

# The Diverse Use of Cord Blood for Hematologic & Non-Hematologic Indications

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U.S. Department of Health and Human Services (HHS) Advisory Council  
on Blood Stem Cell Transplantation (ACBSCT) Strategic planning session

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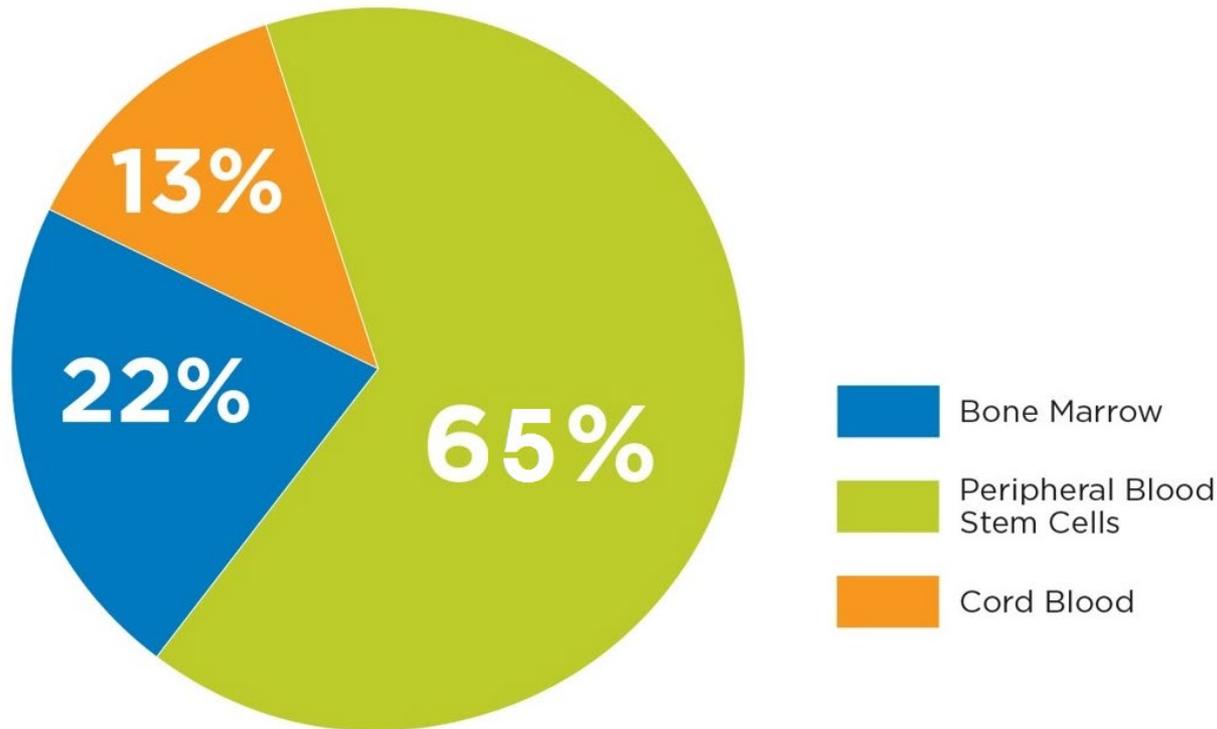
# Umbilical Cord Blood Background



- Over **35,000** cord blood units have been released by public banks for allogeneic transplantation. (WMDA)
- Approximately **every 3 minutes** one person in the United States (US) is diagnosed with a blood cancer.
- Cord blood is used in **13%** of all stem cell transplants
- Cord Blood is an option for patients with **uncommon HLA types**, including many of African American ancestry(only 30% of patients have a match within their family)
- Cord Blood is an option for patients who have an **immediate need** for a transplant

