

WORK GROUP REPORT: ADVANCING HEMATOPOIETIC STEM CELL TRANSPLANTATION FOR HEMOGLOBINOPATHIES

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ACBSCT meeting

September 11, 2015

Working Group Members

- Naynesh Kamani, MD, Chair
- Andrew Campbell, MD
- Colleen Delaney, MD, MSc
- Nancy DiFronzo, PhD
- Mary Eapen, MD
- Lewis Hsu, MD
- Bert Lubin, MD
- Robertson Parkman, MD
- Mark Walters, MD
- Jeffrey McCullough, MD, ACBSCT Chair
- Edgar Milford, ACBSCT Chair, Emeritus
- HRSA Staff

Charge to Working Group

- Identify barriers to transplantation and opportunities to more fully realize its potential for individuals with sickle cell disease and thalassemia.
- Submit for consideration and adoption by ACBSCT recommendations regarding high priority actions

Barriers to transplantation in SCD

- Disease related: Heterogeneous nature of disease with lack of clinical/laboratory/genomic predictors of poor prognosis
- Patient/family barriers
 - Fear of transplant related mortality and morbidity
 - Fear of risk of long-term complications (GVHD/infertility)
 - Comfort with transfusion programs for those with complications
 - Gaps in knowledge about natural history/progressive organ damage
 - Mistrust of medical professionals
- Health care provider barriers
 - Provider reluctance to recommend HSCT
 - Gaps in knowledge about role of HSCT
- Donor availability
 - Lack of matched sibling donors
 - Lack of well matched unrelated donors for the majority of patients
- Lack of Insurance coverage – assessment of gaps in coverage

Working Group Meetings

Meetings: Workgroup conference calls

- 12/18/2015:
 - Presentation by Dr. M. Hsieh on NIH experience with MSD/haplo transplants for SCD
 - Update on BMT data submission to CIBMTR
- 6/16/2015:
 - Update on CMS coverage for hemoglobinopathies, esp. SCD
 - Update: CIBMTR data on BMT for SCD
 - Review of educational opportunities at provider conferences

Working Group Meetings

Meetings: Workgroup conference calls

- 7/20/2015:
 - Presentation by Dr. R. Brodsky on JHU experience with haplo transplants for SCD
 - Update on CMS coverage for SCD and review of previous recommendations
 - Review of educational opportunities at provider conferences

