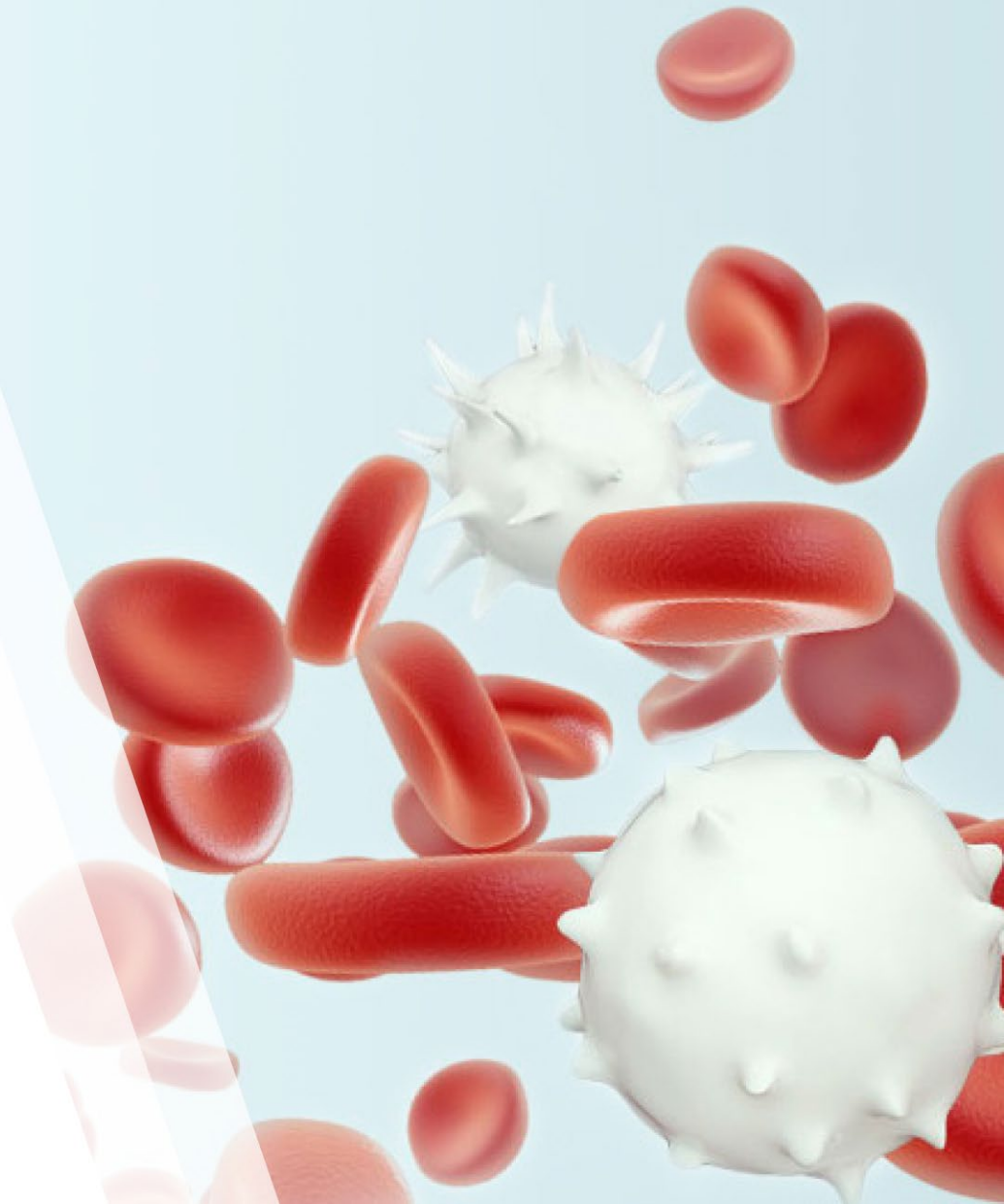




# Company Overview

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December 2018



# 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.

# Company Highlights

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## **NiCord®: Product candidate for bone marrow transplant with curative potential**

- Phase 3 registration study in patients with hematologic malignancies ongoing
- Received FDA Breakthrough Therapy Designation and Orphan Designation from FDA and EMA
- Preparing for potential U.S. launch

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## **Proven, proprietary nicotinamide (NAM)-based cell expansion technology**

- Phase 1/2 study of NiCord in severe aplastic anemia ongoing
- Phase 1 study of NAM-NK in non-Hodgkin lymphoma and multiple myeloma

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## **Wholly-owned clinical pipeline sets the stage for multiple milestones through 2020**

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## **Experienced executive team; recently established U.S. headquarters**

# Gamida Cell Has Worldwide Rights to Its Clinical Pipeline

PRODUCT	PRECLINICAL	PHASE 1/2	PHASE 3	MILESTONES
NiCord®	High-Risk Hematologic Malignancies			Top-line data 1H20
	Severe Aplastic Anemia*			Preliminary data 2019
NAM-NK	Hematologic Malignancies			Additional data 1H19

# The Gamida Cell Executive Team Has Substantial Experience

Julian Adams, Ph.D. — Chairman & Chief Executive Officer



Josh Hamermesh — Chief Business Officer



Shai Lankry — Chief Financial Officer



Tzvi Palash — Chief Operating Officer



Tony Peled — Chief Scientific Officer



Ronit Simantov, M.D. — Chief Medical Officer



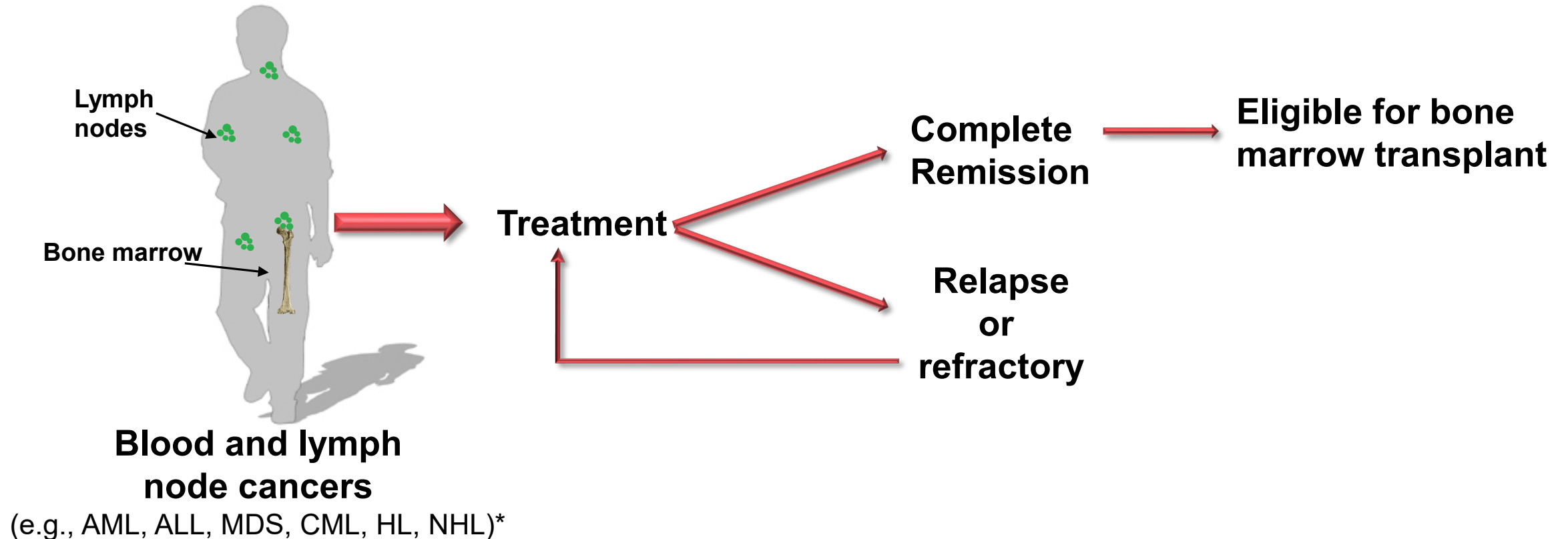
# NiCord®

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Hematologic Malignancies

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# Bone Marrow Transplant May Be Curative in Hematologic Malignancies

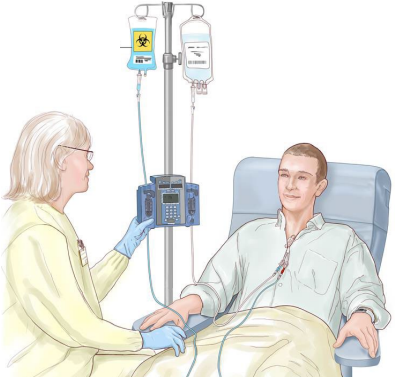


- Allogeneic stem cell transplant (HSCT) is an effective treatment for patients with life-threatening blood cancers
- >70% of allogeneic HSCT in the US are performed in patients with acute leukemia or myelodysplastic syndrome<sup>1</sup>

\*AML: acute myelogenous leukemia; ALL: acute lymphoblastic leukemia; MDS: myelodysplastic syndrome; CML: Chronic myelogenous leukemia; HL: Hodgkin lymphoma; NHL: non-Hodgkin lymphoma.

