

Scientific Symposium Summaries

Summary of Day 1

Presented by Sharon Savage, M.D., FAAP, National Cancer Institute

Lessons from the presentations on the first day of the meeting include the need for studies to learn about the patterns of **bone marrow failure** disorders in different populations. Following patients over the long term is critical to understand how **bone marrow** failure disorders develop and how these disorders change over time.

The studies that use genomic techniques to study all of the hereditary information in patients with bone marrow failure disorders are exciting. Researchers are now using these techniques to predict the course of a patient's disease and help doctors choose the best treatments for their patients. One challenge for researchers is narrowing down the vast amount of information on genetics that studies are generating.

Researchers are using new techniques to study issues that have been looked at in the same way for a long time. These new approaches have helped experts identify potential targets, such as spliceosomes, or groups of proteins involved in splicing pre-messenger RNA, for new treatments.

Summary of Day 2

Presented by Michael Pulsipher, M.D., University of Utah

Research has made tremendous advances in understanding the genetics and biology of myelodysplastic syndromes (**MDS**), and many wonderful studies in this area were presented at this symposium. Research has also shown the relationships between many biological features of MDS and outcomes in patients. This biological research is important because it leads to targeted therapies that will benefit patients. A downside is that the outcomes of many new therapies aren't good enough, so more research is needed. One challenge is to better understand the roles of treatment before hematopoietic **stem cell transplant** (HSCT), HSCT itself, and treatment after HSCT.

Some wonderful research has been done to shed light on the development of paroxysmal nocturnal **hemoglobinuria** (**PNH**), and a very active agent—eculizumab (Soliris®)—is now available for this disease. A research goal is to find drugs that inhibit the abnormal activities of complement proteins, which are part of the immune system, in patients with PNH. A challenge in this field is the high cost of eculizumab treatment, but hopefully this problem can be solved.

Good therapies are available for **aplastic anemia**, and these treatments keep patients alive longer than in the past. The major challenge in aplastic anemia is that experts don't understand the biology of the disease well enough. More biological markers are needed to make progress in this field. A disappointment is the lack of major progress with treatments that suppress the immune system. The ability of eltrombopag (Promacta®) to expand existing **stem cells** is exciting, but not enough patients have responded to this treatment in studies so far.

Tremendous advances have been made HSCT, and its outcomes have improved dramatically in recent years. Problems with HSCT, such as graft-versus-host disease (a common HSCT complication) still need to be solved, but some nice progress is being made in this area. Researchers are now trying to figure out the best time to do HSCT and how to maximize its chances of success.

One way to make progress in all of these areas is for researchers to work together. Many researchers are now doing this, as shown by the successful European LeukemiaNet MDS Registry.

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