

# **Committed to Cures**

Pioneering next-generation cell therapies for patients with cancer and other serious diseases

August 2021

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This presentation contains forward-looking statements as that term is defined in the Private Securities Litigation Reform Act of 1995, including with respect to timing of initiation and progress of, and data reported from, the clinical trials of Gamida Cell's product candidates, anticipated regulatory filings (including the submission of the BLA for omidubicel to the FDA), commercialization planning efforts, the potentially life-saving or curative therapeutic and commercial potential of omidubicel, and Gamida Cell's expectations regarding its projected cash runway. Any statement describing Gamida Cell's goals, expectations, financial or other projections, intentions or beliefs is a forward-looking statement and should be considered an at-risk statement. Such statements are subject to a number of risks, uncertainties and assumptions, including those related to the impact that the COVID-19 pandemic could have on our business, and including the scope, progress and expansion of Gamida Cell's clinical trials and ramifications for the cost thereof; clinical, scientific, regulatory and technical developments; and those inherent in the process of developing and commercializing product candidates that are safe and effective for use as human therapeutics, and in the endeavor of building a business around such product candidates. In light of these risks and uncertainties, and other risks and uncertainties that are described in the Risk Factors section and other sections of Gamida Cell's Annual Report on Form 20-F, filed with the Securities and Exchange Commission (SEC) on March 9, 2021, as amended, and other filings that Gamida Cell makes with the SEC from time to time (which are available at <u>http://www.sec.gov</u>), the events and circumstances discussed in such forward-looking statements may not occur, and Gamida Cell's actual results could differ materially and adversely from those anticipated or implied thereby. Although Gamida Cell's forward-looking statements reflect the good faith judgment of its management, these statements are based only on facts and factors currently known by Gamida Cell. As a result, you are cautioned not to rely on these forward-looking statements.



Committed to Cures: Near-term Promise and Long-term Potential

### Proprietary nicotinamide (NAM) cell expansion platform enables a continuing series of advanced cell therapy programs



**Readying for** commercialization

#### Omidubicel

- Preparing for BLA submission in 4Q21\*
- Potential to be first FDAapproved cell therapy for bone marrow transplantation



# program in NK cells

#### **GDA-201**

- Innate NK cell product with positive Phase 1 data
- Advancing to Phase 1/2 in ٠ NHL in 2H21



**Opening new frontiers in** cancer immunotherapy

#### GDA-301/401/501/601

- **Proof-of-concept for CAR** and CRISPR editing
- Evidence of increased cytotoxicity in preclinical studies
- Potential in blood cancers and solid tumors



## Our NAM-Enabled Advanced Cell Therapy Programs

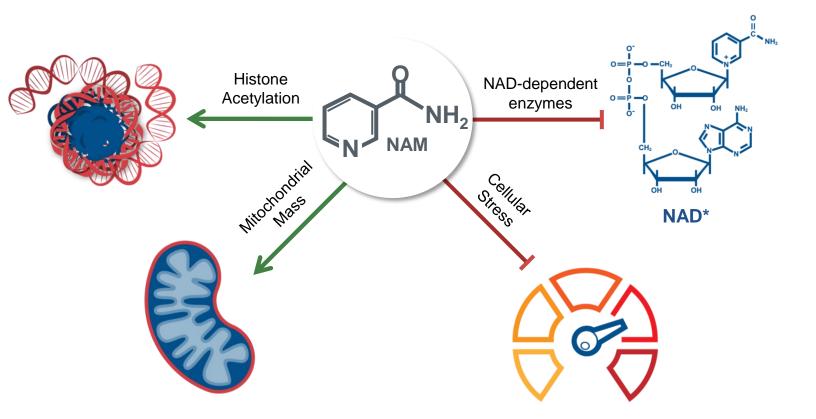




# Pipeline Built on Proprietary NAM Platform Technology

### **NAM Platform Technology**

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





# Omidubicel

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





# **Our Inspiration: Focusing on Cures**

Stacey participated in the first clinical study of omidubicel at Duke University Medical Center after being diagnosed with AML.

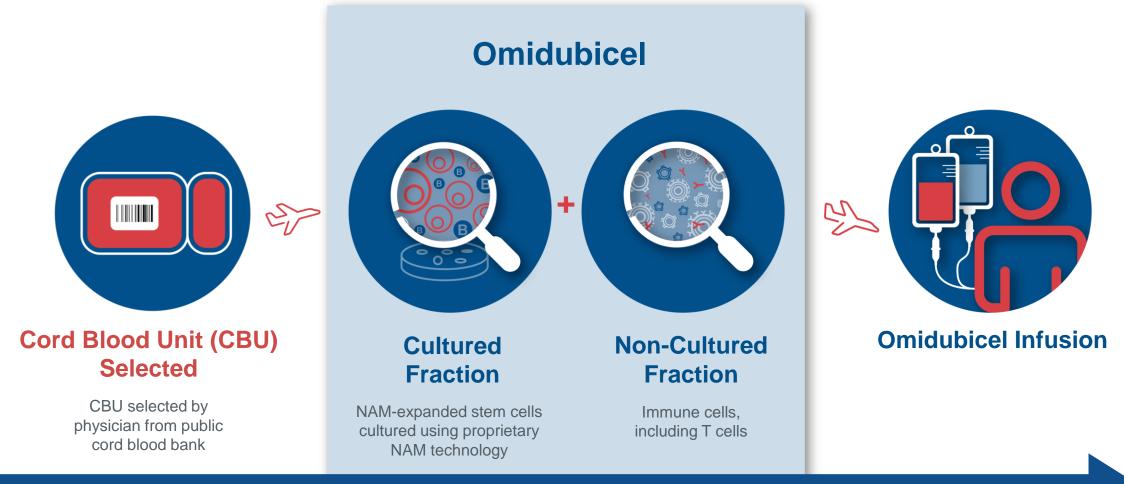
She has been cancer-free since her bone marrow transplant in 2011.

