

# **Committed to Cures**

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

October 2021

#### Disclaimer

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 (including GDA-201), anticipated regulatory filings (including the submission of the BLA for omidubicel to the FDA), and the potentially life-saving or curative therapeutic and commercial potential of its product candidates. 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 <a href="http://www.sec.gov">http://www.sec.gov</a>), 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 forwardlooking 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



# Progressing clinical program in NK cells



# Opening new frontiers in cancer immunotherapy

#### **Omidubicel**

- Preparing for <u>BLA</u> submission in 4Q21\*
- Potential to be first FDAapproved cell therapy for bone marrow transplantation
- Breakthrough Therapy and Orphan Drug status

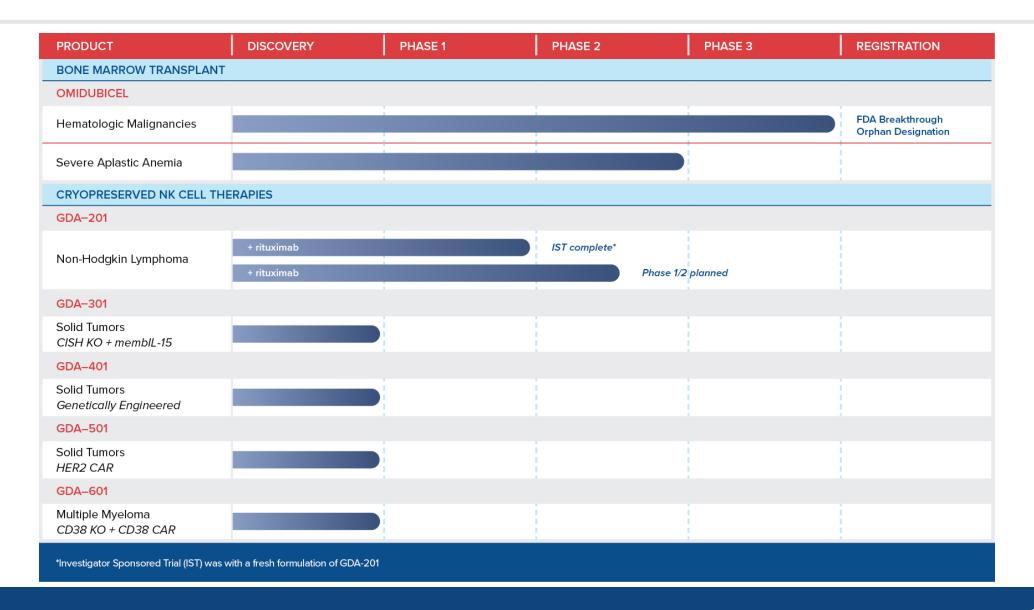
#### **GDA-201**

- Innate NK cell product with positive Phase 1 data
- Submitted IND for a Phase 1/2 trial in NHL
- IND on clinical hold prior to patient dosing pending ongoing discussions with FDA

#### 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

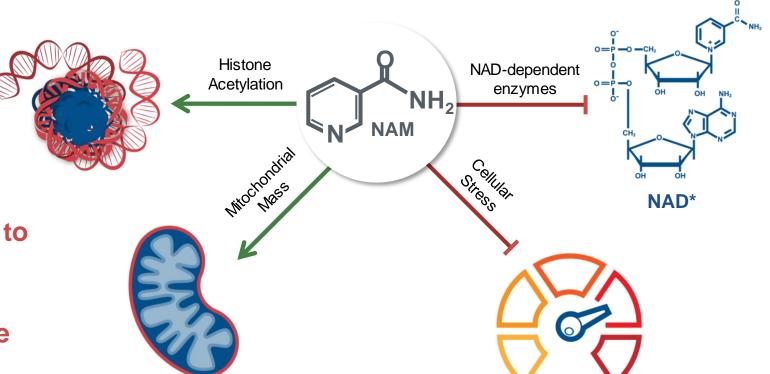
### **NAM Platform Technology**

Enhances the number of allogeneic donor cells

 Enhances cellular functionality and phenotype

 Improves homing and retention to lymphoid tissues

Potential to expand any cell type



# **Omidubicel**

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





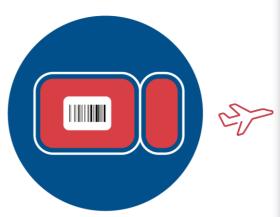
# Our Inspiration: Focusing on Cures

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.

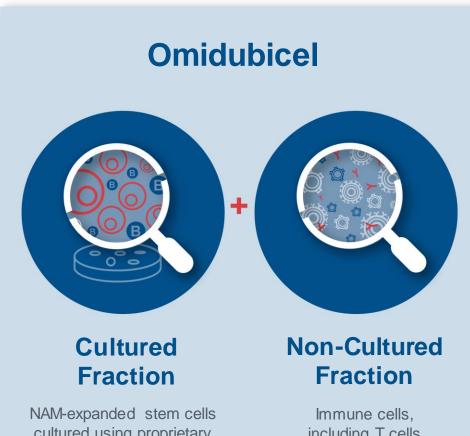
### Omidubicel Is a Cell Therapy Option for Patients in Need of a Transplant



