Center Outcomes Forum - 2023

October 18, 2023

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CENTER FOR INTERNATIONAL BLOOD & MARROW TRANSPLANT RESEARCH

The CIBMTR® (Center for International Blood and Marrow Transplant Research®) is a research collaboration between the National Marrow Donor Program® (NMDP)/Be The Match® and the Medical College of Wisconsin (MCW).

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Agenda – COF 2023

- Purpose
- Agenda Overview
  - Center-Specific Survival Analysis 2023 Summary
  - Research presentations
  - Workgroup presentations and DISCUSSION
- Communication Plans
- Next steps/Wrap Up

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My sincere appreciation

- Center-Specific Analysis “Core” Team:
  - Charimar Santiago-Parrilla, Michael Heim, Molly Allbee-Johnson, Waleska Perez, Sue Logan, Jenni Bloomquist, Eileen Tuschl, Laura Clements, Steve Spellman, Brent Logan, Kwang Ahn, Kristin Page
- Carol Doleysh – Program Manager, SCTOD
- Alicia Halfmann and Carol – Center Outcomes Forum
- Workgroups and presenters:
  - Akshay Sharma, Christopher Strouse, Wael Saber
  - WG leaders: Chris Dandoy, Kristin Page, Mike Verneris
  - WG panelists – See Appendix

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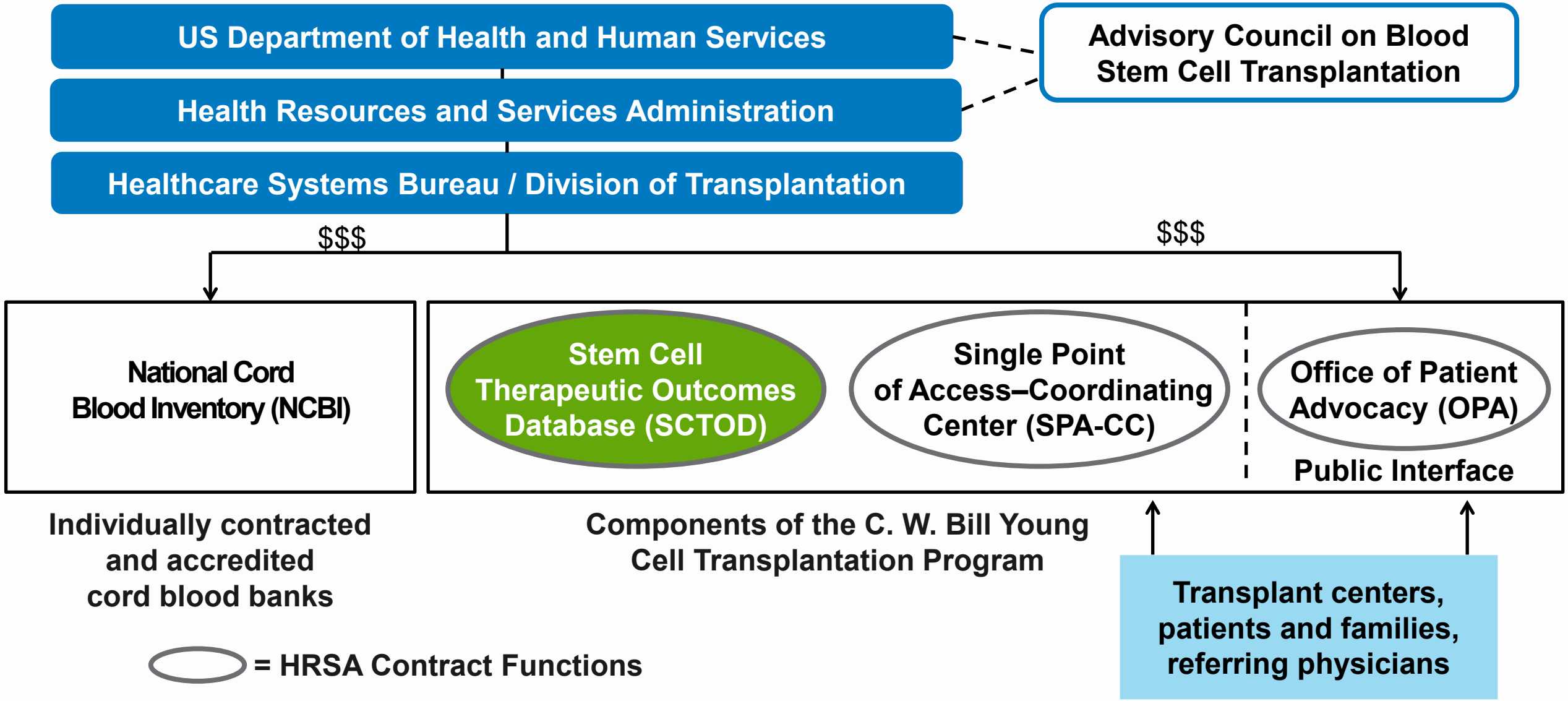
C.W. Bill Young Cell Transplantation Program*



