CIBMTR 2018
What your work makes possible

>475,000 PATIENTS

>2,800 RESEARCHERS

>420 CENTERS

>1,200 PUBLICATIONS

>55 COUNTRIES
A WORLD OF RESEARCHERS
>5,000 researchers, clinicians, and others
>420 transplant centers
>55 countries

WORKING TOGETHER
>220 studies in progress
>1,200 publications
>2.5 million biorepository samples for
>150,000 donors and recipients

FOR LIFE
>475,000 patients registered
~23,000 new patients registered annually
PATIENTS IN THE CIBMTR RESEARCH DATABASE BY REGION

- **United States** (298,244)
- **Europe** (72,655)
- **Canada** (23,420)
- **Central/South America** (18,663)
- **Asia** (16,338)
- **Mideast/Africa** (12,690)
- **Australia/New Zealand** (11,167)
WHO WE ARE - MORE THAN A DATABASE

Scientific and Statistical Expertise

Large Network of Clinical Centers

Unique and Extensive Clinical Database

CIBMTR RESEARCH
WHY WE ARE - IMPROVING PATIENTS' LIVES

Dedicated to improving survival, treatment, and quality of life

Conduct practice-changing research helping patients and physicians

- Select donors, grafts, treatment regimens
- Evaluate patient risk
- Understand the biology of successful outcomes
- Address access to care and future workforce needs
- Identify long-term effects of transplantation and guide medical care for survivors
Collecting clinical outcomes data worldwide for 45 years
6 major areas of research activity
15 Scientific Working Committees

47 HCT global experts chair committees in their field – thousands participate

- 59 publications in 2017
- >2,800 worldwide researchers
- 30 presentations this year
- >175 ongoing studies
Coverage with Evidence Development (CED) studies allow CMS to provide coverage for patients whose data are reported to CIBMTR; CIBMTR generates the data that will inform policy decisions.

Currently engaged in 5 CMS CED studies

<table>
<thead>
<tr>
<th>Disease</th>
<th>Patient Population</th>
<th>Enrollment</th>
<th>Dates</th>
</tr>
</thead>
<tbody>
<tr>
<td>Myelodysplastic Syndrome (MDS)</td>
<td>Elderly patients with MDS</td>
<td>134 centers, 3,425 patients</td>
<td>Launched in 2010</td>
</tr>
<tr>
<td>(10-CMS-MDS)</td>
<td></td>
<td>2,151 patients ≥65 years old</td>
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<tr>
<td></td>
<td></td>
<td>1,046 patients 55-64 years old</td>
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<tr>
<td></td>
<td></td>
<td>228 patients &lt;54 years old</td>
<td></td>
</tr>
<tr>
<td>NCT# 01166009</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Disease</td>
<td>Patient Population</td>
<td>Enrollment</td>
<td>Dates</td>
</tr>
<tr>
<td>--------------------------</td>
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</tr>
<tr>
<td><strong>Myelofibrosis</strong></td>
<td>Patients aged ≥55 years with primary MF or post-essential thrombocytopenia / polycythemia vera</td>
<td><strong>104</strong> centers <strong>56</strong> patients <strong>14</strong> related (matched 6/6) <strong>33</strong> unrelated (matched 8/8) <strong>6</strong> haploidentical <strong>5</strong> unknown</td>
<td><strong>Launched in December 2016</strong></td>
</tr>
<tr>
<td>(16-CMS-MF)</td>
<td>NCT# 02934477</td>
<td></td>
<td></td>
</tr>
<tr>
<td><strong>Multiple Myeloma</strong></td>
<td>Elderly patients with Stage II or III MM or primary plasma cell leukemia</td>
<td><strong>71</strong> centers <strong>3</strong> patients</td>
<td><strong>Launched in July 2017</strong></td>
</tr>
<tr>
<td>(17-CMS-MM)</td>
<td>NCT# 03127761</td>
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</tr>
</tbody>
</table>
## MEDICARE CLINICAL TRIALS AND STUDIES