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



Scalable manufacturing and delivery of omidubicel



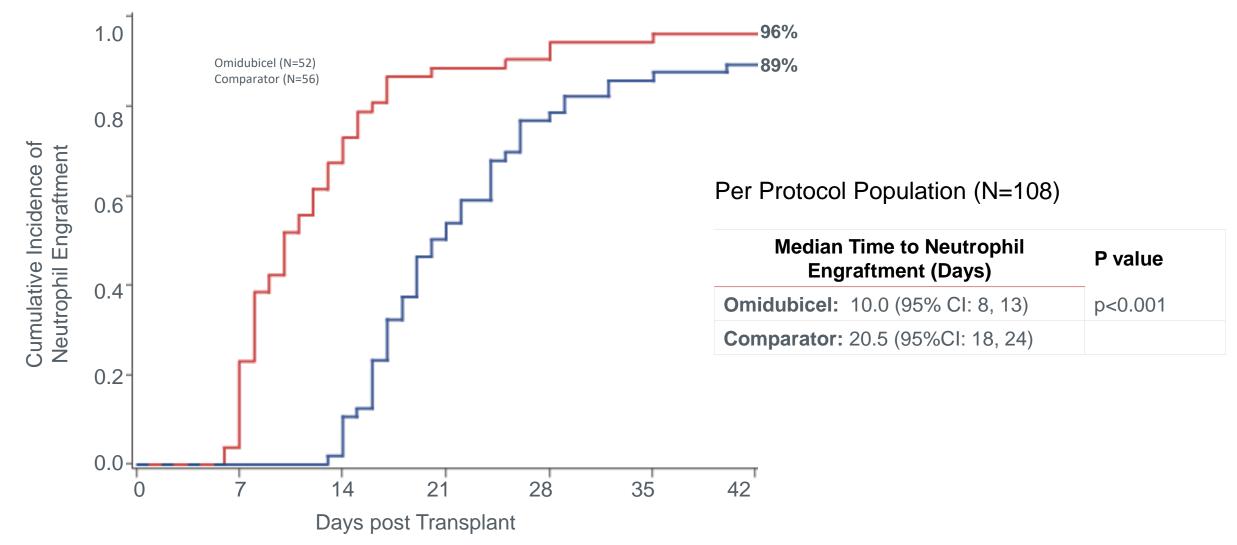
### Global, Randomized Phase 3 Study Primary Endpoint: Omidubicel Significantly Reduced Time to Engraftment

- 125 patients randomized at 33 sites
  - Age 12-65
  - High-risk hematologic malignancies
  - Eligible for allogeneic bone marrow transplantation
  - No matched donor
- 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)	

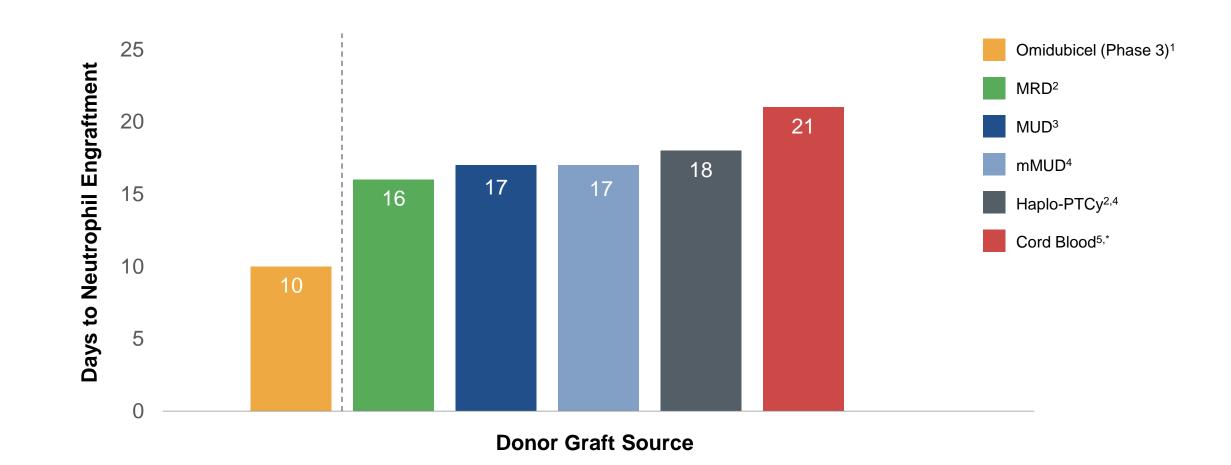


## **Cumulative Incidence of Neutrophil Engraftment**



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

# Omidubicel has Shortest Neutrophil Engraftment Time Compared to Published Results for Other HSCT Donor Sources



1. Clinica trial; 2. Salvatore D, et al. Haematologica. 2018; 103(8):1317-28; 3. Mary M. Horowitz, MD, MS. Haploidentical Transplantation:

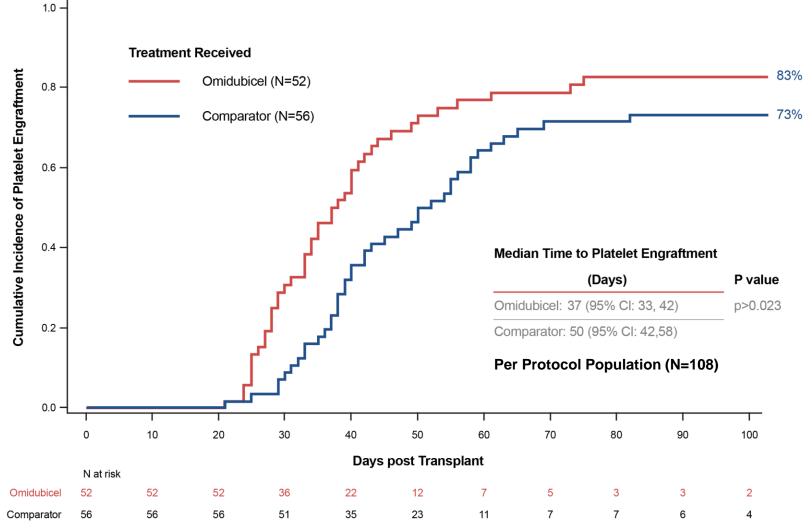
The Answer to our Donor Problems? CIBMTR, Medical College of Wisconsin. January 2017; 4. McCurdy SR, et al. Adv Hematol. 2015; 1-9.; 5. Horwitz ME, et al. J Clin Oncol. 2018; 37(5):367-74. \* Results represent double-cord transplants



Omidubical is investigational and cafety and officaey have not been established by any agoney.

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### Phase 3 Secondary Endpoints: Day 100 Platelet Engraftment

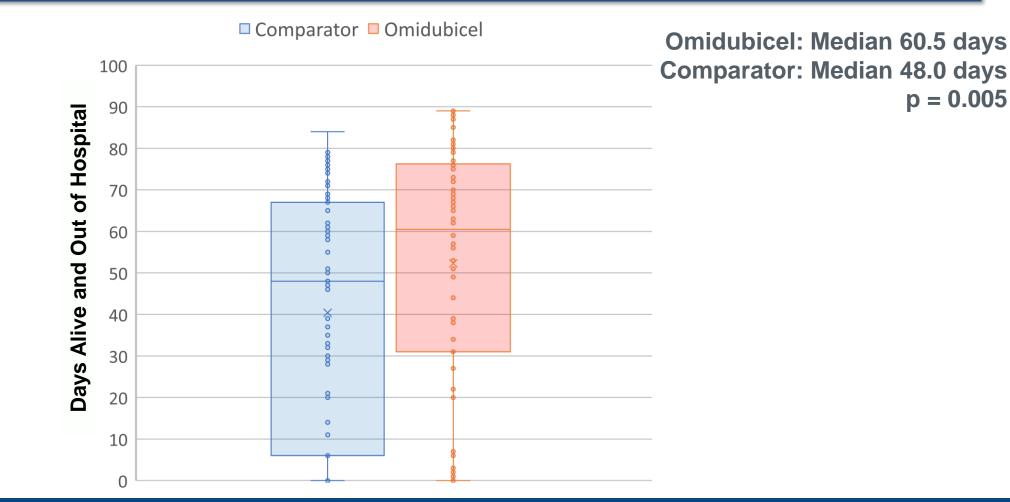


Per protocol population: received transplantation with omidubicel or comparator per protocol.



