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Inspired to Cure

January 2020

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Inspired to Cure with Next Generation Cell Therapies

We are pioneering new standards of care for patients with hematological malignancies

Omidubicel

Holds curative potential for patients in need of bone marrow transplant

GDA-201

Harnesses the curative power of NK cells to treat non-Hodgkin lymphoma

Late-Stage Company with a Phase 3 Data Readout in 1H20

CANDIDATE	PRECLINICAL	PHASE 1	PHASE 2	PHASE 3	UPCOMING MILESTONES
OMIDUBICEL					
High-Risk Hematologic Malignancies	**FDA Breakthrough Designation**				❑ Topline Data 1H20 ❑ BLA submission 2H20
Severe Aplastic Anemia					❑ Additional Data 2H20
GDA-201					
Non-Hodgkin Lymphoma, Multiple Myeloma					❑ Additional Data 1H20 ❑ IND Submission 2H20

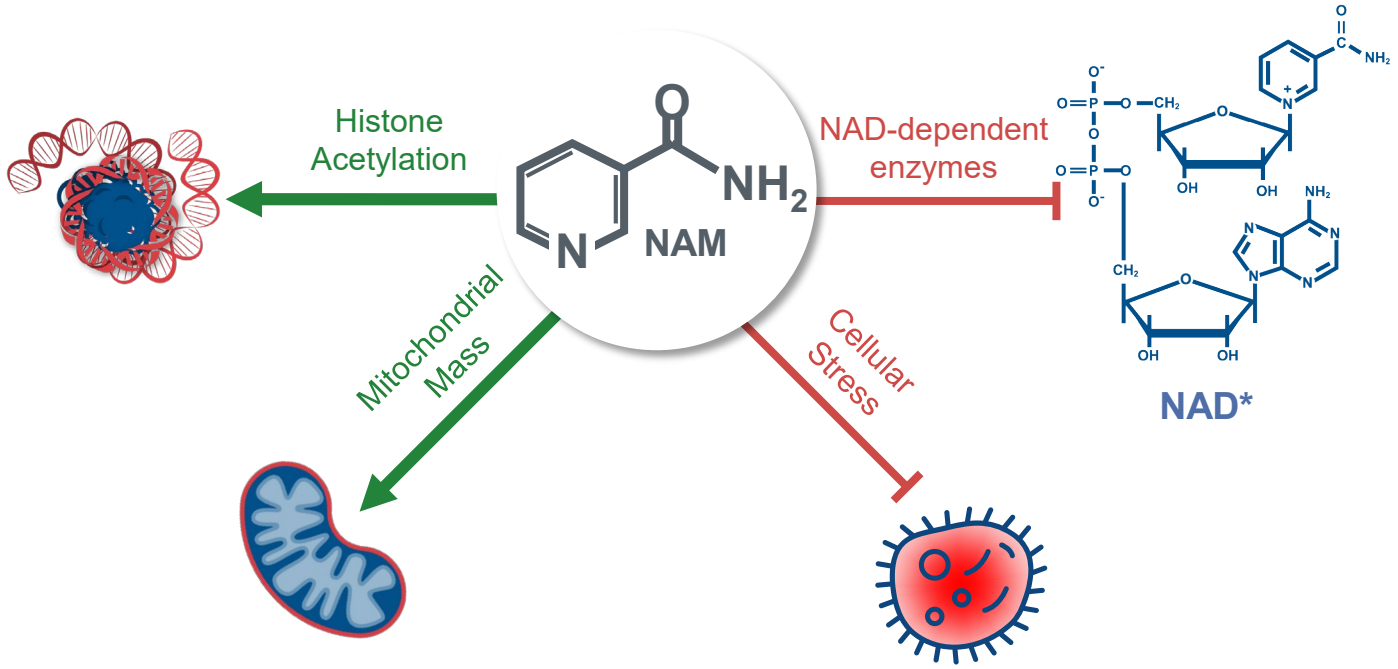
*The Aplastic Anemia Investigational New Drug (IND) application is currently filed with the FDA under the brand name, CordIn, which is the same investigational development candidate as omidubicel.

Technology Platform Designed to Enhance the Number and Functionality of Allogeneic Donor Cells

NAM has the potential to expand any cell type, including stem cells and natural killer (NK) cells

Importance of NAM

- Plays a key role in metabolic reprogramming of cells
- Preserves cellular functionality and phenotype during expansion



Omidubicel

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

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Meet Stacey

Stacey participated in the first clinical study of omidubicel at Duke University Medical Center after being diagnosed with AML. She is now eight years post-transplant.

"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.

Need for Innovation in Bone Marrow Transplant

Patients with hematologic malignancies eligible for transplant annually

(Annually in U.S.)

