

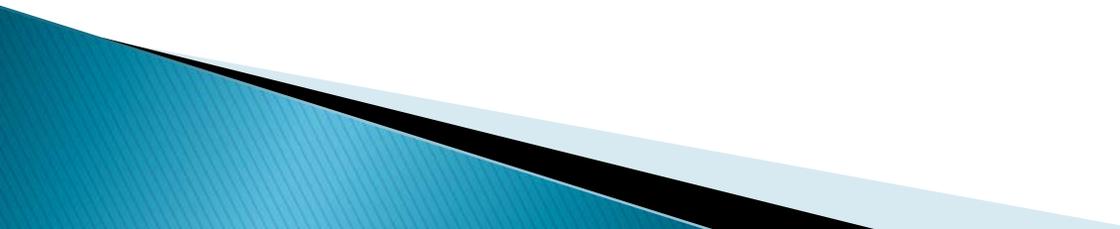
# Work group report: Advancing Hematopoietic Stem Cell Transplantation for Hemoglobinopathies

Naynesh Kamani, MD  
ACBSCT meeting  
May 29, 2014

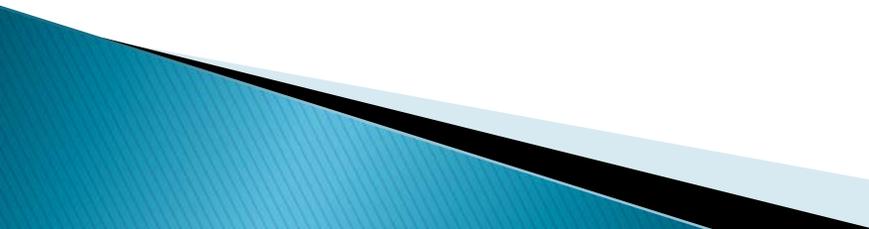
# Charge to Working Group

- ▶ 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
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# Working Group Members

- ▶ Naynesh Kamani, MD, Chair
  - ▶ Mark Walters, MD
  - ▶ Bert Lubin, MD
  - ▶ Andrew Campbell, MD
  - ▶ Lewis Hsu, MD
  - ▶ Mary Eapen, MD
  - ▶ Nancy DiFronzo, PhD
  - ▶ Edgar Milford, ACBSCT Chair
  - ▶ HRSA Staff
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# Working Group Meetings

- ▶ Meetings: Workgroup conference call held on 3/7/2014
  - ▶ NHLBI guidelines: Have not been published to date and will not cover the role of HCT in SCD in any detail
  - ▶ Discussion item: Consider writing a “Perspectives” article in BLOOD in order to inform health care providers about evidence-based recommendations for use of Hematopoietic cell transplantation in the management of sickle cell disease.
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# NMDP educational outreach efforts