CBU selected by physician from public cord blood bank

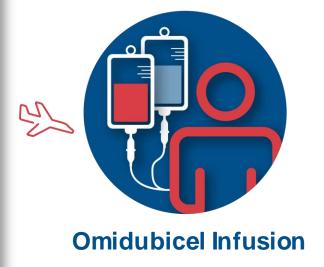
**Cord Blood Unit (CBU)** 

Selected



cultured using proprietary NAM technology

including T cells



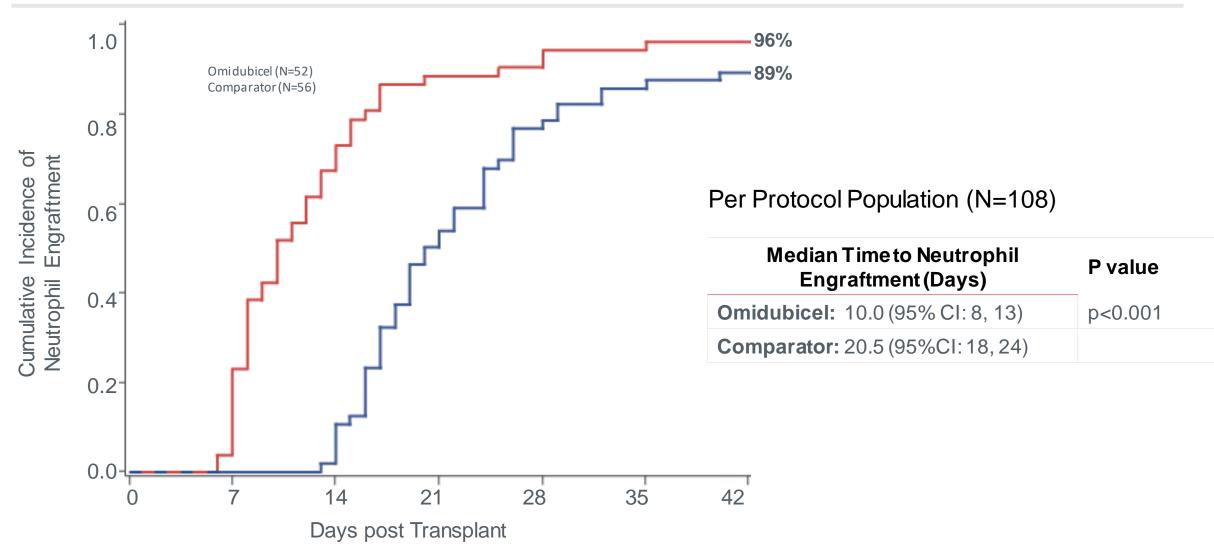
Scalable manufacturing and delivery of omidubicel

# 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

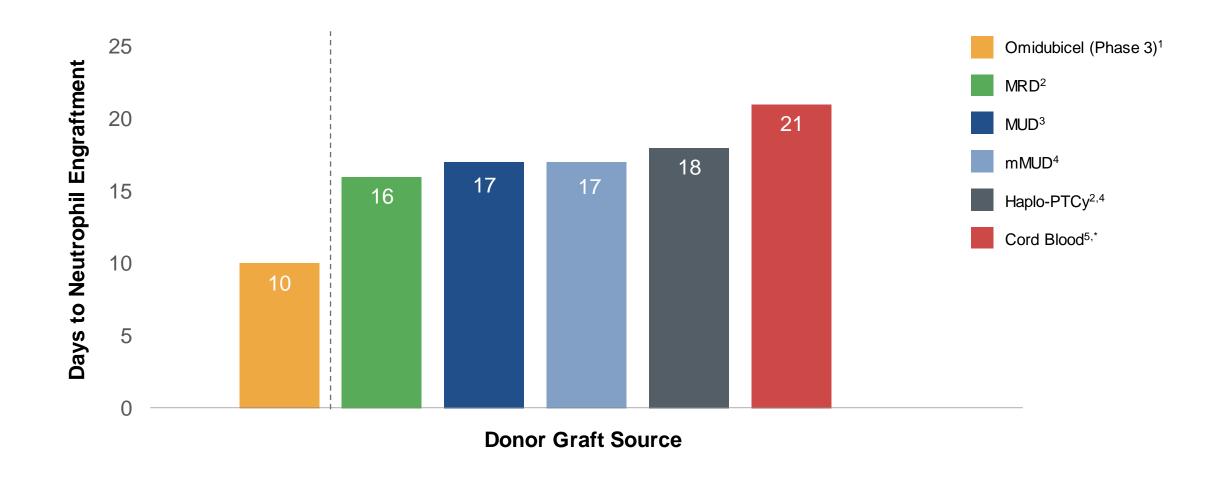
INTENT-TO-TREAT	MEDIAN TIME TO NEUTROPHIL ENGRAFTMENT (DAYS)	95% CI	p-VALUE
Omidubicel (N = 62)	12.0	(10.0, 15.0)	p<0.001
Comparator (N = 63)	22.0	(19.0, 25.0)	

### Cumulative Incidence of Neutrophil Engraftment



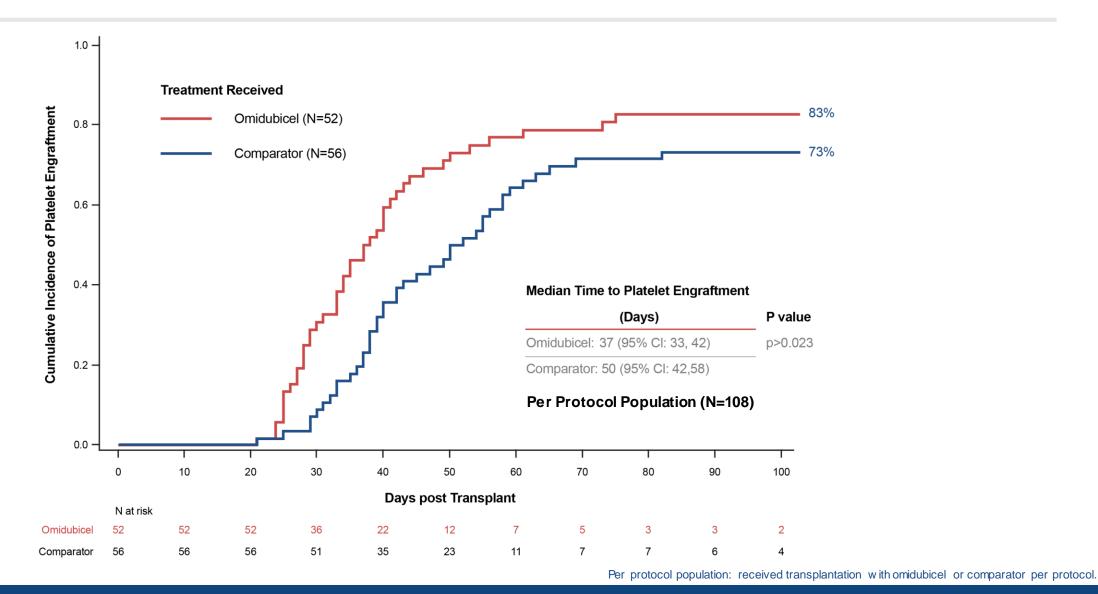
Per protocol population: received transplantation with omidubicel or comparator per protocol.