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

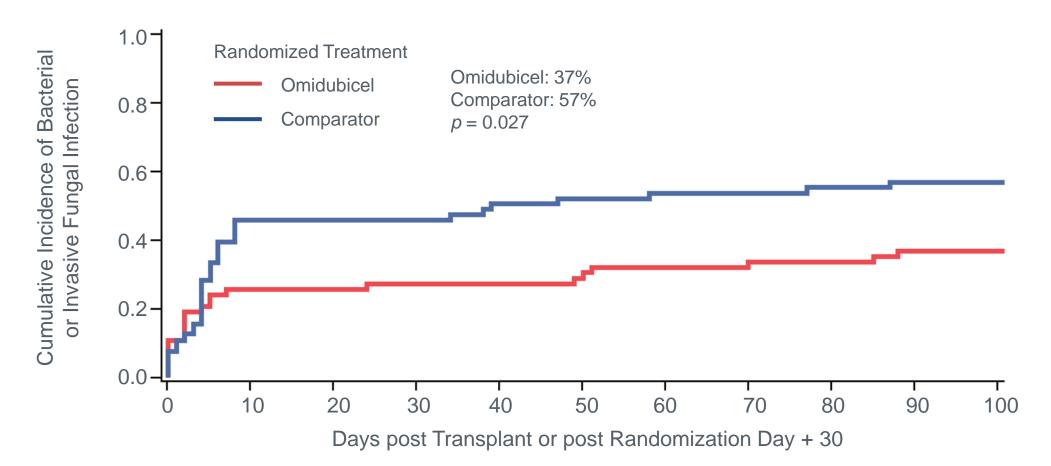
#### ALIVE AND OUT OF HOSPITAL IN FIRST 100-DAYS



Population: ITT

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

#### INCIDENCE OF SERIOUS BACTERIAL OR FUNGAL INFECTIONS BETWEEN RANDOMIZATION AND 100 DAYS<sup>1</sup>

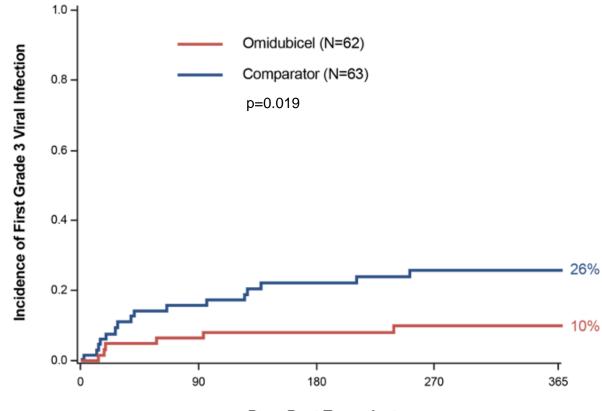


1. Proportion (%) of patients with any grade 2-3 bacterial infection or invasive fungal infection between randomization and 100 days following transplantation

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### Fewer Viral Infections in Recipients of Omidubicel