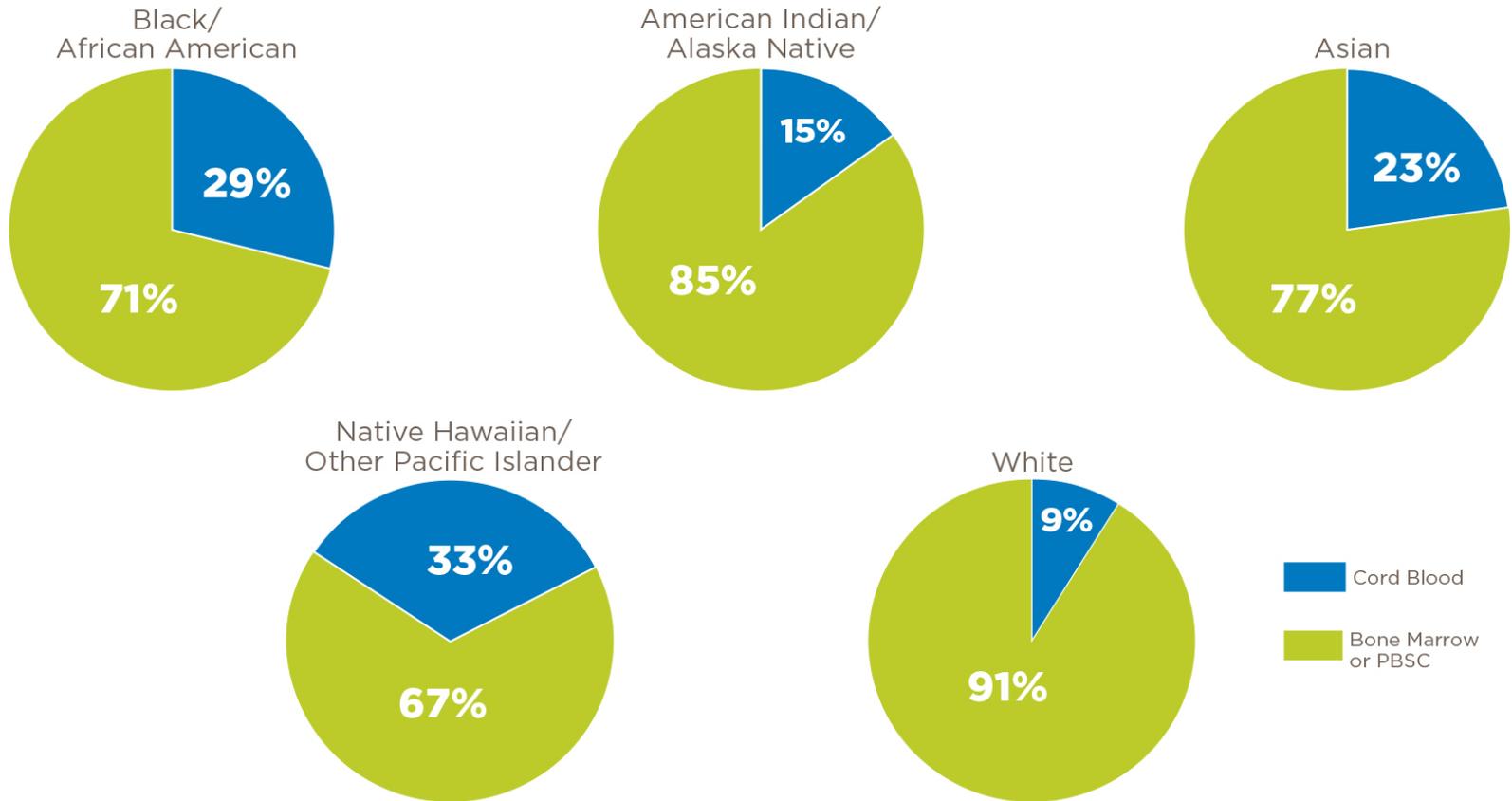
# Cord blood is used in 13% of all stem cell transplants

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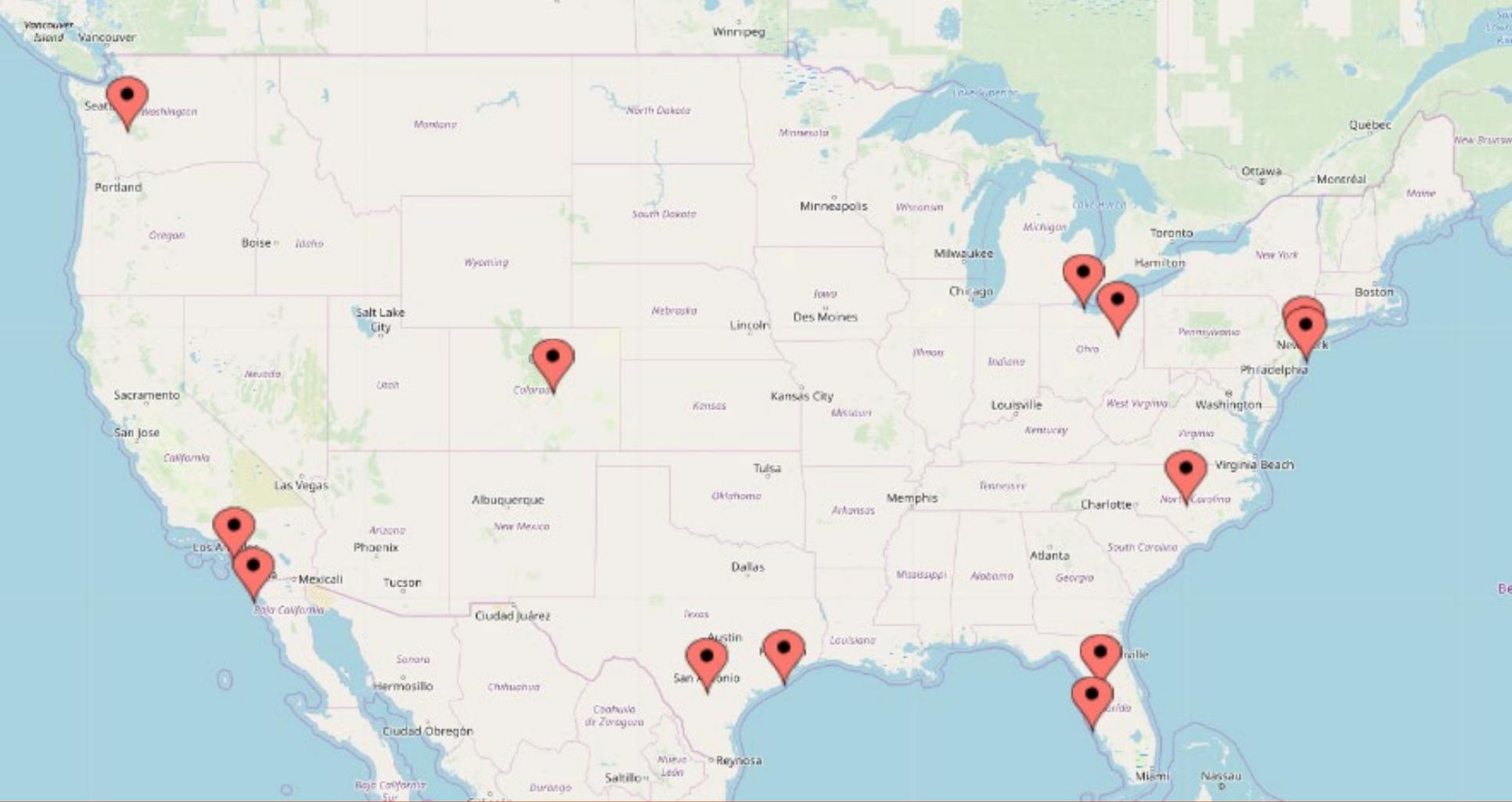