<sup>1</sup>D'Souza A, Fretham C. Current Uses and Outcomes of Hematopoietic Cell Transplantation (HCT): Center for International Blood and Marrow Transplant Research (CIBMTR) 2017 Summary Slides (the "CIBMTR Slides") <http://www.cibmtr.org>.

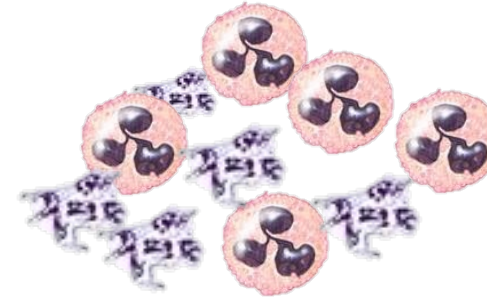
# Allogeneic Bone Marrow Transplantation Must Be Improved



**Conditioning**



**Stem Cell Infusion**



**Engraftment**

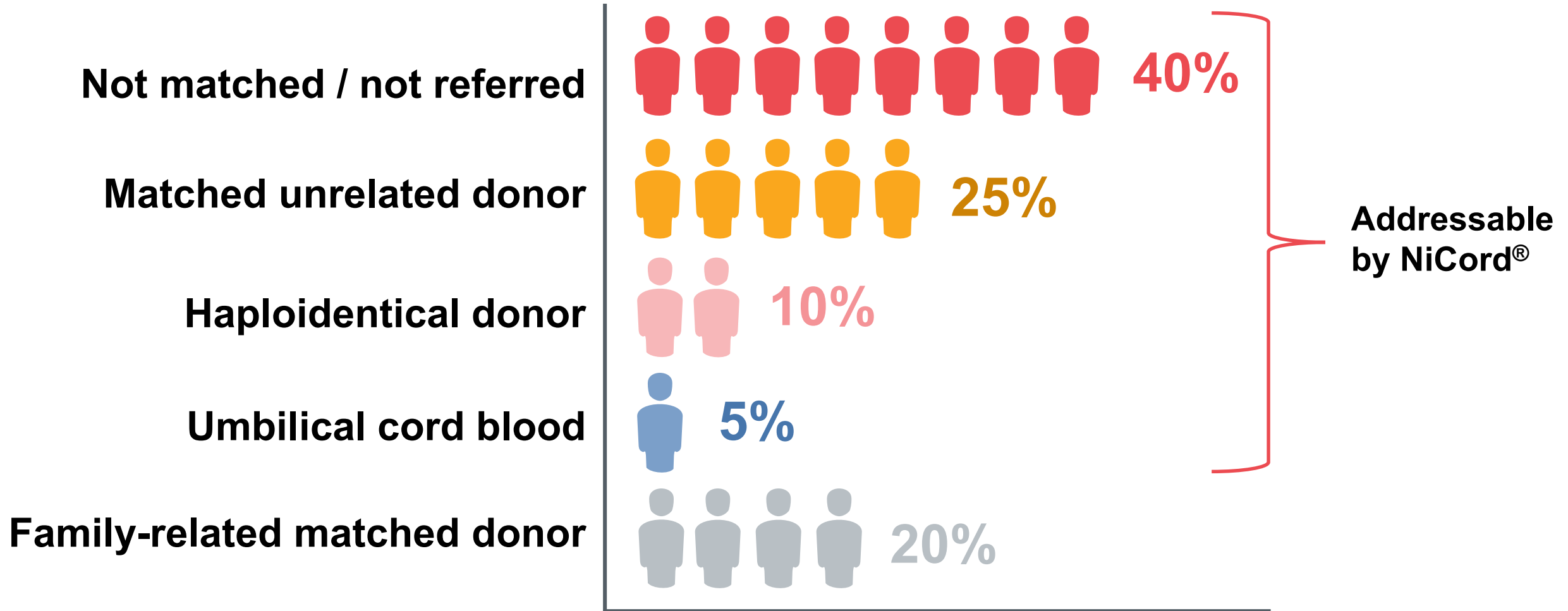


## Medical Need

- Patients have a high risk of infections and other complications such as graft vs host disease
- Many patients never get to transplant or have a significant wait to get a match



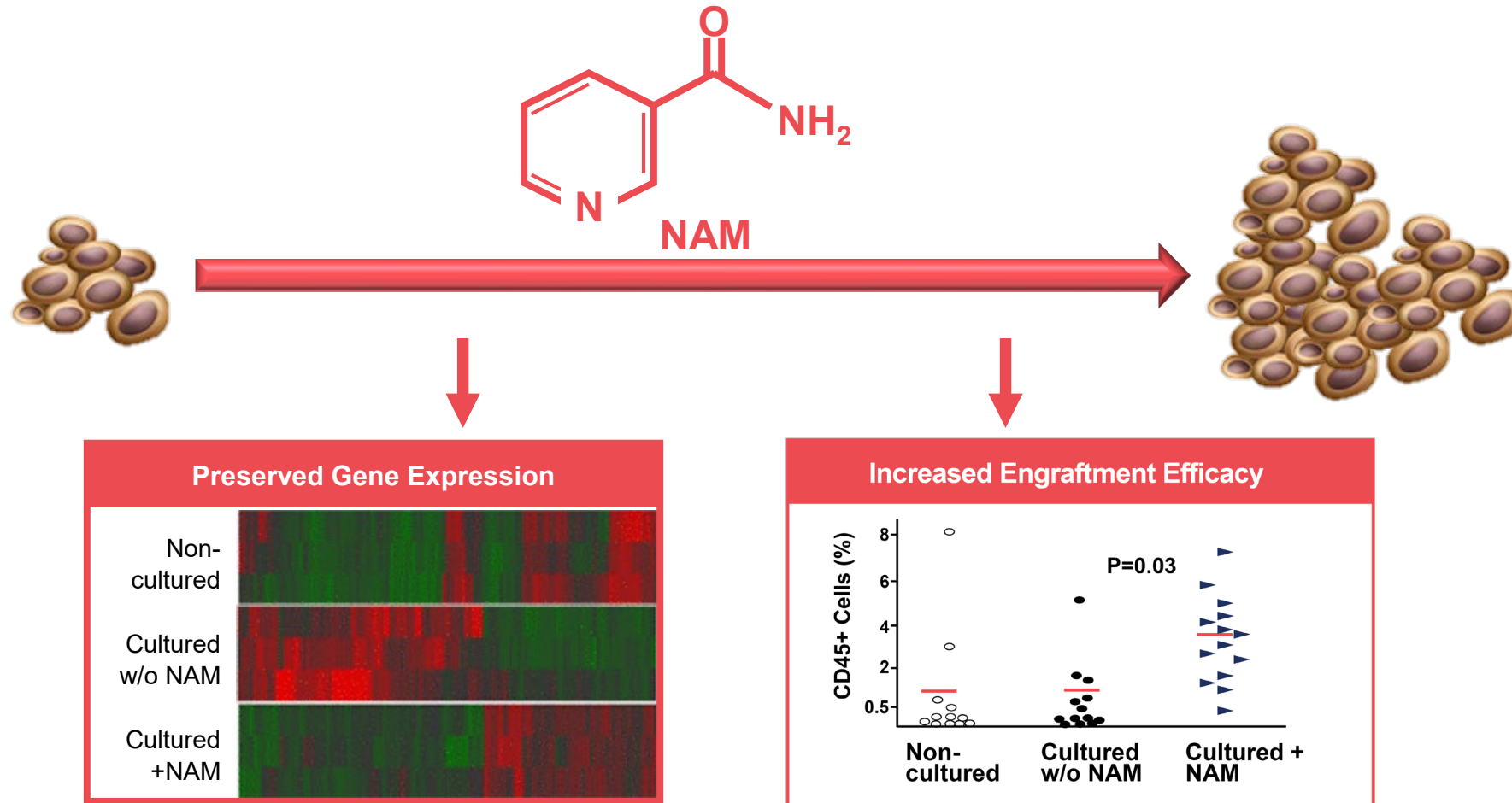
# HSCT Candidate Patient Segmentation



Not matched / not referred based on: Besse K, et al. Estimating Demand and Unmet Need for Allogeneic Hematopoietic Cell Transplantation in the United States Using Geographic Information Systems. Journal of Oncology Practice Volume 11 Issue 2 March 2015 (the "2015 Besse Paper") and The Nemetz Group LLC Crowdsourced Survey of US Allo-HSCT Transplanters April 2018 (the "Nemetz April Survey").  
All other segments: CIBMTR Slides.