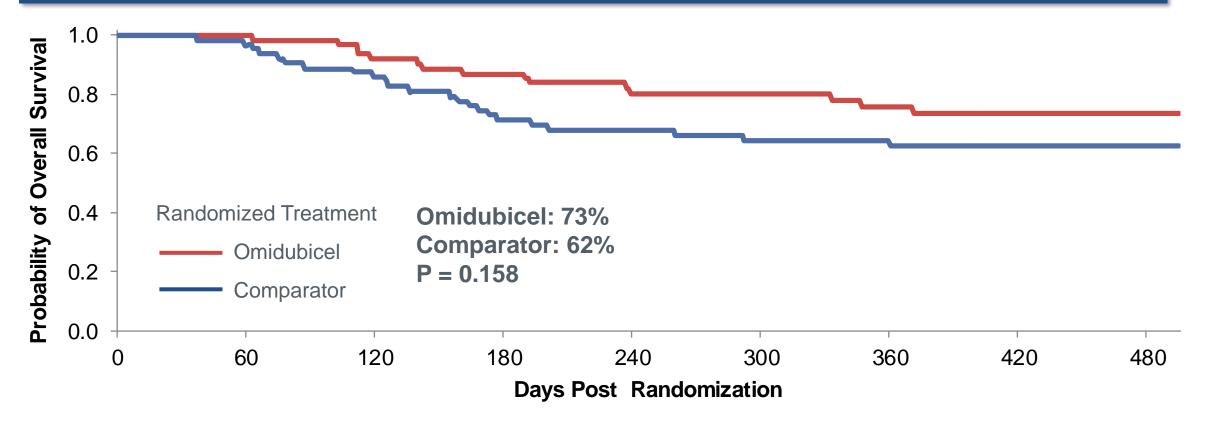
Days Post-Transplant





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

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



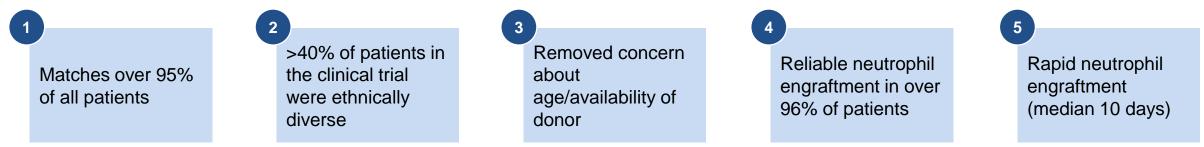
# Omidubicel

Commercial Potential and Launch Readiness



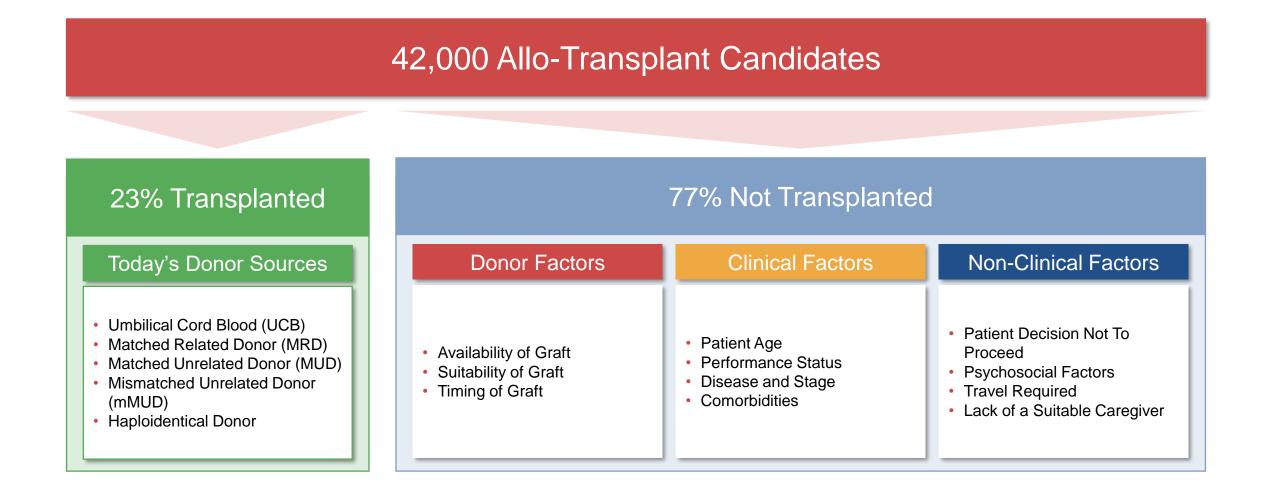
# Omidubicel may be the next-generation cell therapy for allogeneic transplant that delivers a universal solution for a cure

#### Supporting Reasons to Believe:



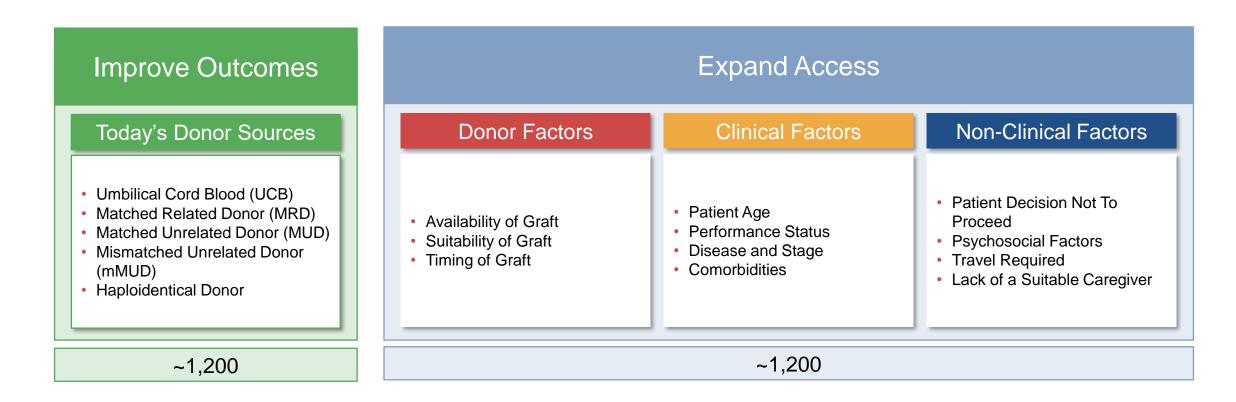


# Due To Roadblocks Along The Way, Only 23% Of Patients Ultimately Receive An HSCT





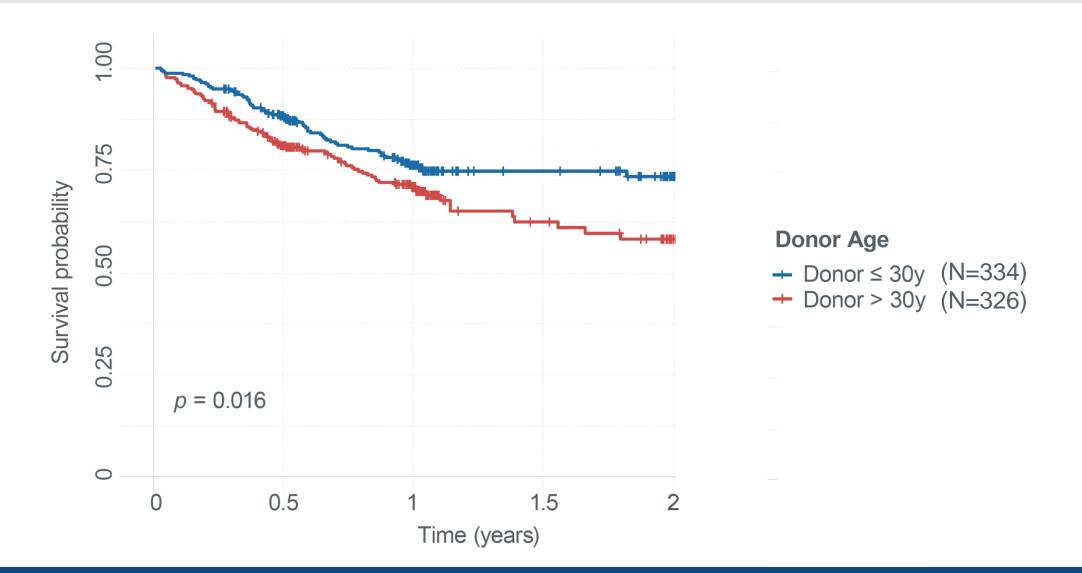
# In market research, physicians indicated that omidubicel would expand access and improve outcomes



20 Source: ZS Associates, Quantitative Demand Study, n=107 US transplanters; CIBMTR transplant volume. Numbers estimated in 2025 based on peak share.