Allogeneic stem cell transplants

~40%

of patients don't receive a transplant

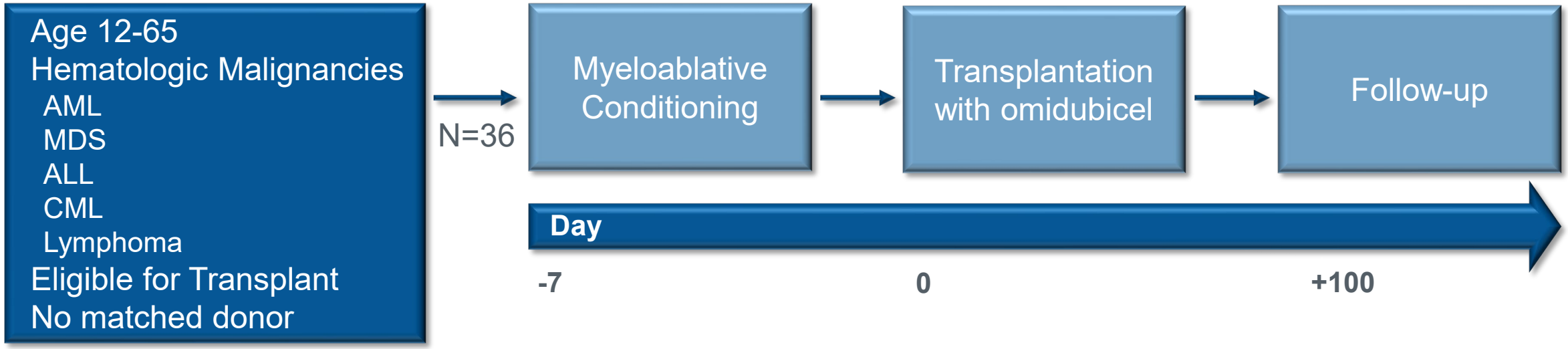
Internal Market Research Studies and Data Analysis, https://bloodcell.transplant.hrsa.gov/about/general_faqs/index.html.

There Is a Significant Population That Omidubicel Can Address

~13,000 patients with hematologic malignancies are eligible for transplant annually

		Patients	Challenges		Unmet Need
Omidubicel opportunity	Not Matched / Not Referred	5,200	• Numerous including: • Access to care • Access to graft source	→	**Expand Access**
	Matched Unrelated	5,200	• Availability of graft source • Quality of graft source • Time to engraftment • Risk of GvHD • Potency of GvL effect	→	**Improve Outcomes**
	Haploidentical				
	Cord Blood				
	Matched Related	2,600	• Availability of sibling donor		

Phase 1/2 Study of Omidubicel in Hematologic Malignancies



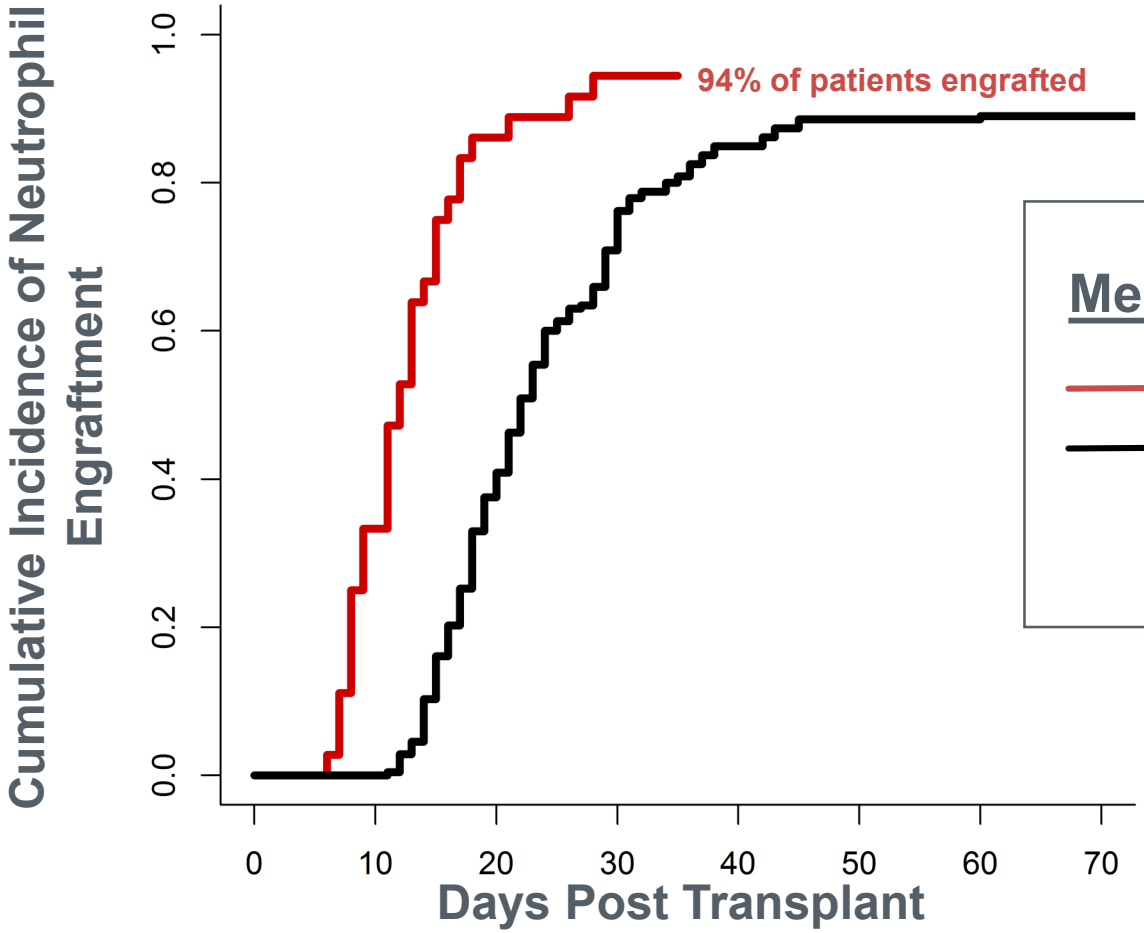
13 sites: U.S., EU, Asia

- **Primary endpoint:** Neutrophil engraftment
- **Secondary endpoints:** Platelet engraftment, acute GvHD, chronic GvHD, infections, hospitalization, non-relapse mortality, overall survival, disease-free survival

Clinicaltrials.gov identifier NCT01221857.