<table>
<thead>
<tr>
<th>Disease</th>
<th>Patient Population</th>
<th>Enrollment</th>
<th>Dates</th>
</tr>
</thead>
<tbody>
<tr>
<td>Sickle Cell Disease</td>
<td>Adolescents and young adults with severe sickle cell disease</td>
<td>31 centers, 43 patients</td>
<td>Launched in October 2016</td>
</tr>
<tr>
<td>(BMT CTN 1503)</td>
<td></td>
<td></td>
<td></td>
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<tr>
<td>NCT# 02766465</td>
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<td></td>
<td></td>
</tr>
<tr>
<td>Sickle Cell Disease</td>
<td>Patients aged 15-50 years with severe sickle cell disease</td>
<td>47 centers, 0 patients to date</td>
<td>Launched in November 2017</td>
</tr>
<tr>
<td>(17-CMS-SCD)</td>
<td></td>
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<td></td>
</tr>
<tr>
<td>NCT# 01166009</td>
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</tbody>
</table>
Contract RENEWED with HRSA to track and analyze all allogeneic transplants performed in the US and transplants performed globally with products from the US.
65,290 from unrelated donors and 7,251 from related donors

58,463 from unrelated recipients and 7,574 from related recipients

11,395 from unrelated cord blood units

401,190 samples from 11,395
>6,000 patients on BMT CTN trials

>2.5 million samples
NEW AREAS OF ACTIVITY

- Epigenetic Effects
- Whole genome sequencing
- Direct transition of data from the EHR
- Effect of non-HLA genes on outcome
Treatment Decision Support

- AML MATTERS, with ASH, ASCP, ONS, France Foundation
- Easy to Read Informed Consents
- Payer-Partnered Approach to Referral
- Transplant Physicians’ Perspectives on Palliative Care

Survivorship

- Individualized Survivorship Care Plans
- Patient-Centered Outcomes Research Agenda in HCT
- Stepped Care Self-Management Program for Survivors

Health Economics

- HCT reimbursement/utilization in
  - AML (Medicare; Optum)
  - Multiple myeloma (Medicare)
Blood and Marrow Transplant Clinical Trials Network (BMT CTN)

>10,000 patients enrolled on BMT CTN trials since 2003

Renewed for 4th, 7-year cycle

>80 publications

>400,000 research sample repository

48 clinical trials
Resource for Clinical Investigations in BMT (RCI BMT)

>33,500 patients accrued to

- 8 cohort and survey studies, including 21,794 on a long-term (20 years) donor follow-up study
- One Phase III, 5 Phase II studies
- 6 studies in development

- Began implementation of new electronic patient-reported outcomes (ePRO) system to be used by Survey Research Group to support:
  - Clinical trials
  - Long term follow-up
  - Quality of life assessment for patients and donors
Developing statistical models to use in HCT and cellular therapy research and comparing new models to existing solutions using the CIBMTR Research Database

- **Statistical integrity** of CIBMTR scientific activities ensured
- Articles on HCT-related statistical issues for clinical audiences supported
- Working Committee investigators supported in developing scientific studies
PUBLICATIONS
BY PROGRAM

Clinical Outcomes - Working Committees (59)

Bioinformatics (10)
BMT CTN (7)
Statistical Methodology (4)
Health Services (2)
• Benefit of autoHCT in patients with early progression of follicular lymphoma
• Transplant outcomes in patients 70 and older
• Allele-level matching for cord blood transplantation
• Comparing related and unrelated donor HCT
• Long-term outcomes after autoHCT for multiple sclerosis
• Long-term outcomes of autoHCT for Hodgkin disease
• Long-term outcomes of infants after alloHCT
• Risk of late CNS tumors in children receiving HCT
• Assessing quality of life in HCT recipients
Diverse genetic influences on GVHD, relapse, treatment-related mortality and survival
Plasma biomarkers for GVHD and its outcome
Donor experiences with second donation
Perceptions of the donation experience by children and their parents
Health care utilization after HCT for sickle cell disease
Health care costs with chemotherapy vs HCT for AML in adults
Statistical methods for personalized medicine in HCT
<table>
<thead>
<tr>
<th>Conference</th>
<th>Oral</th>
<th>Poster</th>
<th>Total</th>
</tr>
</thead>
<tbody>
<tr>
<td>American Society of Hematology (ASH) Annual Meeting</td>
<td>15</td>
<td>17</td>
<td>32</td>
</tr>
<tr>
<td>BMT Tandem Meetings</td>
<td>13</td>
<td>6</td>
<td>19</td>
</tr>
<tr>
<td>American Society of Clinical Oncology (ASCO) Annual Meeting</td>
<td>2</td>
<td>1</td>
<td>3</td>
</tr>
<tr>
<td>International Myeloma Workshop</td>
<td>3</td>
<td>0</td>
<td>3</td>
</tr>
<tr>
<td>European Federation for Immunogenetics (EFI)</td>
<td>0</td>
<td>2</td>
<td>2</td>
</tr>
<tr>
<td>Other Meetings and Conferences*</td>
<td>5</td>
<td>7</td>
<td>12</td>
</tr>
<tr>
<td>TOTAL</td>
<td>38</td>
<td>33</td>
<td>71</td>
</tr>
</tbody>
</table>
SHARING KNOWLEDGE - in ways that are useful to you

