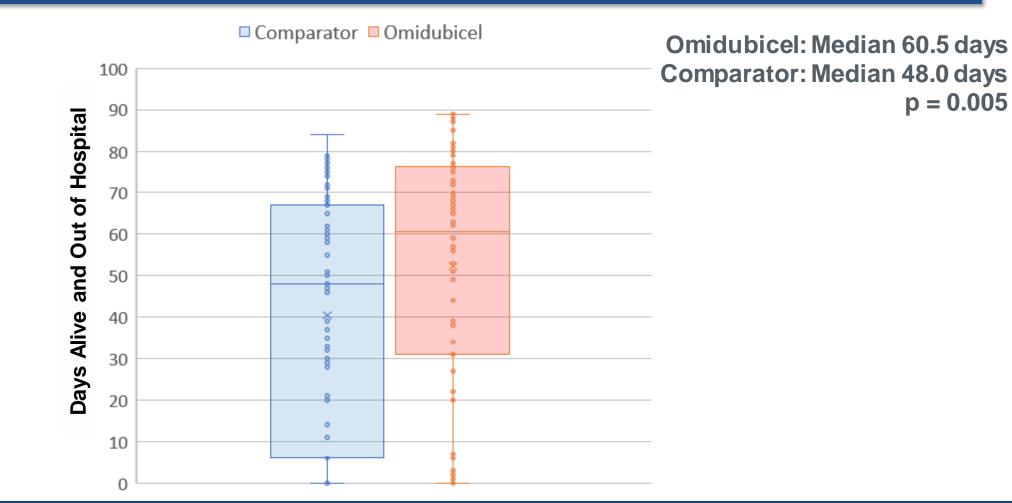
## Cumulative Incidence of Neutrophil Engraftment



Per protocol population: received transplantation with omidubicel or comparator per protocol.

## Phase 3 Secondary Endpoint: Omidubicel Significantly Reduced Total Hospitalization in First 100 Days

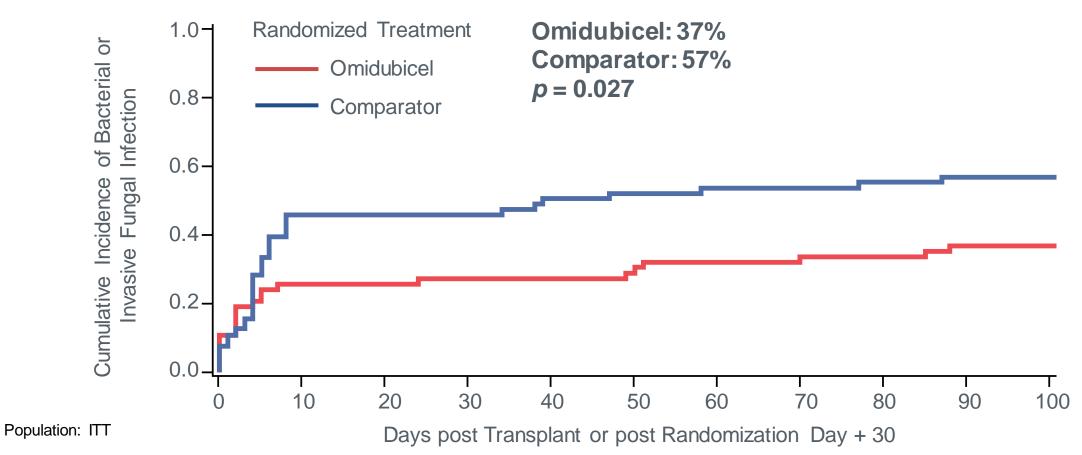
#### ALIVE AND OUT OF HOSPITAL IN FIRST 100-DAYS