# Nicotinamide (NAM) Technology: Mechanism of Action

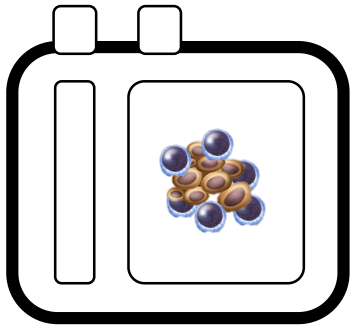
**NAM** causes metabolic reprogramming, leading to cell proliferation while preserving stem cells



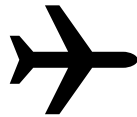
# NiCord® Is a Pharmaceutical-Grade, Universal Bone Marrow Transplant Product

## Manufacturing and Treatment Process For NiCord

### Cord Blood Unit (CBU)

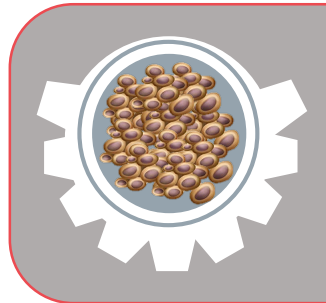


CBU selected by physician from public cord blood bank



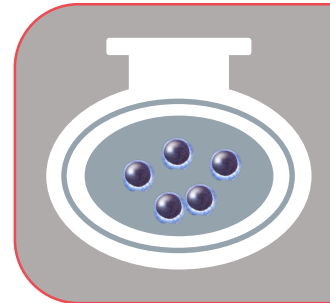
### NiCord

#### NAM-expanded cells



Stem cells cultured for 3 weeks using proprietary NAM technology

