



# Inspired to Cure



September 2020

# Disclaimer

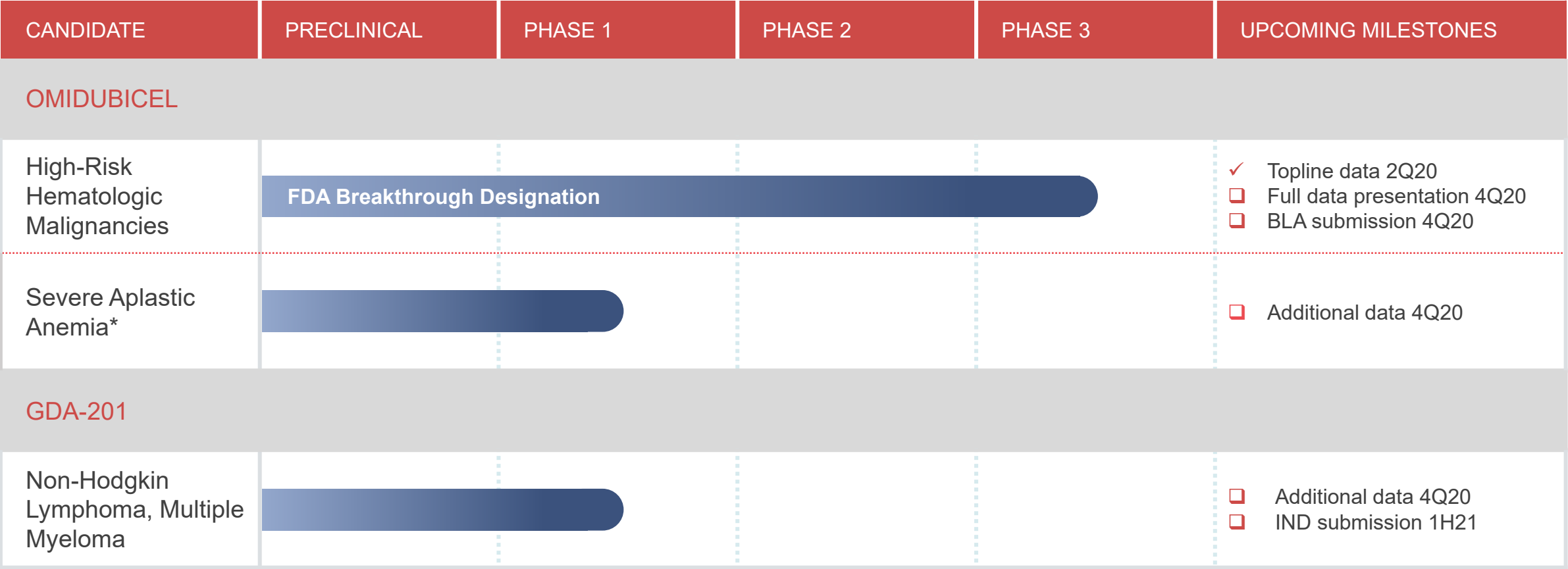
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This Presentation includes certain projections and forward-looking statements as of the date of this Presentation provided by Gamida Cell Ltd (the “company”). The information in this Presentation is current only as of its date and may have changed since that date. These projections and forward-looking statements include, but are not limited to, those regarding the company’s future financial position and results of operations, the company’s commercialization, marketing and manufacturing capabilities and strategy, the company’s intellectual property position, regulatory matters, market size and opportunity and the company’s estimates regarding expenses, future revenues, capital requirements and needs for additional financing. These projections and forward-looking statements are based on the beliefs of the company’s management as well as assumptions made and information currently available to the company. Such statements reflect the current views of the company with respect to future events and are subject to business, regulatory, economic and competitive risks, uncertainties, contingencies and assumptions about the company and its subsidiaries and investments, including, among other things, the development of its business, trends in the industry, the legal and regulatory framework for the industry and future expenditures. In light of these risks, uncertainties, contingencies and assumptions, the events or circumstances referred to in the forward-looking statements may not occur. None of the future projections, expectations, estimates or prospects in this presentation should be taken as forecasts or promises nor should they be taken as implying any indication, assurance or guarantee that the assumptions on which such future projections, expectations, estimates or prospects have been prepared are correct or exhaustive or, in the case of the assumptions, fully stated in the presentation. The actual results may vary from the anticipated results and the variations may be material.



We are pioneering new,  
potentially curative advanced  
cell therapies.

# We Are Developing Advanced Cell Therapies

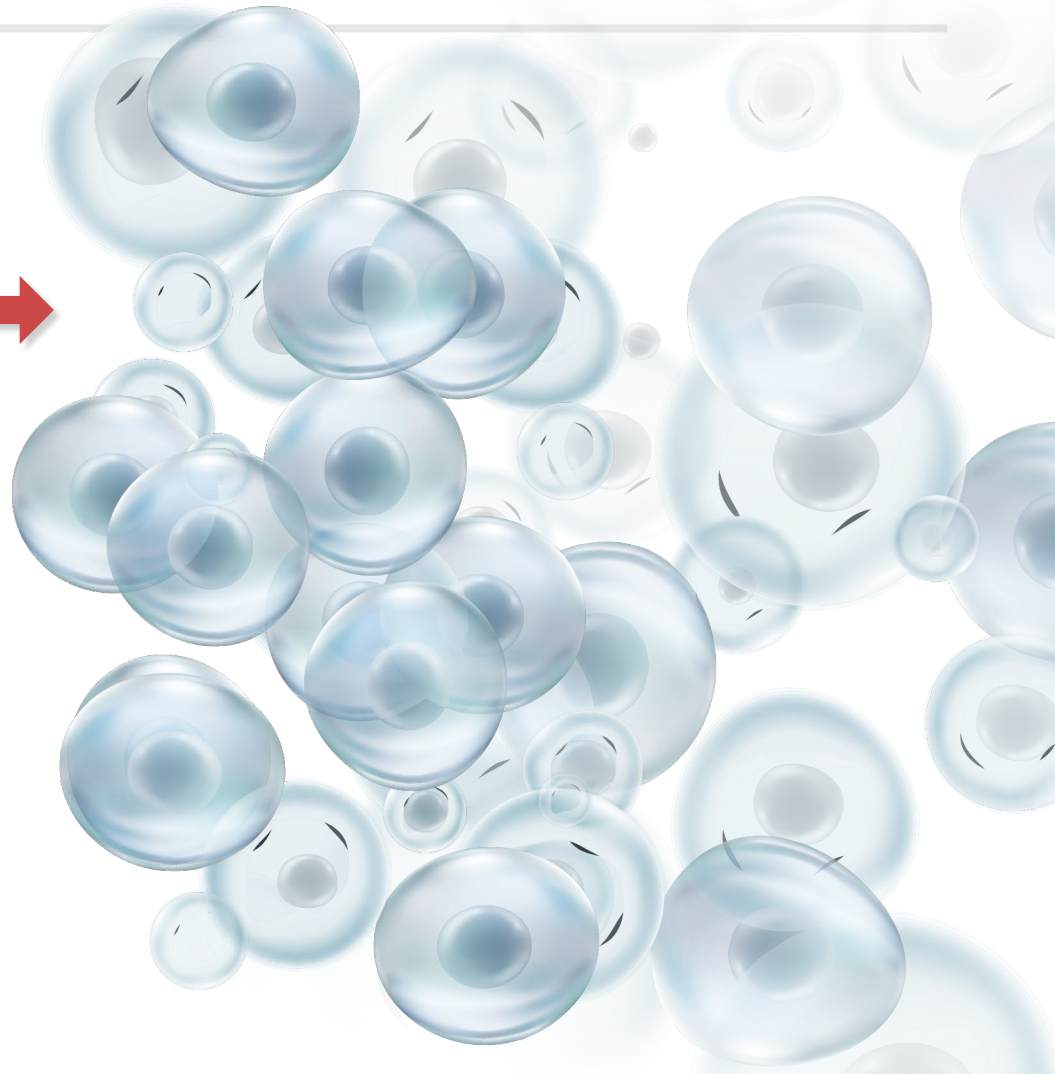
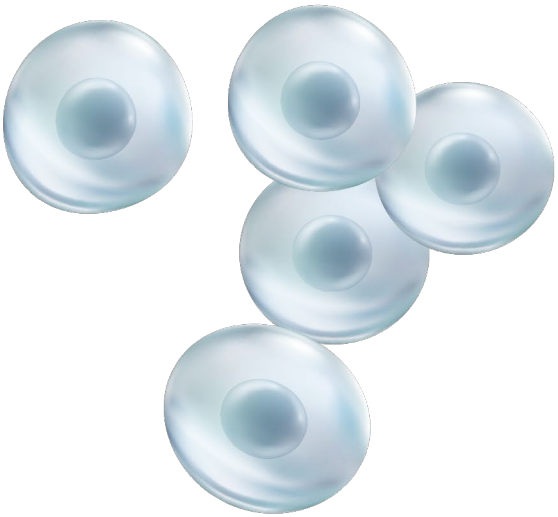