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





# Phase 3 Secondary Endpoints: Day 100 Platelet Engraftment

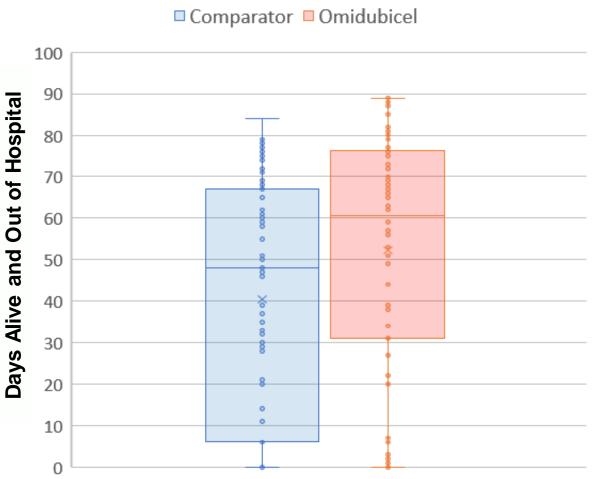




# Phase 3 Secondary Endpoint:

### Omidubicel Significantly Reduced Total Hospitalization in First 100 Days

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



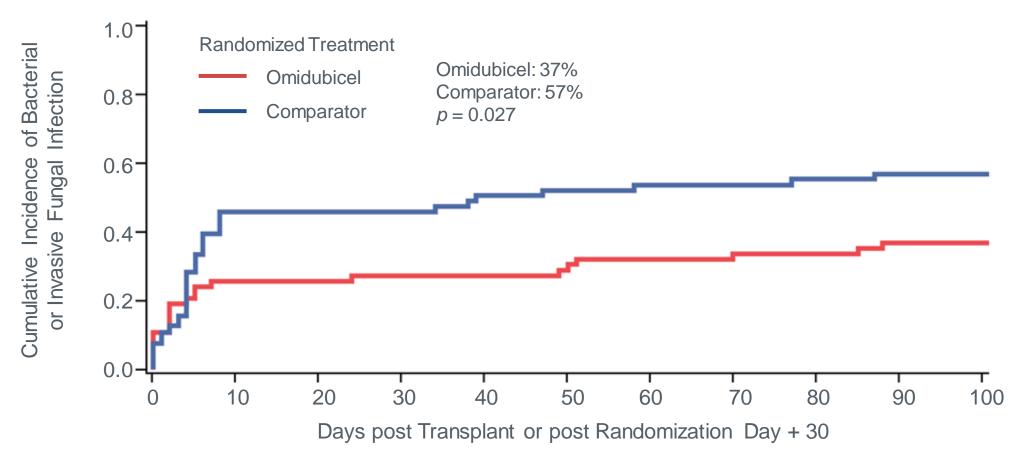
Omidubicel: Median 60.5 days Comparator: Median 48.0 days

p = 0.005

Population: ITT

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

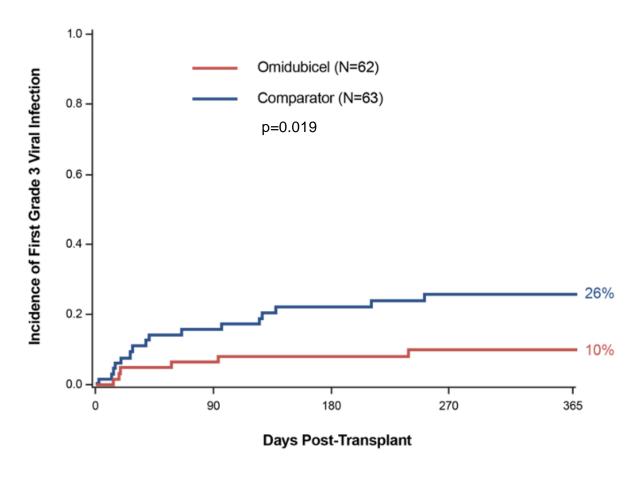
#### INCIDENCE OF SERIOUS BACTERIAL OR FUNGAL INFECTIONS BETWEEN RANDOMIZATION AND 100 DAYS1



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

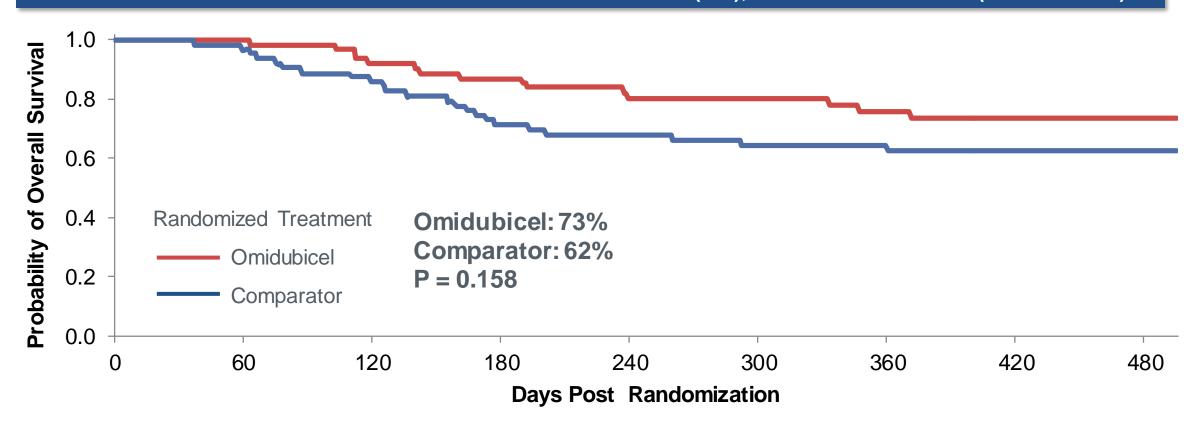


### Fewer Viral Infections in Recipients of Omidubicel



# 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 Vision**

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

#### Supporting Reasons to Believe:

Matches over 95% of all patients

>40% of patients in the clinical trial were ethnically diverse

Removed concern about age/availability of donor

Reliable neutrophil engraftment in over 96% of patients

Rapid neutrophil engraftment (median 10 days)

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

### 42,000 Allo-Transplant Candidates

# 23% Transplanted Today's Donor Sources Umbilical Cord Blood (UCB) Matched Related Donor (MRD) Matched Unrelated Donor (MUD) Mismatched Unrelated Donor (mMUD) Haploidentical Donor

