The Tail of the Comet: Follow-Up to Big Discoveries in Platelet Disorders

By Alice Ma, MD

It happens year after year. A brilliant hematologic discovery is showcased at ASH — a flurry of papers follow, but follow-up questions develop about the utility of the novel test, the indications for the new drug, and what happens when the drugs don't work. These questions will be answered at the Education Session on Platelet Disorders offered today at 2:30 p.m. and tomorrow at 7:30 a.m.

Dr. Jim Bussel will lead off the talks by discussing the novel class of agents used to increase platelet counts — the so-called second-generation thrombopoietic agents. These drugs stimulate the thrombopoietin receptor (TPO-R) while having no sequence homology to native thrombopoietin (TPO), thus avoiding the problem of antibody generation seen with one of the first-generation recombinant TPO products. Much excitement about these drugs has developed after the initial reports that these agents had efficacy in refractory immune thrombocytopenic purpura (ITP), thus presenting a new treatment paradigm for this hitherto frustrating disease. Moreover, these agents have been tried in other thrombocytopenic disorders, such as myelodysplasia, chemotherapy-induced thrombocytopenia, and hepatitis C-associated thrombocytopenia. Dr. Bussel will review the agents currently in development as well as the trials demonstrating their efficacy in the above disorders.

Next, Professor Desmond Fitzgerald will review the sticky topic of aspirin and clopidogrel "resistance." This term, referring to a lack of therapeutic response to these agents is somewhat misleading, since it implies a single clinical definition and mechanism. Professor Fitzgerald will review the biology of these agents, define potential mechanisms underlying lack of therapeutic response, and review measurements used to gauge a pharmacologic response. He will also touch on clinical trials measuring lack of clinical efficacy.

Lastly, Professor Pier Mannucci will address one of the most pressing clinical questions remaining about thrombotic thrombocytopenic purpura (TTP), now that the role of ADAMTS13 in the pathogenesis of TTP has been elucidated. Clinicians who treat TTP still want to know when testing for ADAMTS13 should be done, which test to use, how the test results should be interpreted, and what the pitfalls of testing are.

Attendees interested in platelet disorders may also be interested in the Education Session on Transfusion Medicine, offered today at 7:30 a.m. and 4:00 p.m. Additionally, today at 2:00 p.m. and tomorrow at 9:30 a.m., the Scientific Committee on Clinical Laboratory Hematology will discuss new findings on the pathophysiology of immune-mediated thrombotic disorders, such as TTP, APLA, and HIT. Finally, in the plenary session tomorrow at 1:30 pm, Dr. Terry Gernsheimer will present the results of the use of a novel TPO-R-stimulator, AMG 531, in splenectomized patients with chronic ITP.