\*The Aplastic Anemia Investigational New Drug (IND) application is currently filed with the FDA under the brand name, CordIn, which is the same investigational development candidate as omidubicel.

# Pipeline Built on Proprietary NAM Platform Technology

## NAM Platform Technology

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



# Omidubicel

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

gamida Cell





## Meet Stacey

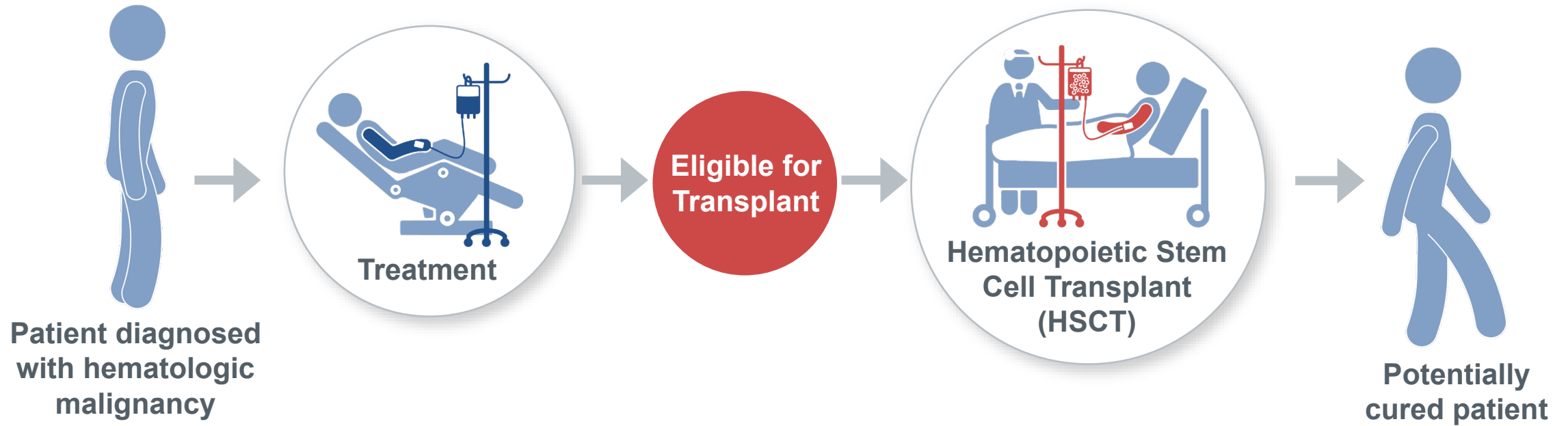
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Stacey participated in the first clinical study of omidubicel at Duke University Medical Center after being diagnosed with AML. She has been cancer-free since her bone marrow transplant in 2011.

*"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."*

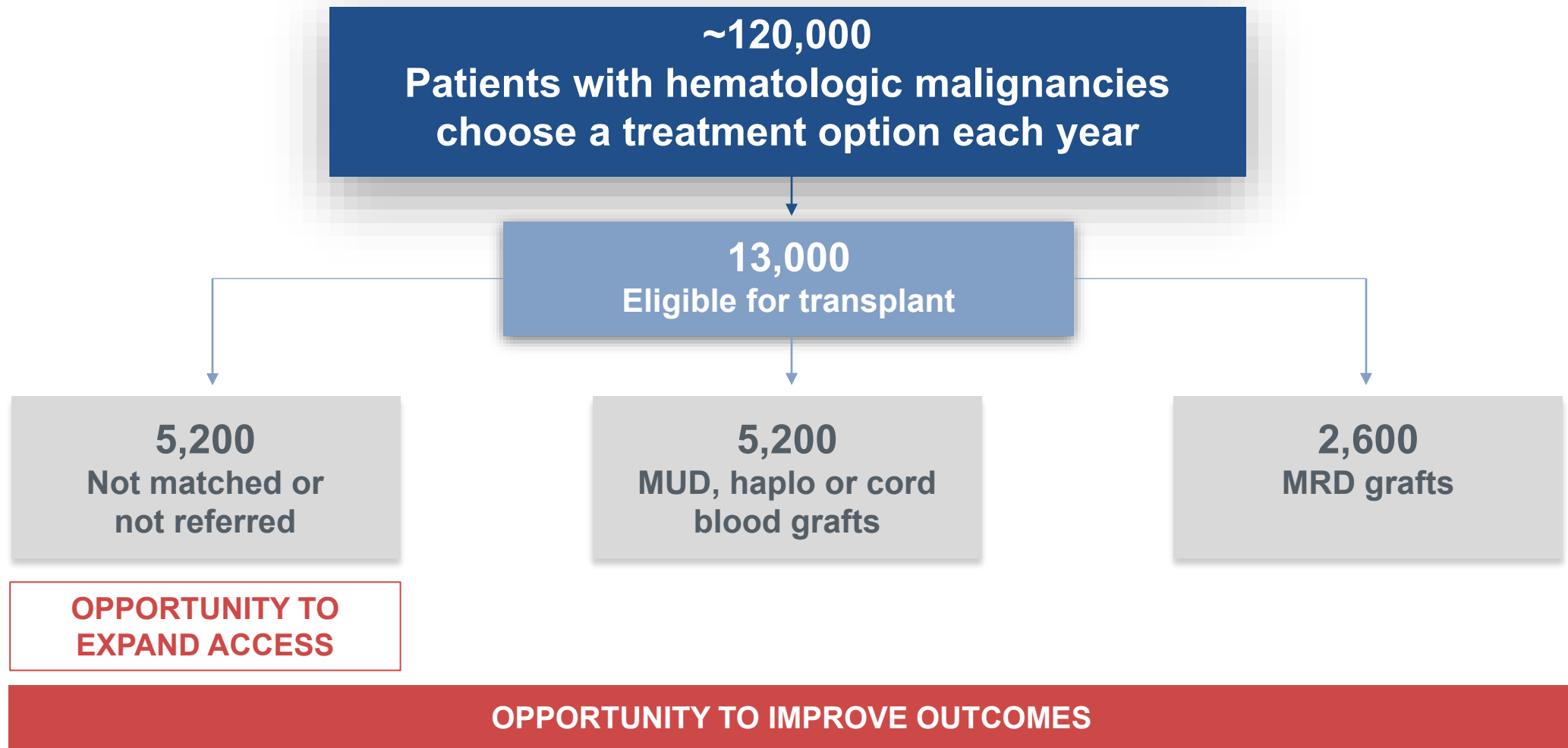
This is one patient and results may not be indicative. Omidubicel is investigational and safety and efficacy have not been established by any agency.