Source: National Marrow Donor Program/Be The Match FY 2017

# ROLE OF CORD BLOOD IN TRANSPLANTS BY PATIENT ETHNIC BACKGROUND



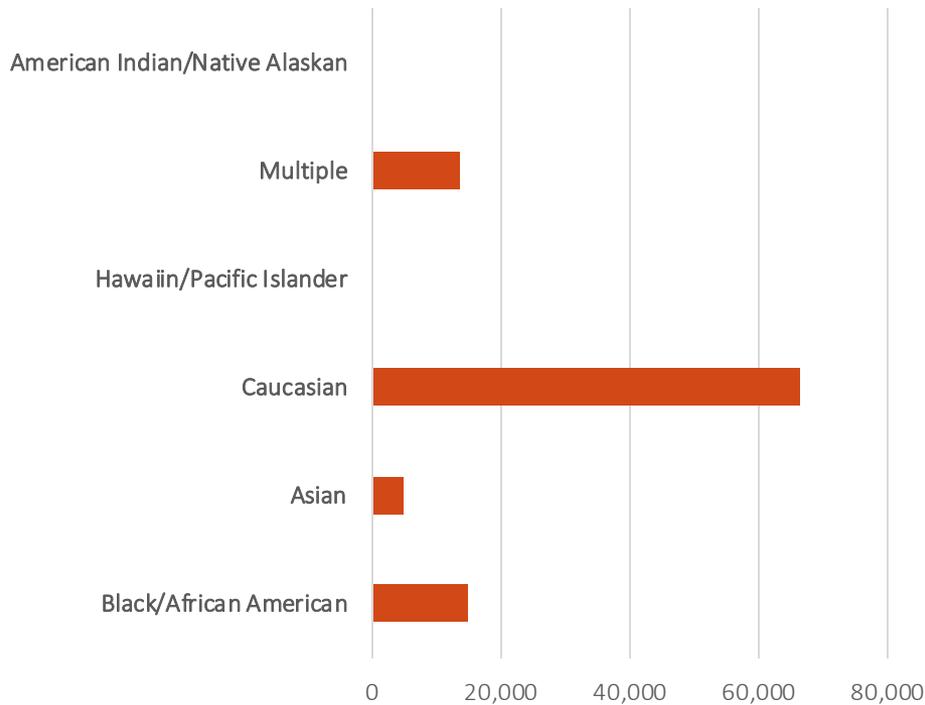
Source: National Marrow Donor Program/Be The Match FY 2017



