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Center Outcomes Reporting:

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Agenda

01

Overview of 2025 Analysis and purpose COF

02

Risk Adjustment Acute Leukemia

03

Optimizing adjustment of comorbidities

04

Refining Transplant related factors

05

Wrap-up/Next Steps

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My Sincere Appreciation

- Center-Specific Analysis "Core" Team:
  - Dylan Liu, Min Chen, Cuyler Huffman, Molly Allbee-Johnson, Waleska Perez, Sue Logan, Jenni Bloomquist, Eileen Tuschl, Laura Clements, Steve Spellman, Brent Logan, Kwang Woo Ahn
- Carol Doleysh – Program Manager, Stem Cell Therapeutic Outcomes Database (SCTOD)
- Alicia Halfmann and Carol and meetings team – Center Outcomes Forum (COF) coordination
- Presenters, committee members, participants

Connect to Purpose

Why do we do it?

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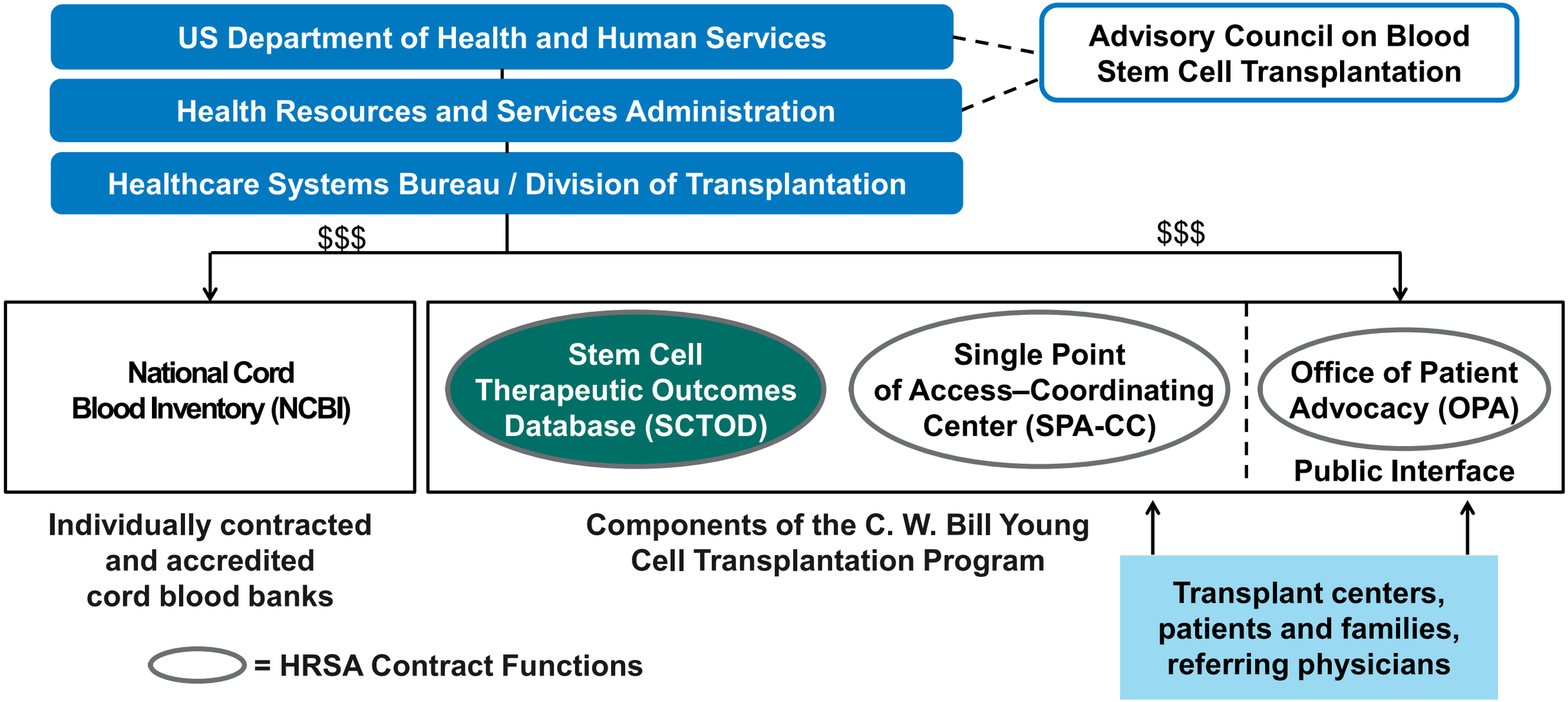
C.W. Bill Young Cell Transplantation Program*



