Center specific outcome reporting for HCT 2013
Quality, Transparency and Patient Safety in BMT
J. Douglas Rizzo, MD
July 29, 2013

Quality (& Safety)
**Highlights of SCTOD expectations**

- Collect data (and specimens)
  - *ALL* allogeneic HCTs with a U.S. recipient or donor
  - Related donor-recipient repository
  - Other cellular therapies
  - Quality of life data
  - Secure, efficient electronic data capture system
- Analyze data
  - Center-specific outcomes for U.S. centers: related and unrelated donor transplants
  - Perform analyses of optimal size for the adult donor registry and cord blood unit inventory
  - Conduct and support other research using the data collected under the contract
- Disseminate data
  - Within the Program
  - To the scientific and medical community
  - To patients, families and the public

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**What is the MAIN goal ?!**

- Provide an equitable, balanced, scientific performance measurement tool(s) that can be used by the profession to define and improve quality. While:
  - Acknowledging limitations
  - Avoiding misuse
  - Striving for continuous improvement
Center Outcomes Analysis: Basic Concepts

- Examination of individual center specific outcomes relative to the overall network
- Risk Adjustment for severity of illness at a given center
- Assessment of center performance needs to account for sampling variability/sample size
- Understandable to public audience

Center Outcomes Cycle and Timeline

Continuous Data Collection, CPI, Data confirmation by centers

- Data File preparation: January - April
- Analysis and Review: May - August
- Draft Report Submitted: September 1
- HRSA review and approval: November
- Publication Centers and Website: Dec - January
Methods

Statistical Methods

• Comparison of observed vs. predicted one year survival probabilities in each center

• **Observed survival probability**: Kaplan-Meier estimates of one year survival, by center

• **Predicted survival probability** (Risk adjustment):
  • Accounts for the types of patients being transplanted at the center
Risk Adjustment Model

- Fit a (pseudo-value) logistic regression model for one year survival to all patients in entire network to predict patient outcomes
- Compute pseudo-values for each recipient by individually removing each recipient from a pooled KM 1 year survival estimate
- Fit fixed effects censored data logistic regression model to the pseudo-values with no center effect
  - Each pt characteristic associated with OR of 1 yr survival
- Direct model for 1 year survival probability which is an alternative to Cox model for hazard rate

Prediction

- Define predicted survival for each recipient based on the odds ratios for their patient characteristics from the regression model
- Generate the predicted survival by center based on recipient characteristics by averaging the estimated survival for all recipients at the center
- Generate the observed one year survival using KM estimation
Statistical Methods

- Predicted survival outcome at a given center is based on the average predicted survival of patients actually 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

Statistical Methods

- 95% confidence interval constructed
  - Range of plausible values for survival probability, if those patients had been transplanted at a generic center in the network
  - Constructed by resampling pseudo-values (Logan et al, Lifetime Data Analysis, 2008)
- If observed survival is outside confidence interval, the center appears to be under- or over-performing relative to the overall network
Statistical Properties

- An “average” center has a <=5% chance that they will be incorrectly identified as overperforming or underperforming (Type I error)
- Type I error rate is not dependent on
  - Case mix, as long as characteristics included in regression model
  - Sample size (because wider intervals for small centers)

Significant Risk Factors

- Disease and stage
- Disease sensitivity (NHL and HL only)
- Co-existing disease
- Race of recipient
- Recipient Age
- Recipient CMV status
- Year of HCT
- Conditioning regimen intensity
- Karnofsky/Lansky perf. score
- Time from dx to tx (ALL and AML not in CR1/PIF only)
- Donor type/graft type and HLA
- Donor Age
- Donor/recipient sex match
- Prior autoHCT
Reporting Results

Centers

Results of risk adjustment model:
- Odds ratios (95% CI’s) for one year survival (>1 means better survival)
- For each center, we include a table with
  - Number of tx
  - Case mix score
  - Observed survival
  - Predicted survival
  - 95% prediction interval
  - An indicator of whether the center is underperforming, performing comparably to, or overperforming the entire network
- Graphical representations can also be helpful
Reporting Results