Unprecedented Time to Neutrophil Engraftment with Omidubicel

~50% reduction in time to engraftment is associated with clinical benefit



Median days to engraftment

Omidubicel: 11 days (95%CI: 9-13d)

CIBMTR Cohort: 21 days (95%CI: 20-23d)

p < 0.001

Journal of Clinical Oncology 2019 Feb 10;37(5):367-374.
Note: CIBMTR registry data of 146 patients who received myeloablative conditioning and unmanipulated cord blood transplantation.

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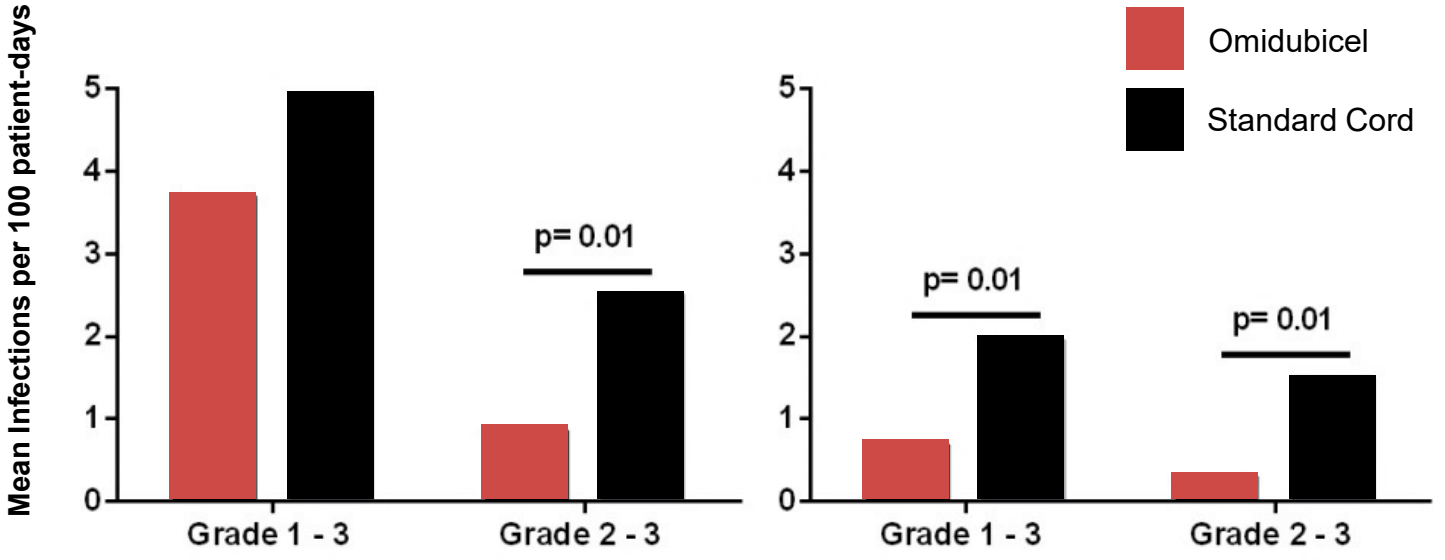
Rapid Engraftment Is Associated with Fewer Infections and Shorter Hospitalizations