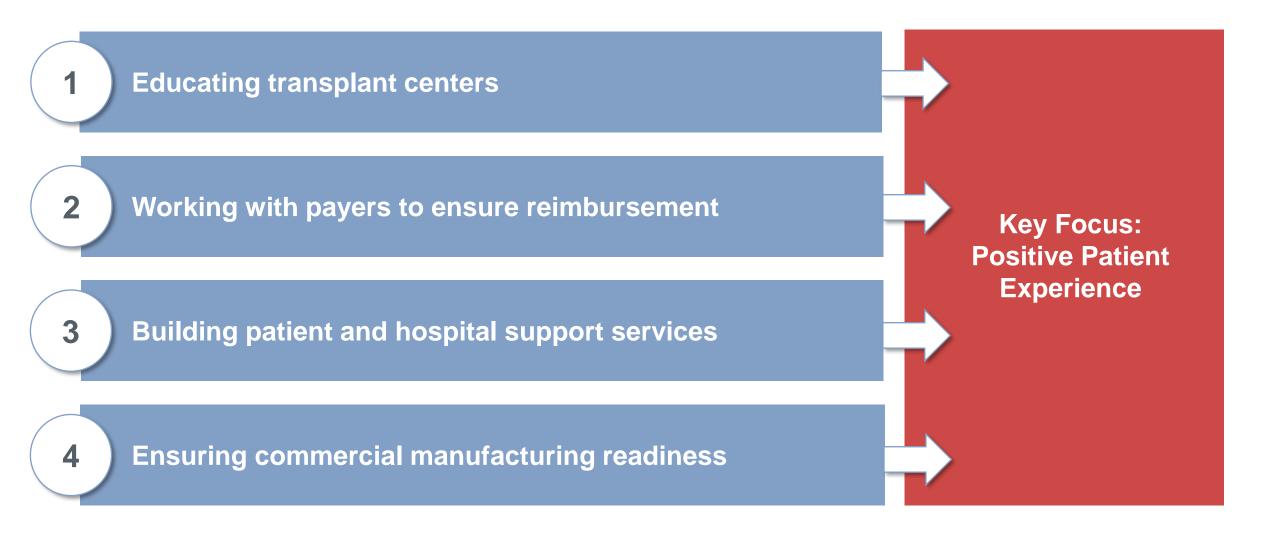


### Overall Survival with Follow up is Associated with Donor Age

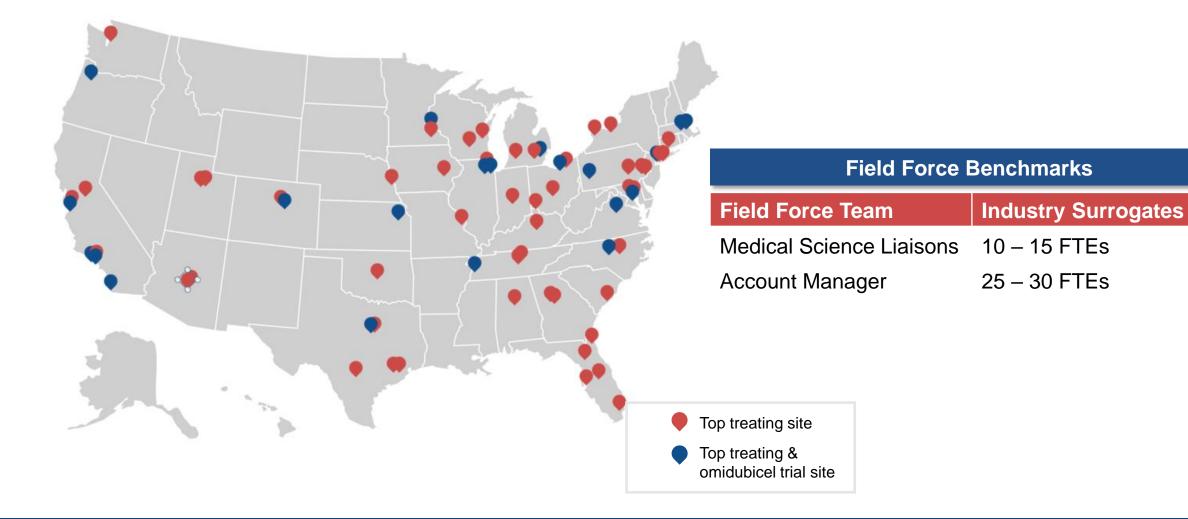




Key Commercial Activities and Infrastructure Build-out Are Underway to Prepare for a Successful Omidubicel U.S. Launch



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



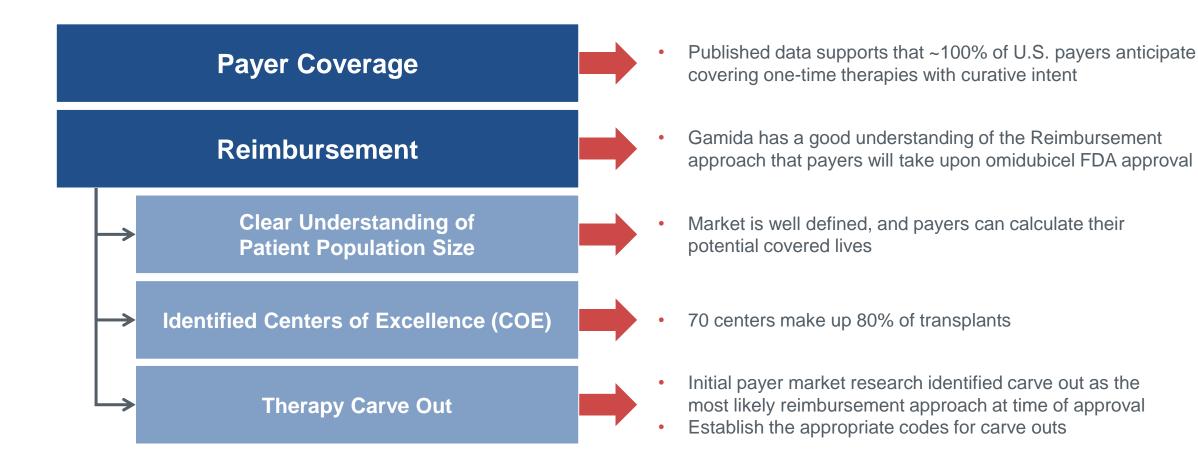
Approximately 70 transplant centers account for ~80% of bone marrow transplants in U.S.





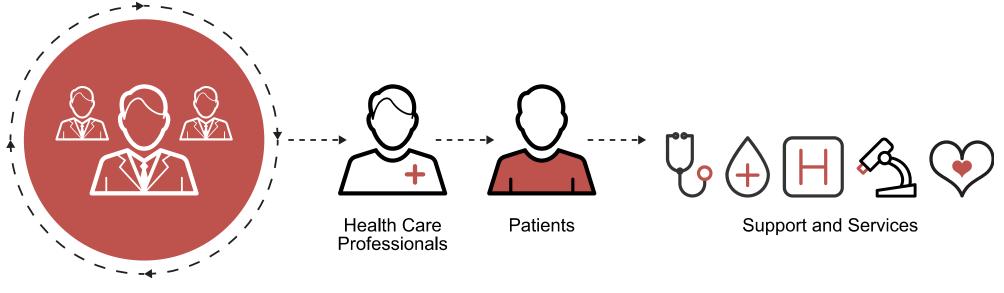
#### **Preparing for Reimbursement**

# Gamida Cell has conducted research to understand the reimbursement approaches that payers will take if omidubicel receives FDA approval



### 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



Single Point of Contact

- We are a support and solutionsoriented 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 who 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.