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* Established by the Stem Cell Therapeutic and Research Act of 2005 and reauthorized by the Stem Cell Therapeutic and Research Reauthorization Acts of 2010 and 2015 and the TRANSPLANT Act of 2021

Center-Specific Survival Analysis 2023

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Center Outcomes Report

Final study population - 2023

- Center outcomes report 2023 includes 3 full years of data:
  - Unrelated and Related HCT 2019 – 2021
- Multivariate analysis adjusts for ‘risk factors’
- Centers must have >90% overall f/u at 1 year
- 178 US centers; 25,532 patients first allogeneic HCT
  - 4 centers (~553 pts) removed because of quality concerns in audits
- Primary outcome: One-year survival
  - Overall: 76.2% (78.1% REL, 74.6% UNR) – 2021: 76.5, 78.3, 75.1

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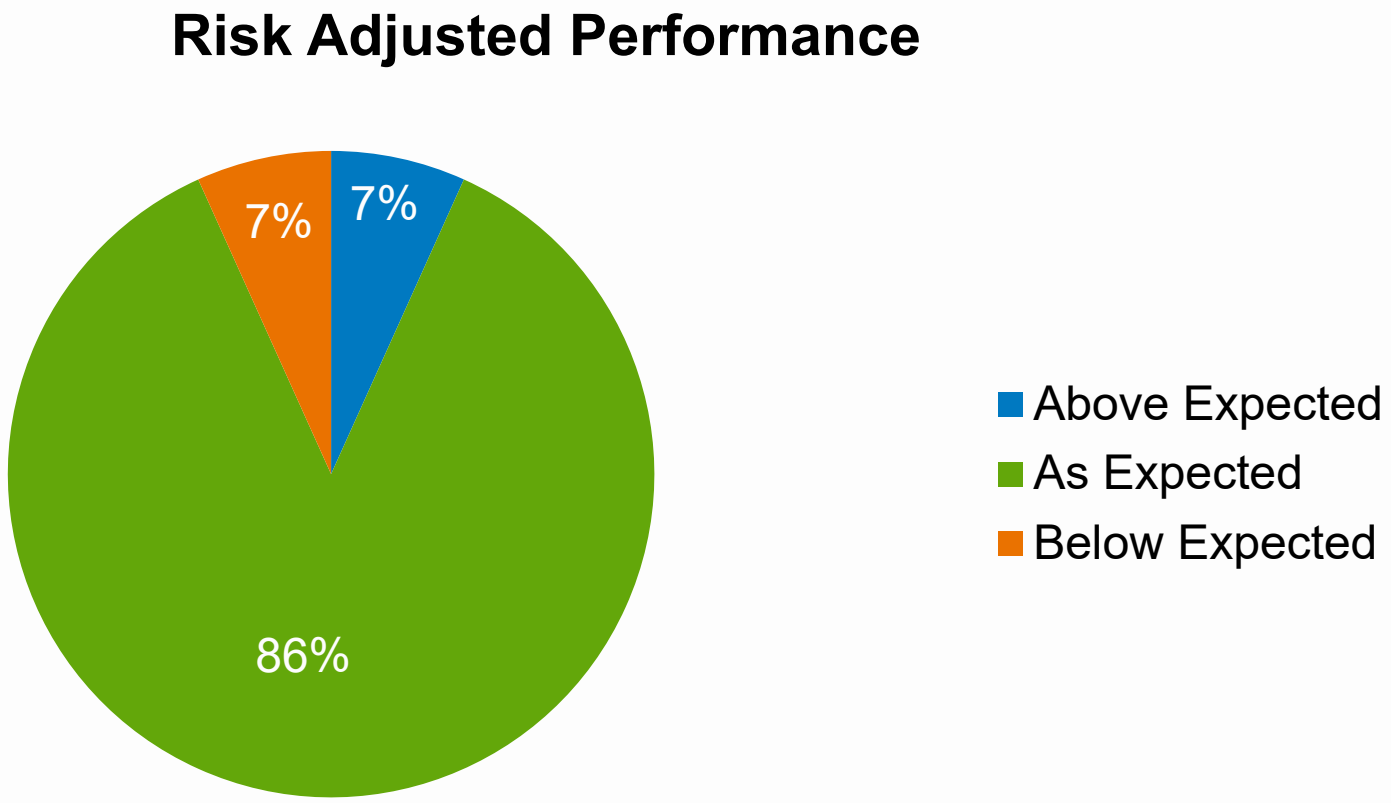
How are US centers doing? 2023