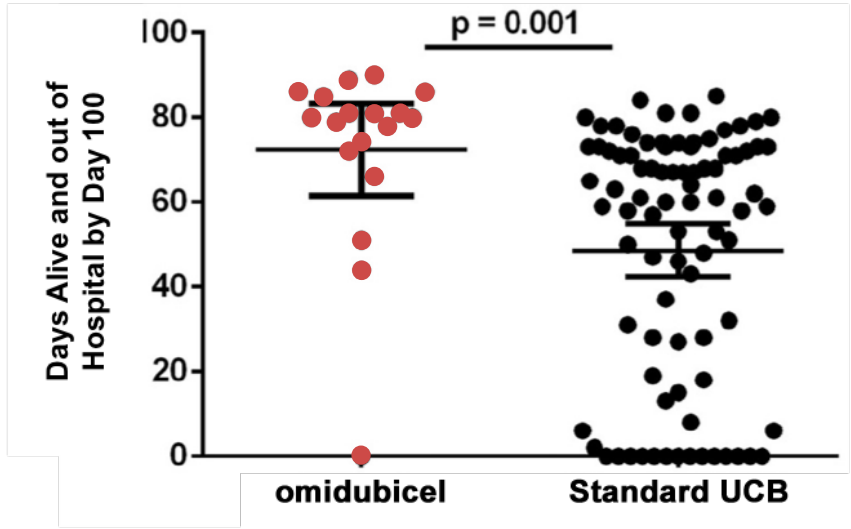
Infection



Total Infections

Bacterial Infections

Hospitalization



~20 fewer days in hospital with omidubicel

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

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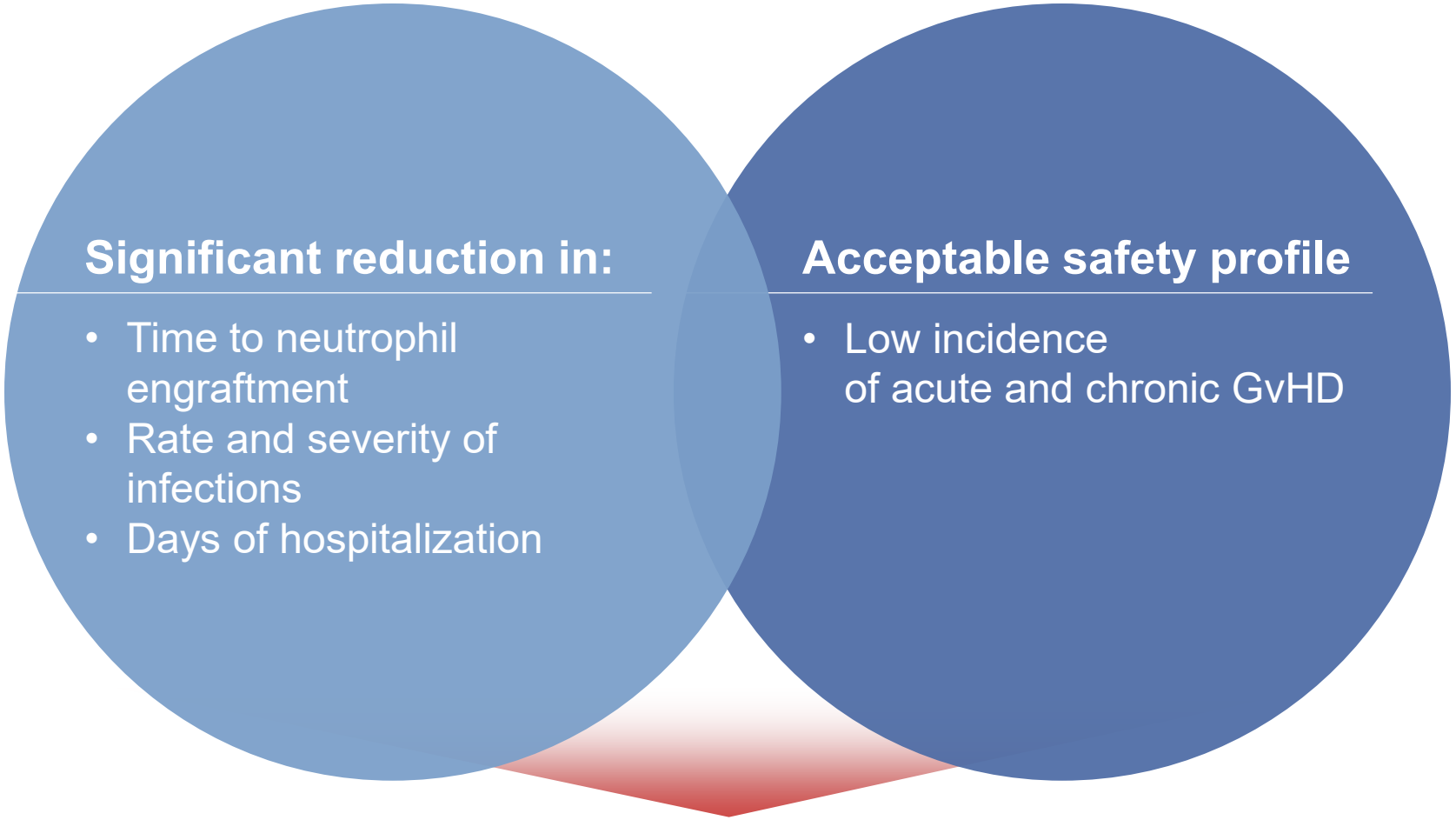
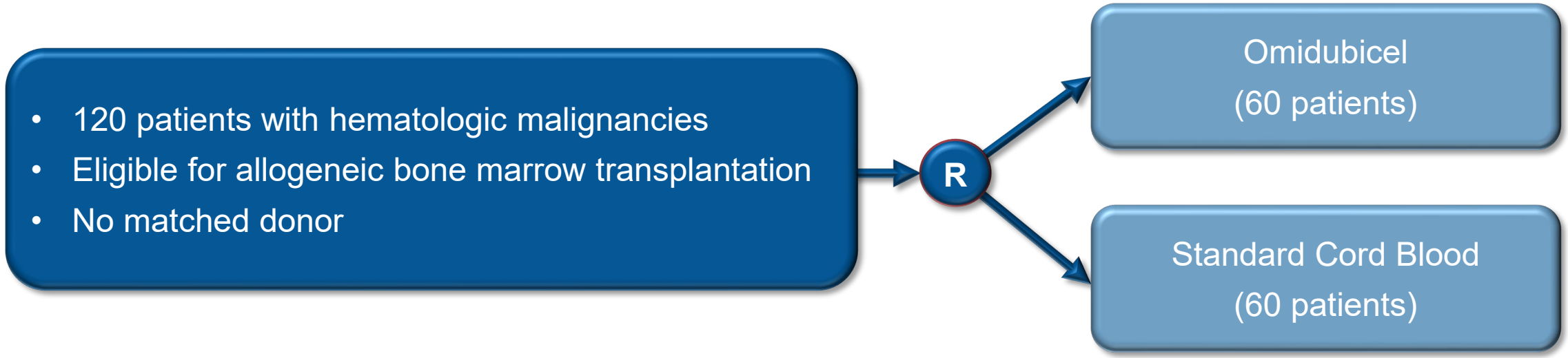
Omidubicel Phase 1/2 Study: Key Takeaways



FDA Breakthrough Therapy Designation

Journal of Clinical Oncology 2019 Feb 10;37(5):367-374.

Topline Data from Phase 3 Study Expected 1H20



Primary endpoint: Time to neutrophil engraftment

Secondary endpoints: Platelet engraftment, acute GvHD, chronic GvHD, infections, hospitalization, non-relapse mortality, overall survival, disease-free survival

- >50 sites globally
- Enrollment completed December 2019

Clinicaltrials.gov identifier NCT02730299.

