METHODOLOGY BEHIND ITP GUIDELINES

Immune Thrombocytopenia (ITP) is a rare autoimmune disorder characterized by a shortage of platelets. Platelets are the part of the blood that the body needs for blood to clot. Normally, your immune system helps your body fight off infections and diseases; however, ITP makes your immune system destroy your body’s platelets by mistake, often causing significant bleeding episodes that range from moderate to severe.

The American Society of Hematology (ASH) believes it is essential to provide updated treatment guidelines that reflect the newest evidence about the disease. ASH formed a multidisciplinary guideline panel that included adult and pediatric clinical experts, methodologists with expertise in ITP, and patient representatives. In partnership with the University of Oklahoma Health Sciences Center, the panel synthesized the evidence to develop new clinical practice guidelines for ITP.

WHAT THE GUIDELINES SAY

1. The guidelines stress avoiding unnecessary treatment in children. Specifically, the guidelines can help physicians avoid giving interventions and treatments that have little evidence of success and may have side effects that outweigh potential benefits. This is important, as bleeding is minimal in the majority of children and the disease often goes away without treatment.

2. Corticosteroids are often used to treat ITP in adults. The guidelines provide new recommendations for the use of corticosteroids, including guidance on when they should be used, for how long, who should take them, and how usage should be monitored. There is guidance for keeping the course of treatment as short as possible, as long-term corticosteroid use can cause harm without additional benefit.

3. The guidelines also reviewed other therapies that may be given if initial therapy does not work, including rituximab, thrombopoietin-receptor agonists (TPO-RAs), and splenectomy (removal of the spleen).
   - The guidelines recommend delaying splenectomy for at least a year due to the life-long side effects that can result from the procedure.
   - When considering TPO-RAs and rituximab, the guidelines recommend considering differences in the route of administration, duration of disease, short versus long-term treatment, and patient co-morbidities to make the best choice for the individual patient.

4. Additional research is needed and could inform how new medicines in development are tested. Specifically, focus should be placed on collecting information on outcomes that are important to patients, applying standard dosing for medications, getting long-term follow-up data, and understanding how to engage patients in shared decision-making.

TOTAL NUMBER OF PANEL RECOMMENDATIONS: 24
The American Society of Hematology (ASH) (www.hematology.org) is the world’s largest professional society of hematologists dedicated to furthering the understanding, diagnosis, treatment, and prevention of disorders affecting the blood. For more than 60 years, the Society has led the development of hematology as a discipline by promoting research, patient care, education, training, and advocacy in hematology.

MULTI-YEAR, STATE-OF-THE-ART, TRANSPARENT AND SCIENTIFIC PROCESS

A panel was vetted and appointed by ASH. Panelists disclosed all financial and nonfinancial interests. Conflicts of interest were managed through panel composition, disclosure, and recusal.

The panel included hematologists, adult and pediatric clinical experts, and ITP experts in evidence synthesis and guideline development.

For more information on these new guidelines, visit: http://www.hematology.org/ITPguidelines