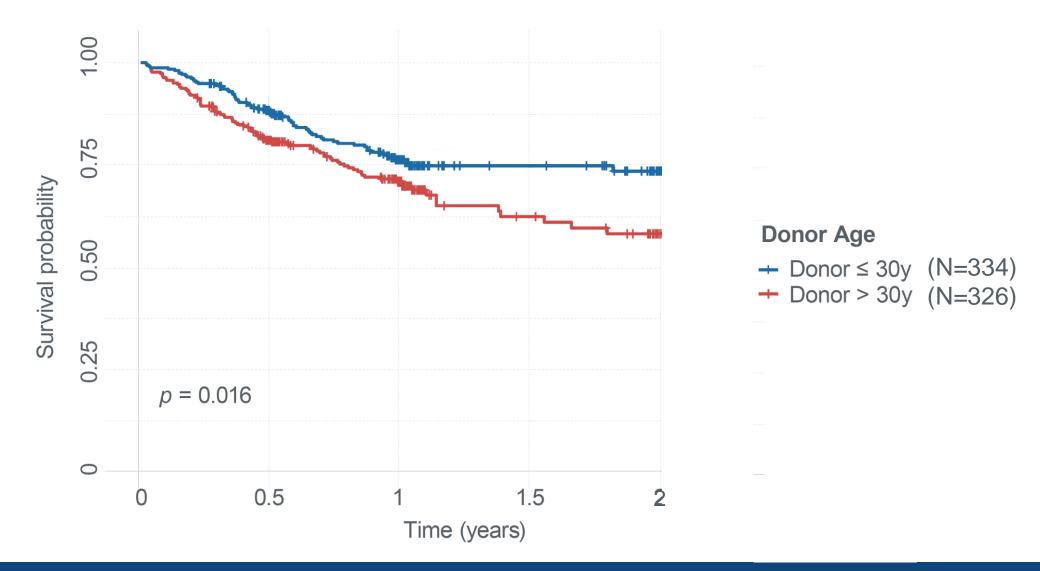
#### 77% Not Transplanted Non-Clinical Factors **Donor Factors** Clinical Factors Patient Decision Not To Patient Age Availability of Graft Proceed Performance Status Suitability of Graft Psychosocial Factors Disease and Stage · Timing of Graft Travel Required Comorbidities Lack of a Suitable Caregiver

### Omidubicel Has the Potential To Expand Access and Improve Outcomes

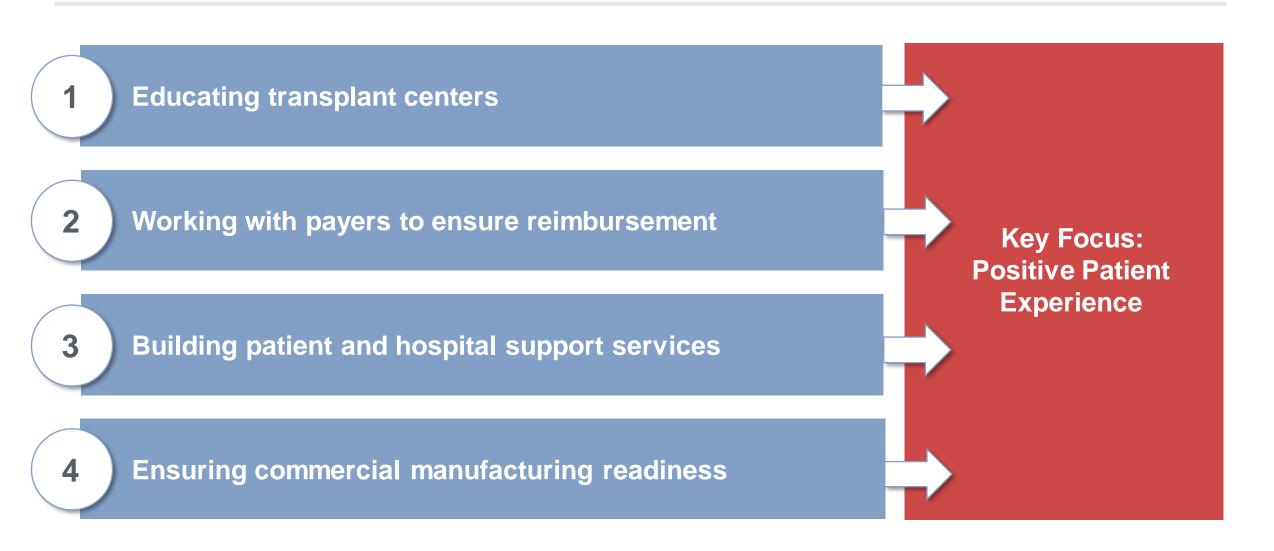
In market research, physicians indicated that omidubicel would expand access and improve outcomes

#### **Expand Access** Improve Outcomes Non-Clinical Factors Today's Donor Sources **Donor Factors** Clinical Factors Umbilical Cord Blood (UCB) Patient Decision Not To Patient Age Matched Related Donor (MRD) Availability of Graft Proceed Performance Status Matched Unrelated Donor (MUD) Suitability of Graft Psychosocial Factors Disease and Stage Mismatched Unrelated Donor · Timing of Graft Travel Required Comorbidities (mMUD) Lack of a Suitable Caregiver Haploidentical Donor ~1.200 ~1,200

