The ITP Support Association Platelet Reprint Series

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Title: **How do haematologists treat ITP patients**

Authors: **James N. George, M.D.,** Oklahoma City, **George R. Buchanan, M.D.,** Dallas

We have written many times about appropriate treatment for patients with ITP. But how do hematologists actually treat their patients with ITP? The only certain way to document what doctors do is to review the medical records of their patients. A simpler way is to send a survey with questions about ITP patients' problems and ask hematologists how they would deal with them. However doctors may respond with what they think is the "right" treatment, not necessarily what they actually do.

Last year surveyed all 101 hematologists-oncologists in the state of Oklahoma who treat adults, asking about their treatment of patients with ITP; 82 responded, an impressive proportion. Most hematologists (68%) reported seeing just 1-5 patients with ITP each year; the others reported seeing more than 5 patients each year. Their years in practice were evenly distributed among less than 5 years (29%), 5-20 years (37%), and more than 20 years (34%). The survey consisted of 3 patient stories, all involving an active, healthy 28 year-old woman who had a low platelet count but whose other blood counts and tests were normal. For each of the stories, the hematologists were asked to select only one option from a choice of treatments. The Table summarizes the patient stories and treatment choices of the 82 hematologists.

Survey of Oklahoma hematologists: Questions about treatment of a 28 year-old active, healthy, non-pregnant woman

Patient story	**Treatment selected by the 82 hematologists**							
	1	**2**	**3**	**4**	**5**	**6**	**7**	**8**
1. Unexpected discovery of a platelet count of 40 on a routine evaluation. No bleeding symptoms	77	0	0	5	0	0	0	0
2. Spontaneous bruising and prolonged menstrual bleeding for 2 months. Platelet count 9	0	0	3	62	17	0	0	0
3. Platelet count responded to prednisone, but platelet count fell again to 9 three months later when prednisone was stopped. IVIg then was effective only for short times.	0	6	0	0	0	30	32	14

Treatment options: each of the 82 hematologists could only select one option		
1. Observation without drug treatment	**4**. Daily prednisone	**7**. Splenectomy
2. Anti-D	**5**. Intermittent high-dose dexamethasone	**8**. TPO agents (romiplostim, eltrombopag)
3. IVIg	**6**. Rituximab	

The first patient story addresses a common question in our current era of routine blood counts: when is drug treatment for ITP appropriate? Since there are no scientific data to guide this decision the hematologist must act on his or

her perception of the risk for bleeding. We know that a platelet count less than 100 is usually required to consider the diagnosis of ITP. Platelet counts between 100 and 150 are considered low but are generally harmless; they may be due to a mild infection or laboratory variation. Even a platelet count of 50 doesn't cause bleeding, unless injury, surgical procedures, or epidural anesthesia for childbirth require higher levels. Platelet counts of less than 10 are almost always associated with easy bruising and petechiae as well as potentially more serious bleeding. So for the first patient story we narrowed the window to between 10 and 50, describing a healthy young woman with an unexpected discovery of a platelet count of 40. Ninety-four percent of hematologists responding to the survey would just observe this woman without drug treatment, although the remaining 6% would begin treatment with prednisone. We think observation without drug treatment is the best decision.

At what platelet count should some treatment be considered? Whereas it is quite appropriate not to treat a young child with a new diagnosis of ITP and platelets under 10, since they often spontaneously and quickly recover, in adults ITP is considered to be more serious so some treatment is usually recommended for patients with very low platelet counts, such less than 20 or 30. In the second patient story, treatment is clearly appropriate, but what should be used? Most hematologists (76%) selected daily oral prednisone, the traditional treatment for many years. It is effective and inexpensive. The problem with daily prednisone is that there are no clear guidelines that help hematologists decide when to decrease the dose and stop it, and too often patients are treated for too long and develop troublesome steroid side effects. Treatment with very high doses of corticosteroids, typically dexamethasone, for short periods of 4 days and sometimes repeated every 2-3 weeks, is another option that was selected by 21% of hematologists. This may be more effective than daily prednisone and it has the advantage of a defined schedule and clear stop point, but the high doses of dexamethasone can cause a lot of symptoms. IVIg, the choice of a few hematologists, is usually reserved for patients with more serious bleeding problems since it is expensive, requires several hours in a clinic for administration, and has many side effects.

In the third story, the same woman has severe and symptomatic thrombocytopenia despite initial responses to prednisone and IVIg The choices of the responding hematologists were divided: 39% selected splenectomy, 37% rituximab, 17% TPO agents, and 7% anti-D. This emphasizes the wide range of opinions with the availability of multiple effective treatment options. Those hematologists who had been in practice longer were more likely to select splenectomy. This may be expected because splenectomy was the first effective treatment (over 70 years ago) and remains the most effective treatment (normal platelet counts without further treatment in two-thirds of patients). The disadvantages of splenectomy are that it is a surgical procedure and is associated with a very rare but real life-long risk of severe infection. Younger hematologists were more likely to select rituximab. Unfortunately long-term responses to rituximab occur in only 20% of ITP patients and rituximab suppresses the immune system for many months. For the past 5 years, the TPO agents have been marketed aggressively for treatment of ITP. Although these agents are very effective (85% of patients respond) and they appear to have few side effects, in almost all patients they need to be taken indefinitely.

This is how hematologists in Oklahoma treat their patients with ITP. Or, at least how they <u>say</u> they treat their patients with ITP!!