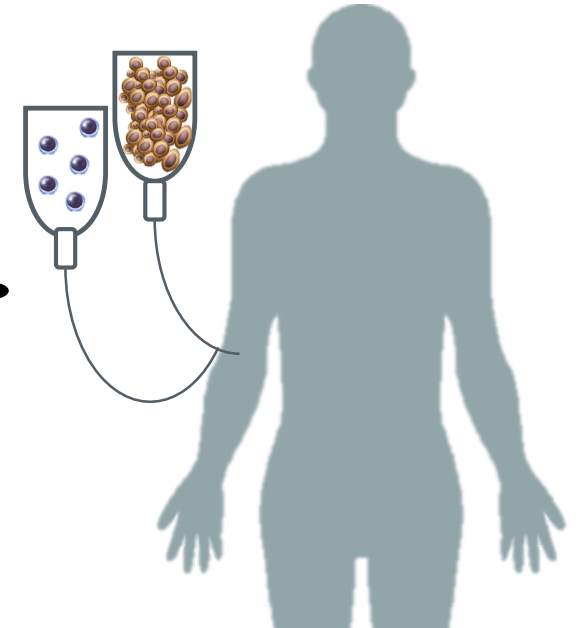
#### Uncultured fraction



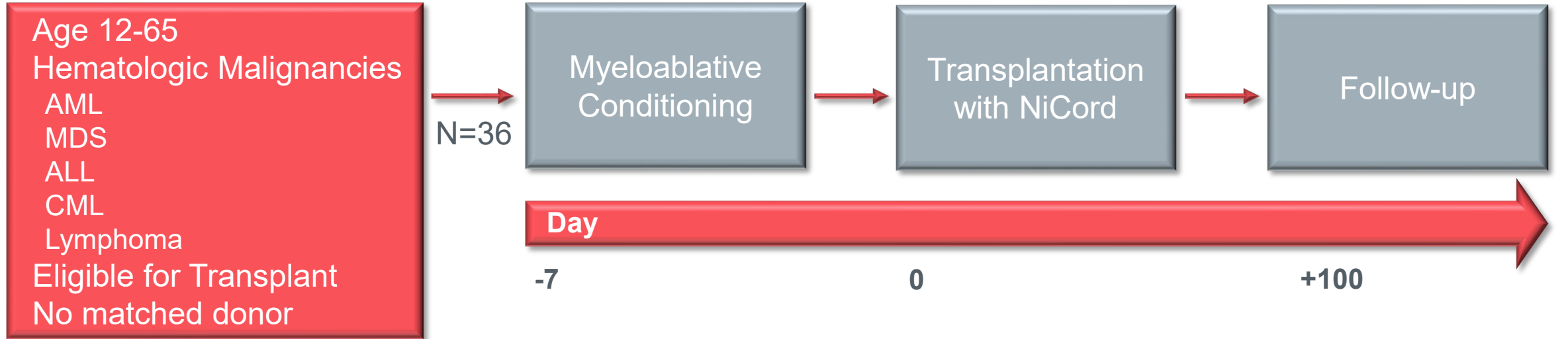
Immune cells, including T cells



### NiCord Infusion



# Phase 1/2 Study of NiCord® in Patients with Hematologic Malignancies

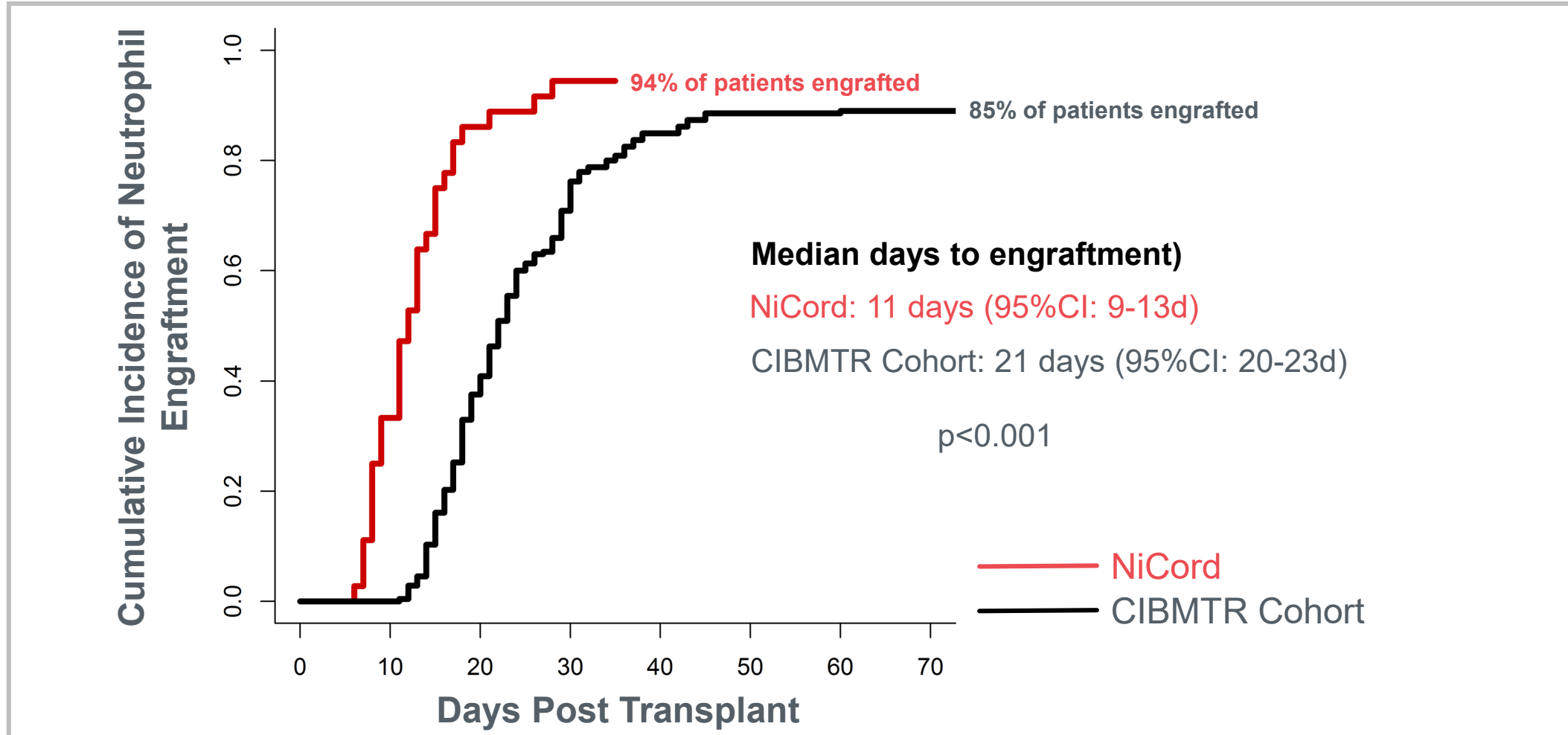


13 sites: U.S., EU, Asia

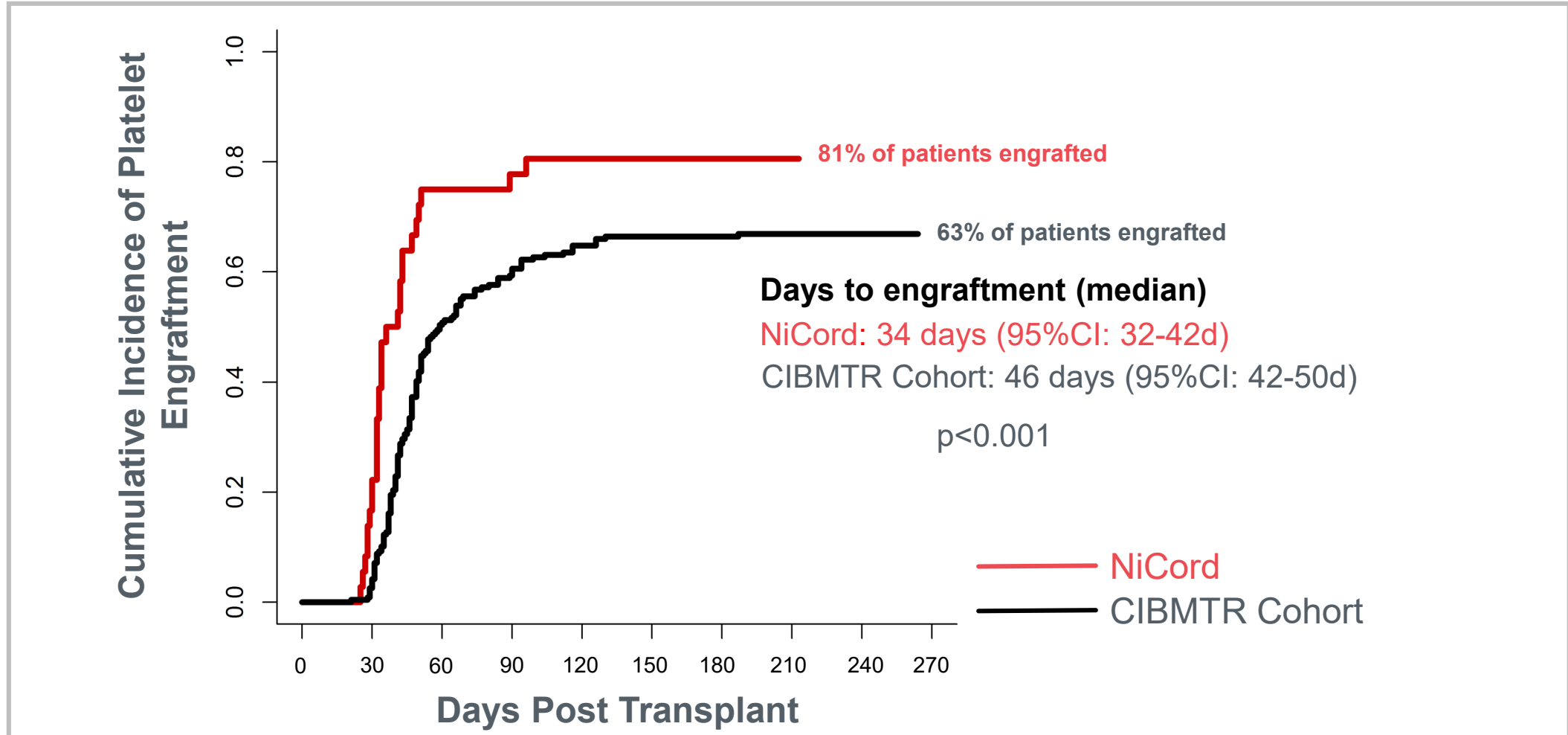
- **Primary endpoint:** Neutrophil engraftment
- **Secondary endpoints:** Platelet engraftment, acute GvHD, chronic GvHD, infections, hospitalization, non-relapse mortality, overall survival, disease-free survival