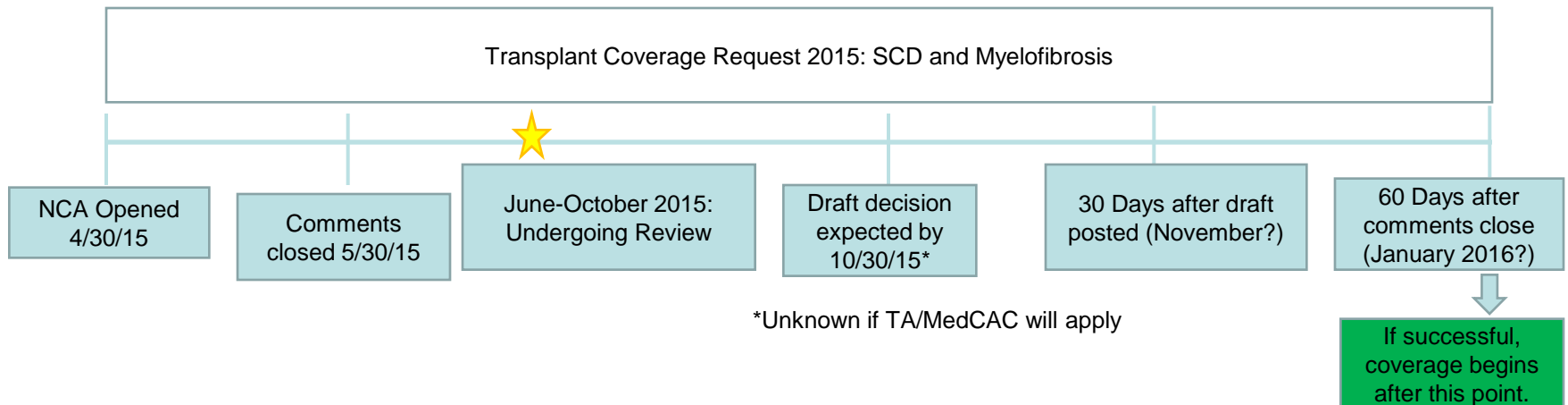
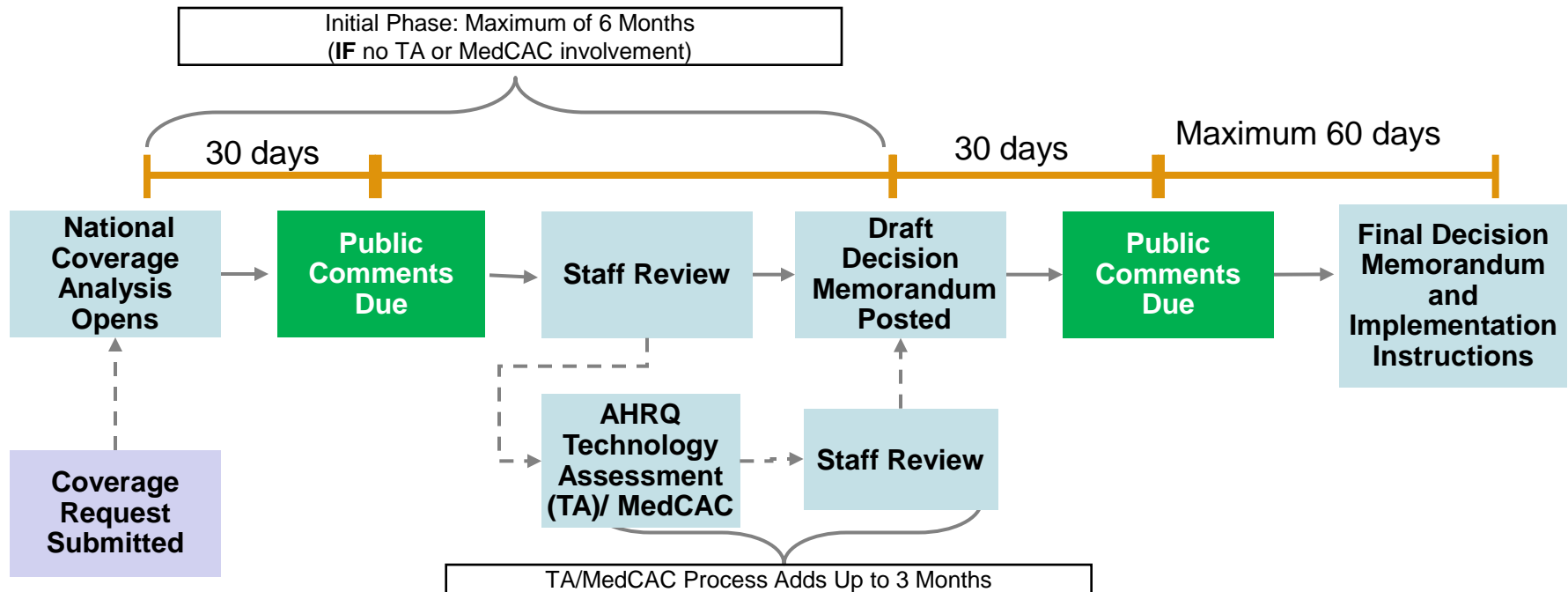
Coverage for HCT for Sickle cell disease

- The overwhelming majority of children with SCD and a significant majority of adults with SCD are covered by state Medicaid insurance
- A number of commercial third party insurers include BMT for SCD in their list of covered indications for their fully funded plans. However, approximately 60% (and growing) of Commercial insurance plans are self-insured (employers determine coverage), which means that there can be huge variations in coverage.
- There is increasing participation in the Health Insurance Exchanges; this may be good news for SCD coverage but whether this is true or not remains to be seen.

