ITP Support Association Platelet Reprint Series

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Title: **What's the Right Thing to Do For a Child with a New Diagnosis of ITP?**
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How do doctors know what is the right thing to do for their patients? How is evidence obtained to guide management decisions? How can doctors distinguish strong evidence from weak evidence? These questions seem more appropriate for lawyers and courts than for doctors and patients. But all medical decisions should be based on evidence, and the best evidence should allow the doctor to be confident in his decision, "beyond a reasonable doubt" . . . the same phrase a jury must use.

How does this relate to the initial management of a child with a new diagnosis of ITP? The question of evidence is important because there are very different opinions among experts about how children with ITP should be treated. Some feel that treatment with IVIG, anti-D, or steroids is essential to increase the platelet count as fast as possible and thereby minimize the risk for serious bleeding; others feel that these treatments have more potential harm for than actual benefit and that the best management is reassurance, supportive care, and observation. The fact is that there is no firm evidence to support either management strategy. But in spite of no firm evidence, doctor's opinions are often very strong indeed.

The best evidence for guiding medical decisions comes from research known as clinical trials. In a clinical trial, patients are randomly assigned to one treatment program or another, and then the outcomes of these patients, both the benefits and the harms from the different treatments, can be accurately compared. The random assignment is the important feature of a clinical trial, because it prevents bias and balances the groups of patients – everything about these groups of patients should be the same except for the treatment being tested. This is the way most new medicines are studied before government approval is awarded. Complementary and alternative medicines have not been tested in this manner, which is why medical scientists sometimes question their value. Clinical trials often contradict both anecdotes and personal experiences, each of which are subject to bias.

We are currently developing a study, a clinical trial, to evaluate the best treatment for children with newly diagnosed ITP. The important question is whether only reassurance and supportive care are as safe and effective as more aggressive treatment with one or another drug treatment when large numbers of children are compared and the frequency of serious bleeding is measured. We believe that measuring the frequency ofserious bleeding is the important outcome. Previous studies have simply measured platelet counts. But a faster platelet count recovery may or may not be important. Avoiding drug treatment costs less, has no side effects, and may be just as safe, even if recovery from ITP is not quite as rapid.

In our planning for this study, we estimated that for two different management programs to be adequately compared for the prevention of serious bleeding, 200 children will need to be analyzed with each management strategy (observation vs. drug treatment). Such a study will likely require 2 or 3 years to complete. Is this worth the effort and expense? Of course. This is the only way to gain the necessary evidence. And without firm evidence, each doctor's personal and often biased opinion will continue to determine what they think is the right thing to do. And the problem with personal opinion is that it is so heavily influenced by our most recent experience. Clinical trials have important dividends in addition to achieving their primary objective of documenting best management. We learn to quantitatively and systematically evaluate symptoms that are most important to children and their parents, even more important than the platelet count. We learn how to objectively evaluate the amount of bleeding, and we can also objectively assess the child's quality-of-life. In the end, we can provide strong evidence so doctors will be more confident about their decisions. Ω