# Overall Survival with Follow up is Associated with Donor Age



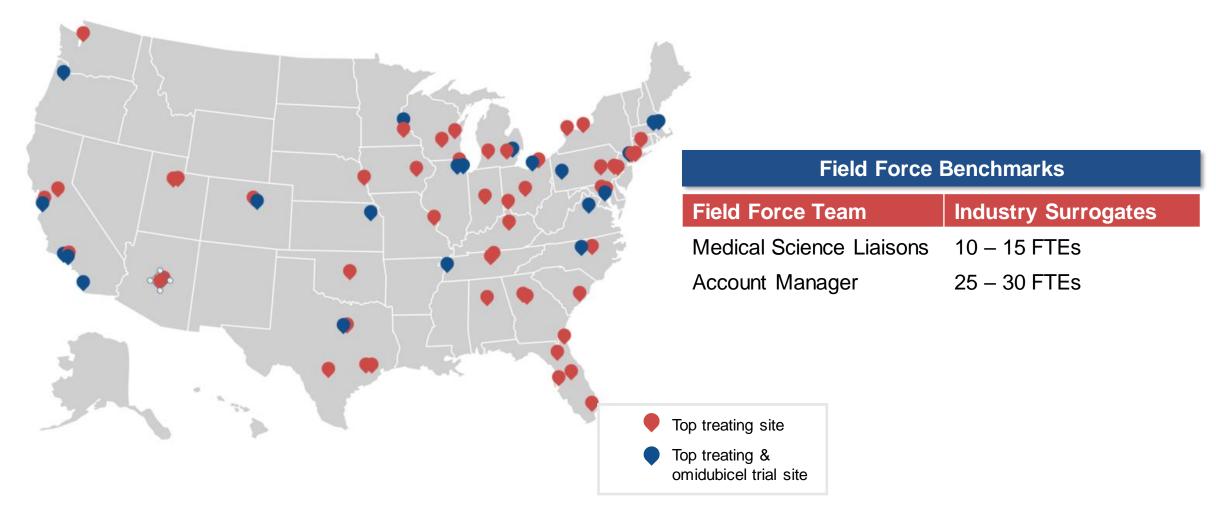
# Key Commercial Activities and Infrastructure Build-out Are Underway to Prepare for a Successful Omidubicel U.S. Launch



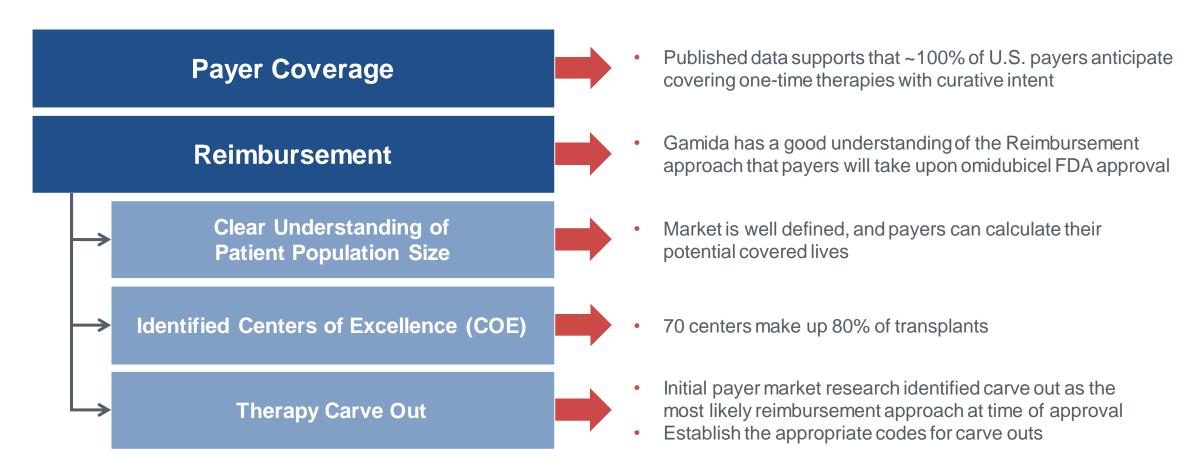
# Gamida Cell Has Initiated Plan for Education of U.S. Transplant Centers



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



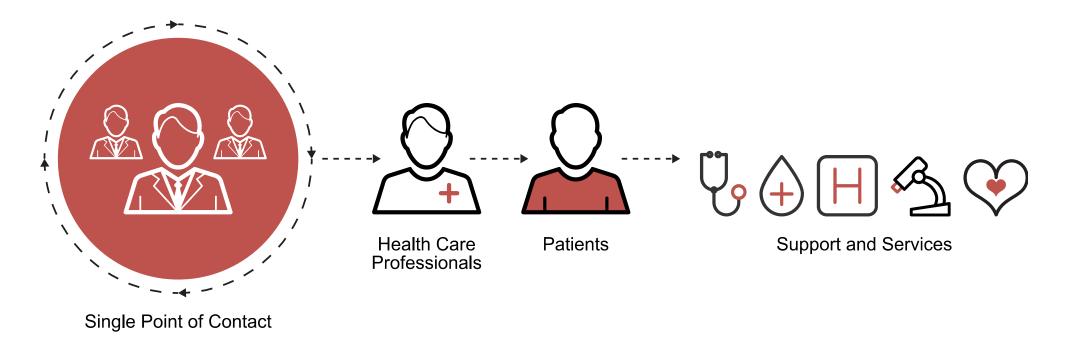
Gamida Cell has conducted research to understand the reimbursement approaches that payers will take if omidubicel receives FDA approval



### 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



- 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

### Manufacturing Readiness on Track to Support Potential Launch Mid-2022



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

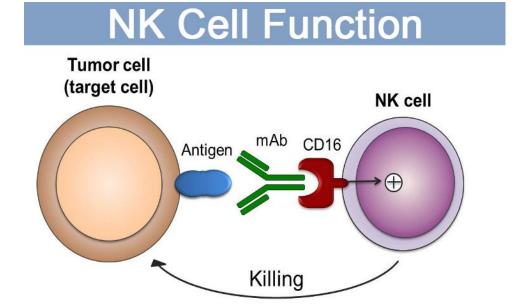
Harnessing Innate Immunity Using Natural Killer (NK) Cells to Treat Cancer



# 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

Allogenic NK cells collected by apheresis





Seed CD3- cells from apheresis material



Proprietary
expansion with
NAM +IL-15
+ autologous
irradiated CD3+
feeder cells

Highly functional NK cells: ~50-100 billion NK cells with purity >99%



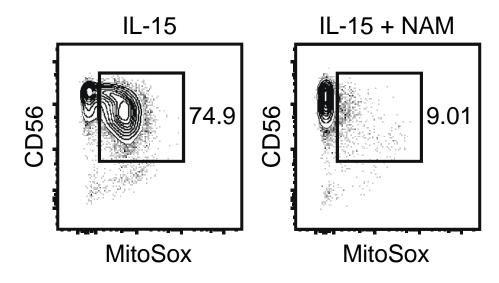
Proprietary

cryopreservation and infusion ready

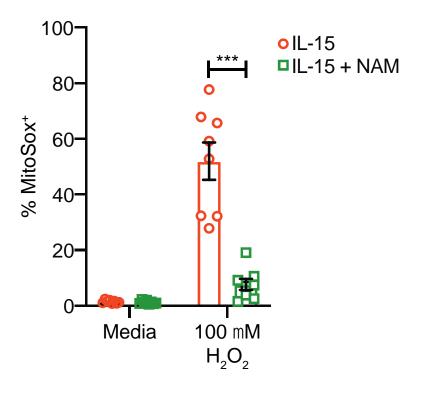
One apheresis procedure can provide several clinical doses