# Bone Marrow Transplant May Be Curative for Certain Hematologic Malignancies





# Omidubicel May Address a Significant Patient Population in the U.S.

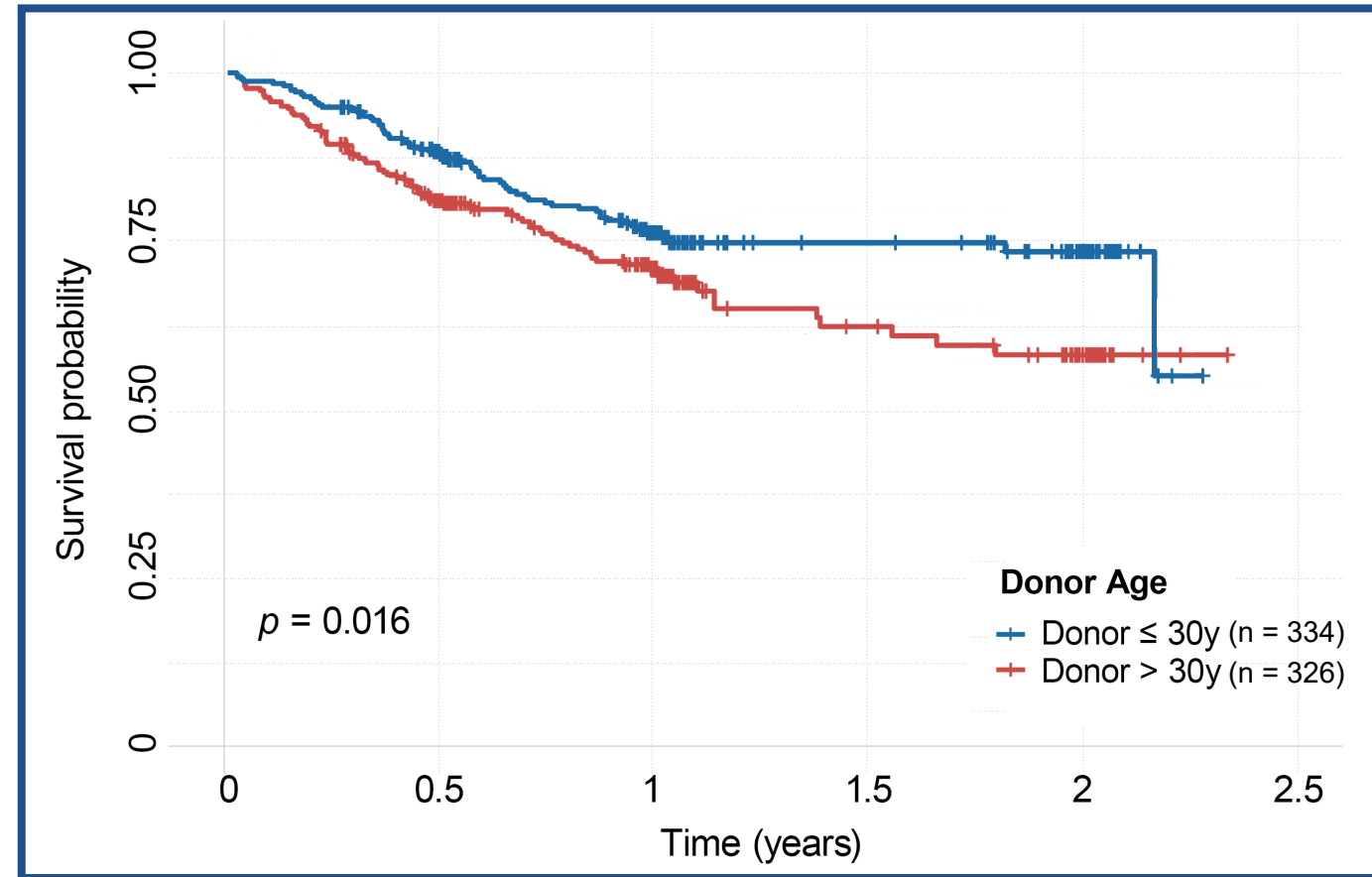


**MUD:** Matched unrelated donor; **haplo:** Haploidentical; **MRD:** Matched related donor

# Overall Survival Is Increased in Patients with Younger Donors

## Methodology

- Ongoing collaboration with CIBMTR to explore outcomes contemporaneous to the Phase 3 study of omidubicel
- Patients underwent myeloablative conditioning and HSCT for hematologic malignancies
- HSCT with three graft types: matched unrelated, mismatched unrelated, and haploidentical donors
- Median age of all donors: 30 years

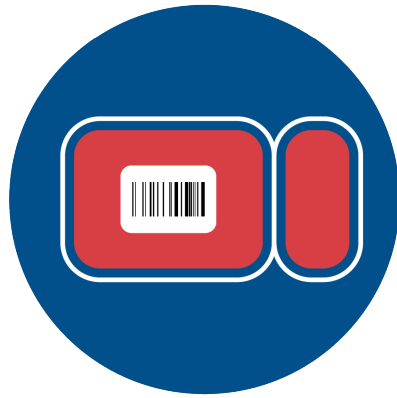


**Stem cells**, the starting point for omidubicel, are the “youngest” cell type used in allogeneic bone marrow transplant

Galamidi E, Joyce A, Simantov R. Impact of Donor Age on HSCT Outcomes. Cord Blood Connect. Sept. 2020.