# **NK Cell Pipeline**

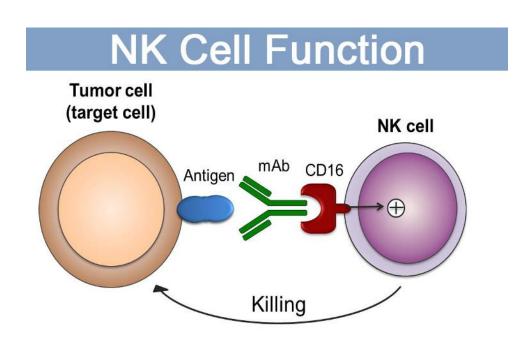
Harnessing Innate Immunity Using Natural Killer (NK) Cells to Treat Cancer



# Putting NK Cells to Work Using Our NAM Technology Platform

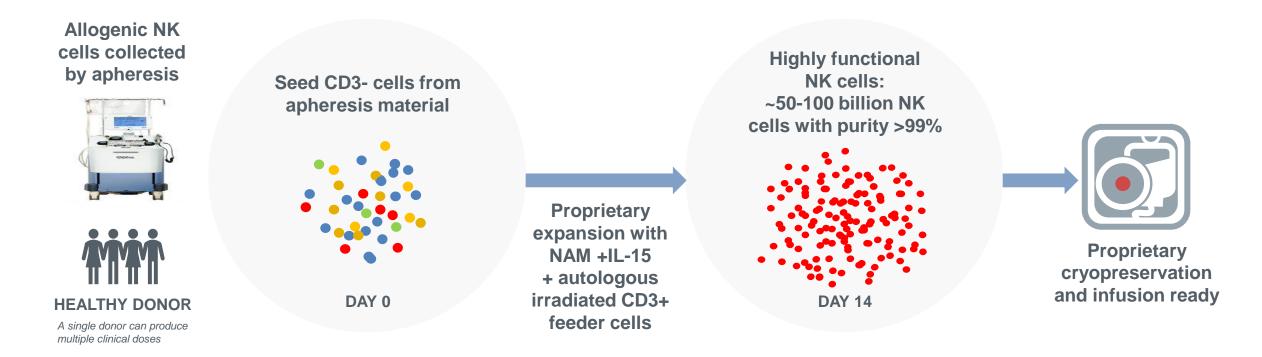
#### **Benefits of NK Cells**

- 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



# Off-The-Shelf Manufacturing with NAM Expansion

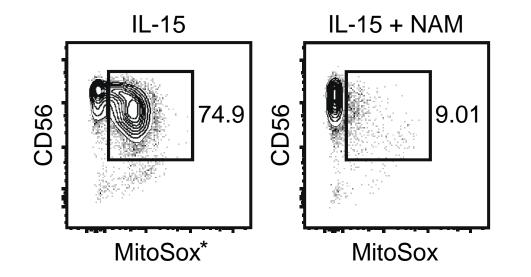
#### NAM rejuvenates NK cell preservation during expansion and cryopreservation



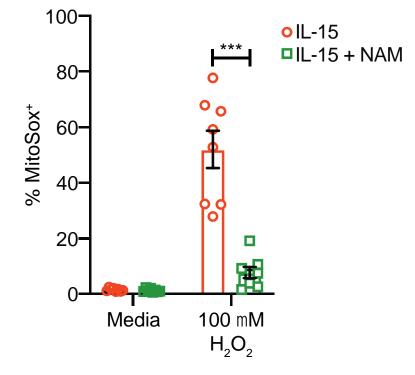
#### One apheresis procedure can provide several clinical doses



#### NK cells were expanded with IL-15 and with or without NAM



NAM-expanded NK cell mitochondria produce decreased levels of lethal superoxide (labeled with fluorescent marker) when the cells are challenged with hydrogen peroxide, reducing oxidative stress.



F. Cichocki, presented at the American Association of Immunologists (AAI) conference, May 2021

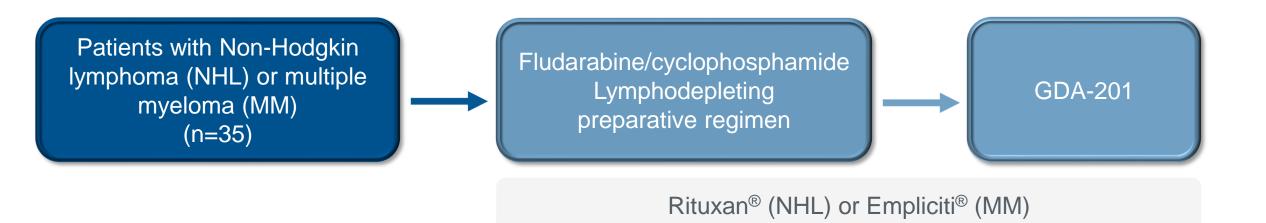


# **GDA-201**

NAM-Enabled NK Cells to Treat Non-Hodgkin Lymphoma



# 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

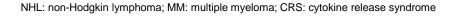
## **Clinical Responses Observed in NHL Cohort**

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%		

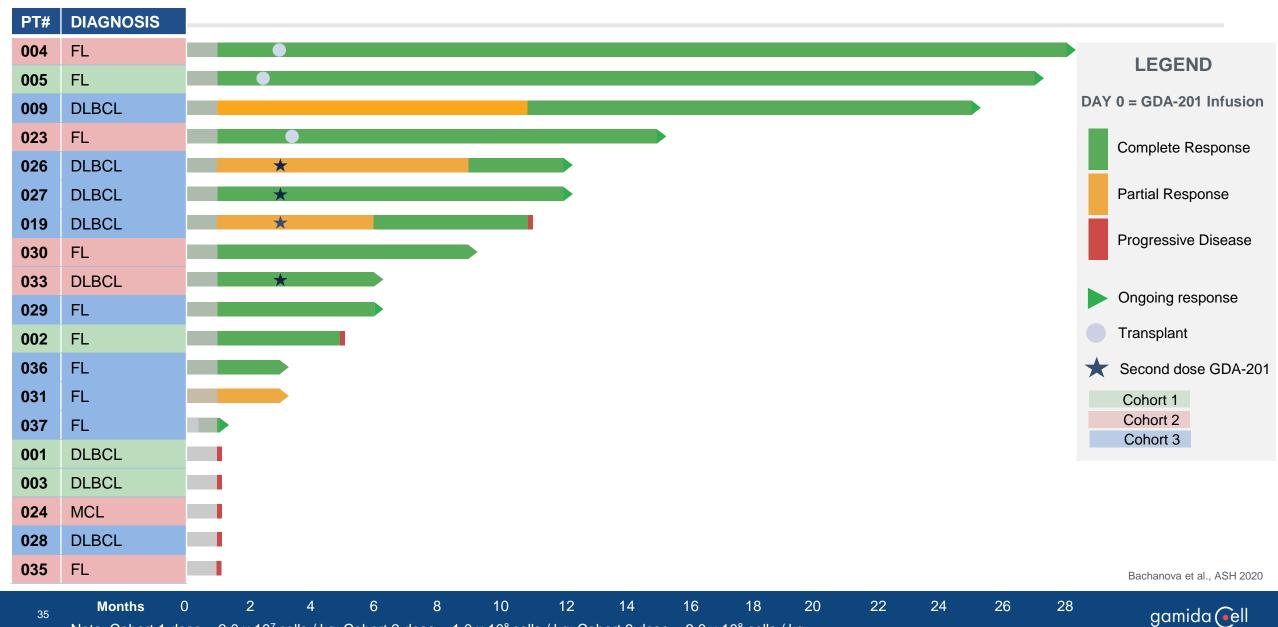


# Safety Summary

- 35 patients treated (19 NHL, 16 MM)
- No dose limiting toxicities
- One patient died of E. coli sepsis, initially reported as CRS
- Most common grade 3/4 adverse events:
  - Thrombocytopenia (n=9)
  - Hypertension (n=5)
  - Neutropenia (n=4)
  - Febrile neutropenia (n=4)
  - Anemia (n=3)
- No neurotoxic events, graft versus host disease, or confirmed CRS