# Strongly Protective Effect Against Oxidative Stress Favors Survivability in the Tumor Microenvironment

#### 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.

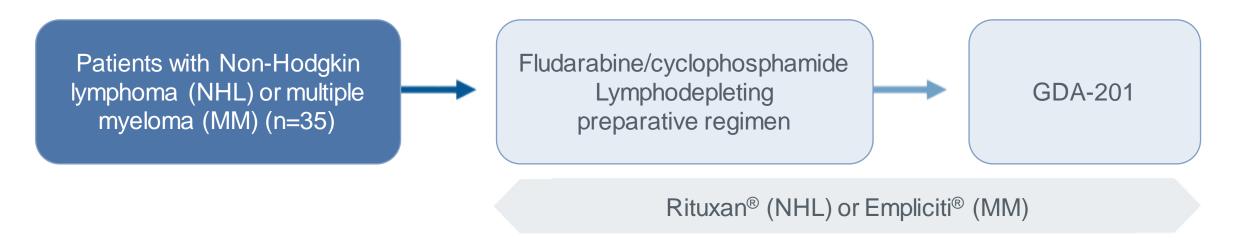


# **GDA-201**

NAM-Enabled NK Cells to Treat Non-Hodgkin Lymphoma

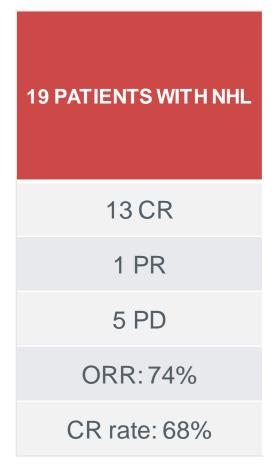


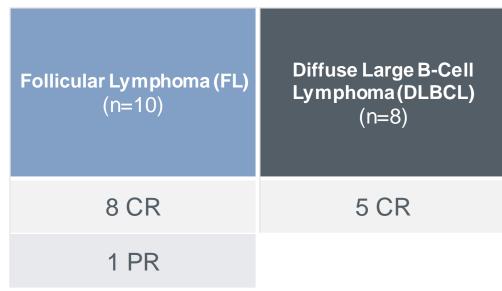
# Phase 1 Study of GDA-201 in Patients with Non-Hodgkin Lymphoma and Multiple Myeloma



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

### Clinical Responses Observed in NHL Cohort

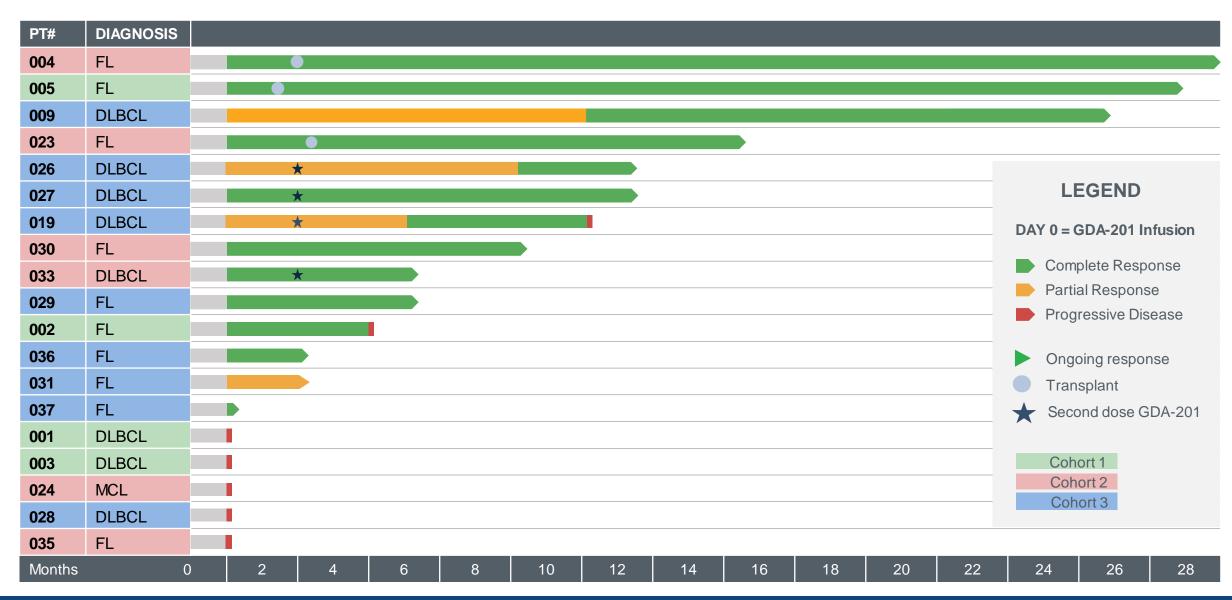




# Safety Summary

- 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

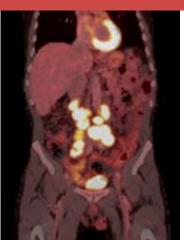


<sup>•</sup> Note: Cohort 1 dose = 2.0 x 10<sup>7</sup> cells / kg; Cohort 2 dose = 1.0 x 10<sup>8</sup> cells / kg; Cohort 3 dose = 2.0 x 10<sup>8</sup> cells / kg

<sup>•</sup> Bachanova et al., ASH 2020

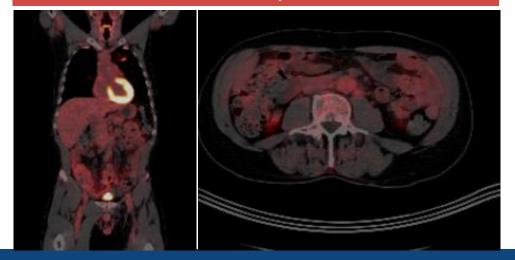
### Complete Response in Heavily Pretreated Lymphoma Patient

#### 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
- 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**



- Clinical proof of concept
- Maximum target dose achieved
- Cryopreserved formulation



