AA&MDSIF Bone Marrow Failure Disease Scientific Symposium Summary Report

Building a Collaborative Research Community That Saves Lives was the theme of the Bone Marrow Failure Disease Scientific Symposium held on March 11-12 in Bethesda, Maryland. Sponsored by the Aplastic Anemia & MDS International Foundation and supported by the Office of Rare Diseases Research and the National Heart, Lung and Blood Institute, the symposium gathered over 120 participants to hear experts from around the world present the latest information about bone marrow failure disease.

The purpose of the symposium was to develop specific recommendations for future basic and clinical research needed to advance the field of bone marrow failure disease. Topics included Genetics and Epidemiology of Myelodysplastic Syndromes (MDS), Genetics and Immunobiology of Aplastic Anemia & Paroxysmal Nocturnal Hemoglobinuria (PNH), Pathophysiology and Molecular Targets in MDS, and Transplant and Non-Transplant Treatments for Aplastic Anemia, MDS and PNH.

Each presentation by the prestigious faculty members was followed by a question-and-answer session. This format was especially appealing to the participants because it allowed for in-depth discussion of important new approaches and opportunities for collaboration. Attendees also appreciated the emphasis on basic science data that is not presented at professional conferences as often as clinical data.

Comments from symposium evaluations reflected how effective the symposium was in advancing the field of bone marrow failure disease research. One participant noted that “it was great to see the breadth of research being done in the field” and another was impressed by the “rapid progression of research.” Many felt it was an excellent forum for collaboration and enjoyed the informal interaction with colleagues as well as the formal sessions.

A poster session enabled young investigators the chance to share their research projects with the established investigators and to further develop their experience in bone marrow failure disease research.

The Symposium Planning Committee included: Richard M. Stone, MD, Dana-Farber Cancer Institute, Chair; Neal Young, MD, NHLBI, Co-Chair; John Barrett, MD, NHLBI; Pamela Becker, MD, PhD, University of Washington; Peter Hillmen, MB, ChB, PhD, Leeds Teaching Hospital, UK; and Jaroslaw Maciejewski, MD, PhD, Cleveland Clinic.

A summary of the symposium findings and recommendations intended for patients is now being edited and will be available in both print and online later this spring. In addition, a summary webcast and booklet is being prepared for health professionals.

For more information, contact Alice Houk, Director of Health Professional Programs, at houk@AAMDS.org or (301)279-7202, ext. 101.