Population: ITT

## Phase 3 Secondary Endpoint: Omidubicel Significantly Reduced Serious Infection Rate

#### **INFECTIONS BETWEEN RANDOMIZATION AND 100 DAYS<sup>1</sup>**

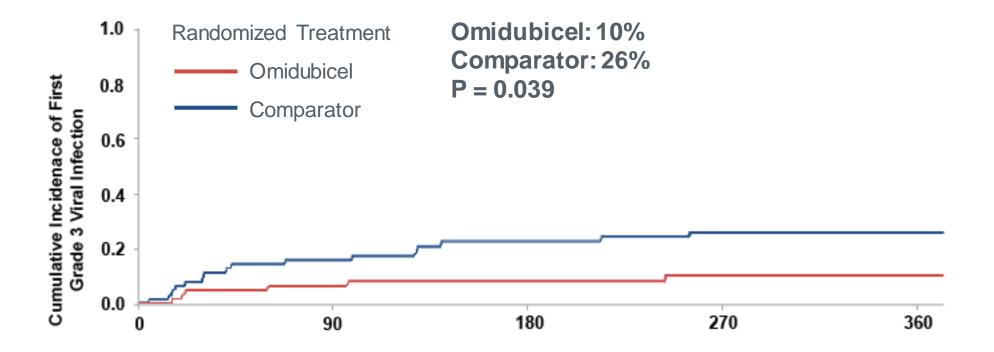


1. Proportion (%) of patients with any grade 2-3 bacterial infection or invasive fungal infection between randomization and 100 days following transplantation



## Phase 3 Exploratory Endpoint Omidubicel Significantly Reduced Viral Infection Rate

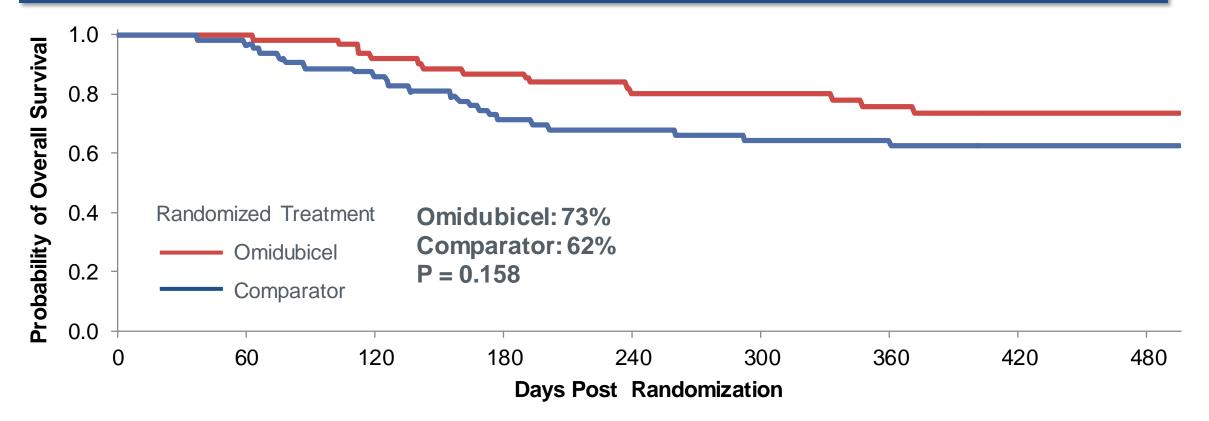
#### CUMULATIVE INCIDENCE OF FIRST GRADE 3 VIRAL INFECTION BY 1 YEAR FOLLOWING TRANSPLANTATION (ITT)





## Phase 3 Exploratory Endpoint: Overall Survival at 15 Months (ITT)

#### OVERALL SURVIVAL AT 15 MONTHS AFTER RANDOMIZATION (ITT), MEDIAN FOLLOW-UP (~10 MONTHS)



# Omidubicel

Commercial Potential and Launch Readiness



Substantial Market Opportunity to Both Improve Known Issues with Existing Donor Source as well as Expand the Market to Treat Untransplanted Patients

~13,000 patients with hematologic malignancies are eligible for transplant annually in the U.S.

		Patients	Challenges		Unmet Need / Omidubicel Opportunity
opportunity	Not Matched / Not Referred	5,200	<ul> <li>Access to care and graft source</li> <li>Limited therapy options</li> </ul>	•	Increase Access
Omidubicel oppor	Matched Unrelated (MUD)		Availability of graft	•	Improve Outcomes
	Mismatched Unrelated (mMUD)	5,200	<ul> <li>Quality of graft source</li> <li>Time to engraftment</li> </ul>		
	Haploidentical		<ul> <li>Time to engraftment</li> <li>Infection</li> <li>Risk of GvHD</li> <li>Potency of GvL effect</li> </ul>		
	Cord Blood				
	Matched Related (MRD)	2,600	<ul> <li>Availability of sibling donor</li> </ul>		



Omidubicel Will Be a Therapy Option for HSCT Patients Who Do Not Have Access to a Matched Related Donor\*

#### **Omidubicel Launch Goals**



Rapid time to peak market share: ~ 3 years to reach peak
Not only capturing share from current modalities, but also increasing access