Coverage for HCT for Sickle cell disease

- State Medicaid programs vary in their coverage of BMT for SCD. A few (e.g. MS, OH, IN) cover SCD. Many do not specify whether SCD is covered or not. Some (e.g. HI) do not provide coverage.
- Medicare is 'silent' on coverage for SCD = 'non-coverage' since decisions are left to contractors who deny coverage because of high costs.
- NMDP/ASBMT has submitted an application for reconsideration of the current National Coverage Determination (NCD) to CMS to expand the scope of coverage for HSCT.

National Coverage Determination Process



Workgroup discussion topics for 2015

Access to HSCT

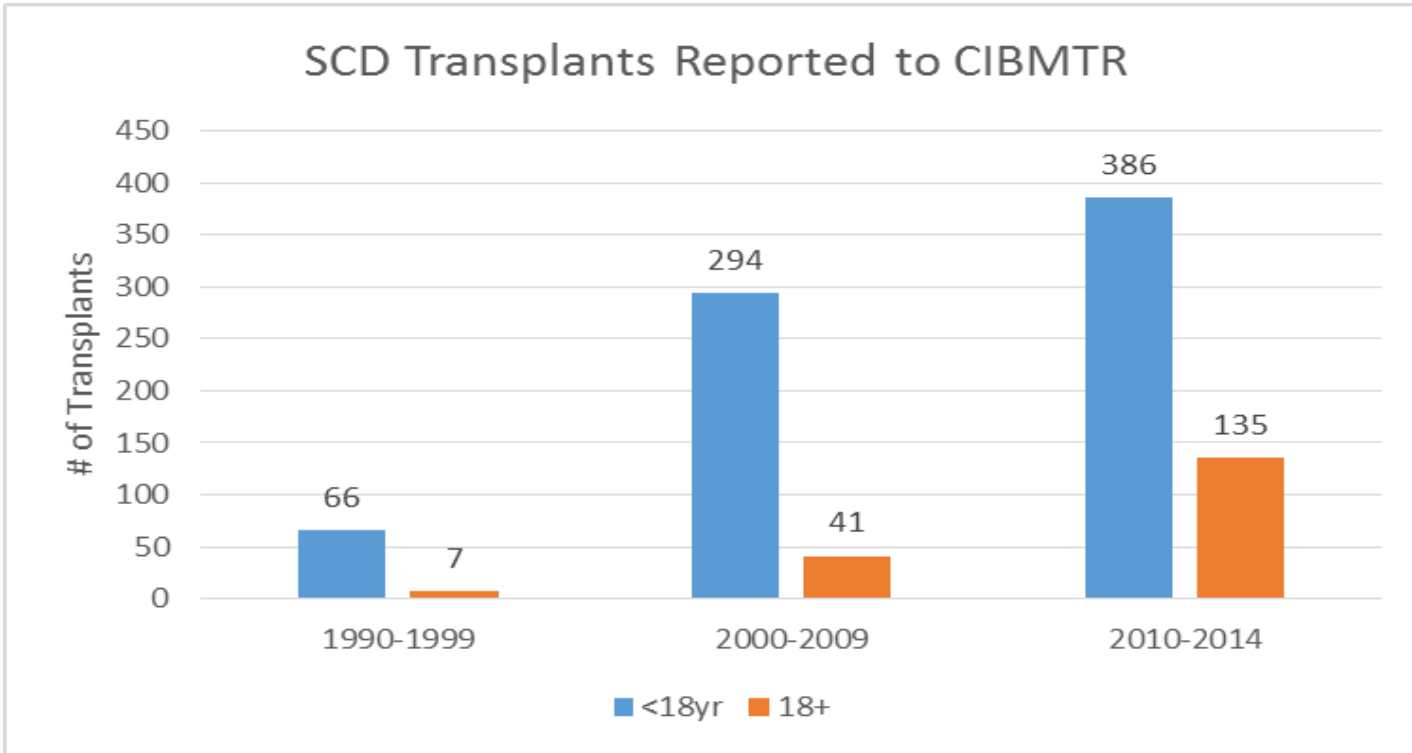
- Increasing Healthcare provider and patient/parent awareness of BMT for sickle cell disease (SCD) will lead to increased referrals and utilization of BMT as therapy for eligible patients with SCD.
- Workgroup feels that additional efforts are necessary to increase awareness
 - Education sessions at health care provider conferences
 - American Society of Hematology - 2016
 - American Society of Pediatric Hematology Oncology - 2016
 - Foundation for Sickle Cell Disease Research - 2016
 - Publication of findings from clinical trials and analyses of CIBMTR data
 - Education/information sessions at parent/patient advocacy organization conferences
 - Patient and Health Professional Services at NMDP Be-The-Match