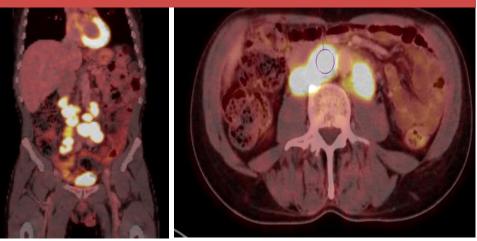
# GDA-201 Is Highly Active in Non-Hodgkin Lymphoma



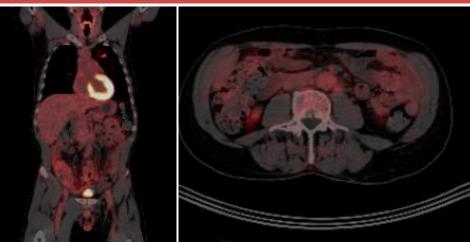
Note: Cohort 1 dose =  $2.0 \times 10^7$  cells / kg; Cohort 2 dose =  $1.0 \times 10^8$  cells / kg; Cohort 3 dose =  $2.0 \times 10^8$  cells / kg

# Complete Response in Heavily Pretreated Lymphoma Patient

#### 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

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- 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.

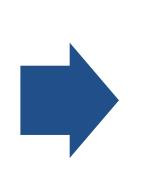
# GDA-201: Encouraging Clinical Activity and Safety Profile Supports Continued Development





• Preclinical proof of principle

- Clinical proof of concept
- Maximum target dose achieved



Phase 1/2 multi-center study in lymphoma for cryopreserved GDA-201 in H2 2021



# Engineered NK Cell Programs

Improving Targeting and Persistence Against Blood and Solid-Tumor Cancers



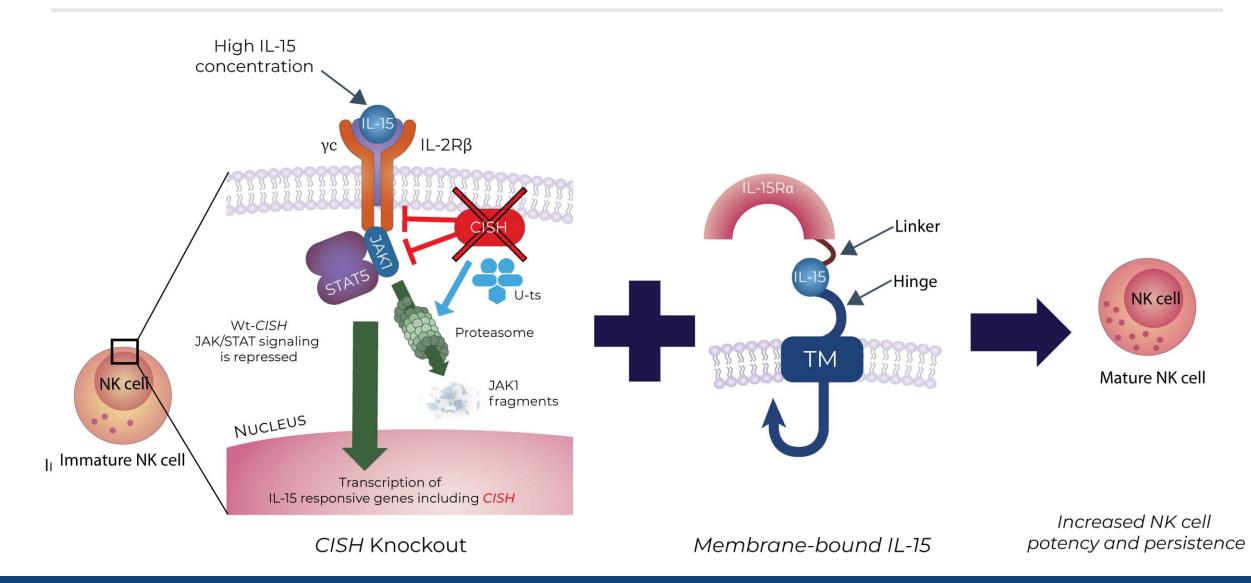
# A Leading Genetically Engineered NK Cell Pipeline

# Four new programs applying a clinically tested NAM-enabled cell source armed with promising strategies for overcoming immunosuppression

PROGRAM	STRATEGY	GENETIC MODIFICATION	INDICATION(S)
GDA-301	Increased potency and persistence	CISH KO + memblL-15	Solid tumors
GDA-401	Undisclosed	Undisclosed	Undisclosed
GDA-501	HER2 Targeting	HER2 CAR	HER2+ solid tumors
GDA-601	CD38 Targeting	CD38 KO + CD38 CAR	Multiple myeloma

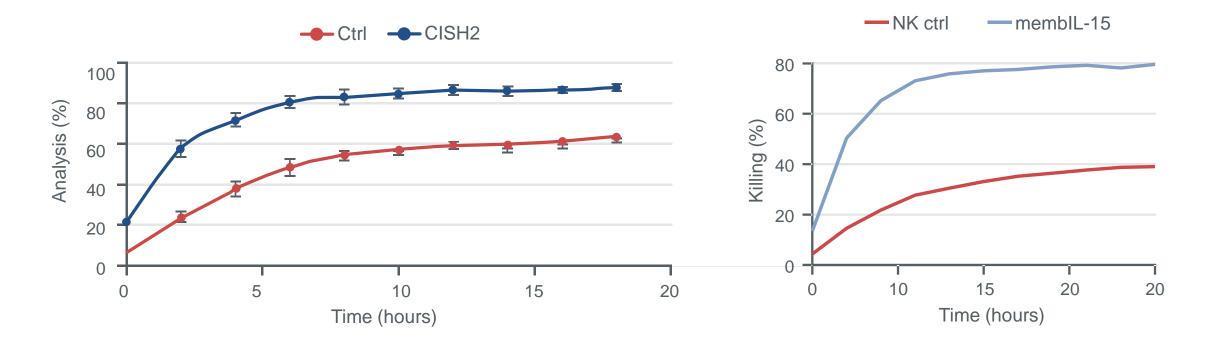
memb-IL15 = Membrane-bound IL-15 KO = Knockout CAR = Chimeric antigen receptor HER2 = Human epidermal growth factor receptor 2