Table 4. Center-Specific Results

<table>
<thead>
<tr>
<th>Center</th>
<th>n Range</th>
<th>Case Mix Score</th>
<th>Survival (%)</th>
<th>Predicted (%)</th>
<th>Actual (%)</th>
<th>Lower (%)</th>
<th>Upper (%)</th>
<th>Performance by Report Year</th>
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Reporting Results

Predicted and Actual Survival Rates for Transplant Centers with 11–50 Transplants
Performance Improvement

- Aside from center specific survival report, CIBMTR provides additional data to center directors
  - Unadjusted survival at 100 days, 6 mos, 1 year by transplant type, conditioning intensity and year for center and US as a whole
  - Demographic tables by year comparing center to US as a whole

RESEARCH QUESTION: Can we identify center characteristics that affect performance?

- One goal of center survival reporting is to promote performance improvement at centers
- What do we know about:
  - Volume
  - Modifiable factors that can be adopted
- What can we learn from high-performing centers that can be used by other centers to improve
Reporting Results

Public

Reporting Results - Public

- Results are posted online and accessible through
  - HRSA website
  - Be the Match
  - CIBMTR

- Format may change in next year or two
What is on the Website?

- [http://bethematch.org/access](http://bethematch.org/access)
- Demographics of program
- Estimated search and HCT costs
- Transplant experience
- Center specific analysis
- Actual (not KM) survival by disease and age strata

Reporting Results

Center-Specific Analysis

This analysis is based on transplants performed from Jan. 1, 2007 through Dec. 31, 2009 using unrelated donors and transplants performed from Jan. 1, 2008 through Dec. 31, 2009 using related donors. It only includes patients who underwent their first alloparentic transplant within these respective time periods and who had at least 100-day follow-up.

1. This center reported survival status data for 189 patients.
2. The actual one-year survival of these patients was 70%.
3. The predicted one-year survival was 69% (with a 95% confidence limit that the predicted survival was between 60% and 77%).
4. This center’s actual results are similar to the predicted range for this center.

For help with understanding these statistics, please see [How to Understand Transplant Center Statistics](http://bethematch.org/access).
Center Outcomes Report
Final study population - 2012

• Centers must have >90% overall f/u at 1 year
  • No centers excluded in 2012
  • Excluded: 4 in 2011, 11 in 2010
• 169 centers; 18,947 patients first HCT
• Primary outcome: One year survival
  • Overall: 64.6% (69% REL, 61% UNR)
• Censoring:
  • 1448 (7.6%) had less than one year of follow-up
• Detailed demographics are given in the report

Center Outcomes Report
2012

• 3 year rolling time window
• Center outcomes report 2012 include:
  • Unrelated HCT 2008 – 2010
  • Related HCT 2008 - 2010
• Full data on HCT Comorbidity Index (Sorror, et al)
• Outcome: 1 year survival
• 11 centers performance above expected, 26 centers below
What do we debate about?

- Time window / reporting interval for analysis
- Best/most appropriate outcome
  - Why
- Adjustment for risk
  - Can we have a standard group to be evaluated and leave the “special” patients out
- Data collection burden vs variables to adjust
What do we debate about?

- Is it fair for pediatric centers to allow combined adult and pediatric centers?
- Handling small centers/pediatric centers
- Unintended consequences
  - Not intended to compare centers
- Translating results into improvement
- Conveying data to the non-statistician
- Medical community acceptance

Exploratory Analyses
What’s new from 2010 to 2012

- Completeness of follow-up criteria now 90% or higher
  - No center excluded by this criteria 2012
- Combined data for Related and Unrelated HCT in statistical model
  - Complete data (all 3 years) for related and unrelated HCT
- Three year window for analysis

What’s new from 2010 to 2012

- Test new variables for inclusion
- Modifications of risk adjustment model
  - Full set of HCT-CI data now available vs. Yes/No previously
  - Finer resolution of upper age categories
  - Breakdown of nonmalignant disease types
- More information in reports to center directors
What have we tested in last 2 years?

- Factors associated with related and unrelated HCT essentially same
  - Single combined model pools sample size
  - Combined model nearly same predicted accuracy as separate models

What have we tested in the last 2 years?