NMDP educational outreach efforts

- The NMDP Patient and Health Professional Services has continued its educational/ outreach efforts to the SCD patient and provider community
- Objectives:
 - To increase awareness of HCT as a treatment option for SCD
 - Identify strategies for informed, shared decision-making
- Target audience:
 - Both patients and health care providers
- Poster/plenary presentations
 - SCDAAs annual meeting Podium presentation: Oct 2014
 - NMDP Council Meeting November 2014
 - FSCD Research Symposium April 2015
 - SCDAAs annual meeting Podium presentation: Sept 2015
 - SCD Community gathering, Seattle, WA – Sept 2015

Current clinical trials for BMT in SCD

- SCURT (BMT CTN 0601): 1st matched unrelated donor BMT multi-institutional trial – 2008-14 (Abstract ASH 2015)
- STRIDE (R34 grant funded): Reduced toxicity BMT trial for young adults with SCD 2011-2015 (Abstract ASH 2015)
- U01 (NHLBI) for a BMT-CTN trial (1503) to enroll 60 young adults ages 15-40 y with SCD to undergo matched/MUD BMT with a comparison cohort of 120 adults undergoing standard of care treatment.
- Haploidentical donor trial: adults with SCD – NIH (JAMA. 2014; 312:48; update on matched sib donor BMT)
- Haploidentical donor trial for patients with SCD –JHU (Blood. 2012; 120:4285)
- Haploidentical BMT for children and adults with severe SCD approved by BMT-CTN steering committee in June 2015



The data presented here are preliminary and were obtained from the Statistical Center of the Center for International Blood and Marrow Transplant Research (CIBMTR). The analysis has not been reviewed or approved by the Advisory or Scientific Committees of the CIBMTR.

ACBSCT recommendations (9/2014)

- **Recommendation 25**

ACBSCT recommends that the Secretary recognize hematopoietic transplantation for sickle cell disease as a covered benefit for all Federal programs for which the Secretary has appropriate responsibility and oversight.

- **Recommendation 26**

ACBSCT recommends that the Secretary direct HRSA and other HHS agencies to collaborate with the CIBMTR to review research level data collection on allotransplants performed for sickle cell disease (SCD) and consider appropriate reimbursement to optimize research data collection. This review should include SCD-specific data elements collected, the completeness of data collection and mechanisms for reimbursement.

Discussion Topics for consideration by Advisory Council

- Is there a need for a recommendation to CMS specifically to expand the scope of the current NCD for HSCT for sickle cell disease?
- Additional efforts/charges to WG to pursue discussions to improve access, mitigate barriers and increase utilization of HSCT for SCD?
- What are the important scientific questions that need to be answered to advance HSCT?