## **GDA-301: Increasing NK Potency and Persistence**





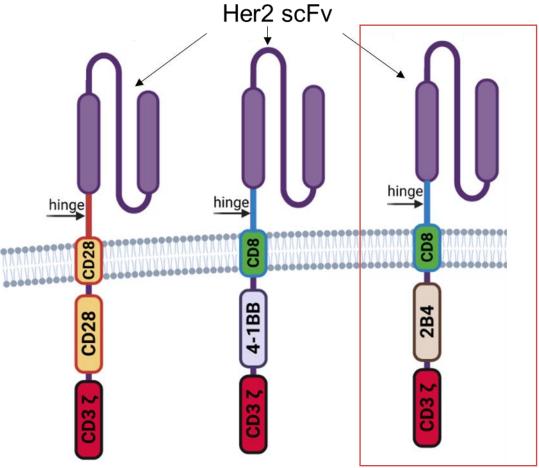
# GDA-301: CISH KO and Membrane-Bound IL-15 Increase Cytotoxicity Against Tumor Cell Lines





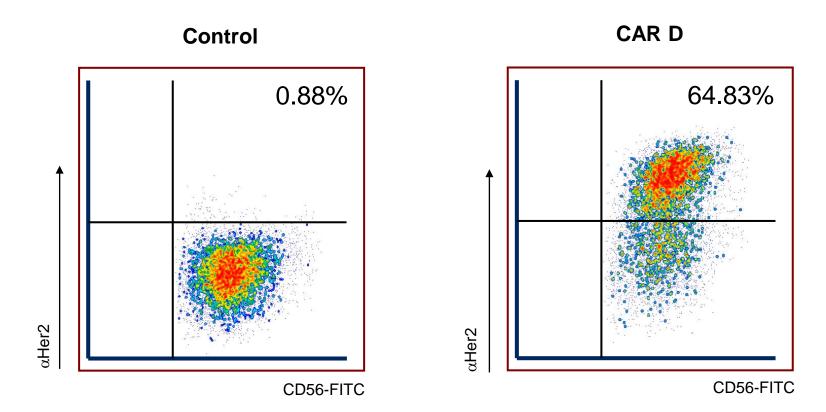
# GDA-501: Targeting HER2+ Solid Tumors

Three CAR constructs were developed to target and activate NK cells against HER2+ tumors



### GDA-501: HER2 CAR Constructs Proof of Concept

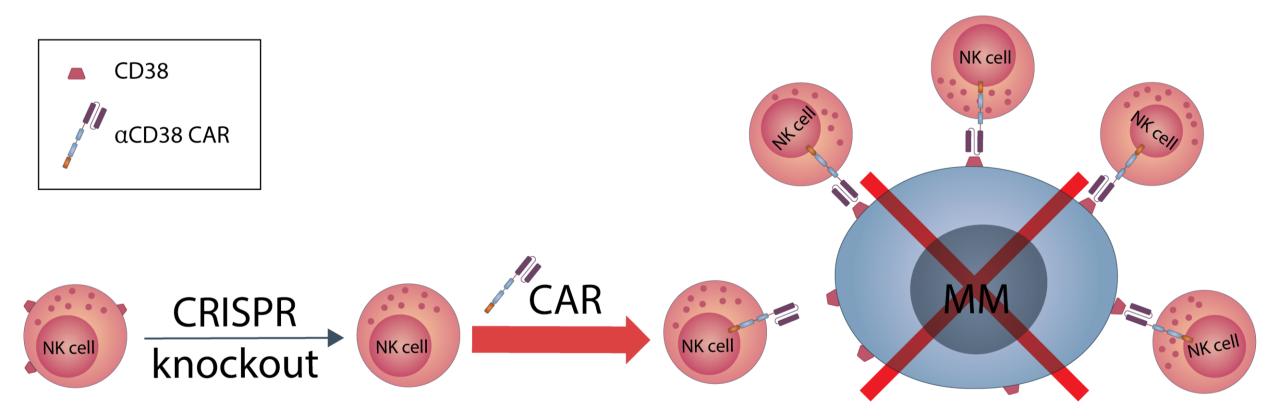
### CAR construct is expressed by NK cells and recognizes the HER2 protein





# GDA-601: CD38 Targeting by Combined Knockout and CAR

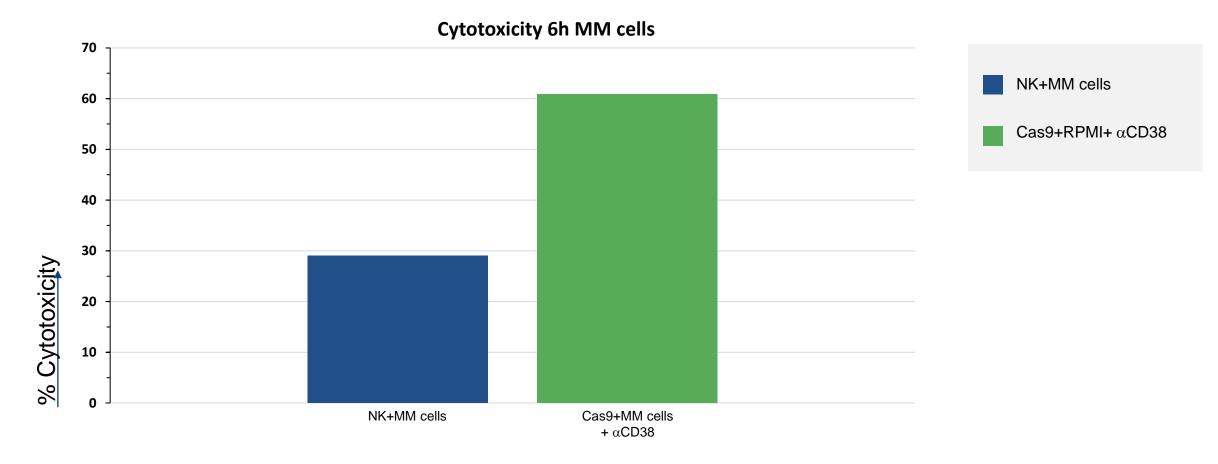
Addition of *a*CD38 CAR to CD38 KO NKs resulted in better MM (Multiple Myeloma) killing





# GDA-601: CD38 KO & $\alpha$ CD38 CAR — Increased Cytotoxicity against MM

### Flow cytometry – Cytotoxicity analysis





# We are Committed to Cures: Looking Ahead

# Making an impact with multiple <u>advanced cell therapy</u> programs that leverage our proprietary NAM 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
- Preparing for BLA submission in 4Q21 based on compelling Phase 3 clinical profile
- Launch readiness activities underway for potential launch mid-22



# GDA-201: Harnessing natural killer cells to fight non-Hodgkin lymphoma

- Promising Phase 1 clinical data with an ORR of 74% and CRR of 68%
- Initiating a Phase 1/2 clinical study in NHL in 2H21

# GDA-301/401/501/601: Engineered NAM-enabled NK cells

- Proof-of-concept for CAR and CRISPR editing
- Combination strategies show evidence of increased cytotoxicity in preclinical studies
- Potential in blood cancers and solid tumors



#### Well-positioned to execute goals

- \$150.2 million cash position to support capital needs into 2H22\*
- Approximately 150 employees





# **Committed to Cures**

August 2021

