

WORK GROUP REPORT: ADVANCING HEMATOPOIETIC STEM CELL TRANSPLANTATION FOR HEMOGLOBINOPATHIES

Naynesh Kamani, MD

ACBSCT meeting

September 15, 2014

Working Group Members

- Naynesh Kamani, MD, Chair
- Andrew Campbell, MD
- Nancy DiFronzo, PhD
- Mary Eapen, MD
- Lewis Hsu, MD
- Bert Lubin, MD
- Robertson Parkman, MD
- Mark Walters, MD
- Edgar Milford, ACBSCT Chair
- 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

Working Group Meetings

- Meetings: Workgroup conference calls:
- 7/18/2014:
 - Healthcare provider and patient/parent awareness of BMT for sickle cell disease (SCD)
 - Presentation on insurance coverage for hemoglobinopathies, esp. SCD by Stephanie Farnia (Director, Payer Policy, NMDP)
- 8/8/2014:
 - Discussion on new recommendation for Insurance coverage for SCD
 - Discussion on collection of research level data on all SCD transplants reported to CIBMTR

Workgroup discussion topics for 2014

Access to HSCT

- Study current status of third party coverage for HCT for SCD in children and adults and evaluate insurance barriers

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 SCD

- 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/CIBMTR is planning to submit an application for Coverage with evidence development(CED) to CMS to consider BMT coverage for SCD. CED means that coverage will be provided for patients on a study where additional patient data collection supplements standard claims data.

New recommendation

Recommendation 10 (passed Nov 2010):

“ACBSCT recommends that the Secretary recognize hematopoietic transplantation for generally accepted indications as a covered benefit for all Federal programs for which the Secretary has appropriate responsibility and oversight.”

The workgroup agreed that it was important to list sickle cell disease specifically in any such recommendation in order to promote access to HCT.

Proposed new recommendation:

“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.

Workgroup discussion topics for 2014

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.
- NIH/NHLBI has just published the Evidence-Based Management of Sickle Cell Disease – Expert Panel Report 2014. These guidelines *make limited mention* of HCT for SCD as a therapeutic option for patients with SCD.
- Workgroup feels that additional efforts are necessary to increase awareness
 - ‘Perspectives’ article outlining role of BMT in SCD
 - Education sessions at health care provider meetings and parent/patient advocacy organization conferences
 - Publication of findings from clinical trials and CIBMTR data
 - Other initiatives

