

NIH-NHLBI/NIDDK Thalassemia Workshop: Clinical Priorities and Clinical Trials

**Fishers Lane Conference Center
5635 Fishers Lane
Rockville, Maryland**

May 20-21, 2009

Draft Agenda

Wednesday, May 20, 2009

Clinical Priorities in Thalassemia

8:30am

Welcome and Opening Remarks

Suthat Fucharoen, MD and
Cheryl Hillery, MD, Co-Chairs

Susan Shurin, MD, Deputy
Director, NHLBI

W. Keith Hoots, MD, Director,
Division of Blood Diseases and
Resources, NHLBI

Reports from Sub-committees

9:00am

Iron Overload: Clinical Monitoring and
Chelation Therapy - **Alan Cohen, MD**
and **John Porter, MA, MD, FRCP**
FRCPPath

11:00am

Fetal Hemoglobin - **Jeff Miller, MD** and
Yogen Sauntharajah, MD

12 Noon -1:00 pm

Lunch (on your own)

1:00pm

Endocrine Disorders/Bone
Disease/Growth and Development -
Maria Vogiatzi, MD and **Ellen Fung,**
PhD, RD

2:00pm

Stem Cell Transplantation - **Franco**
Locatelli, MD and **John Tisdale, MD**

3:00pm

Gene Therapy - **Michel Sadelain, MD**
and **Arthur Nienhuis, MD**

4:00pm

Adjournment

Thursday, May 21, 2009

CLINICAL TRIALS IN THALASSEMIA

8:00am

Successful Clinical Trials in Thalassemia:
How can we best ensure feasibility? -
Elliott Vichinsky, MD

9:00am

The Thalassemia Clinical Research
Network: Accomplishments and
Discouragements - **Janet Kwiatkowski,**
MD and **Ellis Neufeld, MD, PhD**

10:00am

Facilitating NIH-Funded International
Clinical Trials in Thalassemia - **Francis
Crawley, MA, FFPM**

11:00am

Presentation by Cooley's Anemia
Foundation - **Ms. Gina Cioffi**

11:30am

Public Comments

12:30pm-1:30 pm

Lunch (on your own)

1:30pm

Priorities for Observational/Epidemiologic
Studies and Phase II and Phase III
Clinical Trials.

Chelation

Organ damage

Patient outcomes

Transplantation

Observational studies

3:00pm

Summary and Recommendations

4:00pm

Adjournment