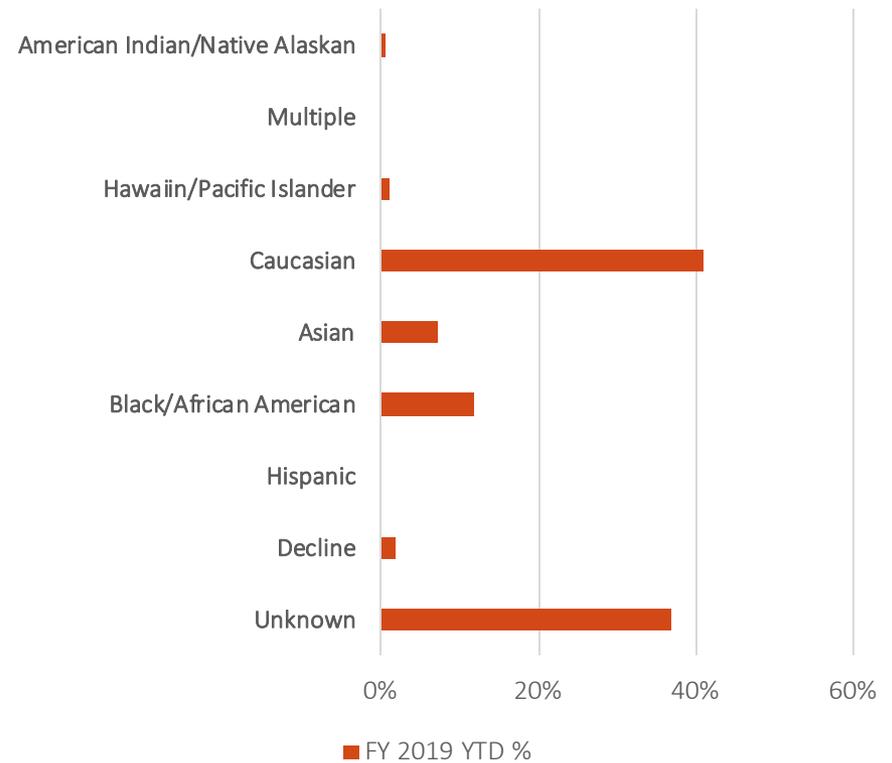
# NCBI Cord Blood Banks

# NCBI Inventory & Recipients by Race

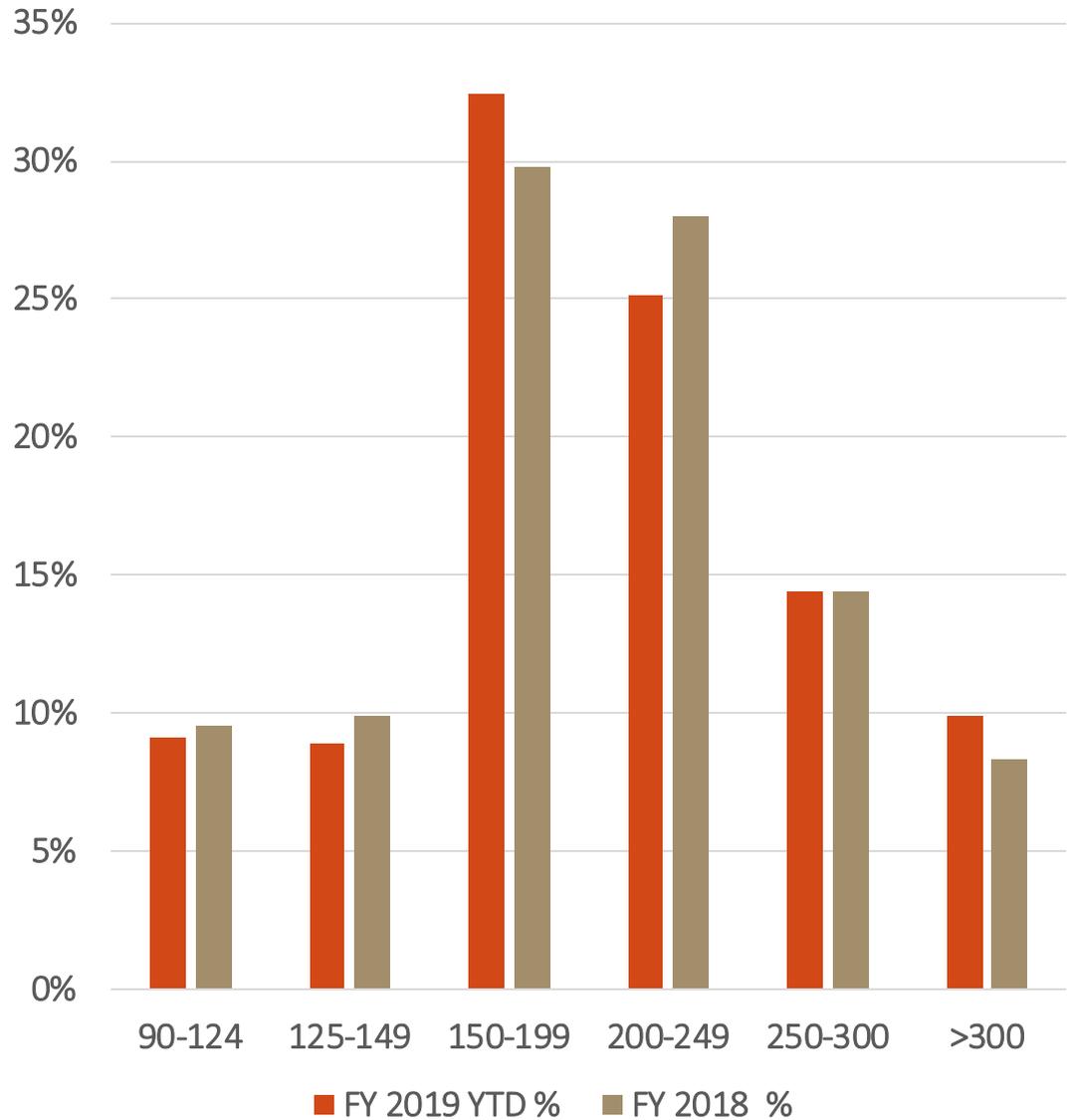
NCBI Inventory



Recipient Race- NCBI Shipments



# TNC Count of NCBI Shipped CBU





# Use of Cord Blood for Hematologic Indications



# Hematologic Diseases Treated with Cord Blood

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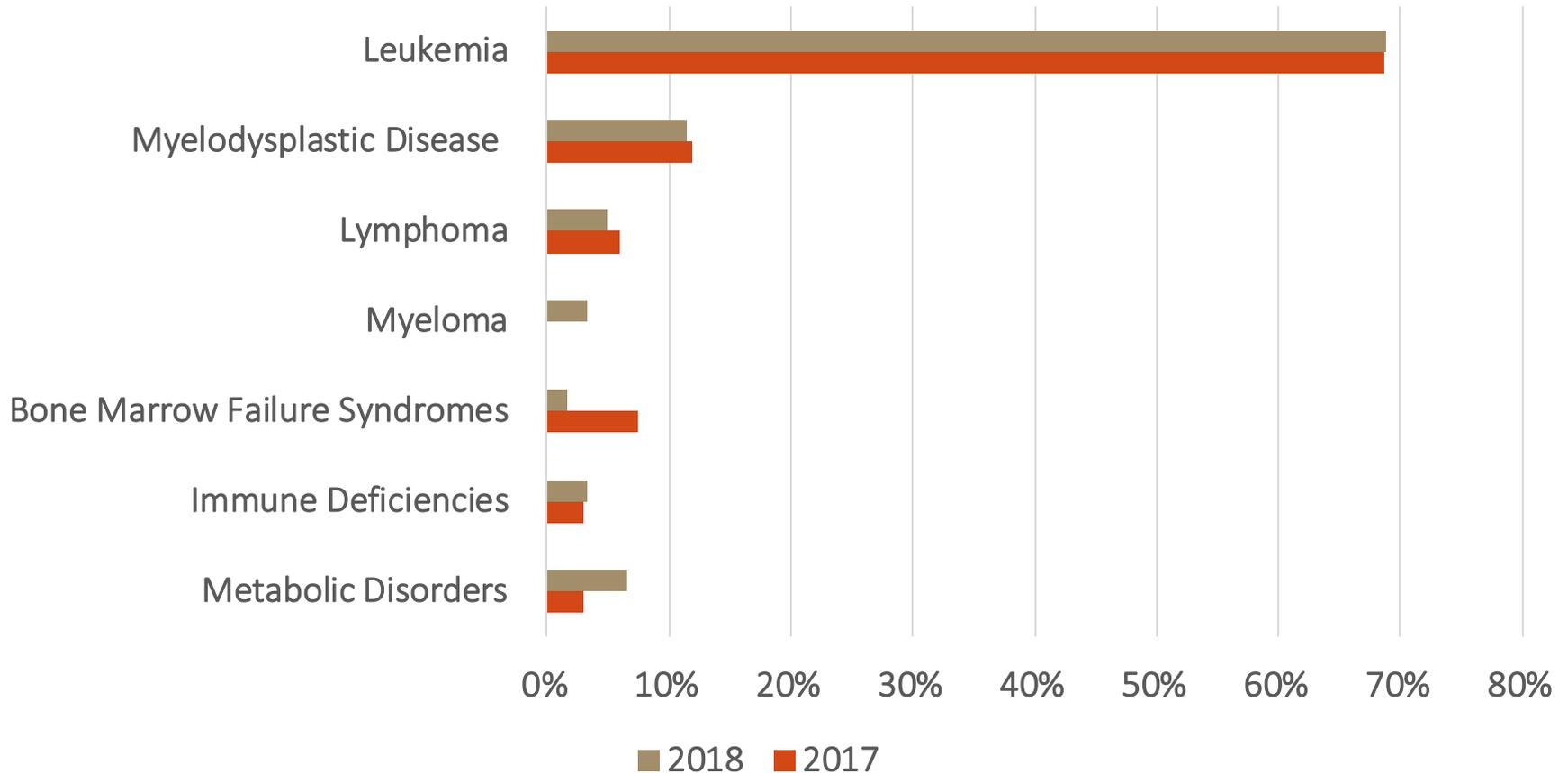
## MALIGNANCIES

- Leukemia
  - ALL
  - AML
  - CML
- Myelodysplastic Disease
- Lymphomas
  - Non Hodgkin's
  - Hodgkin's Disease
- Myeloma

## NON-MALIGNANT DISORDERS

- Hemoglobinopathies
  - Thalassemia
  - Sickle Cell Disease
- Bone Marrow Failure Syndromes
  - Severe Aplastic Anemia
  - Fanconi Anemia
  - Diamond-Blackfan Anemia
- Immune Deficiencies
  - Wiscott-Aldrich Syndrome
  - SCID
  - DiGeorge Syndrome

