Children as Stem Cell Donors in Research

When is it ethical? When is it approvable?

Friday, March 13, 2015

AGENDA

WELCOME AND INTRODUCTIONS

8:00 Nirali N. Shah, MD, MHS, Dave Wendler, PhD

SESSION I: REGULATIONS GOVERNING PEDIATRIC RESEARCH

8:10-8:30 **Review of Regulations Governing Pediatric Research**
Robert "Skip" Nelson, MD, PhD
Deputy Director and Senior Pediatric Ethicist, Office for Pediatric Therapeutics, Food and Drug Administration

8:30-9:00 **Applying the Regulations to Minor Donors**
Dave Wendler, PhD
Head, Unit on Vulnerable Populations, Department of Bioethics, NIH

9:00-9:20 **Discussion; Q&A**

9:20-9:30 Break

SESSION II: RISKS & BENEFITS TO DONORS AND NOVEL USES AND INDICATIONS

9:30-10:00 **Medical Risks and Benefits for a Minor Donor**
Michael Pulsipher, MD
Chair, Pediatric Blood and Marrow Transplant Consortium

10:00-10:30 **Psychosocial Risks and Benefits for a Minor Donor**
Galen Switzer, PhD
Professor of Medicine, Psychiatry and Clinical and Translational Science, University of Pittsburgh

10:30-11:00 **Evolving Indications for Transplantation and Novel Uses for Donor Products**
Terry Fry, MD
Head, Hematologic Malignancies, Pediatric Oncology Branch, National Cancer Institute

11:00-11:15 **Discussion; Q&A**

11:15-12:15 Lunch

SESSION III: CURRENT STRATEGIES ADDRESSING DONORS IN RESEARCH

12:15-12:35 **NMDP Approach to Adult Donors on Research Protocols**
Roberta King, MPH
Vice-President, Center for International Blood and Marrow Transplant Research

12:35-1:00 **Proposed Algorithm for Minor Donors on Research**
Nirali N. Shah, MD, MHS
Clinical Investigator, Pediatric Oncology Branch, National Cancer Institute

SESSION IV: WORKING GROUPS

1:00-1:45 **Working Groups**

- Risk Analysis: Establishing Standard Care Practices to Enable Defining Risk
  Co-Chairs: Michael Pulsipher, MD, Terry J. Fry, MD & Roberta King, MPH

  Goals: In the growing era of novel uses for donor products and increasing indications for cellular therapy and transplantation, the goals for this group are to help define what procurement procedures and therapies currently fall within a well-established standard of care. Identifying current standard practices and associated risks will help to serve as a metric against which donor risk assessment for future studies can be measured.

- Justifications for Donation by Minors in the Clinical and Research Context
  Co-Chairs: Steven Joffe, MD, Nirali N. Shah, MD & Galen Switzer, PhD

  Goals: The best transplant outcomes are in those settings where there is an available matched sibling donor, which is why siblings, even if they are minor are chosen over unrelated adult donors. These siblings will be coming from families where they have been faced with the challenges of having complicated medical issues impact their family, even prior to being considered as a stem cell donor. This group will review the justifications for donation by minors in the clinical and research setting, and will discuss if such donors can be considered to have a "generalizable condition," as a sibling donor.

- Guidance for "Healthy Donor" Research
  Co-Chairs: Robert "Skip" Nelson, MD, PhD; Jerry Menikoff, MD & Dave Wendler, PhD

  Goals: The Federal regulations governing pediatric research currently do not incorporate a well-established mechanism for addressing the role of healthy donors on research protocols. The goals for this group are to brainstorm innovative approaches to either streamlining the 407 processes, considering a waiver for healthy donor research, or consider updating the federal regulations to incorporate guidelines for healthy donors.

1:45-3:00 **Working Group Discussions**

3:00-3:15 Break

SESSION V: FUTURE DIRECTIONS

3:15 **Next Steps**