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What else did we test in the 2023 Report

- COVID Infection pre-HCT
- Previous Solid Organ Tx
- Distinct disease category for BM Failure syndrome
- Updated AML ELN 2022
- Time from dx to tx for AML PIF
- Updated ALL cytogenetic /molecular risk stratification
- MRD for AML and ALL
- Updated other acute leukemia categories and dz status
- Updated MM cytogenetic risk
- R-ISS for MM

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What was changed in the 2023 Report

- Additions:
  - Previous Solid Organ Tx
  - Distinct Disease category for BM Failure syndrome
  - AML ELN 2022
  - ALL cytogenetic/molecular risk stratification
  - Updated “other acute leukemia” categories and dz status
- No change or removed:
  - MRD for AML and ALL
  - Updated MM cytogenetic risk
  - R-ISS for MM
  - Therapy-related AML and MDS

What are our plans for 2024 Analysis?

- Re-assess MRD for AML, ALL based on best available existing data
- Test new variables based on COF 2018/2020 recommendations for:
  - Hemoglobinopathies – TRJB, LIC, RBC dependence, iron chelation
  - Immune system disorders – active/recent viral infection, ever PJP
  - Histiocytic disorders - active/recent viral infection, ever PJP
- Test new variables for pediatric pts – Peds GFR, Cong heart disease

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What are our plans for 2024 Analysis?

- Test serum ferritin, albumin, plt at transplant, poverty, prior cell therapy
- Incorporate, where possible, ideas brought forward from this Center Outcomes Forum

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What else are we doing? - Research

- SC 21-02 (Sharma A, et al)
  - Impact of Center Specific Analysis scores on Hematopoietic Cell Transplant Center Volumes
  - Transplant Cellular Therapy 2023 Aug;29(8) 523-528.

- SC 21-01 (Strouse C, et al)
  - Impact of Publicly Reported Center Specific Analysis on Patient Selection Practices for HCT.
  - ASH 2023 oral abstract

Workgroup 1 Charter

I. Key Questions for the workgroup to address:
   a. What revisions should CIBMTR consider making to current processes to collect recommendations about data (especially disease-specific) to be used in risk adjustment for the center specific survival analysis? This is essential to maintain up-to-date and relevant risk adjustment
   b. What (if any) additional approaches to communication should be made by CIBMTR to inform relevant stakeholders about the center specific survival analysis and ongoing changes to risk adjustment?

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Workgroup 2 Charter

I. Key Questions for the workgroup to address:
   a. What additional information already collected by the CIBMTR should be used for disease-based risk adjustment for AML, ALL, and MDS?
   b. Should CIBMTR begin to use MRD as a risk adjustment in AML and ALL, and if so, what criteria should be used to define MRD?
   c. What changes in data collection are recommended soon to improve risk adjustment for AML, ALL, and MDS while being respectful of practical issues related to data collection at centers?

Workgroup 3 Charter

I. Key Questions for the workgroup to address:
   a. Should CIBMTR collect specific information about patients' participation in transplant-related clinical trials to inform future analyses about the impact of trials-based care on clinical outcomes?
   b. What specific types of information should be collected by CIBMTR for this objective? What barriers must be overcome?
      i. Phase of trial development?
      ii. Type of intervention (eg directly related to transplant procedure (prep regimen, graft source/manipulation, gvhd prophylaxis), supportive care, treatment of early complications, or other) ?
      iii. Identify discrete, unambiguous, and readily available data that can provided by centers to achieve this objective
   c. Beyond traditional patient-, disease-, and transplant- characteristics, should CIBMTR test information related to clinical trials participation for inclusion in future center specific risk adjustment model?
      i. Are there potential negative consequences that can be anticipated?

Thank you

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