# Cleveland Cord Blood Center Shipments- Indications



# Research Efforts in Transplant for Hematologic Indications

- Improve Immune Reconstitution
- Speed Engraftment
- Reduce Relapse
- Expand Cord Blood
- Derive MSCs from Cord Blood
- Augment Homing





# Potential Uses of Cord Blood for Non- Hematologic Indications



# Emerging Uses for HPC-C in Non-Hematologic Disorders

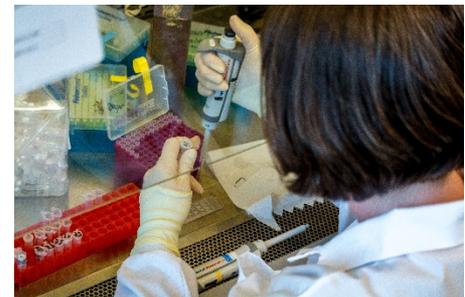
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## Allogeneic

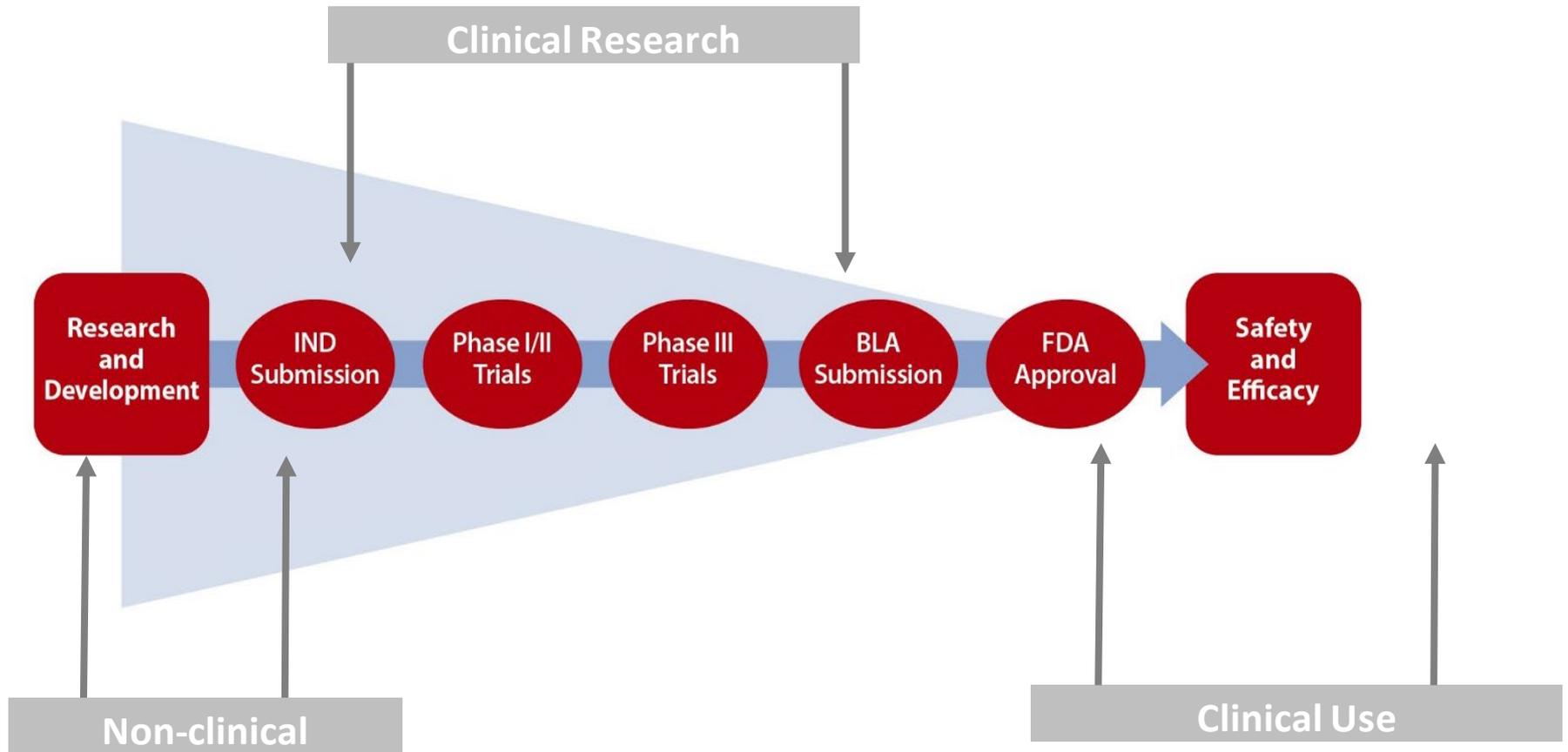
- Inborn errors
- Stroke
- Autism
- Multiple Sclerosis

