PDSA
PLATELET DISORDER
SUPPORT ASSOCIATION

ITP in Adults

FREQUENTLY ASKED QUESTIONS

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Q **I've just been diagnosed with ITP. What is that?**

A ITP, immune (idiopathic) thrombocytopenia, is an autoimmune disease. In autoimmune diseases, the body mounts an immune attack toward one or more seemingly normal organ systems. In ITP, platelets are the target. They are marked as foreign by the immune system and eliminated in the spleen and sometimes, the liver. In addition to increased platelet destruction, some people with ITP also have impaired platelet production.

Q **What are platelets?**

A Platelets are relatively small, irregularly shaped components of our blood. They are required to maintain the integrity of our blood vessel walls and for blood to clot. Without a sufficient number of platelets, a person with ITP is subject to spontaneous bleeding or bruising.

Q **What is a normal platelet count?**

A Normal platelet counts range from 150,000 to 400,000 per microliter of blood. People with platelet counts below 100,000 may have ITP. People with platelet counts under 10,000 have a severe case of ITP. For many, a count of 30,000 is sufficient to prevent a catastrophic bleed. Individual reactions to low platelet counts differ. Determining a safe platelet count is a decision to be made in consultation with an experienced treating physician and is based on many factors.

Q What causes ITP?

A The specific cause of ITP is usually unknown. Some cases appear after a viral or bacterial infection, after immunizations, after exposure to a toxin, or in association with another illness such as lupus or HIV. It is important to recall what was happening in your life before you began having symptoms of low platelets. This information may be useful to your physician in diagnosing and treating your low platelet count.

"I woke up the morning of the 4th of June with blisters in my mouth and red spots on my skin. I also had bruises on my legs and arms. My family doctor acted quickly, and the blood tests confirmed what he thought. I had ITP."
— Greg

Q Is my blood type a factor?

A None of the blood types have been linked to ITP. Being Rh+, however, allows treatment with IV anti-D (i.e., WinRho SDF® or Rhophylac®).

Q Can you inherit ITP?

A ITP is not usually considered a disease that can be passed from one generation to another. There are cases in which multiple family members have been diagnosed with ITP, but most researchers consider these a misdiagnosis.

Q What about underlying diseases?

A Some people may have secondary ITP, meaning that their ITP is a result of some other condition. Secondary ITP can be caused by inherited immune disorders, systemic autoimmunity (the body attacks other cells as well as platelets), ongoing infections (like HIV, Hepatis C and the stomach bacteria, *H. pylori*) and lymphoproliferative disorders (immune cells multiply without stopping, causing an overactive immune system).

Q What are the symptoms of ITP?

A The symptoms vary greatly from person to person. Most people with ITP experience spontaneous bruising. Some find they have petechiae (pe-TEEK-ee-ay), tiny red dots on the skin caused by broken blood vessels or leaks in a capillary wall. If your platelet count is very low you may have other bleeding symptoms including blood blisters on the inside of your cheeks or blood in your urine or stool. In general, the more bleeding symptoms you have, the lower your platelet count.

Q How is ITP diagnosed?

A ITP is a diagnosis of elimination. Your doctor will do tests that rule out other causes of low platelets. If no other cause is found, then the diagnosis is often ITP. There is no accurate, definitive test for ITP. ITP-specific treatment such as IVIg will confirm ITP if there is a good response.

Q What is a "bone marrow aspiration" test and why is it done?

A Platelets are produced in your bone marrow. This test is done to confirm that the platelet production process is working properly. The test is typically done at the hip bone. First a shot of novocaine or other numbing agent is given. Then a needle is pushed through the bone and into the marrow. Some of the marrow is then suctioned out and examined. While some people experience little or no pain, others find this test painful. It, however, provides unique information as to how well the patient can make platelets and if there are other problems in the marrow preventing platelet production.

Q Can ITP be cured?

A While there is no cure for ITP, many patients find their platelet count improves following treatment. What proves difficult for many ITP patients is finding the treatment that works for them without unwanted side effects. Some patients report that changing their diet or lifestyle helps them feel better. The disease can go into remission for a long time, perhaps for the remainder of a person's life. ITP can also recur. There is currently no way to predict the course of the disease.

Q Is ITP contagious? Can it be spread to family and others?

A No, ITP is not a contagious disease and it cannot be spread to others like a cold.

Q What treatments are available?

A There are many treatments for ITP. They all have different risks and benefits and some are very toxic. It is important to understand both the success rate and potential side effects before beginning a treatment.

Hematologists may use several treatments at once to increase their success rate.

Treatments include (in alphabetical order) anti-D (WinRho SDF®, Rhophylac®), azathioprine (Imuran®), corticosteroids (e.g., dexamethasone, methylprednisolone, prednisone), cyclophosphamide (Cytoxan®), cyclosporine (Sandimmune®), danazol (Danocrine®), gamma globulin (e.g., IVIg), mycophenolate mofetil (Cellcept®), rituximab (Rituxan®), splenectomy, and vinca alkaloids (e.g., vincristine). Other treatments are in clinical trials.

Some patients report success with complementary therapies such as vitamins, supplements, diet changes, herbs and energy work, such as Reiki. There are no controlled trials demonstrating utility or safety with any of these agents.

Q **Are there treatment guidelines?**

A ITP treatments vary with the severity of the disease, age of the patient, the experience of the hematologist and other factors. Both the American Society for Hematology and the British Society for Haematology have published guidelines for treating ITP. A new International Consensus Report on the Investigation and Management of Primary Immune Thrombocytopenia was published in January 2010 in the publication *Blood*. A pdf copy of the new treatment guidelines can be viewed at *http://bloodjournal.hematologylibrary.org/cgi/content/full/115/2/168*

Q **Is there one treatment that is usually recommended?**

A An initial course of prednisone is often given to newly diagnosed patients. Prednisone suppresses the immune system. It is hoped that suppressing the immune system will cause the patient's platelet count to increase and remain elevated after the patient stops taking prednisone. Sometimes a short course of dexamethasone, another corticosteroid, is used instead of prednisone.