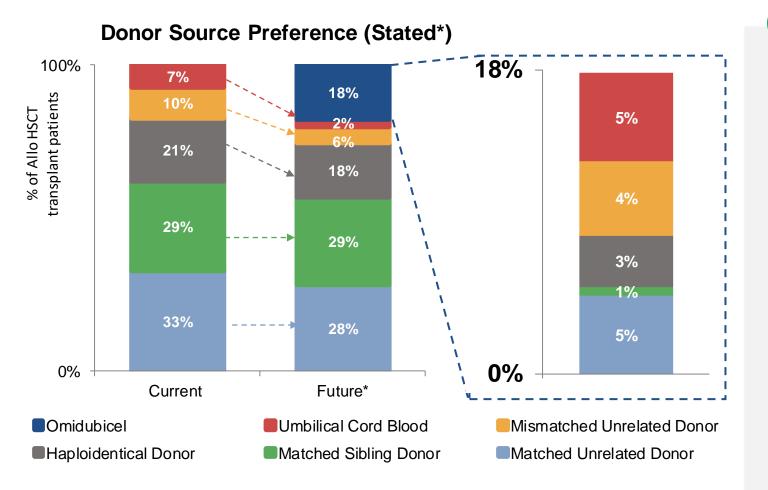
2,000+ patients treated with Omidubicel per year in the U.S., upon reaching peak (supported by market research)



Positive patient and transplant center experience with omidubicel



## Omidubicel Market Share Expected to Exceed 20%, with 18%\* Coming from Current Modalities and Additional Share from Increased Access



#### **Omidubicel's Competitive Advantage**

#### vs.UCB:

- Better efficacy (neutrophil engraftment time, average days in the hospital, and neutrophil recovery)
- Eliminates the need to order 2 cords and risk running out of cells due to engraftment failure

#### vs. MMUD:

- Less risk of infections
- Speed
- Overall trend of decreasing MMUD use

#### vs. Haplo:

Lower GVHD

#### vs. MRD:

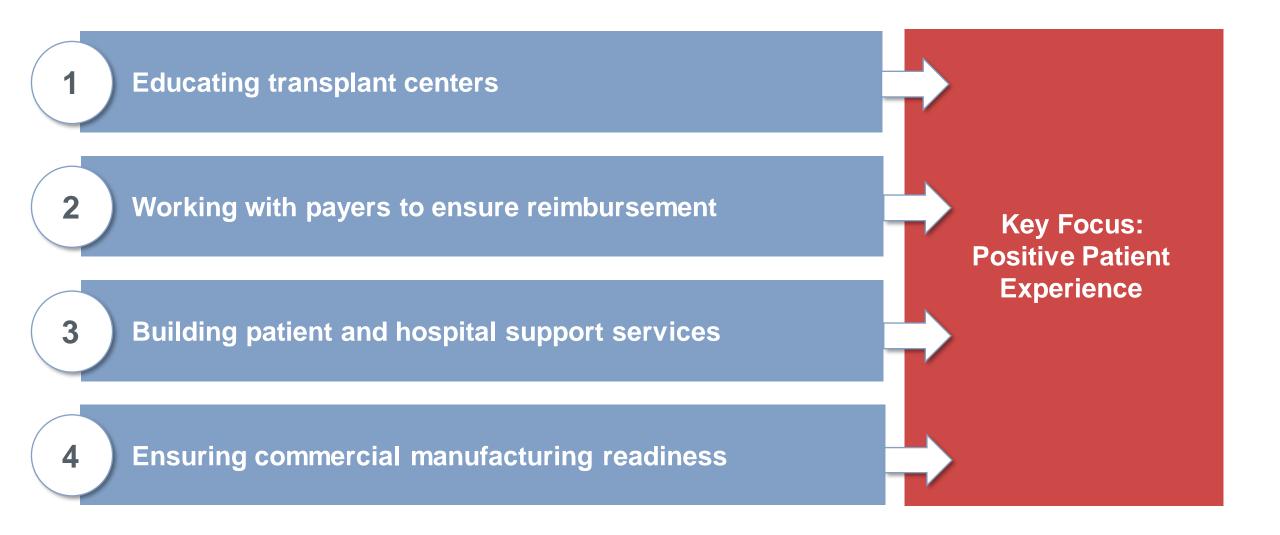
Availability, as not every patient has a fit sibling donor

#### vs. MUD:

- Speed, especially important for patients whose disease is progressing rapidly
- Lack of donor follow through for MUD

