



NHLBI 04

**APLASTIC ANEMIA & MDS INTERNATIONAL FOUNDATION, INC.**  
*Fighting Bone Marrow Diseases through Patient Support & Research Since 1983*

**BONE MARROW FAILURE SCIENTIFIC SYMPOSIUM**

**2005 AGENDA**

**MONDAY, OCTOBER 17**

**7:00 p.m. Cocktail Reception**

*Chris  
Drew*

**TUESDAY, OCTOBER 18**

**7:30 a.m. Breakfast**

**8:30 a.m. Welcome and Introductory Session**

Jaroslav Maciejewski, M.D., Ph.D., Cleveland Clinic Foundation, Taussig Cancer Center, Cleveland, OH;

Neal S. Young, M.D., National Heart, Lung and Blood Institute, Bethesda, MD; and

Robert Carroll, Ed.D., AA&MDSIF President

**8:45 a.m. Plenary Sessions:  
Stem Cell Transplantation**

Chair: Theo de Witte, Ph.D., Radboud University, Nijmegen Medical Centre Central, The Netherlands

**Conventional and Alternative Donor Transplantation**

David Margolis, M.D., Medical College of Wisconsin, Milwaukee, WI

**The Role of Graft Engineering in Transplantation**

Rupert Handgretinger, M.D., St. Jude Children's Research Hospital, Memphis, TN

**Immunosuppression and Stem Cell Transplantation**

Andrea Bacigalupo, M.D., Ospedale San Martino, Genova, Italy

**10:15 a.m. Break**

**10:30 a.m. Long-Term Outcomes after HSCT**

Gerard Socie, M.D., Ph.D., Hospital St. Louis, Paris, France

**Stem Cell Transplantation for MDS: Reduced Intensity, Better Outcome?**

Theo de Witte, M.D., Ph.D., Radboud University, The Netherlands

AA&MDSIF

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John Barrett, M.D.  
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Alan List, M.D.  
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Cleveland Clinic Taussig Center

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Stephen Nimer, M.D.  
Memorial Sloan-Kettering Cancer Center

Ronald Paquette, M.D.  
UCLA Medical Center

Joel Rapoport, M.D.  
Yale University School of Medicine

Mikhael A. Sekeres, M.D., M.S.  
Cleveland Clinic Lerner College of Medicine

Neal Young, M.D.  
National Institutes of Health

## BONE MARROW FAILURE SCIENTIFIC SYMPOSIUM

### 2005 AGENDA

**11:45 a.m. Lunch Break**

**12:45 p.m. Immunosuppressive Treatment of Bone Marrow Failure Syndromes**  
Chair, Neal S. Young, M.D., National Heart, Lung and Blood Institute, Bethesda, MD

**Short and Long-Term Outcomes after ATG Therapy**

Neal S. Young, M.D., National Heart, Lung and Blood Institute, Bethesda, MD

**Advances in Current Immunosuppressive Therapies in AA: European Experience**

Andre Tichielli, M.D., Kantonsspital Basel, University Clinics, Basel, Switzerland

**Immunosuppressive Therapy of MDS**

John Barrett, M.D., National Heart, Lung and Blood Institute, Bethesda, MD

**2:15 p.m. Break**

**2:30 p.m. Novel Therapies**

Chair: Alan List, M.D., H. Lee Moffitt Cancer Center & Research Institute, Tampa, FL

**New Agents and Therapeutic Principles for MDS**

Alan List, M.D., H. Lee Moffitt Cancer Center & Research Institute, Tampa, FL

**Novel Therapeutic Approaches in PNH**

Peter Hillmen, M.D., Leeds Teaching Hospitals NHS Trust, Leeds, UK

**Low-Intensity vs. High-Intensity Therapy for Treatment of High-Risk MDS**

Elihu Estey, M.D., M.D. Anderson Cancer Center, Houston, TX

**4:00 p.m. Cocktail Reception, Poster Viewing and Discussion Session**

**6:30 p.m. Dinner**

Richard Stone, M.D., Chairperson, AA&MDSIF Medical Board

## BONE MARROW FAILURE SCIENTIFIC SYMPOSIUM

### 2005 AGENDA

#### WEDNESDAY, OCTOBER 19

7:30 a.m.      **Breakfast**

8:30 a.m.      **Plenary Sessions:**  
**Stem Cells and Bone Marrow Failure Disorders**  
Chair: Judith CW Marsh, St George's Hospital Medical School,  
London, England