## Autologous

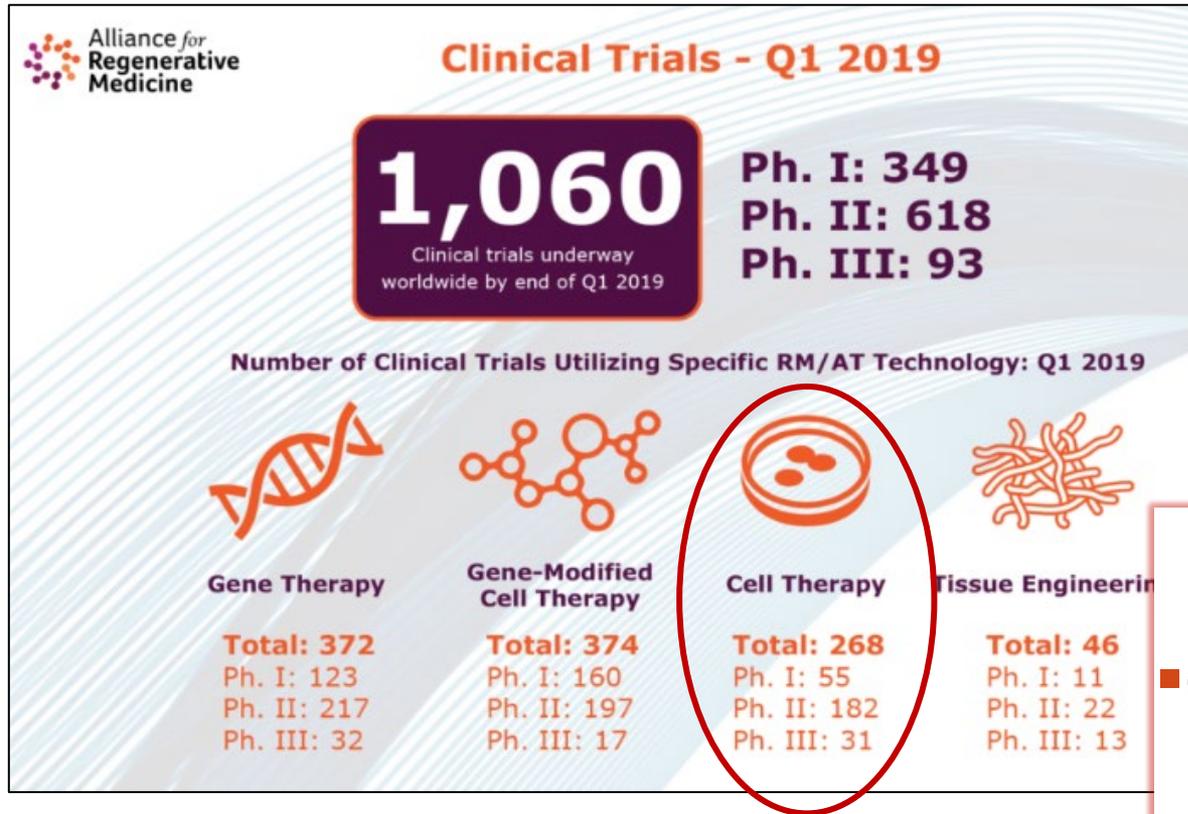
- Neural injury
- Cerebral palsy
- Autism
- Type I Diabetes



# Clinical Development Pathway



# Clinical Research- US



- ### Cell Therapy Clinical Trials:
- Over 100 CB
    - Over 60 Phase I/II
    - 3 in Phase III

# Research Examples for Non-Hematologic Disorders

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- Diabetes
- Parkinson's Disease
- Chronic, Non-Healing Wounds
- Autism
- Cerebral Palsy
- Ischemic Stroke