# Rapid Neutrophil Engraftment with NiCord®

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

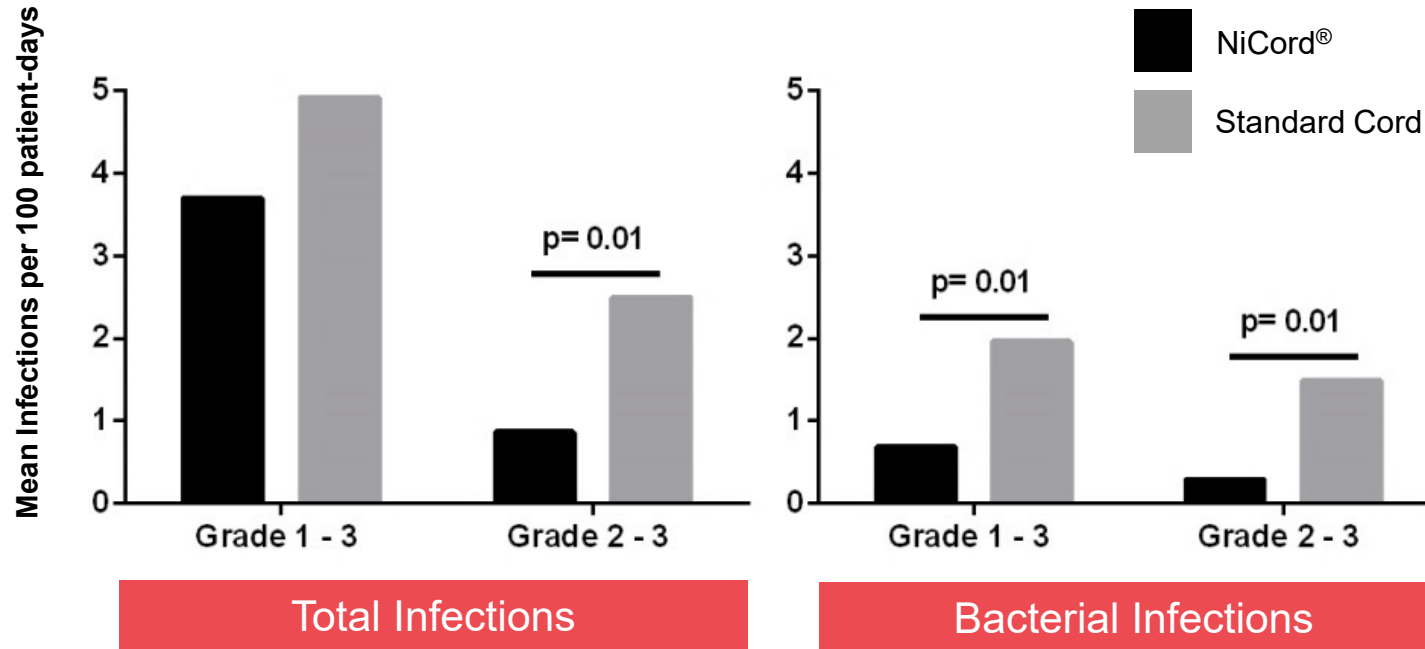


# Significantly Improved Platelet Engraftment with NiCord®

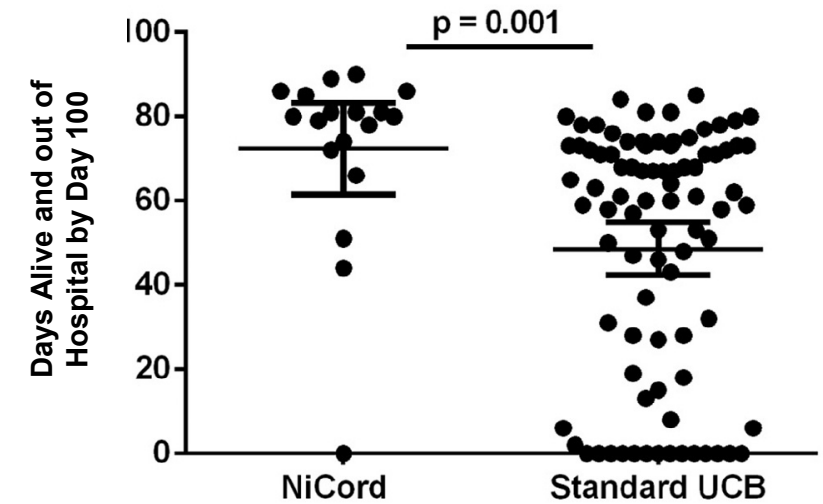


# Rapid Engraftment Is Associated with Fewer Infections and Shorter Hospitalizations

## Infection



## Hospitalization



# Phase 1/2 NiCord® Study: Secondary Endpoint Results

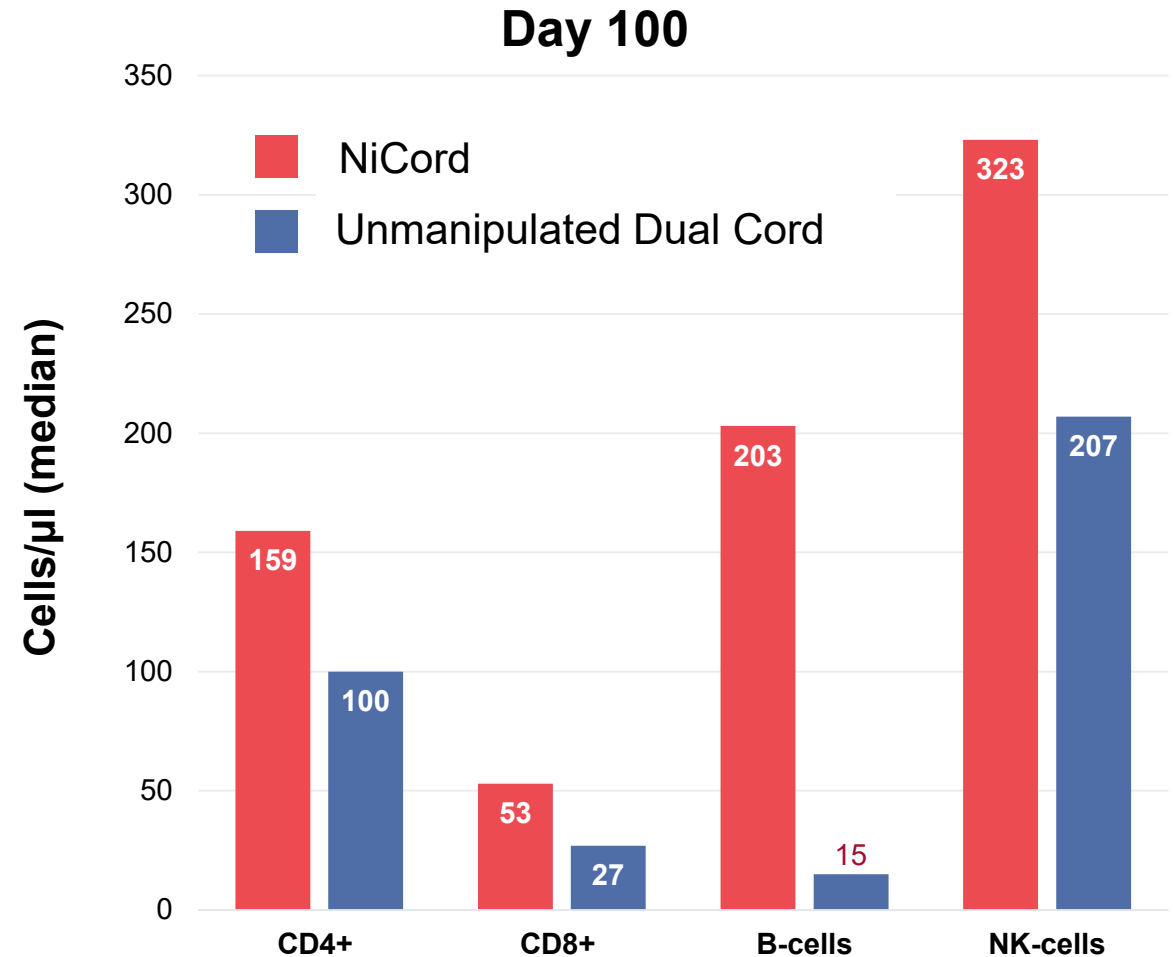
Chimerism	Full donor (>95%): 97% Mixed chimerism: 3%
aGvHD: Grade III-IV at 100 days	11%
cGvHD: Moderate-Severe at 1 year	10%
Number of days of primary hospitalization	19d
Number of days alive and out of hospital by Day 100 (median)	73d

Median follow-up 14 months (range 5-37m)



# Phase 1/2 NiCord® Study: NiCord Recipients Experienced Rapid and Robust Immune Reconstitution (IR) Following Transplantation

- NK, B-cells and monocyte reconstitution faster than transplant with unmanipulated cord blood (CB) and bone marrow (BM)
- At 100 days post transplantation with NiCord, CD4+ reconstitution is at least as fast as transplant with unmanipulated CB and BM in young adolescents
- Optimal comparison of IR in randomized Phase 3 study of NiCord is underway



# NiCord® Phase 1/2 Case Study: Patient #1

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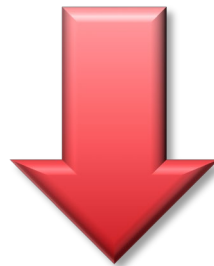
- 63 y/o retired male and avid golfer with acute myeloid leukemia, first complete remission
- No matched donors in National Marrow Donor Program (NMDP) registry
- Neutrophil recovery on Day 14 and discharged from hospital early on Day 16
- Grade II GvHD, resolved with short course prednisone
- Disease free for 7 years and returns to Duke for annual visits

# NiCord® Phase 1/2 Study: Key Takeaways

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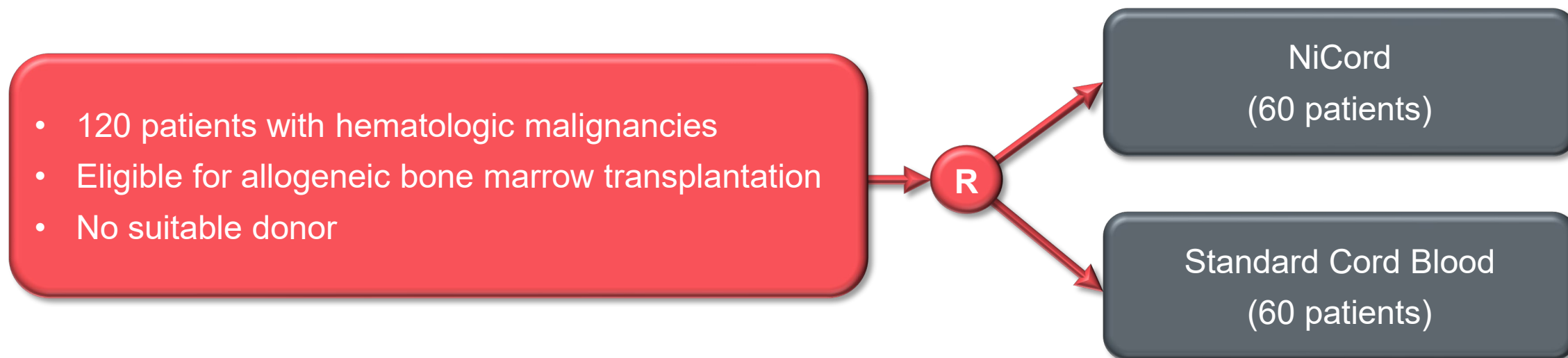
## **Results demonstrated potential of NiCord to be an effective transplantation solution for patients with high-risk blood cancers**