# Omidubicel Is a Potentially Curative Cell Therapy Product

## Omidubicel



### Cord Blood Unit (CBU) Selected

CBU selected by  
physician from public  
cord blood bank



### NAM-Expanded Cells

Stem cells cultured using  
proprietary NAM technology



### Uncultured Fraction

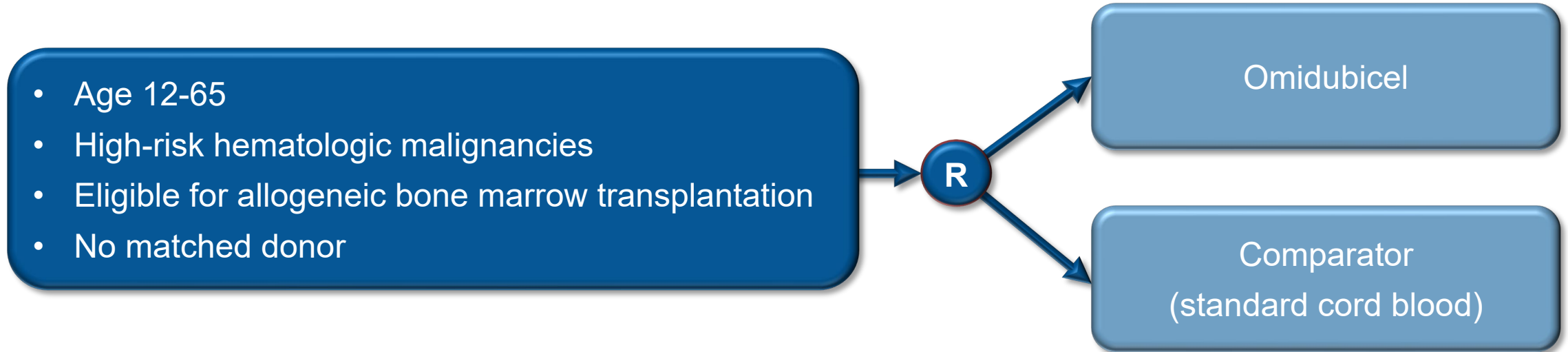
Immune cells,  
including T cells



### Omidubicel Infusion

**Scalable manufacturing and delivery omidubicel**

# Phase 3 Global, Randomized Study Conducted at Over 50 Sites



**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

Clinicaltrials.gov identifier NCT01221857.

# Phase 3 Primary Endpoint

## Omidubicel Significantly Reduced Time to Engraftment

Intent-to-treat	Median Time to Neutrophil Engraftment (Days)	95% CI	
Omidubicel (N = 62)	12.0	(10.0, 15.0)	p<0.001
Comparator (N = 63)	22.0	(19.0, 25.0)	

- Demographics and baseline characteristics were well-balanced in the two arms
- Omidubicel was generally well tolerated

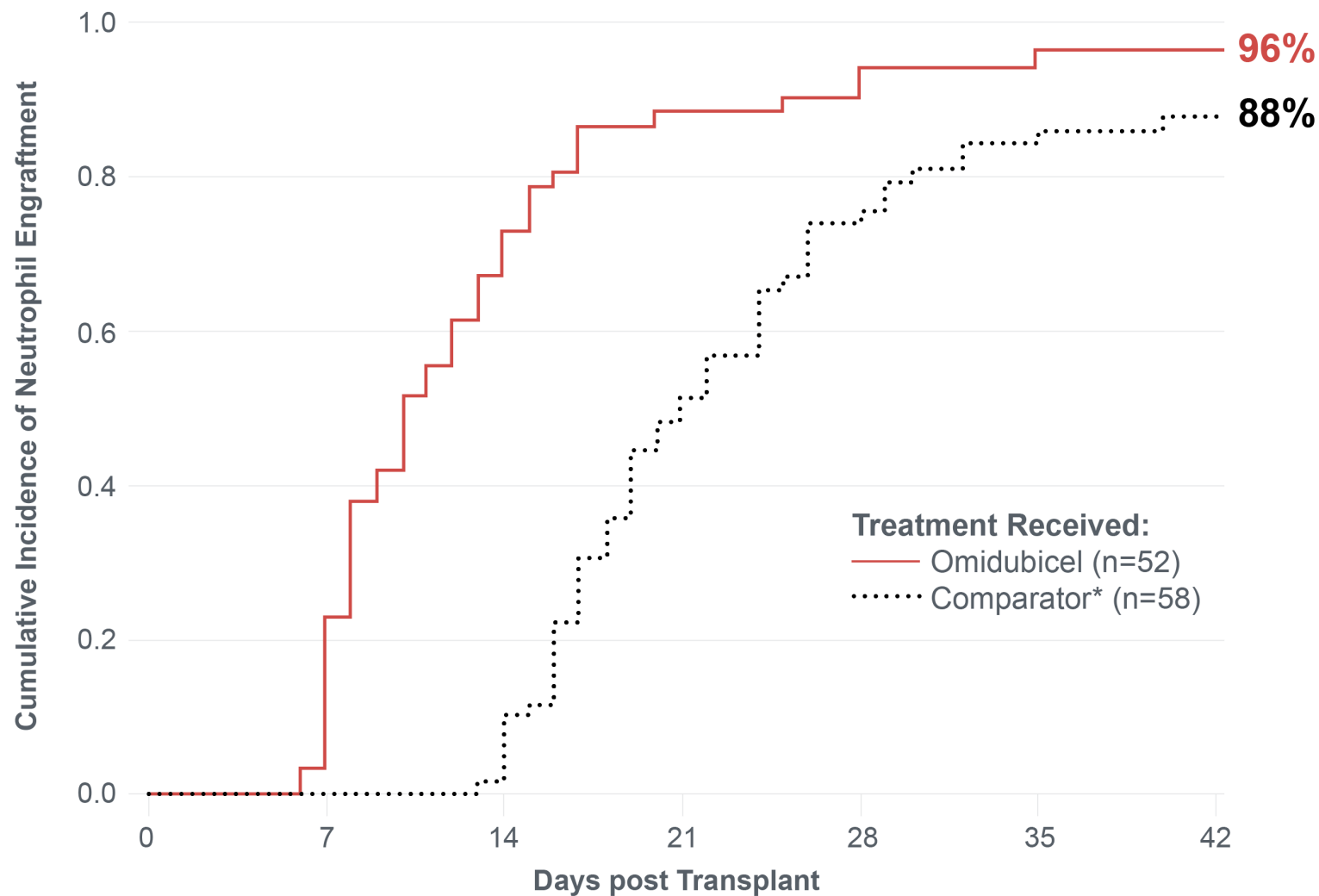
**Engraftment** is a key milestone in recovery  
**Rapid engraftment** is associated with fewer infections and shorter hospitalizations<sup>1</sup>

Anand et al. *BBMT* 23:1151-7, 2017.