Preparing for a Successful Omidubicel Launch

1. Engaging top transplant centers
2. Building patient and hospital support services
3. Working with payers to ensure reimbursement
4. Ensuring commercial manufacturing readiness

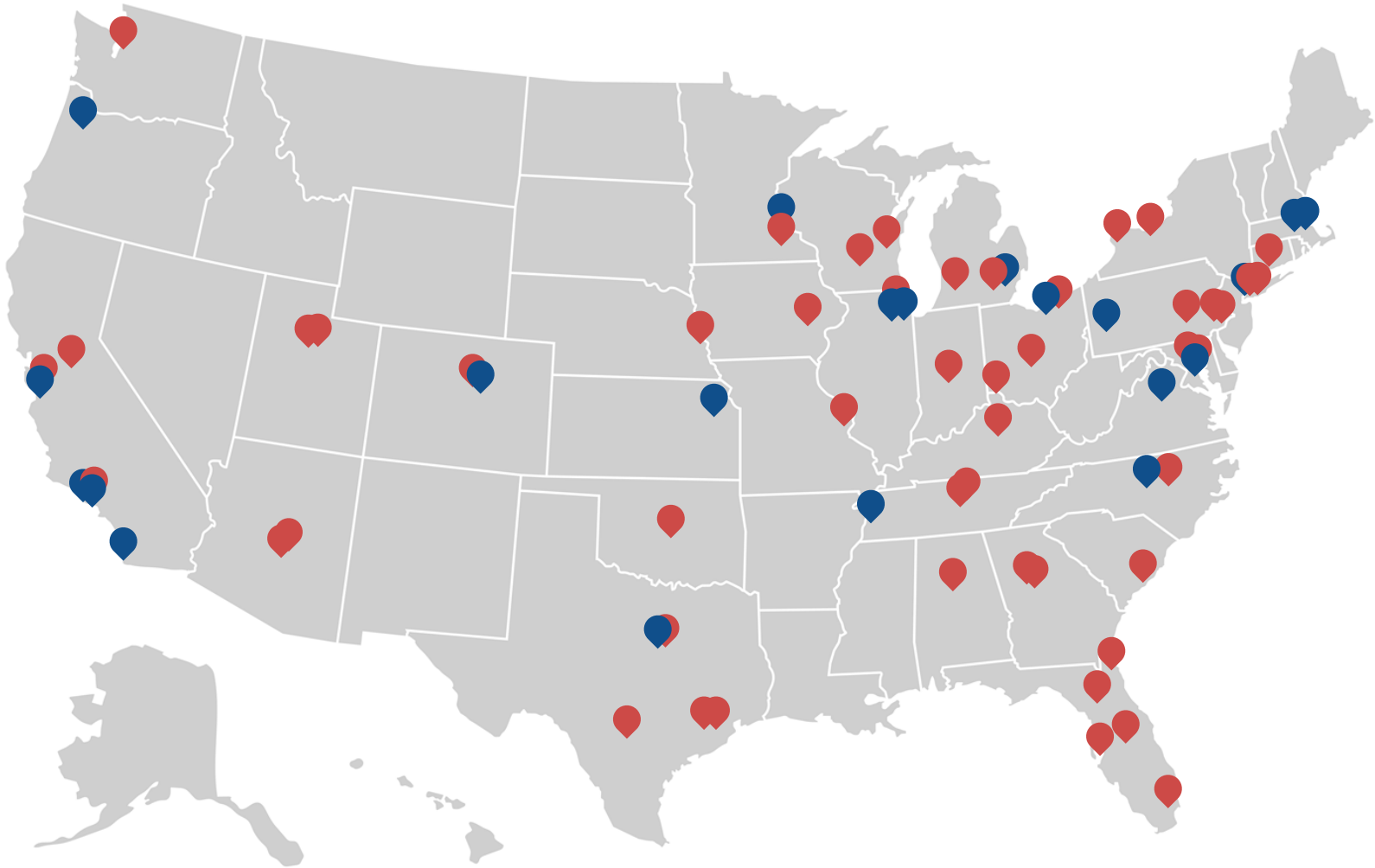
Preparing to submit Biologics License Application to FDA in 2H20*

*If data are positive.

Bringing Omidubicel to Patients

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

- Top treating site
- Top treating & omidubicel trial site



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Our Goal Is to Bring Omidubicel to Every Patient Who Needs It

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 appeal

Omidubicel Key Takeaways

- Unprecedented time to neutrophil engraftment and acceptable safety profile observed in Phase 1/2 clinical study
- Phase 3 enrollment completed with topline data expected 1H20
- Construction ongoing at two manufacturing facilities to ensure reliable commercial supply
- Pre-commercial activities underway to prepare for possible 2021 launch

GDA-201

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

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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.”