- Median time to neutrophil engraftment: 11 days (vs. 21 days for CIBMTR control)
- Significant reduction in rate and severity of infections as well as significantly shorter hospital stays
- Acceptable safety profile, with low incidence of acute and chronic GvHD



**FDA Breakthrough Therapy Designation**

# Phase 3 Registration Study of NiCord® for Allogeneic Transplantation in 120 Patients with Hematologic Malignancies

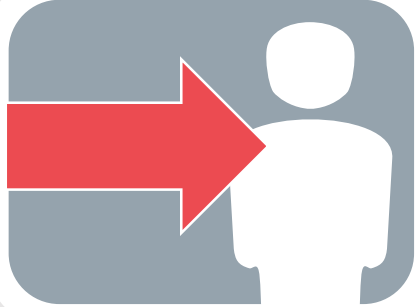


**Primary endpoint:** Time to neutrophil engraftment

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

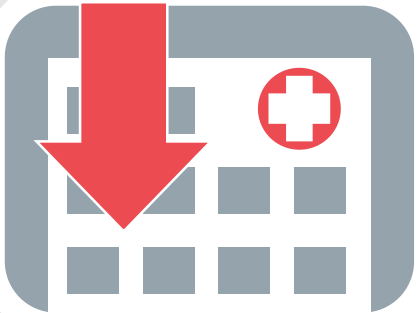
- ~25 sites initiated in the U.S., EU and Asia\*; Expect to open 15-20 additional sites
- Expected enrollment completion: 2H19; Topline data anticipated 1H20
- Concurrent collaboration with CIBMTR to collect real world outcomes in patients following bone marrow transplantation

# NiCord® Designed to Provide Significant Benefits to Patients, Physicians, Providers and Payers



## Patients and Physicians

- Better clinical outcomes
- Rapid hematopoietic recovery
- Reduced morbidity



## Providers (Hospitals)

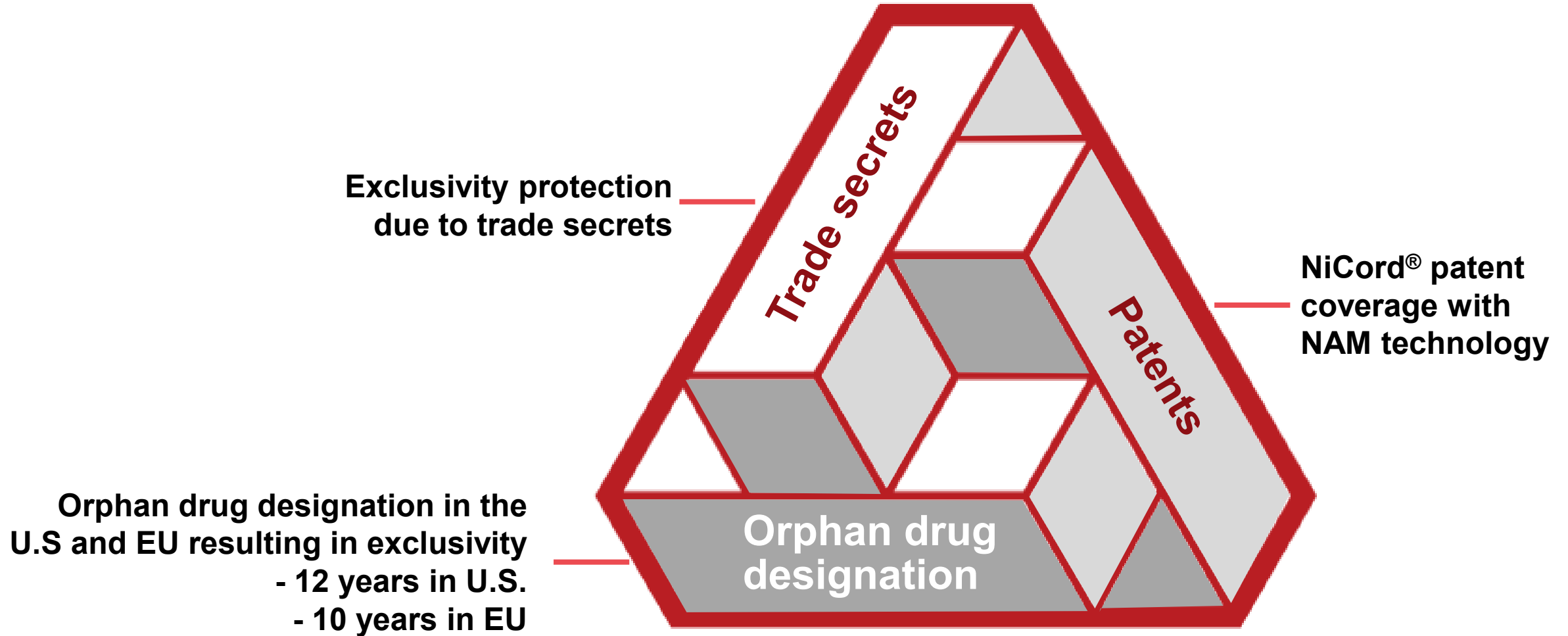
- Shorter hospital stay by ~20 days
- Improved efficiency in beds utilization, allows more patients to receive treatment
- Decrease in resource utilization
- Primarily a commercial insurance patient population



## Payers

- One time infusion with improved and potentially durable outcomes for their beneficiaries
- Potential decrease in total cost of care

# Intellectual Property and Exclusivity



# Natural Killer (NK) Cell Development Candidate

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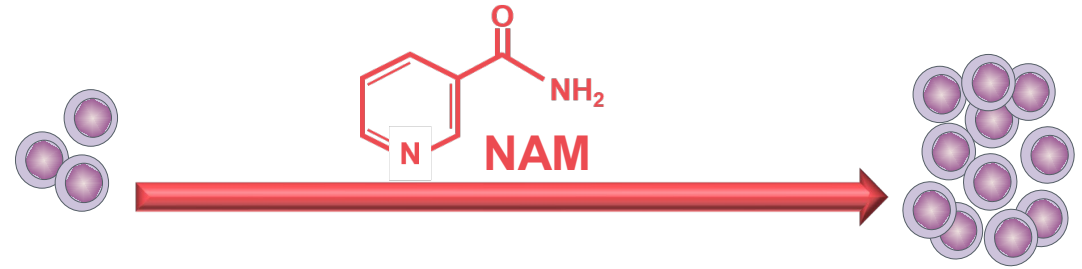
Harnessing Innate Immunity in Cancer

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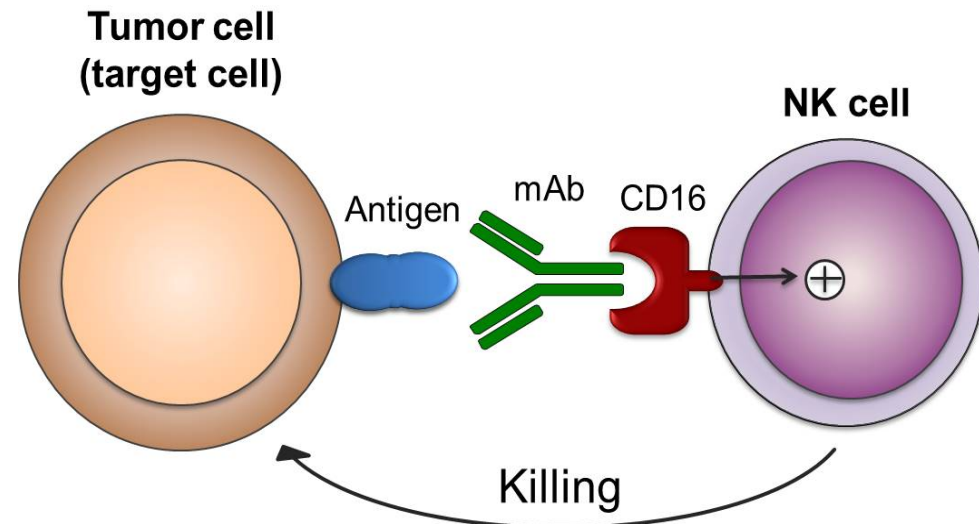
# NAM-NK Immunotherapy