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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

Why Do Center Outcomes Reporting?

- Contractual obligation of the SCTOD for US Centers
- There are some studies suggesting public reporting / transparency improves outcomes – but overall evidence is inconsistent
- Inform patients who are searching for centers
  - Medical literature: Utilization variable
- Provide high quality data to support centers' quality improvement efforts
  - FACT or JACIE accreditation
  - Some international centers participate

What is the MAIN Goal?

- Provide an equitable, balanced, scientific performance measurement tool(s) that can be used to define and improve quality.
- While:
  - Acknowledging limitations
  - Avoiding misuse/misinterpretation
  - Striving for continuous improvement
- Be a resource to support the HCT community

CSA Methodology

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Statistical Methods (abbreviated)

- First allogeneic HCT only
- Observed survival probability: Kaplan-Meier estimates of one year survival, by center
- Predicted survival probability (Risk adjustment):
  - Multivariate modeling accounts for the types of patients being transplanted at the center
  - Fixed effects censored data logistic regression model allows for incomplete follow-up (within reason)
    - No center effect
  - Includes calculation of 95% confidence limits around the predicted survival probability
- Comparison of the observed to the 95% CI of the predicted survival probability in each center

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Statistical Methods (abbreviated)

- Predicted survival outcome at a given center is based on the average predicted survival of patients transplanted at that center
  - Directly comparable to unadjusted K-M estimate to assess center performance
- This represents what we would have expected to happen to the patients at that center if they had been transplanted at a "generic" center in the network (i.e. no center effect)
- Need to account for sampling variability in comparing observed and predicted outcomes
  - Confidence limits built from predicted survival estimates

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Center Outcomes Report

Final study population - 2025

- Center outcomes report 2025 includes 3 full years of data:
  - Unrelated and Related HCT 2021 – 2023
- Multivariate analysis adjusts for ‘risk factors’
- Centers must have >90% overall f/u at 1 year
- 182 US centers; 26,422 patients first allogeneic HCT
  - 6 centers (~1,083 pts) removed because of quality concerns in audits
  - Final population 176 centers, 25,298 patients
- Primary outcome: One-year survival
  - Overall: 77% (79% REL, 76% UNR)

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Significant Risk Factors Included

- Patient:
  - Age
  - Race
  - CMV status
  - Year of HCT
  - KPS/Lansky Score
  - HCT-CI in adults
  - Peds HCT-CI (malignant, non-malignant)
  - BMI (adult underweight)
  - Serum albumin pre-HCT
  - Platelet count pre-HCT (exclude relevant dz)

- Patient:
  - Hx of mech ventilation (adult)
  - Hx invasive fungal infection (adult)
  - Previous autoHCT,
  - Previous Solid organ transplant
  - Recipient median household income (deciles)

Significant Risk Factors Included

- Disease:
  - Disease and stage/status
  - AML: ELN 2022 risk (+TP53), transformed from MDS/MPN, # induction for CR1, time from dx to HCT (CR2, CR3, relapse)
  - ALL: Cyto/Molec risk, # induction for CR1, time from dx to HCT (CR2, CR3, relapse)
  - MDS: IPSS-R, therapy related MDS, TP53 mutated, MDS with predisposing conditions
  - NHL subtype
- Disease
  - Disease sensitivity (NHL,HL)
  - Viral infection within 60 days HCT (immune disorders)
- Transplant:
  - Donor type/graft type and HLA matching
  - Donor Age unrelated and related (excluding sibs/twins)
  - Donor/recipient sex match

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What Else Did We Test in the 2025 Report ?