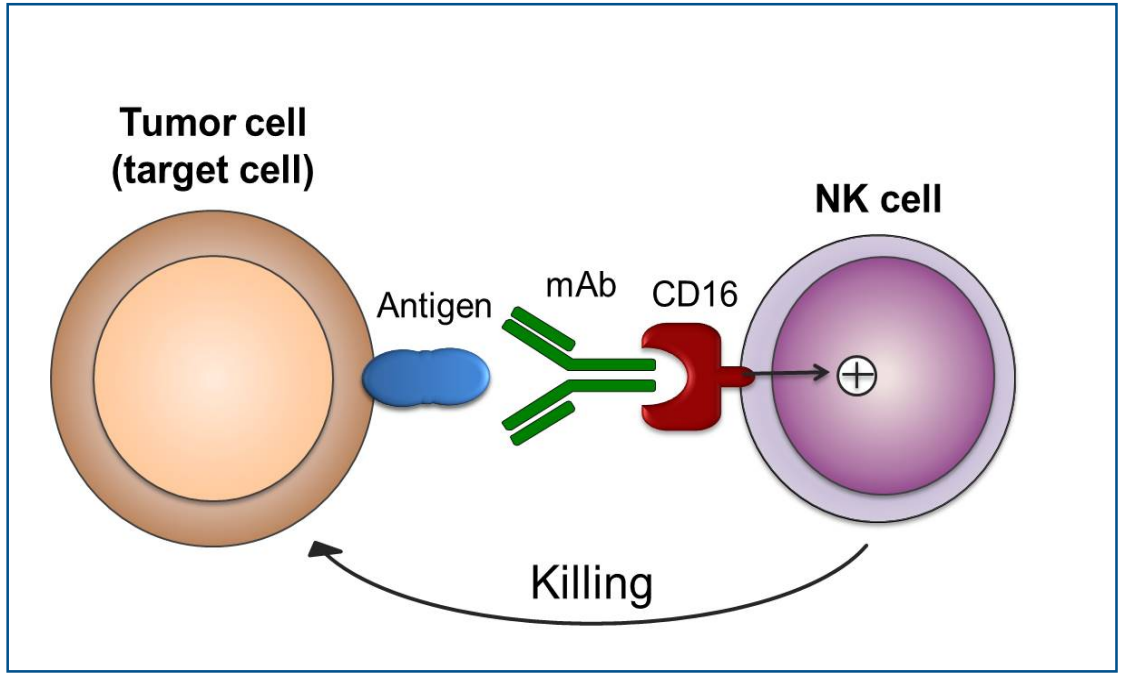
This is one patient and results may not be indicative. Omidubicel is investigational and safety and efficacy have not been established by any agency.