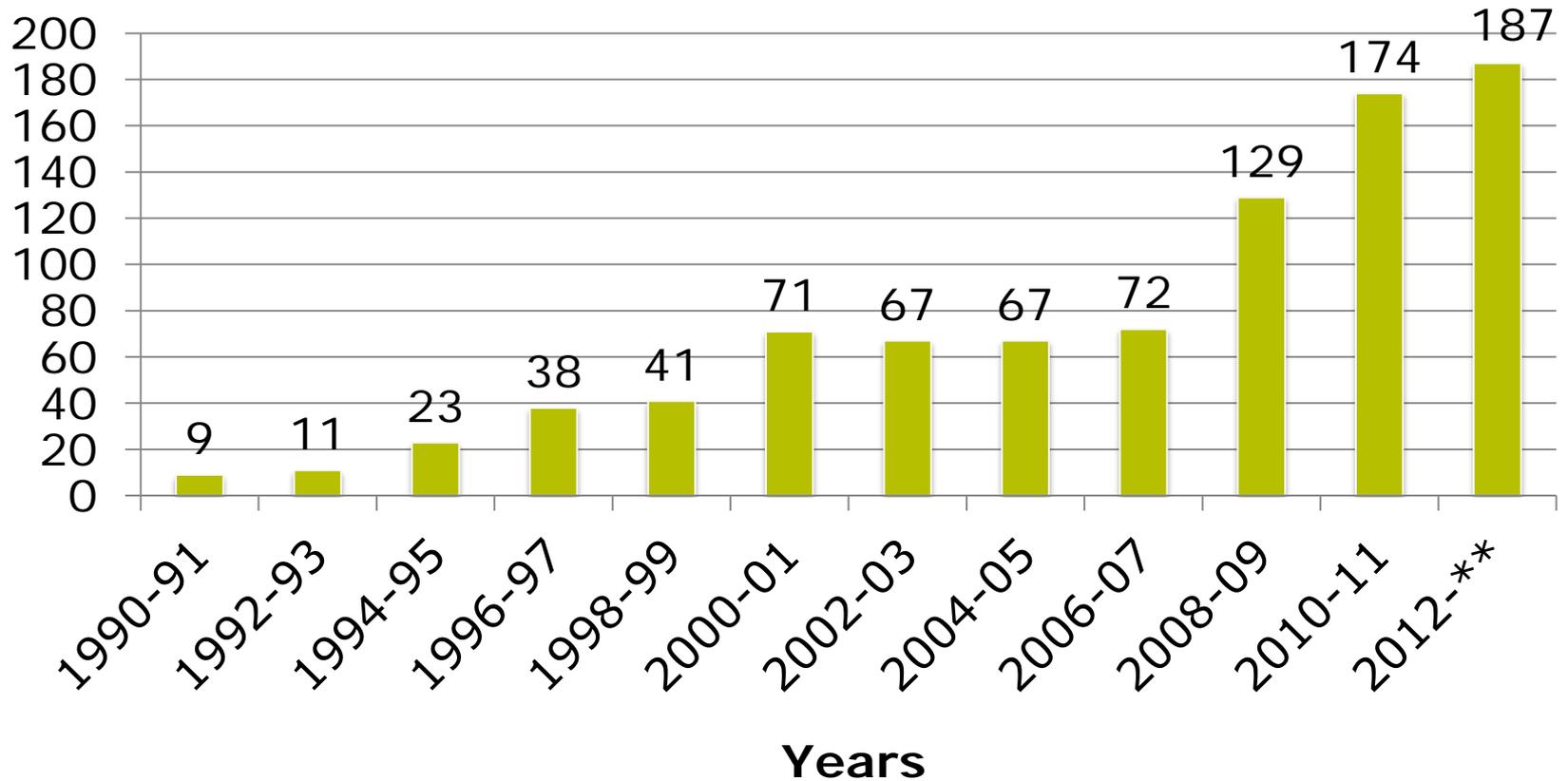
- ▶ The NMDP has continued its educational/ outreach efforts to the SCD patient and provider community
  
- ▶ Objectives:
  - To increase awareness of HCT as a treatment option for SCD
  - Identify strategies for informed, shared decision-making
- ▶ Target audience:
  - Both patients and health care 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
- ▶ Haploidentical donor trial: adults with SCD – NIH
- ▶ Haploidentical donor trial for patients with SCD – JHU
- ▶ Limited institutional and single institution trials