PUBLIC WEBSITE
>370,000 views

INFORMATION REQUEST SERVICE
> 400 custom analyses

DATA SHARING and ANALYTIC TOOLS

eDBtC - >260 users, >2,800 sessions, >500 downloads
Disease Risk Index (DRI) calculator - >8,200 views
Survival Calculator - >3,200 views
Center Performance Analytics - >90 users, >450 sessions
Data for RFI - >60 users, >325 sessions
CIBMTR

The Future

Everyone has a donor

CAR-T cells

Gene Therapy

New indications

New Regimens
EVERYONE HAS A DONOR

- HLA-identical sibling BM/PB
- HLA-mismatched relative BM/PB
- HLA-matched and mismatched unrelated BM/PB
- Unrelated umbilical cord blood

The Challenge:
Delivering the right graft from the right donor at the right time
It’s not just about HLA anymore!
Outcomes after alloHCT for AML in CR1, Age 20-45

RELAPSE IS OUR MOST IMPORTANT ISSUE

<table>
<thead>
<tr>
<th>Year</th>
<th>TRM</th>
<th>Relapse</th>
</tr>
</thead>
<tbody>
<tr>
<td>1980-89</td>
<td></td>
<td></td>
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<tr>
<td>1990-99</td>
<td></td>
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<tr>
<td>2000-09</td>
<td></td>
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<tr>
<td>2010-14</td>
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</tr>
</tbody>
</table>
NEW REGIMENS TO PREVENT RELAPSE

- **Antibodies**: rituximab, daratumamab, inotuzumab, blinatumomab
- **Targeted drugs**: imatinib, nilotinib, ibrutinib, FLT3 inhibitors
- **Hypomethylating agents**: azacitidine, decitabine
- **Immune active agents**: lenalidomide, checkpoint inhibitors

Most of these are disease/subdisease-specific.

**The Challenge:**
Delivering the right graft from the right donor at the right time with the right pre- and posttransplant regimen.
OTHER CELLULAR THERAPIES

- Genetically modified stem cells
- CAR-T cells for cancer
- Virus or Tumor-specific cytotoxic lymphocytes
- Mesenchymal cells

The Challenge:
Understanding both early and late effects
State of the Art Cellular Therapy Outcomes Registry established, designed to address the needs for long-term follow-up of patients receiving genetically manipulated cells

- Integrated with HCT outcomes registry
- **Pilot study** of cellular therapy data collection **completed**
- **New versions** of cellular therapy data collection forms using input from the pilot study, industry partners and regulatory agencies
- Time studies to estimate appropriate reimbursement schedules
- Regenerative Medicine Outcomes Registry strategy meeting
NEW INDICATIONS

- Autoimmune diseases
- Sickle cell disease and other hemoglobinopathies
- Solid tumors
- Tissue repair - regenerative medicine

The Challenge: Adapting our systems to accommodate diseases with different kinds of endpoints
TOGETHER, WE CAN MEET THE CHALLENGE
thank you
ONLINE RESOURCES

CIBMTR.ORG

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theCIBMTR