Putting NK Cells to Work Using Our NAM Technology Platform

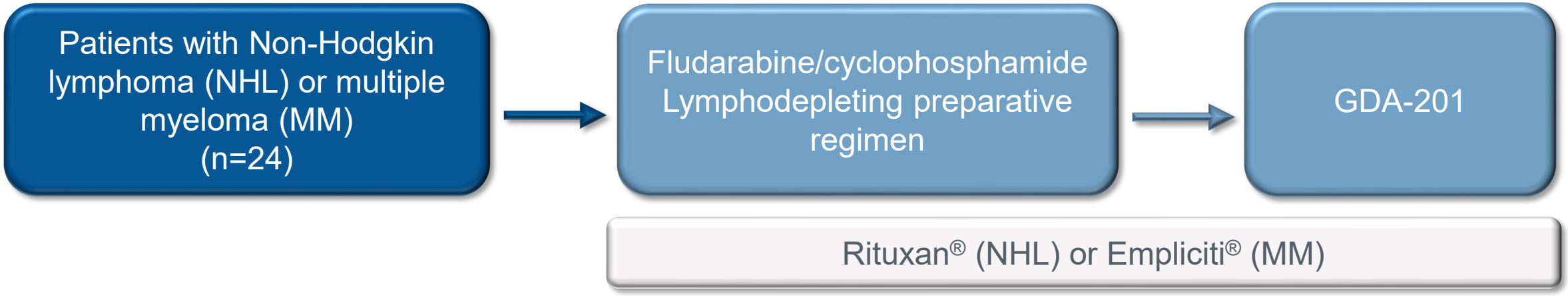
Benefits of NK Cells

- 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



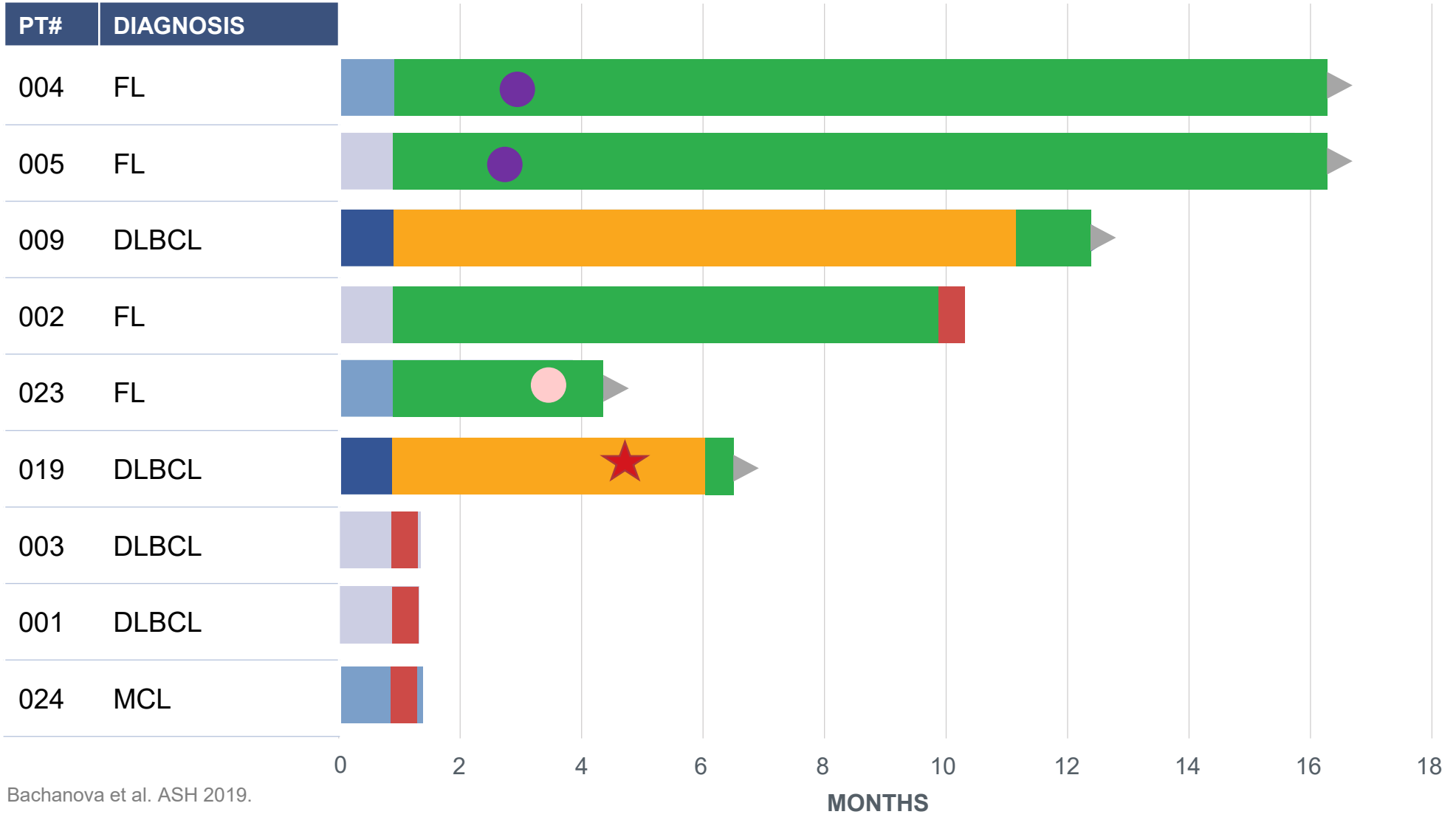
Phase 1 Study of GDA-201 in Patients with Non-Hodgkin Lymphoma and Multiple Myeloma



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

ClinicalTrials.gov Identifier NCT03019666.

GDA-201 Is Highly Active in Non-Hodgkin Lymphoma



LEGEND

Cohort /Dose Level

Low Medium High

Complete response

Partial response

Progressive disease

Continued response

Allogeneic transplant

Autologous transplant

2nd GDA-201 infusion

FL: Follicular lymphoma;
DLBCL: diffuse large B cell lymphoma

Bachanova et al. ASH 2019.

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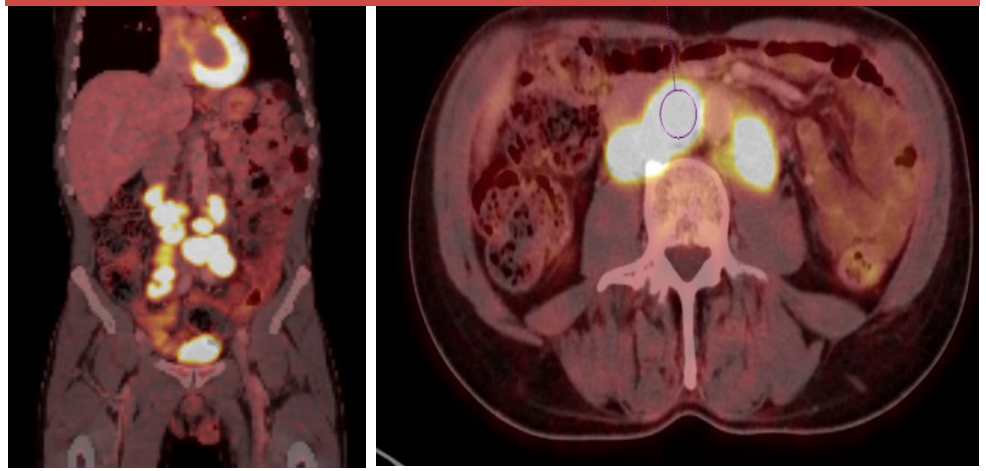
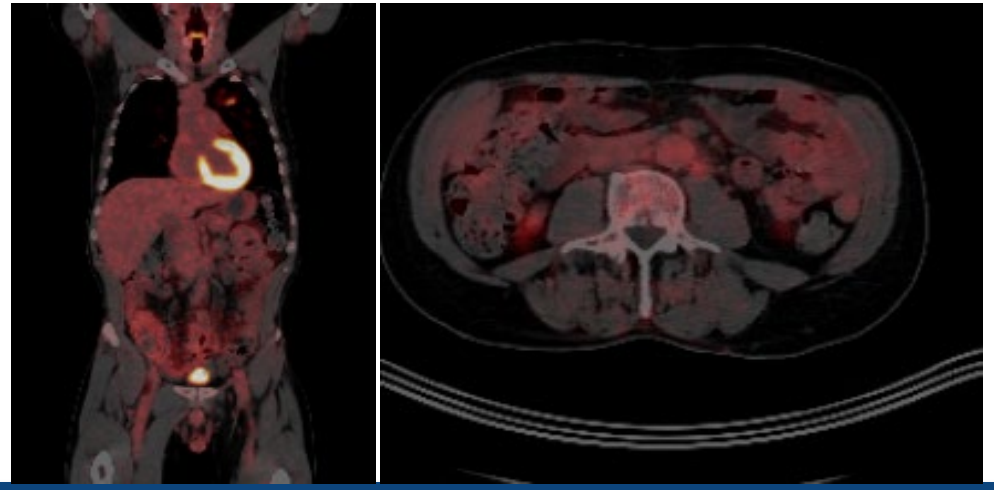
Patient 009