- Median household income from zipcode
- Distance from HCT center
- Cytogenetics risk category AML
Modifications of risk adjustment model - 2012

- HCT-CI
- Age categories at upper end
- Nonmalignant disease categories

Transparency
In Flight
Center Outcomes Beyond 2013

- Review of 2013 report underway
- Center Outcome Forum September 2014
- Re-design of center specific data display on public website by OPA
- Online calculator to provide survival estimate based upon known risk factors (in process)
- Scientific agenda to define “modifiable” factors

CENTER OUTCOMES FORUM 2012

- Engage the relevant stakeholders
  - HCT community, patients, payers, government collaborators
- Provide recommendations to CIBMTR regarding data to be collected, analyses, and presentation of results
- Generate ideas for research re: processes and resources that affect performance, particularly those that may be modifiable.
Center Outcomes Forum 2012
Topics

- HCT-CI
  - Validation studies confirm value in multivariate adjustment
  - Suggestions to improve reliability and data collection at centers
  - Endorsed value of collecting HCT-CI
- TED revision – Changes to data collection to improve risk adjustment models?
  - Patient
  - Disease
  - HCT factors

Center Outcomes Forum 2012
Topics

- Current and future research
  - Modifiable center factors associated with outcome
  - How well can we predict future performance?
- What reports or data can CIBMTR provide centers to assist with performance improvement?
- How to display the results on public websites to increase understanding and avoid misuse?
Limitations

- Outcome is 1 year survival
- Combined Pediatric and Adult centers
- Autologous HCT are NOT included
  - Full representation essential
- Conveying data to the non-statistician
  - Misunderstandings & misrepresentation
- Unintended consequences
  - Not intended to compare centers
  - Translating results into improvement

Limitations

- Can only adjust for those factors collected on all patients
- What about “Value”?
  - No cost data – increasingly of interest to payers, patients, policy makers
  - Costs among most rapidly growing
    About $500,000 billed first 180 days after alloHCT (Friedman, Optum)
  - Cost variation ??? related to risk
- Report issued only once annually
Information on cibmtr.org

- Summary of all Center Outcomes Forum meetings (3)
  - Found under “meetings” tab
- Summary of Center outcomes analysis methodology
  - Found under “slides and reports”

Transparency
(Upcoming Change)
**Why haven’t we publish Center outcomes results as a list?**

- Risk of promoting the unintended consequence of this report of directly comparing centers to each other
- One of the most frequent questions brought to our information request resources
  - “Trying to decide between center A and center B for my condition....”
  - Center B says “CIBMTR rates them as the best ....”

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**Considerations re: unblinded center outcomes reports**

**Benefits**
- Centers won’t be asked by multiple payers to forward data
- Prevent mistakes in “transcription”
- Transparency – consistent with SRTR and likely HRSA expectations

**Risks**
- Pressure to compete with other centers
- Unintended consequence of avoiding “risky” HCT
Why don’t we offer benchmarks?

- Comparisons of centers to each other very problematic
  - Heterogeneity of HCT recipients at centers
  - Incomplete measurement of risk factors
  - If a benchmark were created with a “standard” group of patients, the smaller numbers will lead to very large confidence intervals

What data (and datasets) should we make publicly available?
Data found on .gov website

- Query tools to display:
  - Volumes of HCT for disease by center
    - Geographic basis
  - Volumes of HCT by disease
    - Additional selection by disease status, age, gender, race, cell source and year of transplant
  - Survival at 100d, 1 year, 3 years after HCT
    - By disease, donor type, age, gender, race, cell source

Proposed changes to .gov

- Addition of Annual statistical report containing “static” demographic tables similar to those available by query
- Provide downloadable, de-identified dataset of all data contained in “center volumes” report/query tools
- Add survival to the center volumes dataset to be downloaded
  - Use this dataset to drive all queries on .gov website
Publicly Available Task Force

- Review information currently available on CIBMTR and .gov websites
- Make recommendations for future state of information and datasets to make publicly available
- Consider benefits and uses, risks, and CIBMTR effort to maintain

Inclusive TF representation

- HCT centers
- Researchers (CIBMTR WC)
- Public (CIBMTR CAC and OPA)
- Cord blood (CBDWG)
- ASBMT Quality outcomes committee
- Payers
- CW Bill Young program
  - CBCC
  - BMCC
  - OPA/SPA
- NIH/HRSA
- Legal
Questions??

Contact information:
Drizzo@mcw.edu
414-805-0700