

# Immune (or Idiopathic) Thrombocytopenic Purpura

## *A Guide for Parents*

The following information is provided in the form of a pamphlet to the parents of all newly diagnosed ITP patients seen in our center. It describes the diagnostic and management strategies employed by our physicians, emphasizing what has been called the “non-interventionist” or “wait and see” approach. This is often preferred to administration of corticosteroids, intravenous immunoglobulin, anti-D, or other drug treatments.

# IMMUNE THROMBOCYTOPENIC PURPURA (ITP)

## *A Guide for Parents*

Prepared by:  
George R. Buchanan, M.D.  
Medical Director – Center for Cancer and Blood Disorders  
Children’s Medical Center Dallas &  
U.T. Southwestern Medical Center at Dallas

Revised October 2007<sup>MA</sup>

Your child has been diagnosed as having Immune, or Idiopathic, Thrombocytopenic Purpura (ITP). The following information about the disease, including its possible causes, treatments, and outcomes, will hopefully prove useful to you.

### Definition of ITP.

**Immune, or Idiopathic, Thrombocytopenic Purpura** (ITP) is a condition characterized by bleeding resulting from a low platelet count due to increased destruction of platelets by the body. "Idiopathic" is a medical term indicating that we do not know the exact cause of the illness. Thrombocytopenic is a medical term meaning a low platelet count. Purpura means bruising. Therefore, ITP is an illness of unknown cause characterized by bruising and other bleeding resulting from a low platelet count. There are two forms of ITP, acute ITP (in which the disease lasts for less than six months) and chronic ITP (where the condition lasts for more than six months and, sometimes, for many years). The distinction between acute and chronic ITP is arbitrary.

**What are platelets?** Platelets are small, sticky particles in the blood which are necessary for blood clotting. A reduction in the number of platelets results in bleeding in the skin, from body openings (nose, mouth, urinary tract, vagina, rectum) and sometimes internally. Platelets are produced (along with the red blood cells and white blood cells) in the bone marrow, the spongy tissue inside the bones.

**Why are the platelets low in ITP?** A reduction in any part of the blood count (including the platelets) can be due either to decreased production in the bone marrow or increased destruction by the body. In ITP the platelet count is low as a result of increased destruction of the platelets. An antibody against the platelets is produced, often as a result of a recent viral infection. The antibody attaches to the platelets and leads to their rapid removal from the bloodstream. In a sense, a child with ITP is “allergic” to his or her own platelets as a result of abnormally increased immunity. That is why many doctors now call the condition “Immune Thrombocytopenic Purpura” rather than Idiopathic Thrombocytopenic Purpura. Even though the bone marrow (which is normal in ITP) may produce platelets at an increased rate, it is unable to “keep up” with those that are destroyed, and the platelet count drops to a level that may result in bleeding. The site in the body where most of the platelets are destroyed is the spleen, an organ under the left rib cage which filters and cleanses the blood.

**What is the Cause of my Child’s ITP?** Many parents are alarmed and frustrated about their child’s ITP because of uncertainty about its cause. They tend to blame themselves for having done something wrong or having overlooked something about their child. Such guilt is common in conditions whose causes are unclear. ITP is not alone among problems where the cause is unknown. After all, we also don’t know the cause of most other diseases, such as cancer, diabetes, kidney problems, asthma, and skin conditions. The best evidence at

present is that there is some type of imbalance in the child's immunity that leads to the production of the antibody against the platelet. This imbalance is either due to a previous viral infection or, in the case of chronic ITP, to a subtle underlying autoimmune disorder.

**So, you couldn't have prevented your child's ITP and you are not at fault. Don't blame yourself! Clearly more research is needed regarding the cause of ITP.**

**History:** ITP can occur in individuals of all ages. It is most common in children between 2 and 8 years of age, although it is seen in infants, teenagers, adults, and even elderly individuals. Most patients with ITP have been previously well but suddenly develop bruises, petechiae (pinpoint red spots on the skin and in the mouth which are tiny hemorrhages), nosebleeds, and perhaps bleeding in other sites. This sudden development of bleeding often occurs one or two weeks after an infection, usually a minor viral infection such as a cold or stomach disorder. Occasionally specific viral infections such as measles, chicken pox, mononucleosis ("kissing disease"), and HIV infection can result in ITP. In some patients with the chronic form of ITP, the bleeding does not come on suddenly but tends to evolve over a period of many weeks or months.

Children with newly diagnosed ITP are usually otherwise well. They typically do not have high fevers, weight loss, or other signs of serious illness. Although ITP-like illnesses occasionally result from certain drugs in adults, drug-induced ITP is rare in children. ITP usually doesn't run in families.