**Qualitative and Quantitative Defects in the Stem Cell  
Function in AA**  
Judith CW Marsh, St George's Hospital Medical School,  
London, England

**PNH as a Stem Cell Disease**  
Lucio Luzzatto, M.D., Ph.D., University of Genova, Genoa, Italy

**Molecular and Cellular Basis of Clonal Evolution**  
Elaine M. Sloand, M.D., National Heart, Lung, and Blood  
Institute, Bethesda, MD

**Application of High Resolution Genomic Scan in MDS**  
Jaroslaw Maciejewski, M.D., Ph.D., Cleveland Clinic  
Foundation, Taussig Cancer Center, Cleveland, OH

10:30 a.m.      **Break**

10:45 a.m.      **Epidemiology and Genetics of Bone Marrow Failure Syndromes**  
Chair: David W. Kaufman, Sc.D., Boston University School of Public  
Health, Boston, MA

**Epidemiology of the Bone Marrow Failure Syndromes**  
David W. Kaufman, Sc.D., Boston University School of Public Health,  
Boston, MA

**Aplastic Anemia in the Orient**  
Surapol Issaragrisil, M.D., Siriraj Hospital, Mahidol University, Bangkok,  
Thailand

**Genetic Risk Factors for Bone Marrow Failure**  
Neal S. Young, M.D., National Heart, Lung and Blood Institutes,  
Bethesda, MD

## BONE MARROW FAILURE SCIENTIFIC SYMPOSIUM

### 2005 AGENDA

- Noon**      **Box lunches distributed to attendees - no break**
- 12:15 p.m.**      **Immune Pathophysiology of Bone Marrow Failure Syndromes**  
Chair: Jaroslaw Maciejewski, M.D., Ph.D., Cleveland Clinic Foundation,  
Taussig Cancer Center, Cleveland, OH
- T Cell Immunity and T Cell Repertoire in Bone Marrow Failure**  
Jaroslaw Maciejewski, M.D., Ph.D., Cleveland Clinic Foundation, Taussig  
Cancer Center, Cleveland, OH
- Autoantigens in Marrow Failure Syndromes**  
Shinji Nakao, M.D., Ph.D., Kanazawa University Graduate School of  
Medical Science, Tokyo, Japan
- Immune Surveillance in MDS**  
Jeffrey J. Molldrem, M.D., M. D. Anderson Cancer Center, Houston, TX
- 1:45 p.m.**      **General Summary Session by Chairs of Plenary Session**
- 2:15 p.m.**      **Symposium Summary**  
Neal S. Young, M.D., National Heart, Lung and Blood Institute, Bethesda,  
MD
- 2:30 p.m.**      **Adjourn**

# Bone Marrow Failure Scientific Symposium

*sponsored by*

***National Institutes of Health  
Office of Rare Diseases  
National Cancer Institute  
National Heart, Lung, and Blood Institute***

**Dr. Jaroslaw P. Maciejewski, Co-Chair**

*Taussig Cancer Center, The Cleveland Clinic Foundation*

**Dr. Neal S. Young, Co-Chair**

*National Heart, Lung, and Blood Institute*

**October 17-19, 2005**

**Loews L'Enfant Plaza Hotel, Washington, D.C.**

## Scientific Speakers

**Dr. Andre Bacigalupo**, Ospedale San Martino, Italy; **Dr. John Barrett**, National Heart, Lung and Blood Institute, Maryland; **Dr. Theo de Witte**, University Hospital Nijmegen, The Netherlands; **Dr. Elihu Estey**, University of Texas MD Anderson Cancer Center, Texas; ; **Dr. Rupert Handgretinger**, St. Jude Children's Research Hospital, Tennessee; **Dr. Peter Hillmen**, Pinderfields Hospital, United Kingdom; **Dr. Surapol Issaragrisil**, Siriraj Hospital, Mahidol University, Thailand; **Dr. David Kaufman**, Boston University School of Public Health, Massachusetts; **Dr. Alan List**, H. Lee Moffitt Cancer Center & Research Institute, Florida; **Dr. Lucio Luzzatto**, Istituto Nazionale per la Ricerca sul Cancro Largo, Italy; **Dr. Jaroslaw Maciejewski**, Taussig Cancer Center, The Cleveland Clinic Foundation, Ohio; **Dr. David Margolis**, Children's Hospital of Wisconsin, Wisconsin; **Dr. Judith Marsh**, St. Georges Hospital Medical School, United Kingdom; **Dr. Jeffrey Molldrem**, University of Texas MD Anderson Cancer Center, Texas; **Dr. Shinji Nakao**, Kanazawa University, Japan; **Dr. Elaine Sloand**, National Heart, Lung, and Blood Institute, Maryland; **Dr. Gerard Socie**, Hopital Saint Louis, France, **Dr. Andre Tichelli**, University Clinics, Switzerland; and **Dr. Neal Young**, National Heart, Lung, and Blood Institute, Maryland.