# BMT for SCD reported to CIBMTR

## Number of BMT for SCD 1990-2012



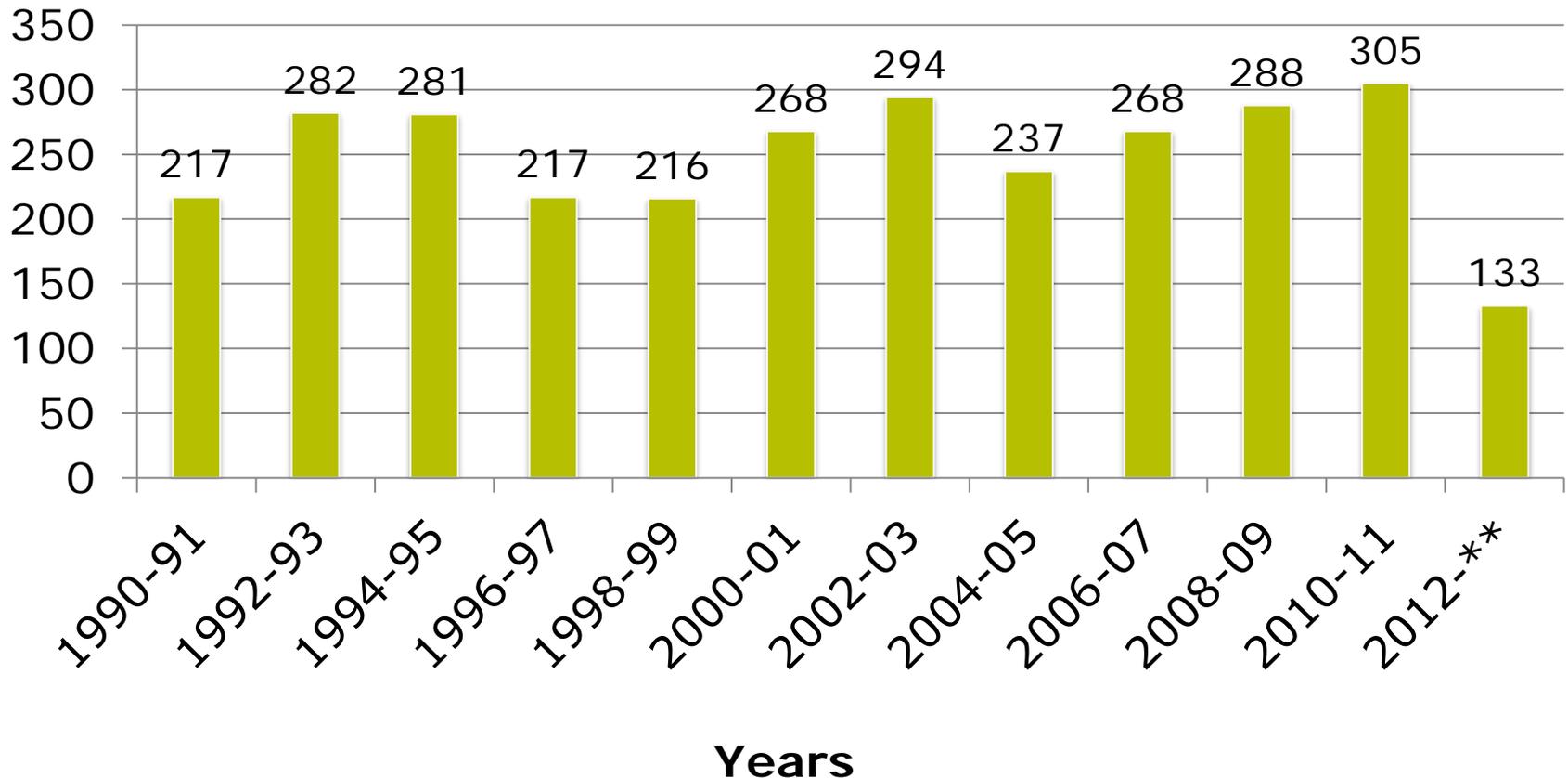
\*\* cases continue to be reported



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.

# BMT for Thalassemia reported to CIBMTR

## Number of BMT for SCD 1990-2012



\*\* cases continue to be reported



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# Workgroup discussion topics for 2014

## ‘Perspectives’ /White paper on role of HCT in SCD

### Access to HSCT

- Study current status of third party coverage for HCT for SCD in children and adults and evaluate insurance barriers
- Assessment of availability of well matched unrelated donors (8/8 MUD and  $\geq 5/6$  UCB) in registry
- Monitor coverage of clinical trials under the ACA