Patient-reported outcomes (PRO) in CAR-T cell therapy

Kathryn Flynn, PhD
Associate Professor of Medicine
Senior Scientific Director for Patient-Reported Outcomes at CIBMTR

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A research collaboration between the National Marrow Donor Program (NMDP)/Be The Match and the Medical College of Wisconsin

Disclosure: I am representing the Center for International Blood and Marrow Transplant Research (CIBMTR).

CIBMTR paid for my travel to attend the meeting.

PROs in hematopoietic cell transplant (HCT)

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Value & Relevance in HCT

- The most accurate measure of the patient's experience with disease and treatment
- Used as primary and secondary outcomes in clinical trials
- Several studies in HCT show that pre-HCT PRO can predict survival and post-HCT quality of life
- When survival and event-free survival differences are small between treatment groups, PRO can indicate best therapeutic options

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BMT CTN PRO collection

- Several BMT CTN studies have collected PROs
  - Primary (n=1) or secondary outcome (n=8) (1 pediatric study excluded) in 18 trials performed since 2004
  - Ten different multi-item measures were used in these studies
    - Most commonly used measures were the SF-36 (n=6) and FACT-BMT (n=7)

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Shaw et al, BMT, 2016

BMT CTN Recommendations for HCT

- Use the same core measures in all research studies of HCT patients
- Use a system that is free and easy to access
- Ensure a low burden for the patient
- Use a single, versatile measurement system for core concepts
  - Supplement with measures addressing specific areas/domains (e.g. GVHD) as necessary
- Core system recommended: PROMIS

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Shaw et al, BMT, 2016

PROMIS® System

- Measures physical, mental, and social health in adults and children
- No licensing fees to use or score
- Rigorous development process
- Validated in multiple languages
- Multiple formats (paper, electronic)
- Reduced participant burden especially with computerized adaptive tests
- Crosswalk to legacy measures (PROsetta stone)

The most appropriate PROs to collect in cell therapy are unknown

Once relevant constructs are identified, can choose appropriate measures and timepoints

Centers need a structure/process to systematically collect PROs

Components of CIBMTR ePRO system

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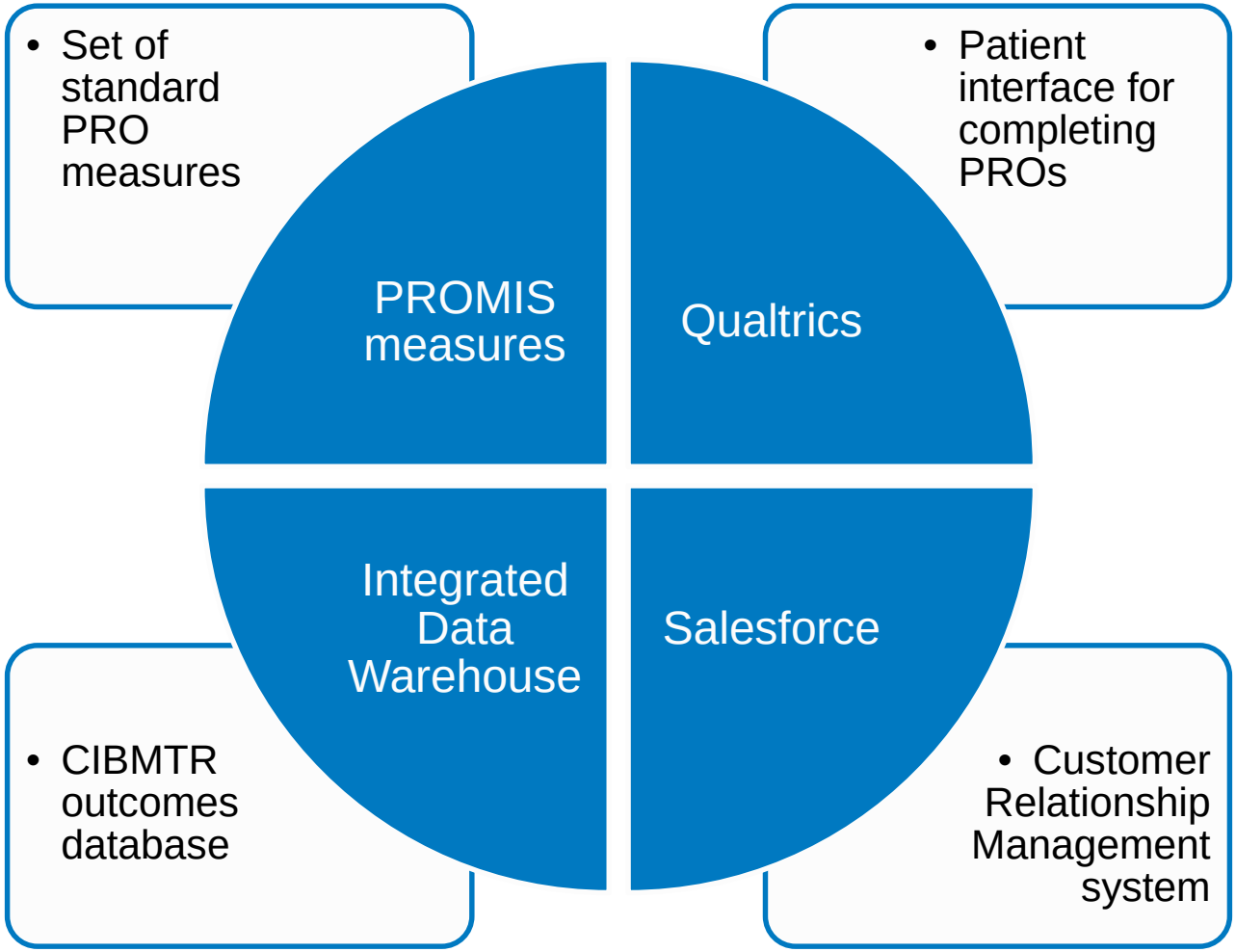
CIBMTR ePRO collection system



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CIBMTR ePRO collection system

- Developed with funding from Navy grant and NMDP
- Development team included CIBMTR and NMDP business and IT members
- Securely collects and stores PRO data from patients; single time point and long term follow-up studies
- Allows direct patient contact so the CIBMTR Survey Research Group (SRG) can follow-up with non-responders
- Stores PRO scores alongside clinical outcomes data for center and researcher access

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Pilot ePRO study – summer 2018

- Pilot trial of new ePRO system
  - Examine QOL in patients on the CMS MDS study (cross-sectional) and explore feasibility of use of ePRO
- Study procedures
  - CIBMTR sends list of eligible patients to site
  - Site returns patient eligibility and contact info form to SRG
  - SRG conducts pre-consent call with patients
  - SRG emails patient with unique link to eICF, with follow-up calls if needed
  - Patient completes and signs eICF
  - SRG emails patient PDF of signed eICF along with unique link to ePRO survey, with follow-up calls if needed

Late Effects Task Force for HCT

- CIBMTR recently organized a multidisciplinary working group
- Goal: To develop a strategy for collection of late effects in patients reported to CIBMTR
  - Which populations should have routine PRO collection?
  - What domains, measures, time points?
- Timeline: ~9 months
  - Will present recommendations at TCT 2019

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