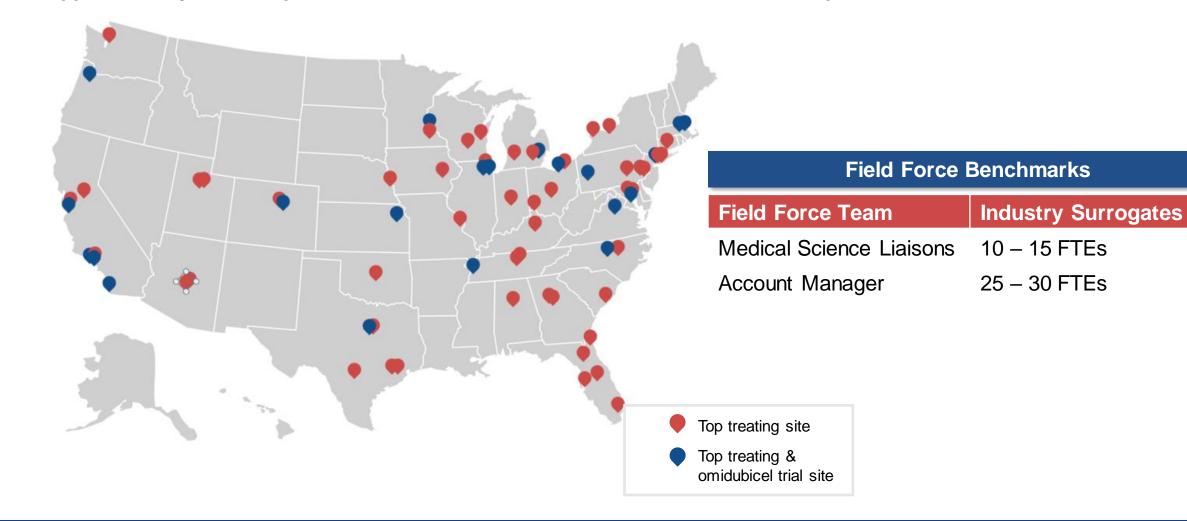


Key Commercial Activities and Infrastructure Build-out Are Underway to Prepare for a Successful Omidubicel U.S. Launch



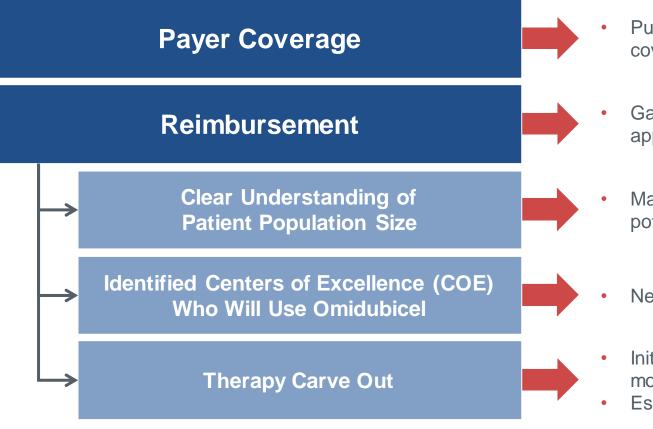


## Gamida Cell Has Initiated Plan for Education of U.S. Transplant Centers



Approximately 70 transplant centers account for ~80% of bone marrow transplants in U.S.

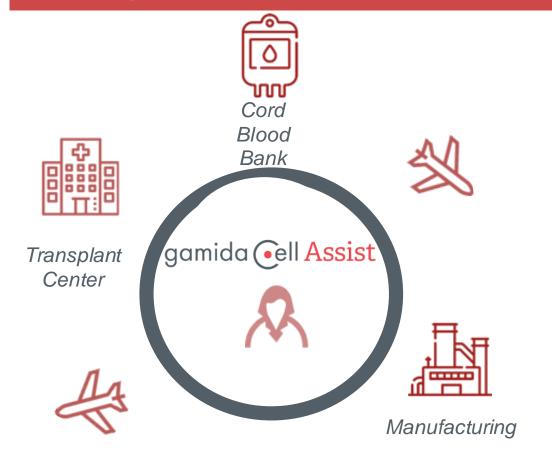




- Published data supports that ~100% of U.S. payers anticipate covering one-time therapies, with curative intent
- Gamida has a good understanding of the Reimbursement approach that payers will take upon omidubicel FDA approval
- Market is well defined, and payers can calculate their potential covered lives
- Needs for COEs: 70 centers make up 80% of transplants
- Initial payer market research identified carve out as the most likely reimbursement approach at time of approval
- Establish the appropriate codes for carve outs