NMDP educational outreach efforts

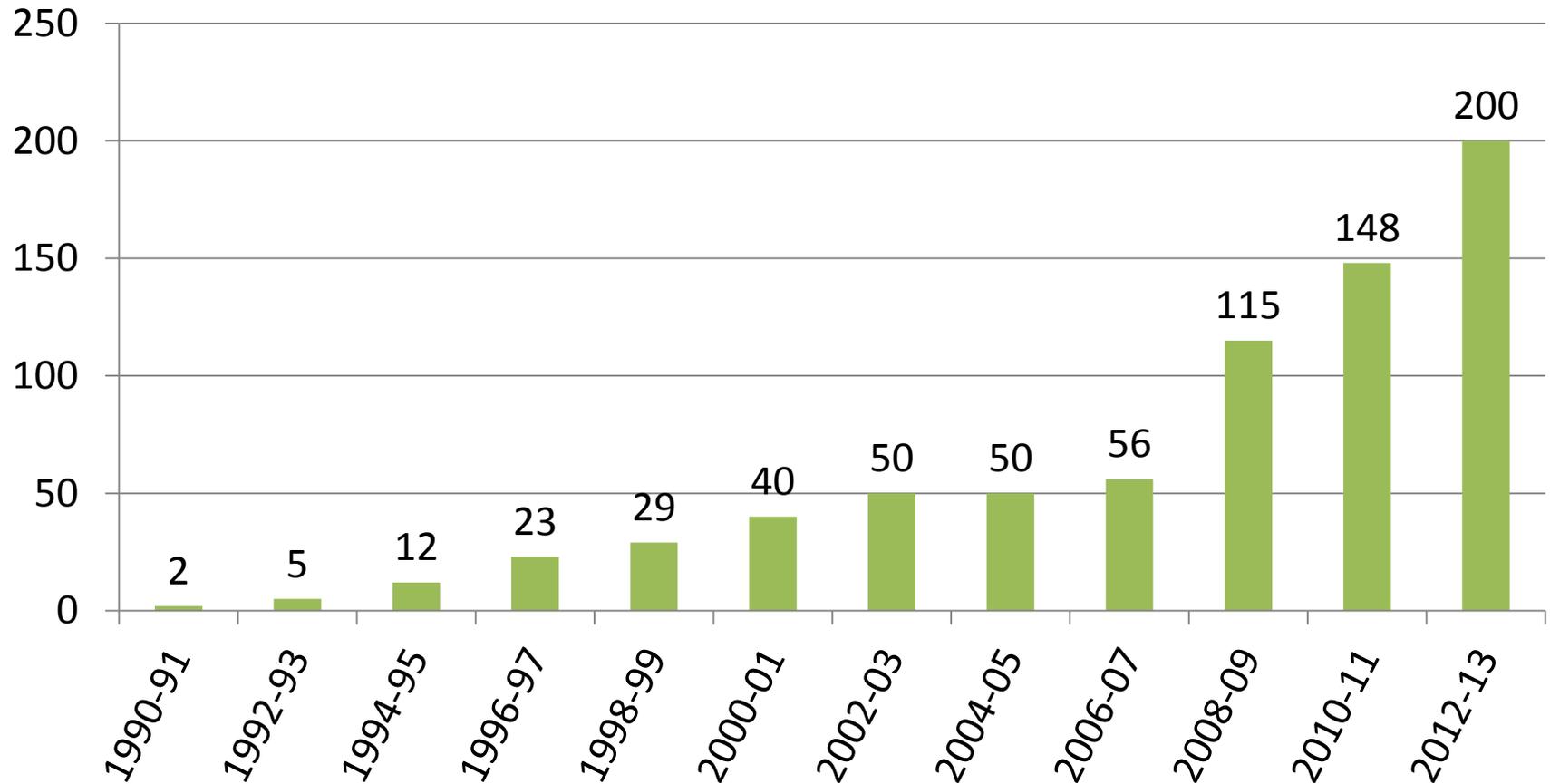
- The NMDP has continued its educational/ outreach efforts to the SCD patient and provider community
- Objectives:
 - To increase awareness of HCT as a treatment option for SCD
 - Identify strategies for informed, shared decision-making
- Target audience:
 - Both patients and health care providers
- Poster/plenary presentations
 - SCD Research and Educational Symposium March 2013
 - SCDAAs annual meeting: Oct 2013
 - BMT Tandem meetings February 2014
 - SCD Research and Educational Symposium April 2014
 - APOSW annual meeting April 2014

Current clinical trials for BMT in SCD

- SCURT (BMT CTN 0601): Pediatric phase II trial for MUD BMT: reached accrual goal March 2014;
- STRIDE (R34 grant funded): Reduced toxicity BMT trial for young adults with SCD;
 - R01 submission planned in concert with BMT CTN (concept approved by BMT CTN Steering Committee); 60 young adults ages 16-40 y with SCD to be recruited for 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)
- Limited institution and single institution trials
-