# Phase 3 Data

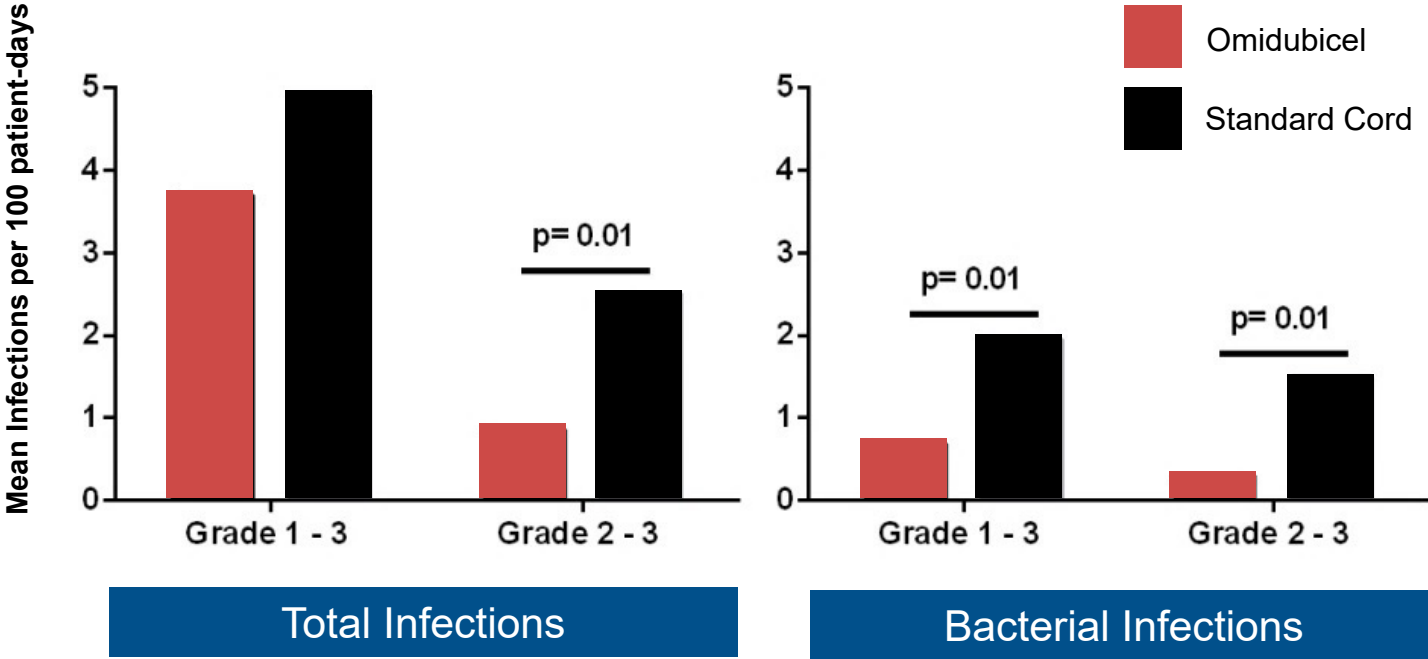
## Cumulative Incidence of Neutrophil Engraftment in As-Treated Population



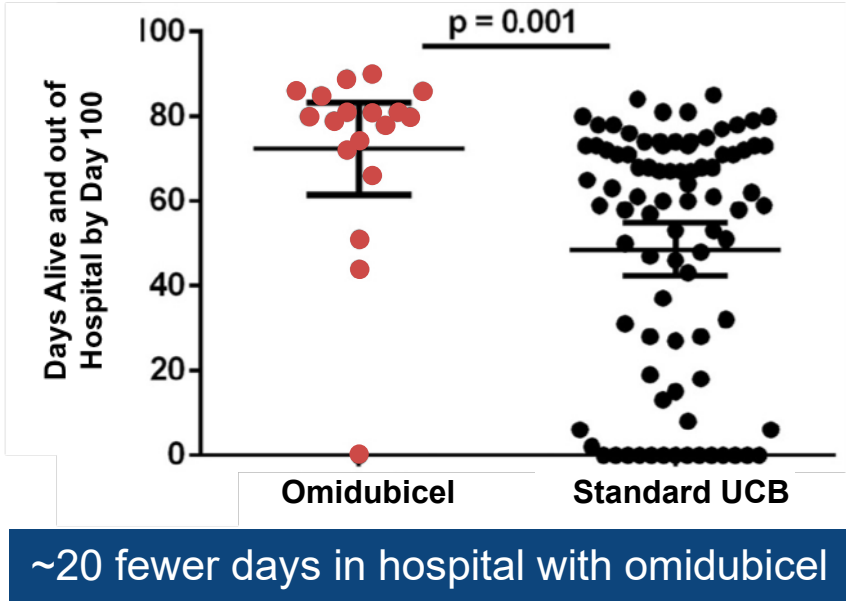
\*Comparator is standard cord blood.  
AT: As treated population (received transplantation with omidubicel or comparator per protocol).

# Phase 1/2 Omidubicel Study Demonstrated That Rapid Engraftment Is Associated with Fewer Infections and Shorter Hospitalizations