### Gamida Cell Assist Will Be a Key Aspect of Our Patient-centric Launch

Building a patient support operation to provide the assistance and services to healthcare professionals, patients, and caregivers that will support access to our therapy and strive to ensure a positive personalized experience



- We are a support and solutions-oriented team that will provide a personalized, high touch experience
- Gamida Cell Assist will provide a single point of contact for patients and health care professionals
  - Through this, we will provide support and services throughout the therapy process
- Our focus is on keeping operations simple with the flexibility and agility needed to address the needs of each patient that requires cell therapy

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## Dual sourcing for manufacturing established for commercialization of omidubicel:

#### Kiryat Gat (Israel)

- Gamida Cell owned facility
- Construction completed in 2020 and hiring complete for initial team
- Qualification for BLA filing underway

#### Lonza (CMO)

- Well recognized cell and gene therapy manufacturer
- Manufacturing partner for the omidubicel Phase 3 study\*



Photo of Gamida Cell-owned facility.



# **GDA-201**

Harnessing Innate Immunity Using Natural Killer (NK) Cells to Treat Cancer





## Meet Wayne

Wayne participated in the Phase 1/2 clinical study of GDA-201 at the University of Minnesota to treat lymphoma. His lymphoma is in remission a year after treatment.

"[The doctors] were finding that the lymphoma appeared to have evaporated, completely gone away, that the lymph nodes were really showing no signs of having any kind of cancer in them."

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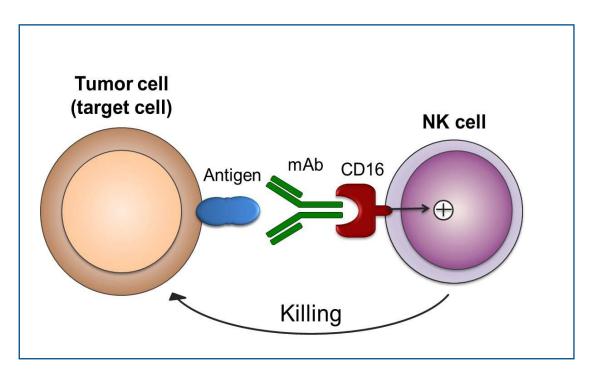


## Putting NK Cells to Work Using Our NAM Technology Platform

#### **Benefits of NK Cells**

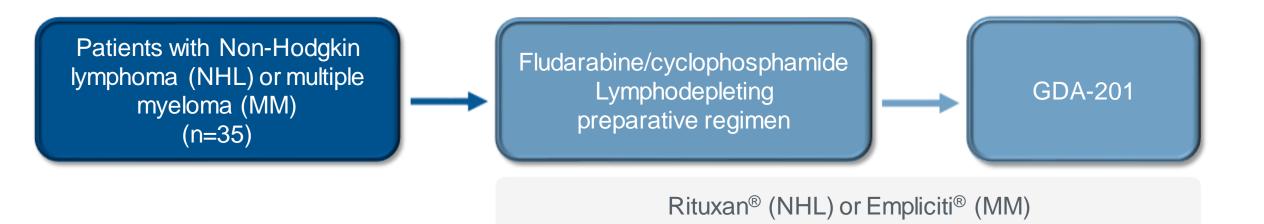
- Natural killer (NK) cells infusion is a promising immune therapy for cancer
  - No HLA matching required
  - Synergy with antibodies
  - Potential for off-the-shelf therapy
- Expansion is necessary to obtain clinically meaningful doses with retained cell function