**Physical Examination:** When a doctor examines a child with ITP, there are usually no abnormal findings except for signs of bleeding (bruises, petechiae on the skin, petechiae and blood blisters in the mouth, nosebleeds, etc.). Specifically, we usually find no enlargement of the liver, spleen, or lymph glands (which would suggest leukemia or another serious cause of a low platelet count).

**Laboratory Tests:** Very important for the diagnosis of ITP is a complete blood count (CBC). Children with ITP usually have a very low platelet count, typically between 1,000 and 20,000 per  $\text{mm}^3$  (or 1 to 20 x  $10^9$  per liter). A normal platelet count is over 150,000 per  $\text{mm}^3$ , and the lowest platelet count that completely protects against bleeding is usually around 50,000 per  $\text{mm}^3$ . The remainder of the blood count is usually normal in children with ITP. This means that there is typically no anemia (reduction in the red blood cell count or hemoglobin) or abnormalities in the white blood cells (which are important in fighting infection).

If we see significant anemia and/or white blood cell abnormalities we need to consider that the child might have leukemia, aplastic anemia, or another serious disorder rather than ITP. In that case, a **bone marrow examination** is sometimes necessary to make the diagnosis. This test consists of removing several drops of bone marrow from the hip bone. Although this procedure may be somewhat uncomfortable for the child, it is not dangerous. Some sedation is given to make the procedure more tolerable for the child. The results are usually available within several hours. It is not necessary to routinely perform a bone marrow test in every child with suspected ITP, but only when there are some atypical features.

Other than a blood count and in some cases the bone marrow examination, other tests are usually not required in children with probable acute ITP. However, in patients with chronic ITP several additional tests are sometimes performed.

## The Treatment and Outcome of ITP.

Children with ITP usually do extremely well since ITP is generally not a serious condition such as leukemia. Children with ITP often show an improvement in their bruising, petechiae, and nosebleeds within a week or so from diagnosis, even though the platelet count may not yet begin to rise. In several weeks, however, the platelet count usually increases, sometimes slowly and sometimes very rapidly. In half of the children with ITP, the platelet count rises to normal (above 150,000 per  $\text{mm}^3$ ) within 6 to 8 weeks from diagnosis. In other children the rise in platelet count (and complete disappearance of bleeding problems) takes a number of months. In about 25% of children with ITP, the platelets remain low for more than six months (see section on chronic ITP).

Despite a low platelet count, hemorrhage is usually not serious in children with ITP. However, a few children develop extensive bleeding from the nose, mouth, or intestines (with vomiting of blood or blood in the stool, which is sometimes black in color). These children can develop significant anemia due to blood loss, as can girls with vaginal bleeding. The most dreaded complication of ITP is bleeding in the brain. Fortunately, this is extremely rare.

Most children with ITP do not need any specific drug treatment. We just need to “wait and see” if the bleeding stops and the platelet count rises to normal. However, in some cases, specific treatment is necessary, with prednisone, intravenous IVIG, or anti-D immunoglobulin.

**Prednisone:** Prednisone is a cortisone or steroid-like medication which is usually given by mouth for 4 to 14 days (or occasionally in high doses by vein for three days). In some children the prednisone may result in a more rapid rise in the platelet count than if no treatment is given. The prednisone often has temporary side effects such as moodiness, weight gain, insomnia, and stomach aches. Long-term use of this medication is not recommended for ITP because of these and other side effects (diabetes, reduced immunity, thin bones, etc).

**Intravenous Immunoglobulin (IVIG):** This is a medication, derived from blood plasma that is given for one or two days by vein over 4 to 6 hour periods. This treatment usually raises the platelet count within a few days. Some doctors use it routinely for treatment of ITP. On the other hand, IVIG is very expensive (usually \$3,000 to \$5,000 for a single dose), requires an IV, and may cause nausea, vomiting, and severe headaches.

**Anti-D Immunoglobulin:** Anti-D immunoglobulin, also called WinRho, is another possible treatment for ITP for children whose red blood cells are Rh positive. It is also derived from blood plasma and works like IVIG by blocking the spleen so that the antibody-coated platelets are less readily destroyed. It does this by “fooling” the spleen to destroy red blood cells rather than platelets. Anti-D is given as a single intravenous injection, so it is easier to administer than IVIG. However, it may cause severe anemia and is expensive.

With all three forms of therapy - prednisone, IVIG, and anti-D - the apparent beneficial effect soon “wears off”, and several days to weeks later the platelet count may again decline to a low level. These medicines raise the platelet count by temporarily “paralyzing” the spleen, so that the platelets that have antibody on their surface cannot be filtered or removed by the spleen. There is a great deal of controversy about whether prednisone, IVIG, or anti-D should be used to treat most children with ITP. Certainly, however, all doctors agree that the occasional child with ITP and serious hemorrhage should receive one or more of these treatments. Unfortunately they sometimes are ineffective in raising the platelet count in such circumstances.