Pt 009: Baseline



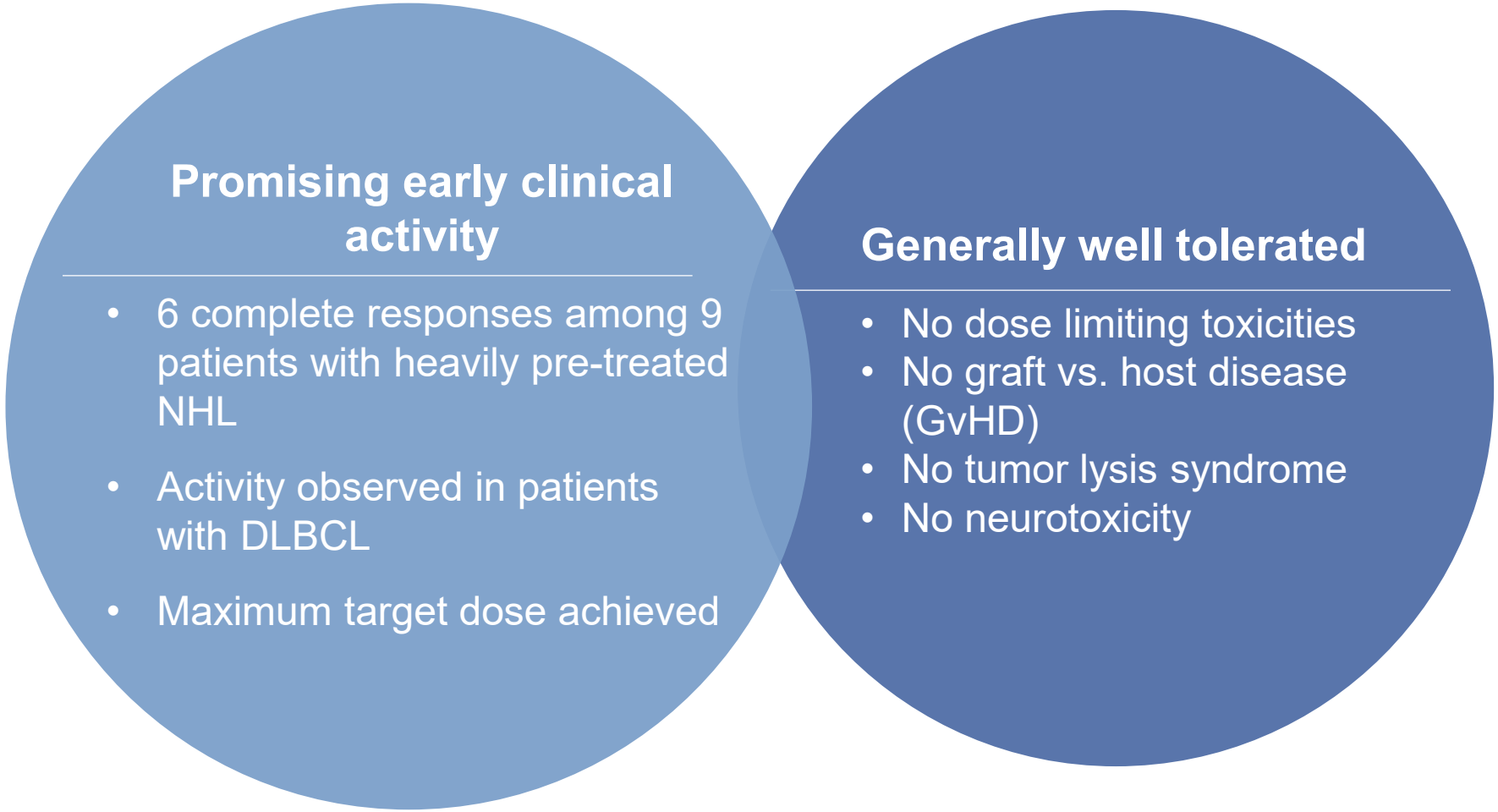
Pt 009: 6-month post GDA-201



- 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



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

Bachanova et al. ASH 2019.

NK Could Be the Next Disruptive Cell Therapy

CAR-T Benefits

- Dramatically changed treatment paradigm
- Demonstrated long-term clinical benefits

CAR-T Limitations

- Complex manufacturing process
- Side effects, including cytokine release syndrome
- Many patients aren't fit enough for treatment

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)

Expected 2020-2021 Milestones

Omidubicel

- ❑ Report topline data from the Phase 3 study in 1H20
- ❑ Present data from the Phase 3 study at a medical meeting in 2H20
- ❑ Submit BLA in 2H20*
- ❑ Report additional data from the Phase 1/2 study in patients with severe aplastic anemia in 2H20
- ❑ Launch omidubicel in 2021*

GDA-201

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

*If data are positive.

Financial Snapshot

- Total cash position of approximately $56 million (unaudited) as of December 31, 2019
- Cash supports capital needs into 4Q20*
- Approximately 90 employees

*Cash runway guidance is based on the company's current operational plans and excludes any additional funding that may be received or business development activities that may be undertaken.

We Are Inspired to Cure

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

Omidubicel — Preparing for Phase 3 data readout and BLA submission

GDA-201 — Stunning early clinical activity in heavily pre-treated patients with lymphoma

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

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