#### **GDA-201: NK Cells + Tumor-specific Antibodies**





# Phase 1 Study of GDA-201 in Patients with Non-Hodgkin Lymphoma and Multiple Myeloma



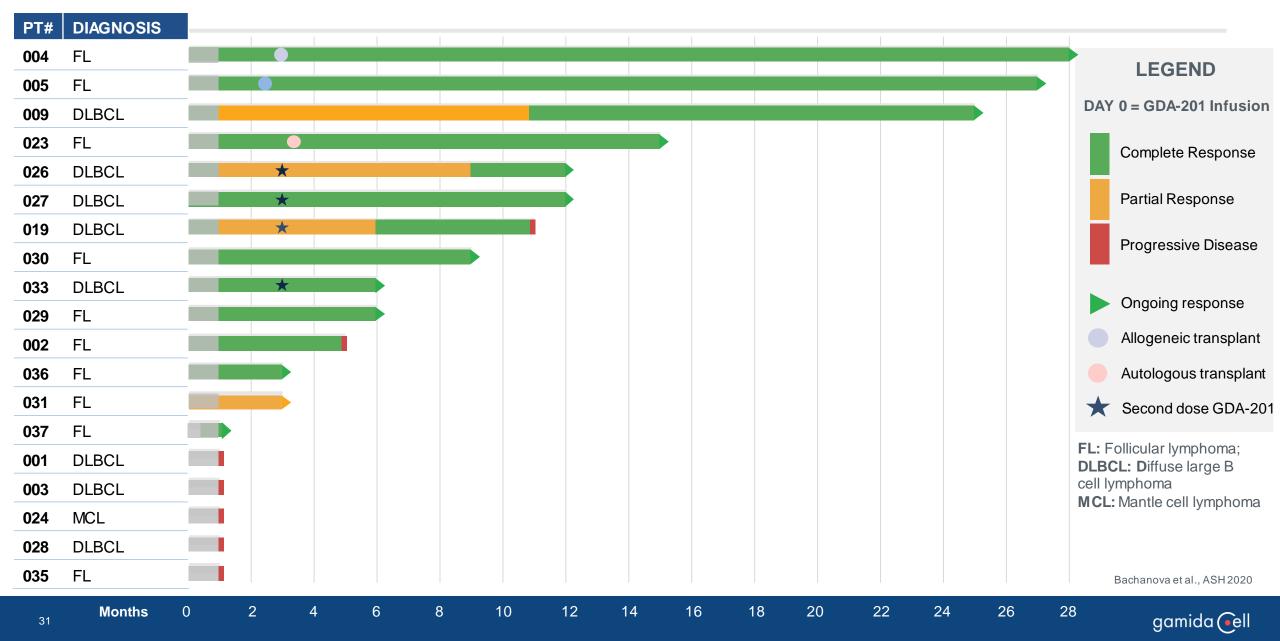
- Primary endpoint: Maximum tolerated dose of GDA-201 (3 doses evaluated)
- Secondary endpoints: Overall response, toxicity

## Response Rates

19 PATIENTS WITH NHL	Follicular Lymphoma (FL) (n=10)	Diffuse Large B-Cell Lymphoma (DLBCL) (n=8)
13 CR	8 CR	5 CR
1 PR	1 PR	
5 PD		
ORR:74%		
CR rate: 68%		

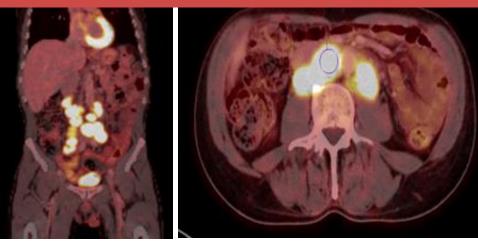


## GDA-201 Is Highly Active in Non-Hodgkin Lymphoma

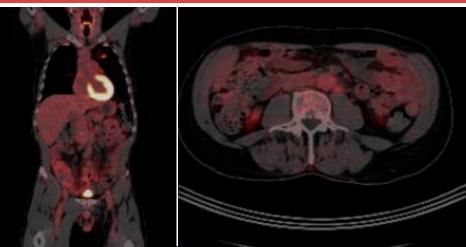


## Patient 009

#### Pt 009: Baseline



#### Pt 009: 6-month post GDA-201



- 57-year-old man with history of CLL and Richter's transformation-large cell lymphoma, measurable retroperitoneal lymph nodes at baseline
- Prior therapy: FCR-light, Rituximab/Bendamustine Ibrutinib/Revlimid, R-CHOP, Venetoclax/Rituximab
- Allogeneic HSCT (matched sibling)
- Relapse at 6 months
- Treated with GDA-201
- 28-day response: Tumor shrinkage
- 6 months: PR with continued tumor shrinkage
- 12 months: Complete response

Bachanova et al. ASH 2019.



## GDA-201: Encouraging Clinical Activity and Safety Profile Supports Continued Development

#### **Key Accomplishments**

- ✓ Preclinical proof of principle
- ✓ Clinical proof of concept
- ✓ Well tolerated
- Maximum target dose achieved

### **Next Steps**

- Complete Phase
   1 study
- Finalize CMC for cryopreserved formulation
- Initiate Phase 1/2 multi-center study in 2021

#### **Future Directions**

- Combine with a broad range of antibodies
- Evaluate in solid tumors
- Genetic modification of NAM-expanded NK cells

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