**Other treatment:** Children with ITP usually don’t need any other therapy. No special vitamins or special diets are required. Clearly the child with ITP should be watched very carefully to try to prevent injury to the head, especially when the platelet count is very low and the child shows other signs of bleeding. Contact sports and other vigorous physical activities should be avoided until the platelet count rises substantially. If the child’s platelet count is low, aspirin or drugs that contain aspirin and ibuprofen (Motrin or Advil) should be avoided, since these medicines may damage the platelets. Sometimes special protective helmets are recommended for active toddlers in order to reduce the risk of head injury.

**Return visits to the doctor:** After the diagnosis of ITP, the child should be seen initially every week or two until the bleeding signs stop and the platelet count begins to rise. Then he or she can be seen less often until the platelet count returns to normal. At each visit a blood count, including a platelet count, will be done. If the child is treated with prednisone, IVIG, or anti-D the platelet count may initially rise, but then decline again several weeks later. Retreatment is usually not necessary in such cases. When the platelet count returns to and remains normal, regular doctor visits or blood counts are no longer necessary since the child is usually considered “cured.” About 1 to 2% of children with acute ITP have another episode of ITP months or even years later. These events are usually similar to the first ITP episode. Children who recover from ITP do not later appear to have an increased risk of leukemia or other blood problems. They usually remain healthy.

## Chronic ITP.

About 25% of children with ITP do not develop a normal platelet count by six months and are arbitrarily designated as having chronic ITP. These children are often, but not always, older, and their initial bleeding problems frequently develop slowly over weeks or months rather than suddenly. The platelet count in children

with chronic ITP varies widely (usually between 20,000 and 100,000 per mm<sup>3</sup>) from day to day and week to week. Therefore, platelet counts are not needed on a regular basis in children known to have chronic ITP, just every few months. Their ITP is often managed very much like the acute form of the disease. Most children require no treatment other than avoidance of aspirin and some restriction of activities, such as not participating in contact sports to avoid head injury. Prednisone is sometimes useful in temporarily raising the platelet count to normal such as following an injury or prior to surgery; long-term prednisone therapy is not advised, however, because of side-effects. Intravenous IVIG or anti-D may also be useful in temporarily raising the platelet count. Some doctors give IVIG or anti-D regularly (once every 3 to 6 weeks) to keep the platelet count up in the range where bleeding does not occur. However, this treatment is inconvenient and costly.

Most children with chronic ITP eventually recover or learn to live with their somewhat low platelet count. Many develop an increased or even normal platelet count by 12 to 24 months after diagnosis. Those children whose platelet counts remain quite low (under 20,000-30,000 per mm<sup>3</sup>) and who have significant bleeding may be considered for splenectomy. We do recommend, however, that all children with ITP, even when they are well and have previously achieved a normal platelet count, have another blood count six months following diagnosis just to “be sure”.

**Splenectomy:** When the platelet counts are low and bleeding signs significant enough to interfere with the child’s lifestyle, splenectomy is often recommended. Splenectomy is surgical removal of the spleen. This usually (but not always) causes a rise in the platelet count to levels where bleeding does not occur and thus appears to cure the chronic ITP. The disadvantage of splenectomy is that it results in a slightly increased risk of blood poisoning, or septicemia, which is potentially fatal. In order to avoid septicemia following splenectomy, one or more special vaccines (shots or immunizations) are given before the procedure, and children are asked to take penicillin twice daily afterwards for 3 years or more in order to prevent these serious infections.

**Other Treatments:**

An entirely new treatment approach being explored is the use of drugs that stimulate the production of platelets by the bone marrow. The few agents that are furthest along in clinical trials are AMG 531, which is given weekly by injection under the skin, and eltrombopag, which is taken by mouth once daily. Preliminary studies in adults with ITP show that these drugs usually raise the platelet count without troublesome side effects. However, these agents are not yet commercially available nor have studies yet been performed in children. On occasion other treatments using so-called “immunosuppressive” drugs such as Rituximab are used in children whose ITP causes serious bleeding despite splenectomy or when splenectomy cannot be performed. Their long-term beneficial and potentially harmful effects are unclear at present.

**Conclusion.**

Fortunately, ITP during childhood is usually more of a nuisance than a truly serious condition. However, it can be troublesome to the child, family, and primary physician. More research into its cause and treatment is definitely needed. Doctors at Children's Medical Center Dallas and UT Southwestern are conducting much of the necessary research. If there are any questions about the information on presented here, please do not hesitate to contact a staff member of the [Center for Cancer and Blood Disorders](#) at Children’s Medical Center Dallas, or at (214) 456-2382.