## Infection

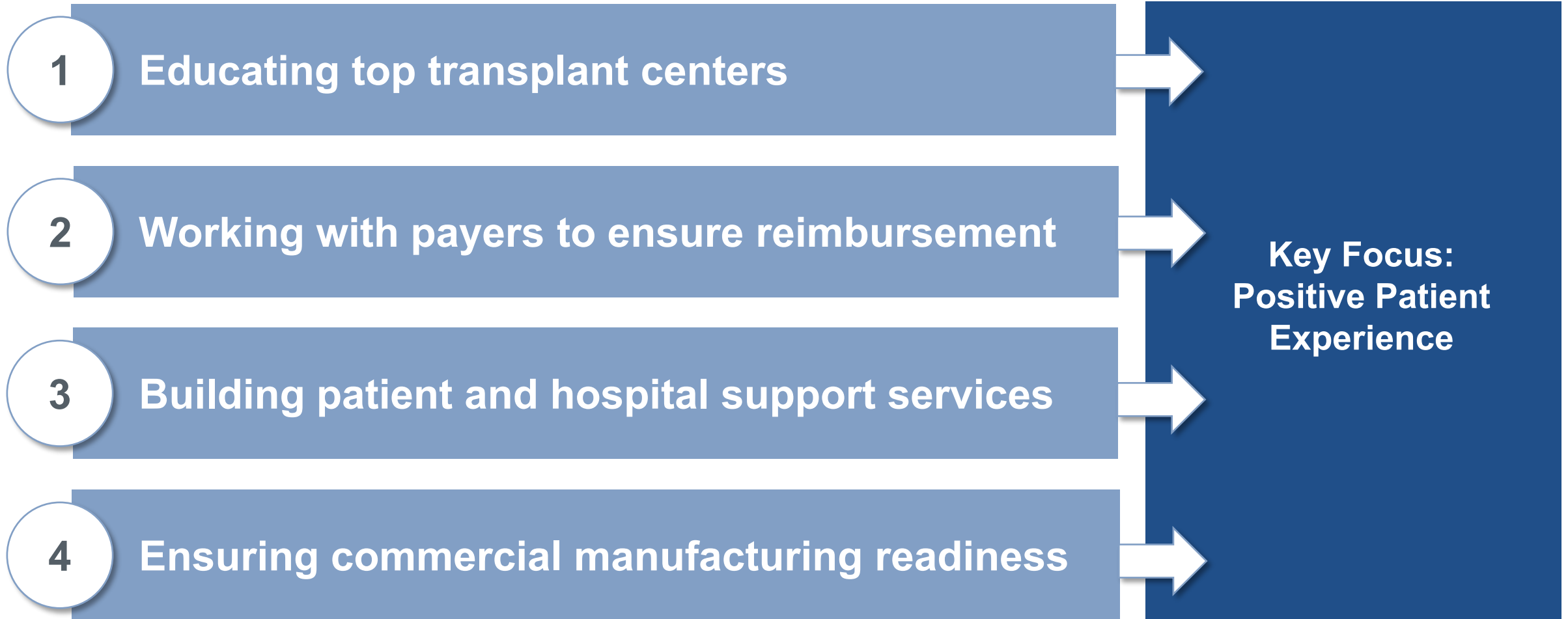


## Hospitalization

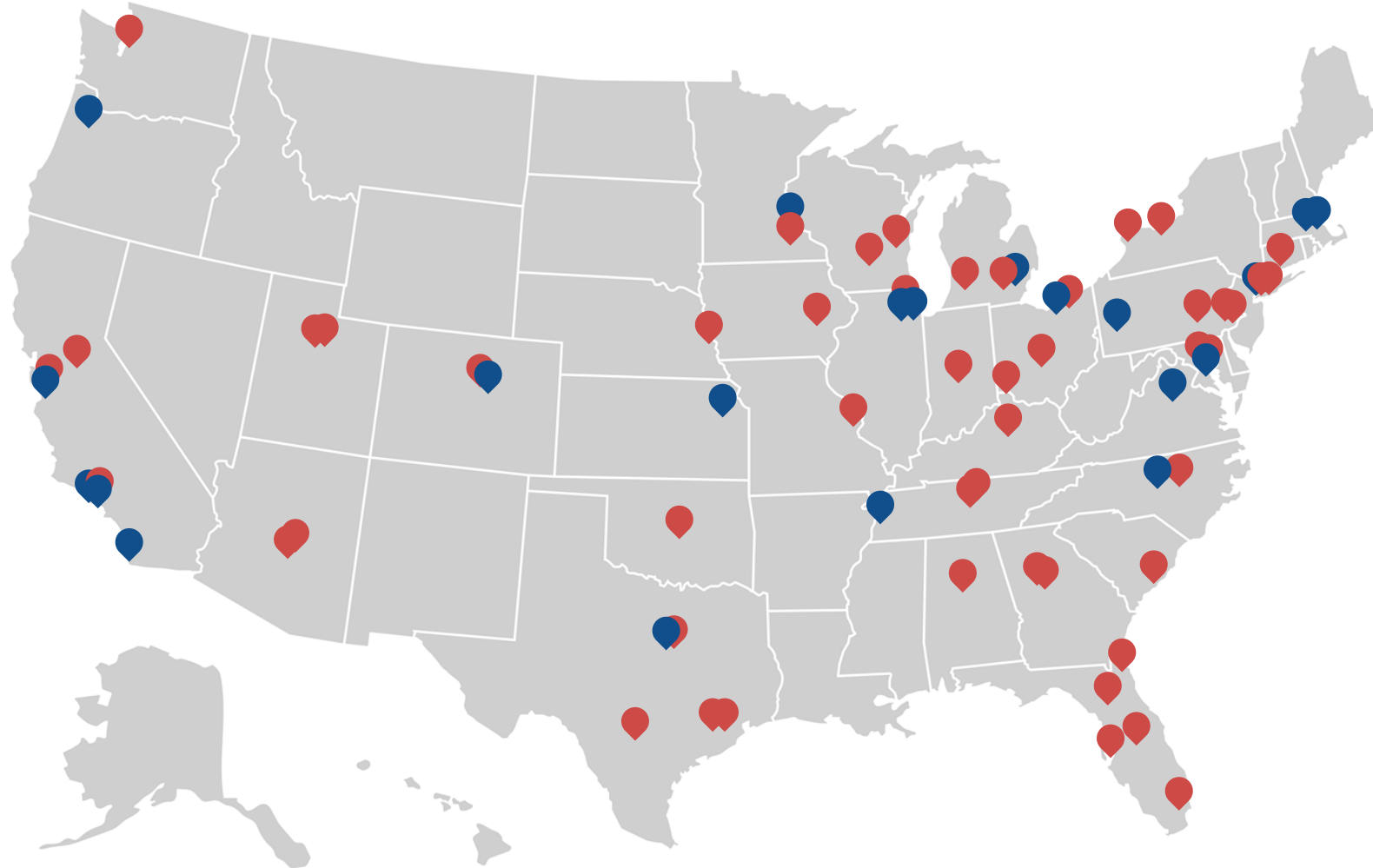


Anand et al. *BBMT* 23:1151-7, 2017.

# Preparing for a Successful Omidubicel Launch



- Top treating site
- Top treating & omidubicel trial site



# Omidubicel Has a Very Compelling Clinical Profile

Performance of Omidubicel (Base Case) vs Current Transplants on Different Metrics (n = 83)



\*Attributes not shown for current transplants.



# Gamida Is Prepared for the Two Aspects of Payer Access: Coverage and Reimbursement

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## Payer Coverage

Published data supports that ~100% of U.S. payers anticipate covering one-time therapies, with curative intent

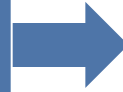
## Reimbursement

Gamida has a good understanding of the Reimbursement approach that payers will take upon omidubicel FDA approval

# We Will Be Prepared for the Potential Reimbursement Approaches

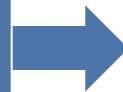
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## Clear Understanding of Patient Population Size



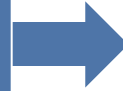
Market is well defined, and payers can calculate their potential covered lives

## Identified Centers of Excellence (COE) Who Will Use Omidubicel



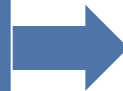
We agree there needs to be COEs:  
70 centers make up 80% of transplants

## Therapy Carve Out



Initial payer market research identified carve out as the most likely reimbursement approach at time of approval. We will establish the appropriate codes for carve outs

## Case Agreements Incorporating New Therapy\*



We will provide the payers and hospitals with the data they need for their confidential discussions

\*Before contract is updated.

# Our Goal Is to Bring Omidubicel to Every Appropriate Patient



## Pre-Infusion and Reimbursement Support

### Prior to infusion:

- Cord blood unit selection
- Benefits verification
- Assistance with prior authorization process



## Patient Coverage Support

### Assistance for patients who are:

- Uninsured
- Underinsured or inadequate insurance



## Travel and Housing Resources

Patients and caregivers travel and housing support services



## Claims Appeals

Support if a claim is denied and requires an appeal

# Manufacturing Readiness On Track to Support Potential 2H21 Launch

- Anticipate initial commercial supply to be produced by Lonza
  - ✓ Technology transfer completed
- Scalable, Gamida Cell-owned manufacturing facility can further enable reliable, consistent supply
  - ✓ Construction complete
  - Validation expected to be complete by year-end 2020



Photos of Gamida Cell-owned facility.

# Omidubicel Key Takeaways

- Potential to be first FDA-approved bone marrow transplant graft
- Compelling clinical profile to date
  - Unprecedented time to neutrophil engraftment
  - Generally well-tolerated
  - Reduced hospitalization time and decreased risk of infection
- Initiation of rolling BLA submission anticipated in 4Q20
- Pre-commercial activities underway for potential 2H21 launch



**After receiving a bone marrow transplant with omidubicel in 2011, Stacey remains cancer free**



# GDA-201

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

gamida ell

A person wearing a motorcycle helmet and jacket riding a motorcycle on a road.