- Serum ferritin pre-HCT
- Pediatric GFR
- Complex congenital heart disease (age 18 or less)
- Prior CT for ALL, Lym, MM
- Hx PJP infection (immune, histiocytic dz)
- Time from dx to tx AML in PIF
- MRD for AML and ALL
- MM cytogenetic and R-ISS
- Recipients with GVHD due to maternal engraft pre HCT (immune system)
- TRJV (sickle, beta thal)
- Liver Iron (sickle, beta thal)
- RBC dependence, Iron chelation (beta thal)

What Changed in the 2025 Report ?

- Additions:
  - Pediatric comorbidity for malignant and non-malignant disease
  - Serum albumin
  - Platelet count pre-HCT
  - Addition of TP53 subgroup AML
  - Addition of TP53 mutated MDS
- No change or removed:
  - MRD for AML and ALL
  - Previous cellular therapy for patients with lymphoma

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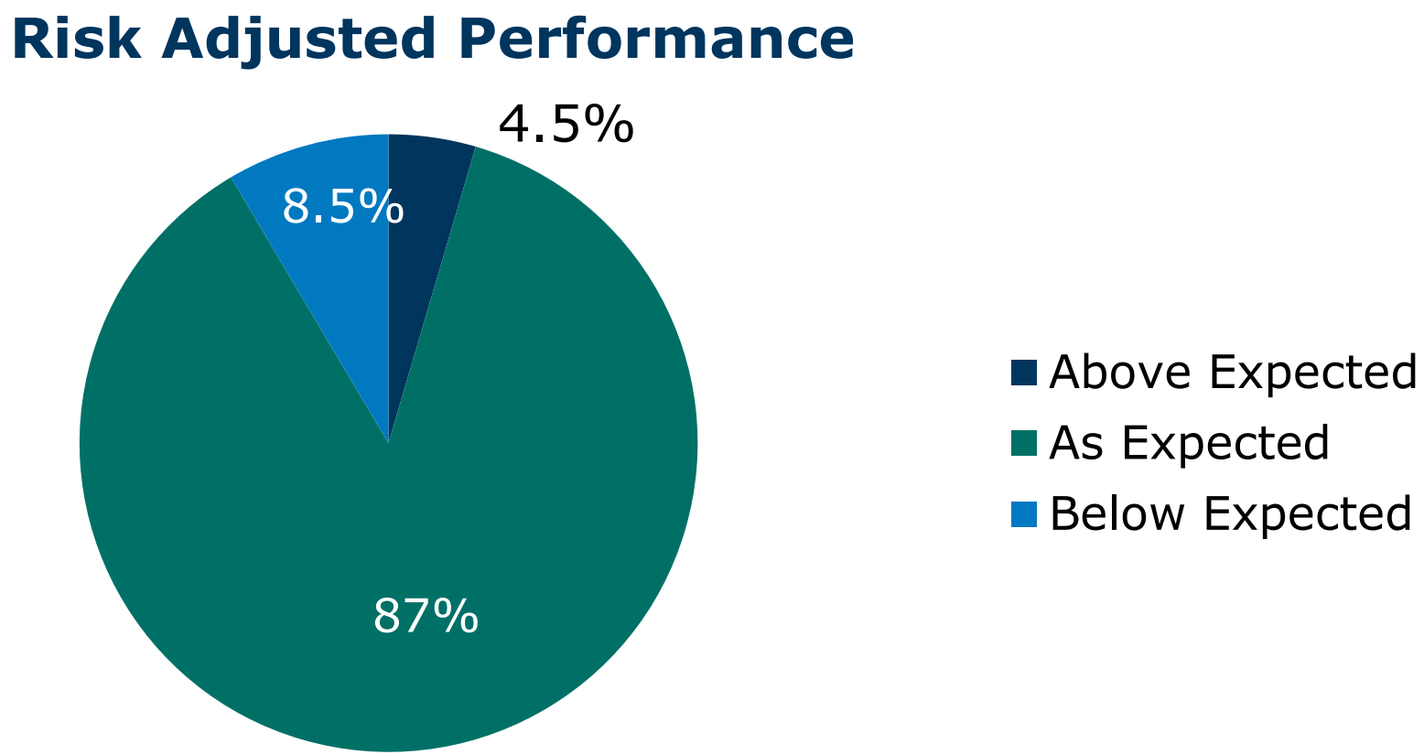
How are US Centers Doing? 2025



