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

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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.

Source: National Marrow Donor Program/Be The Match FY 2017
ROLE OF CORD BLOOD IN TRANSPLANTS BY PATIENT ETHNIC BACKGROUND

- **Black/African American**: 29% Cord Blood, 71% Bone Marrow or PBSC
- **Native Hawaiian/Other Pacific Islander**: 33% Cord Blood, 67% Bone Marrow or PBSC
- **American Indian/Alaska Native**: 15% Cord Blood, 85% Bone Marrow or PBSC
- **Asian**: 23% Cord Blood, 77% Bone Marrow or PBSC
- **White**: 9% Cord Blood, 91% Bone Marrow or PBSC

Source: National Marrow Donor Program/Be The Match FY 2017
NCBI Cord Blood Banks
NCBI Inventory & Recipients by Race

NCBI Inventory

- American Indian/Native Alaskan
- Multiple
- Hawaiin/Pacific Islander
- Caucasian
- Asian
- Black/African American

Recipient Race - NCBI Shipments

- American Indian/Native Alaskan
- Multiple
- Hawaiin/Pacific Islander
- Caucasian
- Asian
- Black/African American
- Hispanic
- Decline
- Unknown

FY 2019 YTD %
TNC Count of NCBI Shipped CBU

NMDP Data As of July 2019
Use of Cord Blood for Hematologic Indications
Hematologic Diseases Treated with Cord Blood

<table>
<thead>
<tr>
<th>MALIGNANCIES</th>
<th>NON-MALIGNANT DISORDERS</th>
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<tbody>
<tr>
<td>Leukemia</td>
<td>Hemoglobinopathies</td>
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<tr>
<td>• ALL</td>
<td>• Thalassemia</td>
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<tr>
<td>• AML</td>
<td>• Sickle Cell Disease</td>
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<tr>
<td>• CML</td>
<td>• Bone Marrow Failure Syndrome</td>
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<tr>
<td>Myelodysplastic Disease</td>
<td>• Severe Aplastic Anemia</td>
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<td>Lymphomas</td>
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<td>• Non Hodgkin's</td>
<td>• Diamond-Blackfan Anemia</td>
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<tr>
<td>• Hodgkin's Disease</td>
<td>• Immune Deficiencies</td>
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<tr>
<td>Myeloma</td>
<td>• Wiscott-Aldrich Syndrome</td>
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<td>• SCID</td>
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<td>• DiGeorge Syndrome</td>
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</tbody>
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Cleveland Cord Blood Center Shipments - Indications

- Leukemia
- Myelodysplastic Disease
- Lymphoma
- Myeloma
- Bone Marrow Failure Syndromes
- Immune Deficiencies
- Metabolic Disorders

0% 10% 20% 30% 40% 50% 60% 70% 80%

2018 2017
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

Allogeneic
- Inborn errors
- Stroke
- Autism
- Multiple Sclerosis

Autologous
- Neural injury
- Cerebral palsy
- Autism
- Type I Diabetes
Clinical Development Pathway

Non-clinical

Clinical Research

Research and Development

IND Submission

Phase I/II Trials

Phase III Trials

BLA Submission

FDA Approval

Safety and Efficacy

Clinical Use
Clinical Research - US

Clinical Trials - Q1 2019

1,060 Clinical trials underway worldwide by end of Q1 2019

Ph. I: 349
Ph. II: 618
Ph. III: 93

Number of Clinical Trials Utilizing Specific RM/AT Technology: Q1 2019

Gene Therapy
Total: 372
  Ph. I: 123
  Ph. II: 217
  Ph. III: 32

Gene-Modified Cell Therapy
Total: 374
  Ph. I: 160
  Ph. II: 197
  Ph. III: 17

Cell Therapy
Total: 268
  Ph. I: 55
  Ph. II: 182
  Ph. III: 31

Tissue Engineering
Total: 46
  Ph. I: 11
  Ph. II: 22
  Ph. III: 13

Cell Therapy Clinical Trials:
- Over 100 CB
- Over 60 Phase I/II
- 3 in Phase III
Research Examples for Non-Hematologic Disorders

- 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

- > 60,000 CBU Collected 100%
- ≈ 62% CBU Unused
- ≈ 15% CBU R&D
- ≈ 23% CBU Qualified for Clinical Use
- ≈ 94% Clinical CBU in Inventory
- ≈ 6% Distributed

Cell Therapy Incubator
- CCBR R&D
- CBU Utilization

Cleveland Cord Blood Center - Save a Cord. Save a Life.
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