## Meet Wayne

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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.”*

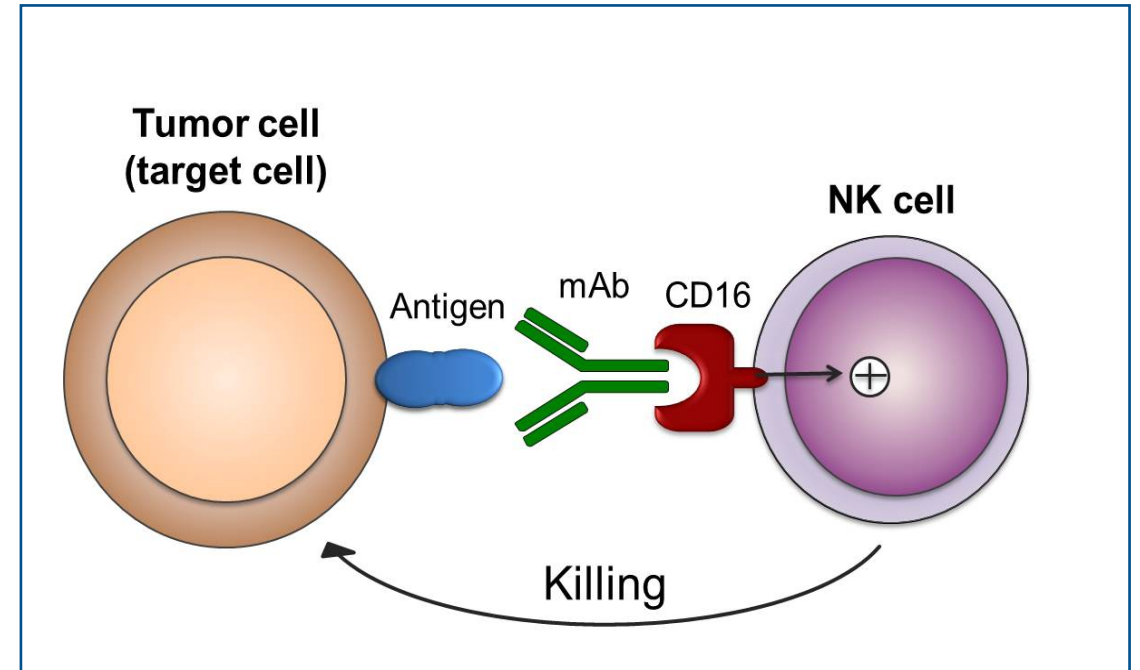
This is one patient and results may not be indicative. Omidubicel is investigational and safety and efficacy have not been established by any agency.

# Putting NK Cells to Work Using Our NAM Technology Platform

## Benefits of NK Cells

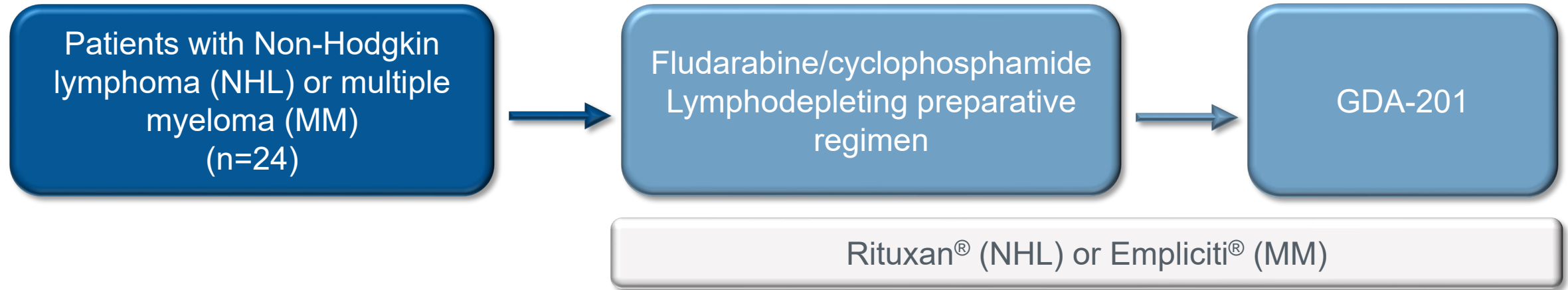
- Natural killer (NK) cells infusion is a promising immune therapy for cancer
  - No HLA matching required
  - Synergy with antibodies
  - Potential for off-the-shelf therapy
- Expansion is necessary to obtain clinically meaningful doses with retained cell function

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



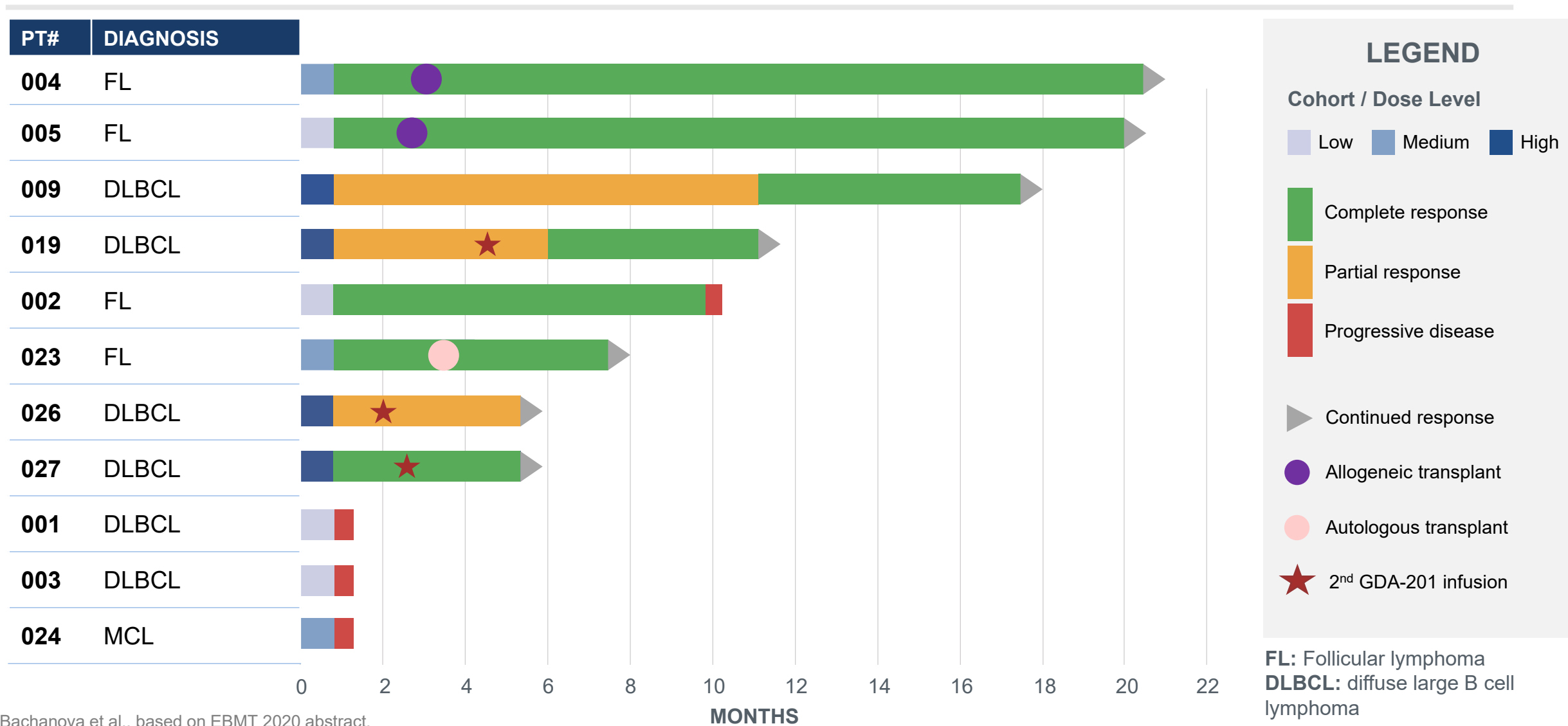
# Phase 1 Study of GDA-201 in Patients with Non-Hodgkin Lymphoma and Multiple Myeloma