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N = 176

Summary Results for CSA 2016-2025

Report year	2016	2017	2018	2019	2020	2021	2022	2023	2024	2025
Years of HCT included	**2012-2014**	**2013-2015**	**2014-2016**	**2015-2017**	**2016-2018**	**2017-2019**	**2018-2020**	**2019-2021**	**2020-2022**	**2021-2023**
Total no. of centers	179	174[a]	173[b]	170	172	173	175[c]	178[d]	172[e]	176[f]
No. under-performing centers	23 (13)	21 (12)	25 (14)	27(16)	23(13)	17(10)[g]	13 (7)	12(7)	18(10)	15(9)
No. centers performing as predicted	139(78)	140(80)	135(78)	126(74)	132(77)	141(81)[g]	151 (86)	154(87)	142(83)	153(87)
No. over-performing centers	17 (9)	13 (7)	13 (8)	17(10)	17 (10)	15 (9)	11 (6)	12(7)	12(7)	8(5)

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How Do We Address Challenges?

Center Outcomes Forum

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What is the Center Outcomes Forum?

- Bi-annual meeting to discuss the center specific survival analysis for hematopoietic cell transplantation (HCT) – the highest impact report produced for the Stem Cell Therapeutics Outcomes Database (SCTOD)
- Began in 2008, 10 events
- Invitees include:
  - HCT centers/community, ASBMT Quality Outcomes Committee, biostatisticians, quality and reporting methodologists, patients, payers, US government representatives
- Various in-person and virtual venues
- High degree of engagement with attendees

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Agenda – COF 2025

- Purpose
- Agenda Overview
  - Center-Specific Survival Analysis 2025 Summary
  - Workgroup presentations and DISCUSSION
    - Enhance risk adjustment in acute leukemia
      - MRD Taskforce recommendations
      - ELN recommendations for AML (time permitting)
    - Optimizing Handling of comorbidities for adults and children
    - Refining Transplant-related factors included in risk adjustment
- Next steps/Wrap Up

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COF Summary and Recommendations

- Executive Summary and Detailed Summary including discussion and recommendations are posted and archived for all COF since 2008

- https://cibmtr.org/CIBMTR/Meetings/Materials-Archive/Center-Outcomes-Forum

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COF 2023 Workgroup 2 Recommendations:

- Updating data collection & risk adjustment - AML, ALL, MDS
  - Data collection should reflect modern classification and risk assignment of hematologic malignancies as published by WHO and other international cooperative groups. The work group strongly recommended updating to the WHO 2022 classification as well as updating the cytogenetic, FISH and molecular data that is used to assign the correct classification.
  - CIBMTR should continue to collect MRD data, recognizing that there are challenges to collecting these data. Since there is an FDA-approved test available (ClonoSEQ), the work group recommended collecting results of this MRD test for patients with ALL. As testing capabilities are continuing to evolve, the work group recommended that we continue evaluate how best to capture these data.

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COF 2023 Workgroup 2 Recommendations:

- Updating data collection & risk adjustment - AML, ALL, MDS
  - The work group also recommended that the data collection tools should be updated to reflect modern disease response criteria (i.e., incorporating MRD status for AML and ALL, recently updated response criteria for MDS).
  - As the recommendations above are implemented, the work group identified questions that have already or will soon become less relevant for the purposes of the CSA analysis and research in general. The work group recommended that these questions be eventually removed from the analysis or retired from the forms.

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Can We Risk Adjust for Measurable Residual Disease in Acute Leukemia?

Wael Saber