- NK cells infusion is a promising immune therapy for cancer:
  - No antigen presentation required
  - No HLA-matching required
  - Low risk of inducing GvHD
  - Synergy with antibodies
  - Immune system recruitment
- Expansion is necessary to obtain clinically meaningful doses

## NAM Expansion of Natural NK Cells



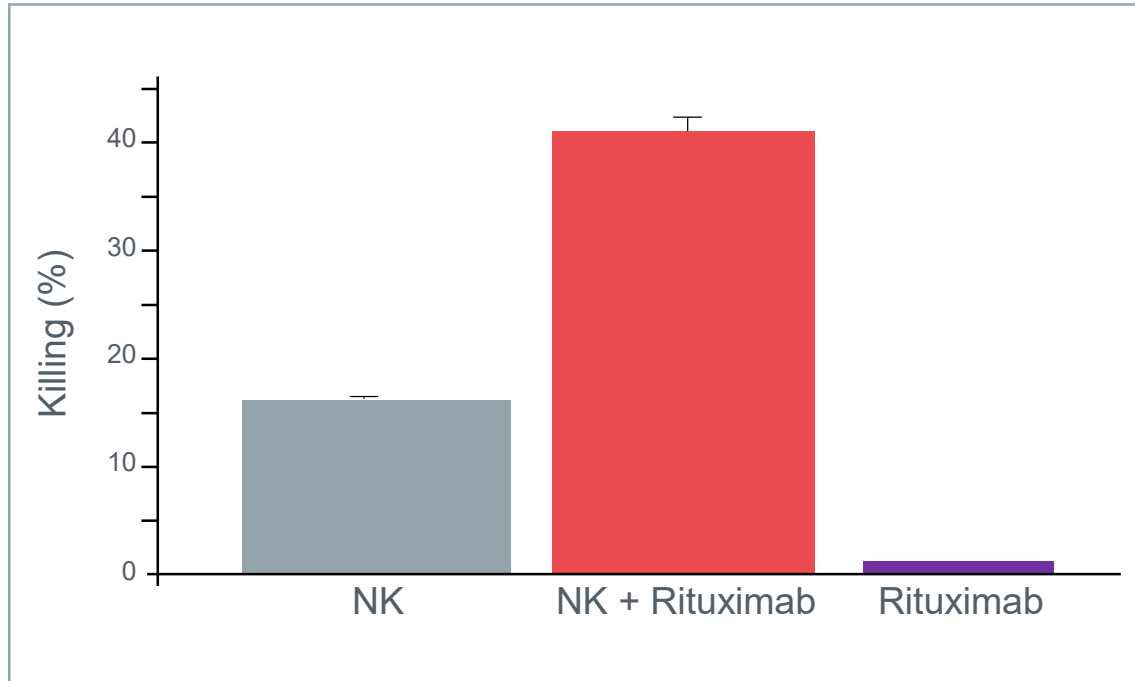
## NAM-NK + Tumor-Specific Antibodies



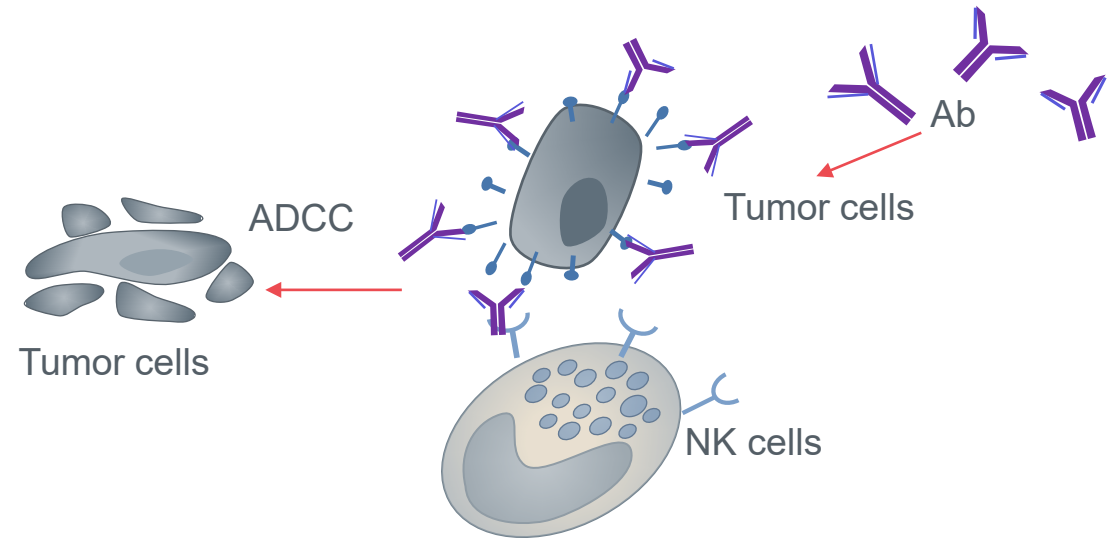


# NAM-NK Cell Immunotherapy Program

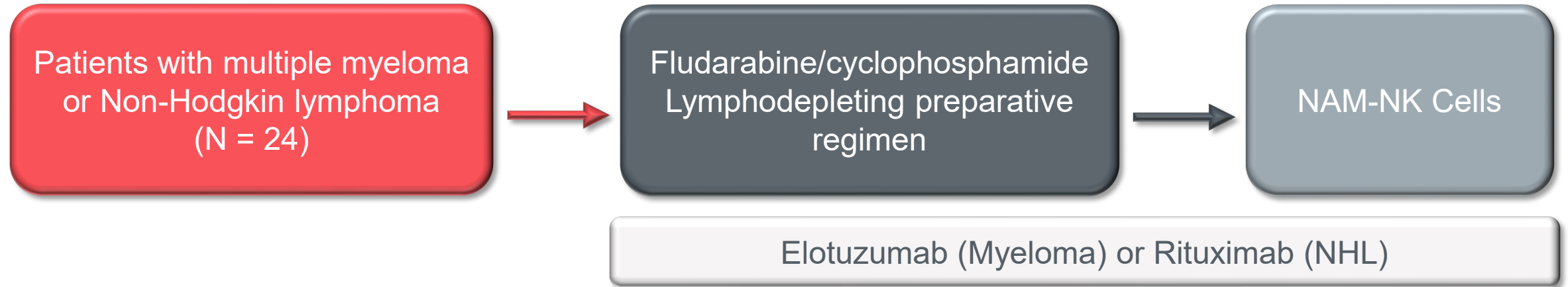
## High Affinity Antibodies Bind NK Cells and Home to Lymphoma Cells



### Antibody dependent cellular cytotoxicity (ADCC)



# Phase 1 Study of NAM-NK Cells in Patients with Multiple Myeloma or Non-Hodgkin Lymphoma



- **Primary endpoint:** Maximum tolerated dose of NAM-NK cells
- **Secondary endpoints:** Overall response, toxicity

# Phase I Study of NAM-NK: Patient Outcomes

## Preliminary Safety Results

- No cytokine release syndrome or neurotoxicity observed in the first patients treated (n=2)
- Expected short-term neutropenia and thrombocytopenia observed
- No dose limiting toxicity; no Grade 3 or Grade 4 adverse events

## Patient 002

- 67 y/o patient with follicular lymphoma diagnosed in Oct 2012; Stage IVA
  - Adenopathy in upper and lower abdomen; bone marrow involved
- Ongoing treatments since 2012; tumor continued to progress

# Phase I Study of NAM-NK: Patient Outcomes

## Patient 002: Radiographic Complete Response



- Key findings:
  - Symptomatic resolution of bulky inguinal lymphadenopathy
  - Complete response by CT/PET scan
  - Biopsy of residual mass showed no evidence of lymphoma
  - Evidence of expansion of donor NK cells in peripheral blood
- Study continues to actively enroll patients with multiple myeloma and NHL

# Corporate Information

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# Financial Snapshot

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Total cash position of \$23.8 million as of September 30, 2018