### **Next Step**



Phase 1/2 multi-center study in lymphoma for cryo-preserved GDA-201

# Engineered NK Cell Programs

Improving Targeting and Persistence Against Blood and Solid-Tumor Cancers



# A Leading Genetically Engineered NK Cell Pipeline

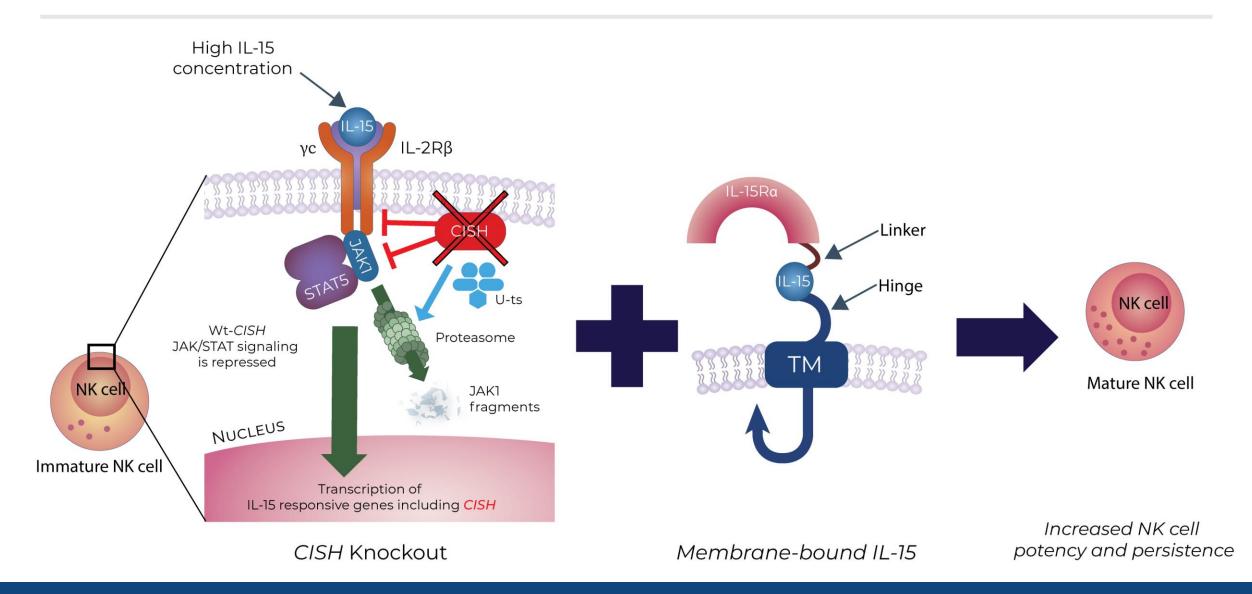
PROGRAM	STRATEGY	GENETIC MODIFICATION	INDICATION(S)
GDA-301	Increased potency and persistence	CISH KO + memblL-15	Hematologic + solid tumors
GDA-401	Undisclosed		
GDA-501	HER2 Targeting	HER2 CAR	HER2+solid tumors
GDA-601	CD38 Targeting	CD38 KO + CD38 CAR	Multiple myeloma



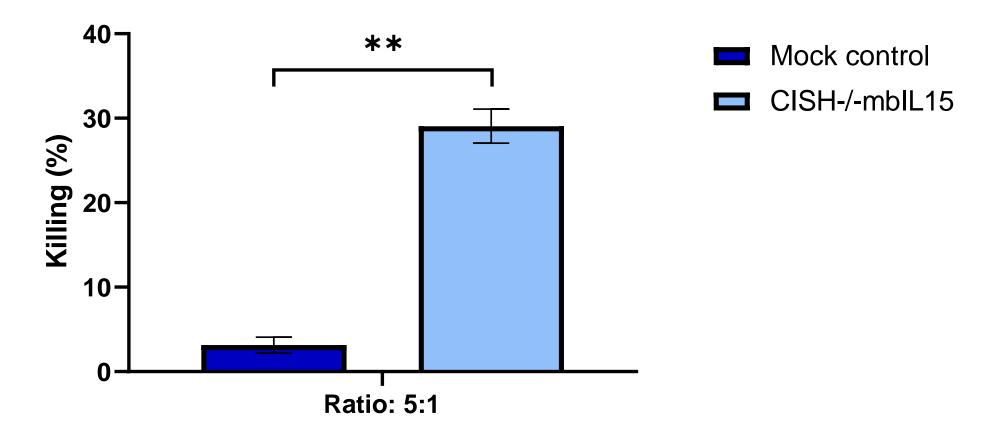
<sup>•</sup> memb-IL15 = Membrane-bound IL-15

<sup>•</sup> HER2 = Human epidermal growth factor receptor 2

# GDA-301: Increasing NK Potency and Persistence



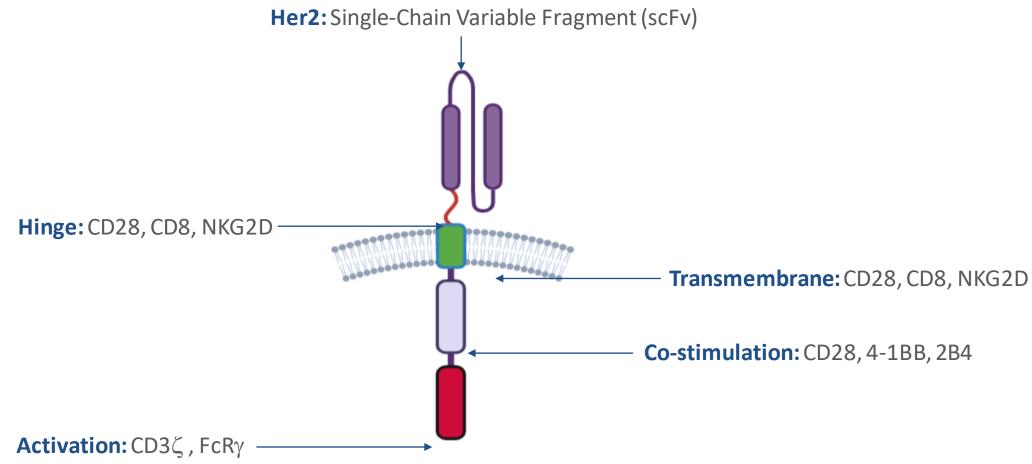
# GDA-301: CISH Knockout NK Cells that co-express mbIL-15 Increase Cytotoxicity Function Against Multiple Myeloma Cell Line (RPMI)