This activity has been planned and implemented in accordance with the Essential Areas and Policies of the Accreditation Council for Continuing Medical Education through the American Society of Hematology, accredited by the ACCME to provide continuing medical education for physicians.

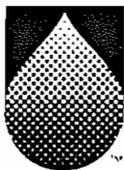
*organized by*

***Aplastic Anemia & MDS International Foundation, Inc.***

Registration:

[www.aamds.org](http://www.aamds.org)

Other Inquiries 800.747.2820 or [baker@aamds.org](mailto:baker@aamds.org)



# APLASTIC ANEMIA & MDS INTERNATIONAL FOUNDATION, INC.

*Fighting Bone Marrow Diseases through Patient Support & Research Since 1983*

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**FOR IMMEDIATE RELEASE**  
March 15, 2005

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## **BONE MARROW FAILURE SCIENTIFIC SYMPOSIUM**

*Washington, DC* ~ The Aplastic Anemia & MDS International Foundation, Inc. has organized an international Bone Marrow Failure Scientific Symposium, sponsored by the National Institutes of Health, Office of Rare Diseases, National Cancer Institute, and the National Heart, Lung and Blood Institute, to be held at the Loews L'Enfant Plaza Hotel in Washington, DC October 17-19, 2005.

This symposium has been designed to provide researchers from around the world a forum for presentation of their work and the opportunity to exchange ideas relating to the etiology, genetics, pathophysiology, epidemiology, and treatment of bone marrow failure diseases. More than 250 physicians with interests in immunosuppressive therapy, stem cell transplantation and basic scientists in cell biology, immunology education are expected to attend.

Dr. Jaroslaw P. Maciejewski, Taussig Cancer Center, The Cleveland Clinic, and Dr. Neal S. Young, National Heart, Lung, and Blood Institute are co-chairing the event. Scientific speakers include; **Dr. Andre Bacigalupo**, Ospedale San Martino, Italy; **Dr. John Barrett**, National Heart, Lung and Blood Institute, Maryland; **Dr. Theo de Witte**, University Hospital Nijmegen, The Netherlands; **Dr. Elihu Estey**, University of Texas MD Anderson Cancer Center, Texas; **Dr. Rupert Handgretinger**, St. Jude Children's Research Hospital, Tennessee; **Dr. Peter Hillmen**, Pinderfields Hospital, United Kingdom; **Dr. Surapol Issaragrisil**, Siriraj Hospital, Mahidol University, Thailand; **Dr. David Kaufman**, Boston University School of Public Health, Massachusetts; **Dr. Alan List**, H. Lee Moffitt Cancer Center & Research Institute, Florida; **Dr. Lucio Luzzatto**, Istituto Nazionale per la Ricerca sul Cancro Largo, Italy; **Dr. Jaroslaw Maciejewski**, Taussig Cancer Center, The Cleveland Clinic Foundation, Ohio; **Dr. David Margolis**, Children's Hospital of Wisconsin, Wisconsin; **Dr. Judith Marsh**, St. Georges Hospital Medical School, United Kingdom; **Dr. Jeffrey Mouldrem**, University of Texas MD Anderson Cancer Center, Texas; **Dr. Shinji Nakao**, Kanazawa University, Japan; **Dr. Elaine Sloand**, National Heart, Lung, and Blood Institute, Maryland; **Dr. Gerard Socie**, Hopital Saint Louis, France; **Dr. Andre Tichelli**, University Clinics, Switzerland; and **Dr. Neal Young**, National Heart, Lung, and Blood Institute, Maryland.

Continuing Medical Education credits will be available, as this activity has been planned and implemented in accordance with the Essential Areas and Policies of the Accreditation Council for Continuing Medical Education through a joint sponsorship of the American Society of Hematology and the Aplastic Anemia & MDS International Foundation, Inc. The American Society of Hematology is accredited by the ACCME to provide continuing medical education for physicians.

Registration fee is \$100.00 per person, \$25.00 in addition for CME's. Register for the symposium online at [www.aamds.org](http://www.aamds.org) or call (800) 747-2820 for more information.