Additional gross proceeds of approximately \$53.2 million from October 2018 IPO

~70 employees

IFRS calendar year basis

# Key Completed and Anticipated Milestones 2018-2020

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## **Nicord®**

- ✓ Received Orphan Drug Designation from FDA\*
- ✓ Presented Phase 1/2 data at ASH and Tandem
- ✓ Initiated Phase 3 study in high-risk hematologic malignancies
- ✓ Initiated Phase 1/2 study in severe aplastic anemia
- ❑ 2019: Report topline severe aplastic anemia data
- ❑ 2H19: Complete enrollment in Phase 3 study in high-risk hematologic malignancies
- ❑ 1H20: Report topline Phase 3 data

## **NAM-NK Phase 1 study**

- ✓ Initiated study
- ✓ 2018: Present preliminary data
- ❑ 2019: Complete patient enrollment
- ❑ 2019: Present additional data

# Our Goal: Deliver Curative Cell Therapies to Patients with Cancer and Rare, Serious Blood Disorders

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- NiCord®
  - Phase 3 product candidate with FDA Breakthrough Therapy Designation
  - Potential to improve the practice of bone marrow transplant
  - Foundation for building a fully-integrated biotech company
- NAM-NK
  - Demonstrates therapeutic potential of NAM platform across different cell types
  - Encouraging preliminary data from ongoing Phase 1 study
  - Opportunity to build future pipeline with NAM-NK + antibody therapy
- Experienced team in place committed to building the company and realizing value for patients and shareholders

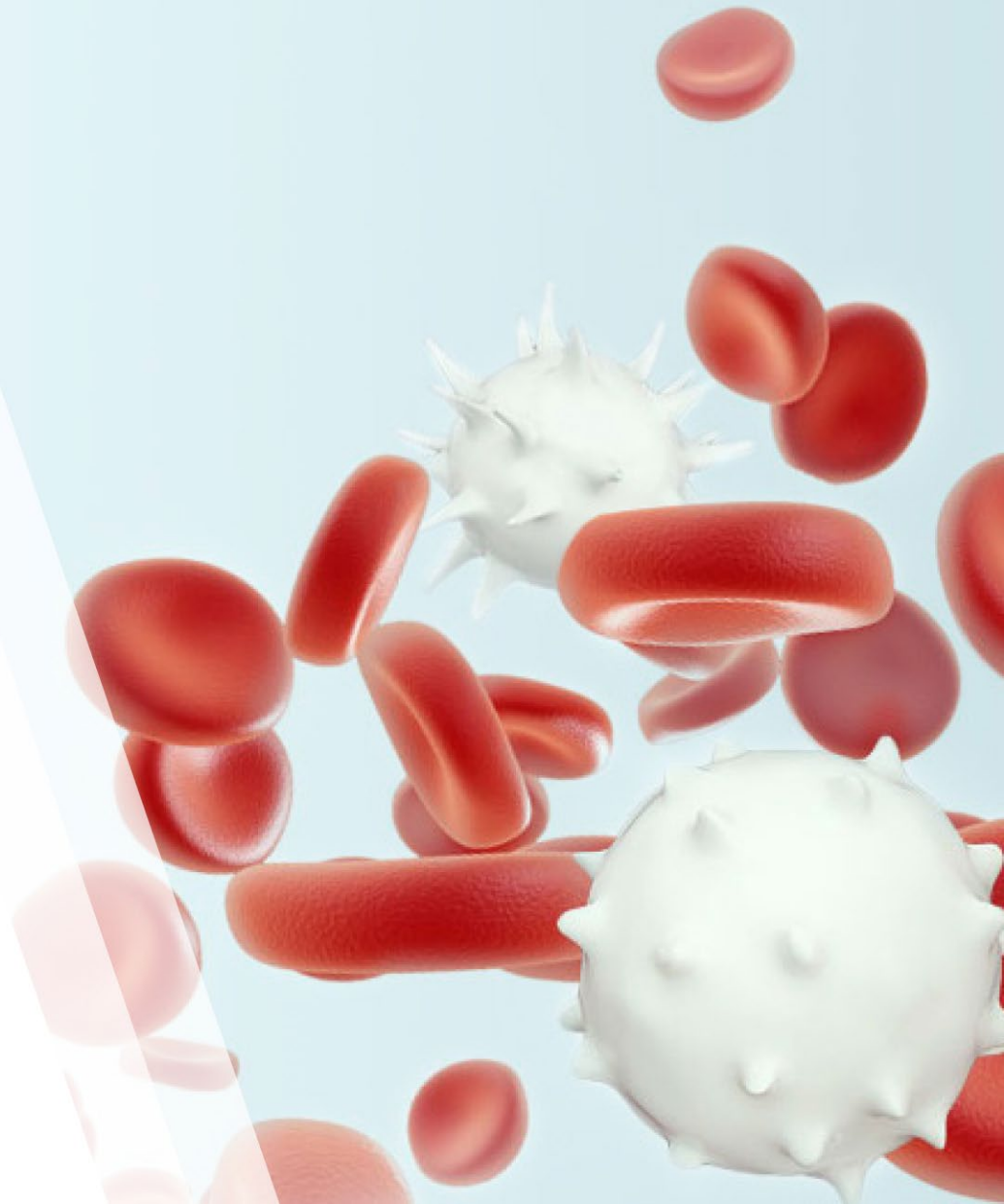




# Company Overview

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December 2018



# Appendix

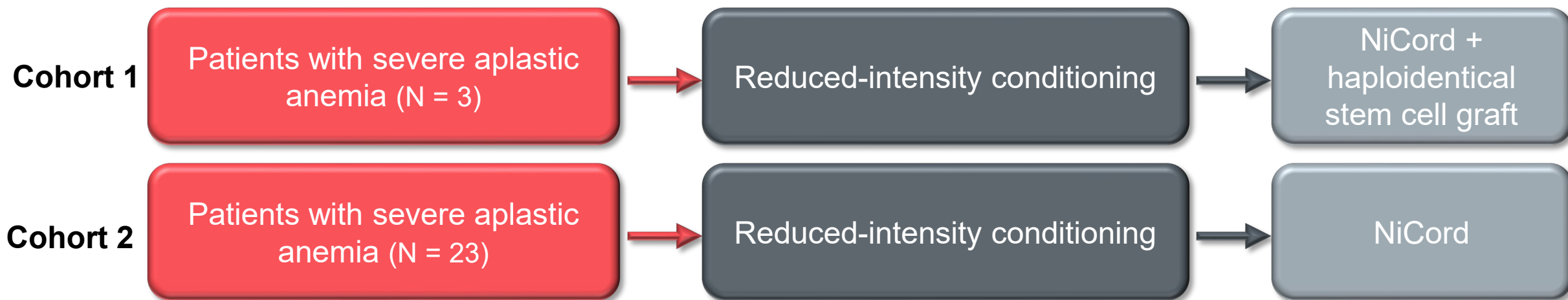
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# NiCord® Potential in Severe Aplastic Anemia

Bone Marrow Transplant Is the Only Known Cure for Severe Aplastic Anemia

**600-900 Americans are diagnosed with aplastic anemia each year<sup>1</sup>**



- Cohort 1 ongoing
- Next steps: Evaluate outcomes after first 3 patients; expansion to additional study sites
- Registration strategy: Single-arm study following FDA discussion

# Financial Snapshot

- Total cash position of \$24.8 million as of September 30, 2018
  - Including IPO proceeds, total cash position of ~\$70 million as of the end of October 2018
- 72 employees
- IFRS calendar year basis

## Income Statement

	Period ended Sep 30, 2018	Year ended Dec 31, 2017
(\$ in thousands)	Reviewed	Audited
R&D	\$17,169	\$15,018
G&A	7,008	4,472
<b>Operating loss</b>	<b>24,177</b>	<b>19,490</b>
OI&E	6,126	479
<b>Net loss</b>	<b>30,303</b>	<b>19,011</b>

## Balance Sheet

	Period ended Sep 30, 2018	Year ended Dec 31, 2017
(\$ in thousands)	Reviewed	Audited
Cash	\$23,829	\$43,622
<b>Total assets</b>	<b>28,512</b>	<b>44,922</b>
<b>Total liabilities</b>	<b>26,068</b>	<b>17,390</b>
<b>Total equity</b>	<b>(4,855)</b>	<b>22,956</b>