The ITP Support Association Platelet Reprint Series

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Title: **Treatment of Children with ITP: Who and When**

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Both the United Kingdom and United States have published guidelines regarding the management of pediatric ITP and together they support that in the absence of significant bleeding, most children can be managed with observation, without any treatment, even if their platelet count is very low. This recommendation is based on several observations. First, most children will recover from their ITP even without any treatment. Second, severe bleeding events in children with ITP are rare, even when their platelet counts are very low. Third, there is no evidence that giving any medication reduces the chance of a child having a severe bleeding event. Lastly, medications are associated with side effects and costs.

So what factors should be taken into account when determining if a child with ITP requires treatment or not? Several things should be assessed each time a child is evaluated for possible treatment. The first thing to take note of is the amount of bleeding. Treatment should be given for children with “moderate” or “severe” bleeding in order to stop the active bleeding. “Severe” bleeding is usually considered bleeding that is life-threatening or results in decline in the red blood cells, however “moderate” bleeding is less easily defined. Efforts are underway to create a way for physicians to accurately assess bleeding and feel more confident in applying this criterion. Despite several published bleeding severity measurement tools, to date there is not one universally accepted measure. In 2013, an International Working Group published a Bleeding Assessment Tool (BAT) specific for ITP, however it has not yet been tested for its accuracy.

Once it has been established that the child has no bleeding requiring treatment, consideration should be given to any upcoming surgery or activity that might require an increase in the platelet count. Children often require procedures such as dental work or tonsillectomy. The hematologist is involved to assist with the creation of a treatment plan that will result in a platelet count adequate for the type of procedure being performed. Unfortunately, there is little published information on the specific platelet count that is considered safe for individual procedures and so often this decision involves close communication between the surgeon and hematologist to determine the amount of bleeding that is likely.

But what about the child with no bleeding and no additional urgent needs for treatment? What factors should guide the decision for treatment in this setting? This can best be answered by conducting a detailed assessment of the child and parent’s health related quality of life (HRQoL). The focus of this assessment might vary depending on how long the child has had ITP. At diagnosis, for example, it might involve questions about the comfort level with the diagnosis of ITP, child activity level, and distance from the medical center. Later, in patients with ongoing ITP the discussion might revolve more around a desire to live without ITP, ability to participate in athletic activities, concerns over treatment or the impact of feeling different from other kids. In older children it is important to make sure that they are part of the conversation and have a chance to communicate their needs and concerns. These conversations help understand the impact of the disease on the patient and allow patients to define their goals and expectations for living with ITP. To assist with understanding HRQoL, a specific ITP questionnaire called the Kids’ ITP Tool (KIT) has been created. This questionnaire asks parents and children questions about living with ITP including how much they worry about the diagnosis and symptoms of ITP. In some settings the amount of bleeding a child has might be closely linked with HRQoL, however often assessment of HRQoL provides additional information. Take for example a nosebleed that causes the child to be sent home from school resulting in the parent missing work. This episode might impact HRQoL regardless of how much bleeding there was.

As clinical scientists when we design studies of new or existing medications it is important to make sure we assess the impact of these medications on bleeding and HRQoL so we can best know how to support patients in decision-making. In the end the decision to treat is individual to each patient and outside of significant hemorrhage there is no single approach that works for every patient. The decision to treat requires frequent discussions between the physician and the patient to understand their goals for quality of life, concerns about the disease, and impact of treatment side effects. Only after these items have been addressed can a true decision about the need for treatment of children with ITP be reached.