HCT for SCD reported to CIBMTR

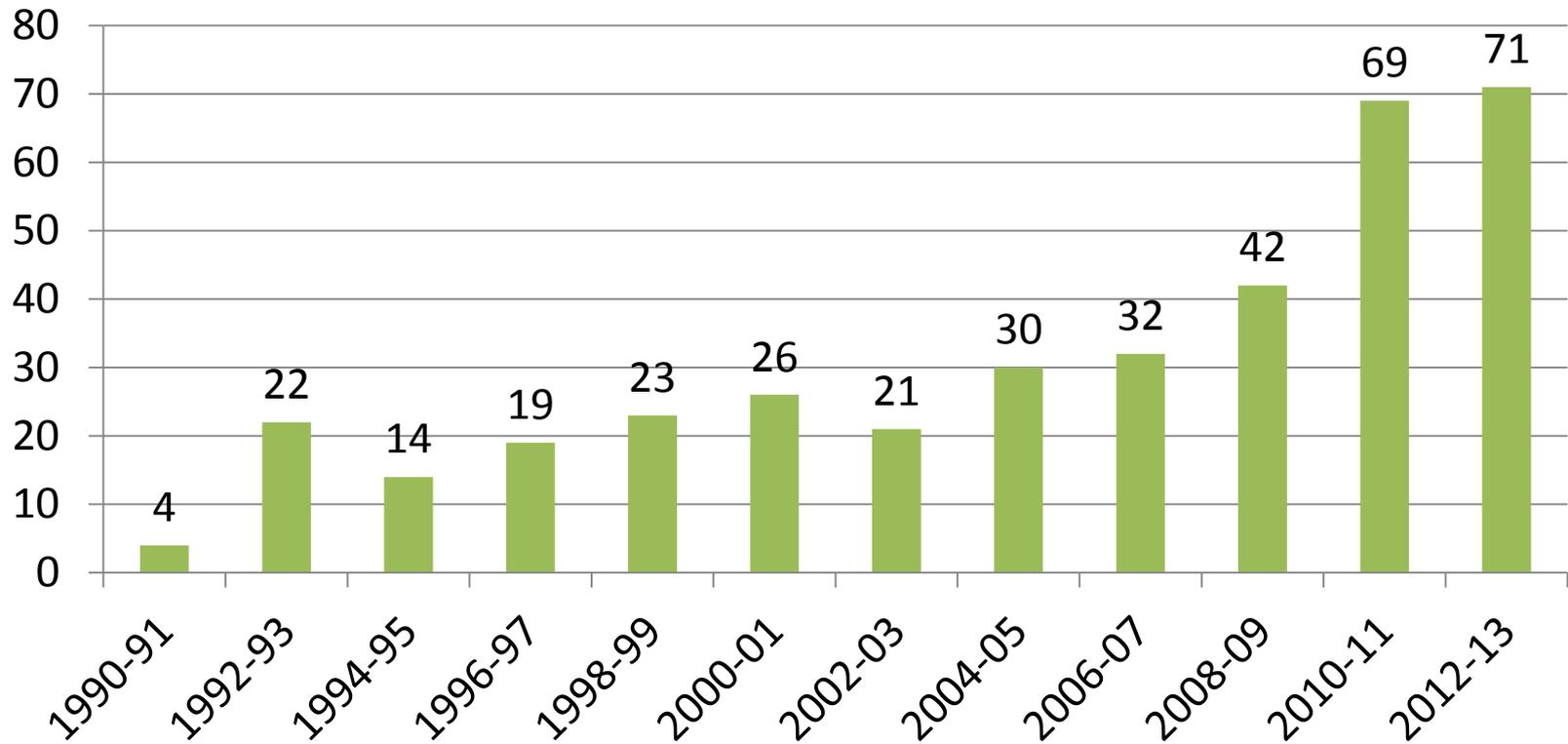
Number of patients who had first allo HCT for SCD, U.S



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.

HCT for Thalassemia reported to CIBMTR

Number of patients who had first allo HCT for Thalassemia, U.S



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.

CIBMTR data on BMT for SCD

- Registration Data on all transplants performed for SCD is reported to the CIBMTR
- Analysis of SCD outcomes requires disease-specific research level data
- Research level data requires submission of detailed data by transplant centers and additional financial resources
- Workgroup recommends that outcomes data analysis is important to identify patient and disease-related factors that impact outcomes and identify future research questions.

Recommendation to HRSA

- Provide financial support to the CIBMTR to enable the prospective collection of sickle cell disease-specific research level data for patients undergoing hematopoietic cell transplantation for SCD at transplant centers that elect to be CIBMTR research center contingent upon OMB approval.