Deletion of CISH and co-expression of mb-IL15 on NK cells enhances their cytotoxicity activity. Killing assay was performed on CISH knocked cells ,24h after the electroporation of mRNA-mbIL-15 that followed a 6hr co-culture of NK cells with RPMI cell line

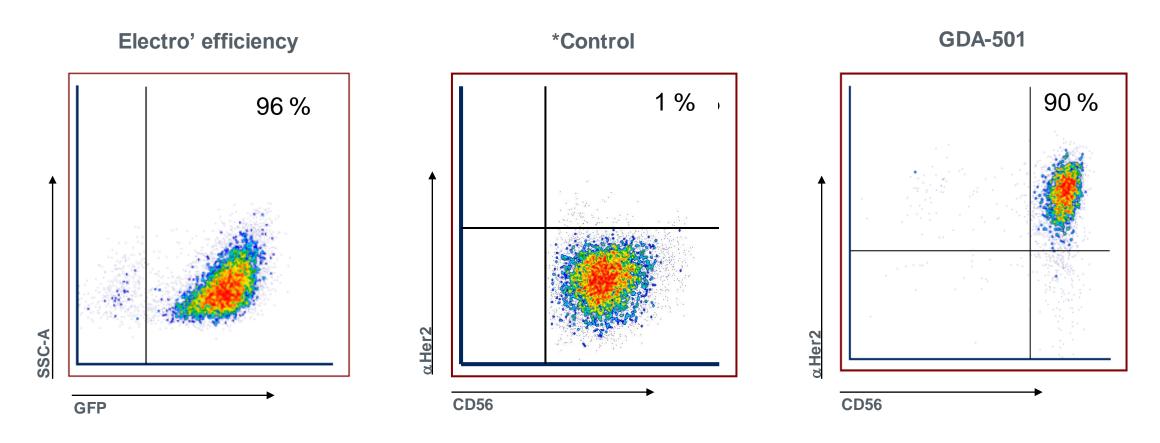
### GDA-501: Targeting Solid Tumors Expressing HER2

# Multiple tailor-made NK CARs were developed to target and activate NKs against HER2+ tumors



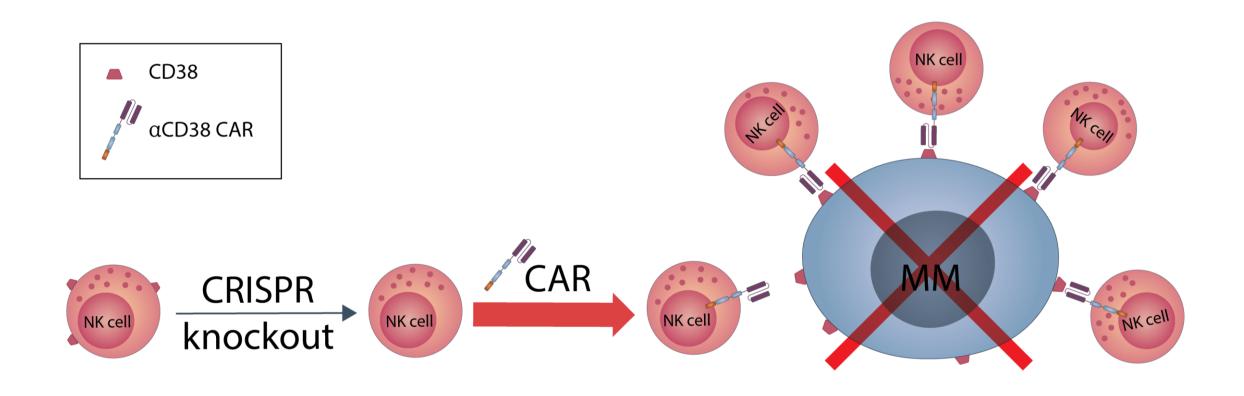
### GDA-501: αHER2 CAR Constructs Proof of Concept

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



<sup>\*</sup> GDA-501 cells were expanded using Gamida's NAM technology, gene editing was done on the NAM expanded cells

### CD38 Knockout and CD38 CAR Targeting Multiple Myeloma

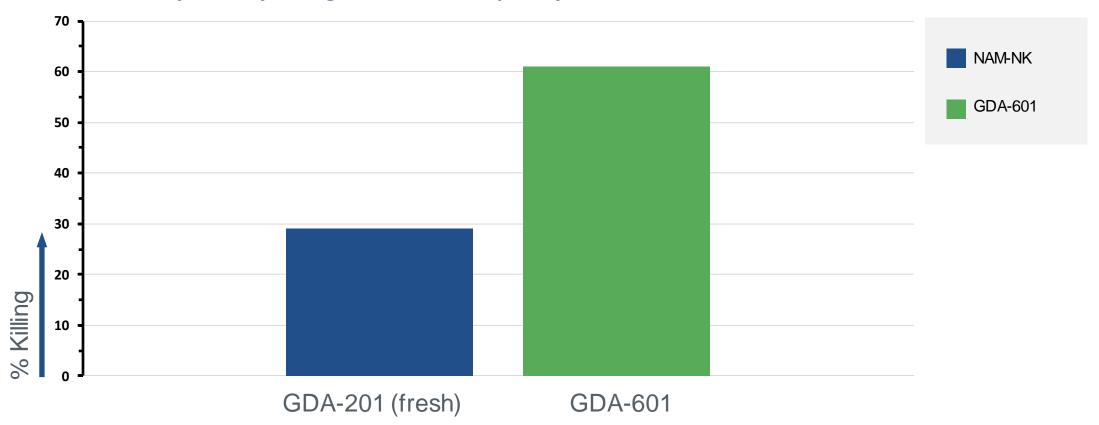


We used the CRISPR/Cas9 system to knockout CD38 in NKs

# GDA-601: CD38 KO & αCD38 CAR — Increased Cytotoxicity Against Multiple Myeloma

### The fratricide rescue and addition of $\alpha$ CD38 CAR improve cytotoxicity

Flow Cytometry Killing, 6h RPMI: Multiple Myeloma Cell Line, E:T-5:1



### 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%
- Submitted IND for a Phase 1/2 trial in NHL
- On clinical hold prior to patient dosing pending ongoing discussions with FDA



# 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**

October 2021