# Research Examples

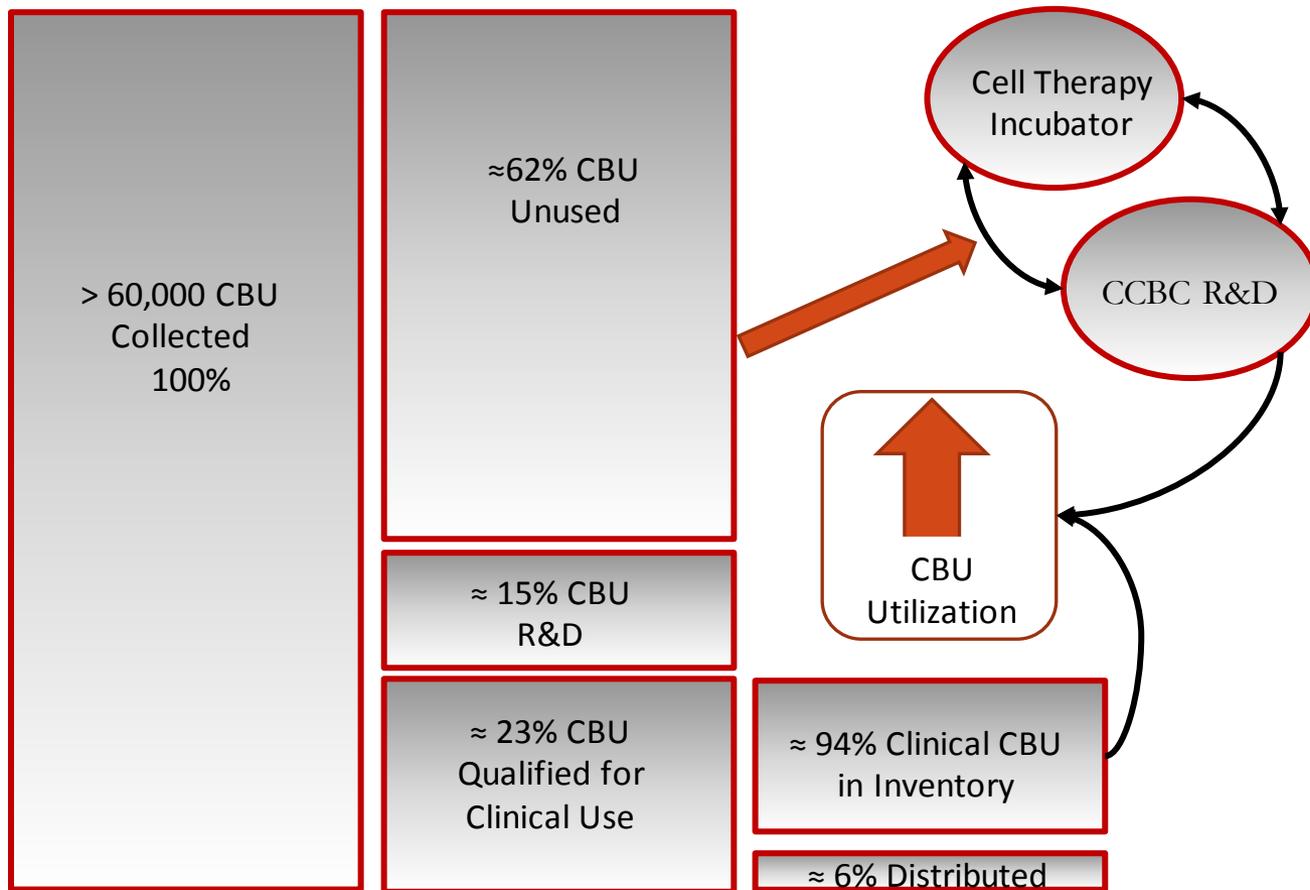
## Non-Hematologic Disorders

Approximately **780,000** Publically Banked Cord Blood Worldwide

- **6.5 million people in the U.S** currently have chronic wounds
- **1 million** Parkinson's disease (PD) patients in the US
- **3.5 million** Americans live with an autism spectrum disorder
- **9,500 children** diagnosed with cerebral palsy each year
- **795,000 people** in the US have a stroke each year

If **ONE** of these research approaches resulted in a safe & efficacious cellular therapy product, the public cord blood inventory could be more fully & effectively utilized.

# Drive Utilization of CB Inventory



# Public Cord Blood Banking Future Directions



## Optimize HPC, Cord Blood Inventory

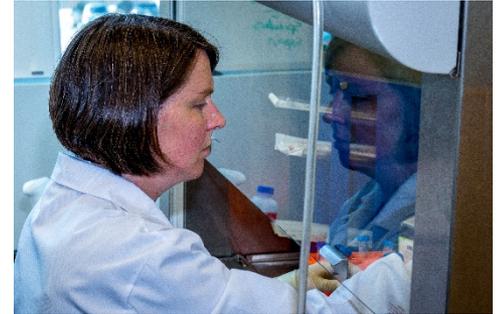
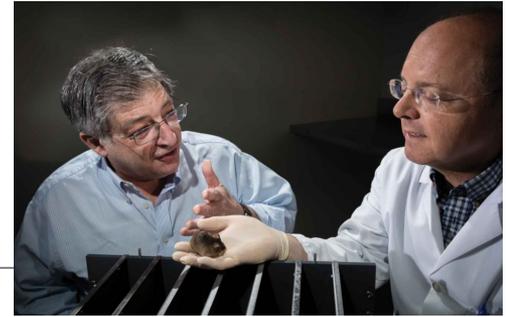
- Identify Industry Drivers
  - High TNC Cord Blood Units
  - Unique HLA Types
- Reduce Cost of Inventory Acquisition
  - Streamline Processing Procedures
  - Optimize Collection Strategies

## Develop Clinical Pathways & Product Pipelines

- In-house Research & Development
- Support External Researchers
  - Make research cord blood units available

## Leverage Existing Resources to Diversify Utility

- Support movement of cord blood derived products from R&D to FDA approval
  - Experience with FDA BLA process
  - Diverse Partnerships
  - Supply chain management



# Questions?

'As ob-gyns... , we're helping make life-giving miracles possible.'

- Hillcrest Hospital



'The highest degree of recycling there can be – I like to think of myself as a wonderful, recycled product.'

- Recipient,  
patient

'Two women were asked to donate their babies' cord blood and two women said 'Yes'!

- Recipient