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

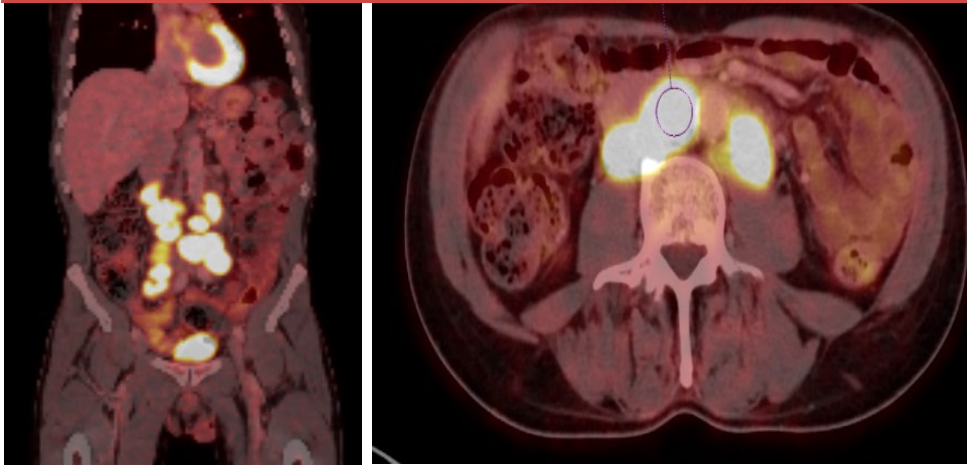
# GDA-201 Is Highly Active in Non-Hodgkin Lymphoma



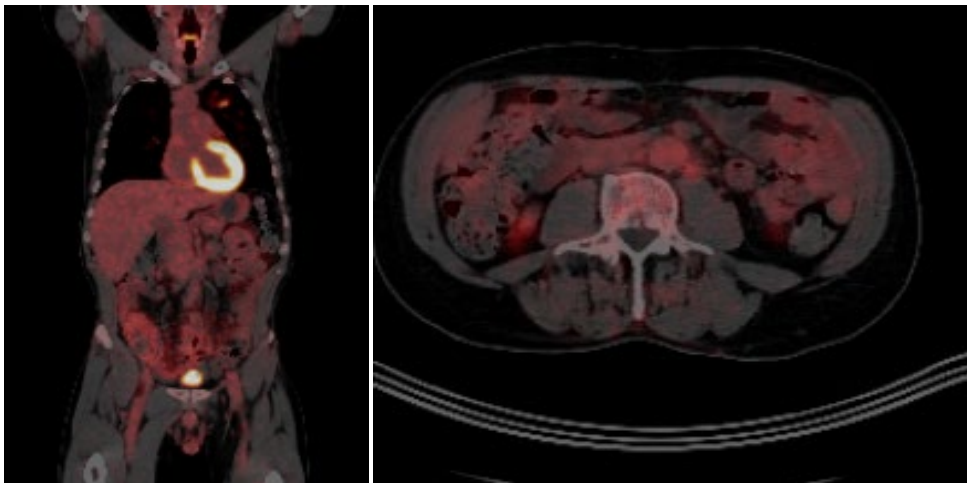


# Patient 009

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 Phase 1 Study: Key Takeaways

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## Promising early clinical activity

- 7 complete responses, 1 partial response among 11 patients with heavily pre-treated NHL
- Activity observed in patients with DLBCL
- Maximum target dose achieved

## Generally well tolerated

- No dose limiting toxicities
- No graft vs. host disease (GvHD)
- No tumor lysis syndrome
- No neurotoxicity

**Data support Phase 1/2 multi-center, multi-dose study in NHL**

Bachanova et al. EBMT 2020 abstract. Bachanova et al. ASH 2019.

# NK Could Be the Next Disruptive Cell Therapy

CAR-T Benefits	CAR-T Limitations
<ul style="list-style-type: none"><li>• Dramatically changed treatment paradigm</li><li>• Demonstrated long-term clinical benefits</li></ul>	<ul style="list-style-type: none"><li>• Complex manufacturing process</li><li>• Side effects, including cytokine release syndrome</li><li>• Many patients aren't fit enough for treatment</li></ul>

Precedent for Rapid Path to Approval for Cell Therapies with Significant Clinical Benefit  
YESCARTA® IND to BLA: 27 Months

2014	2015	2016	2017
IND Submitted (Dec)	ZUMA-1 Ph1 Study First Patient Enrolled (Apr) ZUMA-1 Ph2 Pivotal Study Opens (Nov)	ZUMA-1 Pivotal Study Interim Analysis (Nov)	ZUMA-1 Pivotal Study Topline Primary Analysis (Feb) BLA Submission Completed (Mar) FDA Approval (Oct)

# GDA-201: Encouraging Clinical Activity Supports Continued Development

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## 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
- File IND in 1H21
- 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

# Expected 2020-2021 Milestones

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## Omidubicel

- ✓ Report topline data from the Phase 3 study in 2Q20
- ❑ Present data from the Phase 3 study at a medical meeting in 4Q20
- ❑ Initiate rolling BLA submission in 4Q20
- ❑ Report additional data from the Phase 1/2 study in patients with severe aplastic anemia in 4Q20
- ❑ Launch omidubicel in 2021\*

## GDA-201

- ✓ Present additional data from the Phase 1 study in 1H20\*\*
- ❑ Submit IND in 1H21
- ❑ Initiate a Phase 1/2 clinical study in NHL in 2021

\*Pending BLA submission, acceptance and subsequent FDA approval.

\*\* Data accepted for EBMT2020, which was to be held in March and then postponed due to COVID-19.

# Financial Snapshot

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- June 30<sup>th</sup> cash position: \$88.6 million\*
- Cash supports capital needs into 2H21\*
- Approximately 90 employees

\*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 undertaken.

# We Are Inspired to Cure

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## NAM Platform

Potential to expand any cell type, including stem cells and NK cells

## Omidubicel

Preparing for BLA submission and potential launch

## GDA-201

Promising early clinical activity in heavily pre-treated patients with lymphoma

## Looking Ahead

Potential to launch first-ever FDA-approved bone marrow transplant graft





# Inspired to Cure



September 2020