Q **What are the side effects of these treatments?**

A Side effects have been reported for each of the drugs used to treat ITP. However, side effects will vary from one person to another. You may experience all, some,

or no side effects at all. Descriptions and side effects for frequently used treatments are described below.

Prednisone — Prednisone is a synthetic medicine (i.e., steroid) similar to cortisone, a natural substance produced in the body's adrenal glands. It is used in the treatment of ITP because it has been shown to increase the platelet count while it is being taken.
Possible side effects: Prednisone is generally only given for a few weeks at a time because it can have serious side effects with long-term use. And even when it is given for a short time, you may become more irritable, have stomach upsets, sleep disturbances, increased appetite, weight gain, puffy cheeks, frequent urination, sugar in the urine, loss of bone density, or acne. When the medicine is stopped, most side effects will begin to disappear.

"I have had ITP for five years but the last two years have been the worst. The first three were okay, but for the past two years I have not been able to get my count above 23,000. I am struggling physically and, I must admit, emotionally too."
— Debra

Intravenous gamma globulin (IVIg) — IVIg is a liquid concentrate of antibodies purified from the plasma (the liquid portion of the blood that doesn't contain red blood cells) of healthy blood donors. IVIg is believed to work by overwhelming the spleen with antibody so that it cannot recognize the antibody-coated platelets. IVIg treatment will usually result in a rapid (24 to 48 hours) increase in the platelet count, but any improvement is generally short-lived. Treatment may be repeated until the platelet count improves. IVIg is deliverd by an intravenous infusion directly into a vein in the arm for several hours a day over a period of 1 to 5 days.
Possible side effects: Some patients treated with IVIg experience nausea and vomiting, headaches or fever and rarely, aseptic meningitis, abnormal blood clots or kidney failure.

Anti-Rho(D) immune globulin (WinRho SDF®, Rhophylac®) — Anti-D is also a liquid concentrate of antibodies derived from healthy human plasma. However, this medicine is targeted against the Rh factor* on red blood cells. It is thought that anti-D binds to red blood cells to such an extent that the spleen is fully occupied eliminating red blood cells and does not have much opportunity to remove the antibody-coated platelets. Like IVIg, the response is usually rapid but temporary. If a hematologist

* Most people have Rh-positive blood. This means they produce the Rh factor, an inherited protein found on the surface of red blood cells. A small percentage of people lack the Rh factor. They are considered Rh-negative.

recommends treating you with anti-D, it will be given by intravenous infusion. The procedure takes less than a half hour and can be done during an outpatient visit. Anti-D will generally not work if you are Rh-negative or have had a splenectomy (removal of the spleen). **Possible side effects:** Temporary side effects from anti-D include fever, headache, chills, nausea and vomiting, anemia, and rarely, kidney failure.

"My platelets have stayed in the mid to high 30,000s since February. Even though that's low, it's safe and I haven't had to have any medical treatments other than blood tests."
— ROSELLYN

Monoclonal antibodies — Rituximab (Rituxan®) is a monoclonal antibody approved by the FDA in November 1997 for treatment of lymphoma, a type of cancer. It is increasingly being used to treat ITP. It reduces the number of B cells, a type of white blood cell, in your body as well as changing the character of T-cells (another type of white blood cell). The B cells eliminated are not specific B cells that target cancer or ITP. Rituximab reduces the general population of all B cells with a specific receptor called CD20. After rituximab treatment, the body can take up to a year to replace the eliminated B cells and have the immune system and antibody production back in full working order. Rituximab is given by intravenous (IV) administration. Hypersensitivity reactions do occur in some patients. The manufacturer recommends premedication with acetaminophen (Tylenol®) and diphenhydramine (Benadryl®) before each infusion and prednisone is also helpful.

Possible side effects: Side effects that developed following 7% of infusions included headaches, chills, fever, and body aches. For patients with hypersensitivity to blood products there is a remote risk of anaphylaxis (shock response). If any patients experience back pain, chills, fever, changes in urine output, sudden weight gain, fluid retention/edema, or shortness of breath they should report these symptoms to their doctor immediately. A very small number of patients may experience severe anemia, which requires immediate medical attention. For additional information on rituximab for treatment of ITP, visit the PDSA Web site, *www.pdsa.org* .

Platelet growth factors — Platelet growth factors or thrombopoietin (TPO) receptor agonists are a new class of treatments for ITP that stimulate the bone marrow to produce more platelets. TPO, a protein made in the liver, naturally stimulates platelet production in the bone marrow. TPO receptor agonists bind to the

same receptor as the TPO produced in the body, which prompts the megakaryocytes in the bone marrow to produce more platelets. While ITP is often considered a disease characterized by platelet destruction, recent research has shown that many people with ITP also have low platelet production. The additional bone marrow stimulation prompted by the TPO receptor agonists creates a sufficient number of platelets to overcome the platelet destruction or platelet production problems in most people who receive the treatments. In 2008 two different platelet growth factors, romiplostim (Nplate®) and eltrombopag (Promacta™), received FDA approval for treatment of chronic ITP in teens 18 and over and adults.

The FDA has mandated that both of these new treatments only be available through a risk-assessment program. There is ongoing research in the use of these treatments for children younger than 18. The most common adverse reactions are joint and muscle pain, dizziness, insomnia, indigestion, and 'pins and needles' sensations. Potential exists for patients to develop reticulum (fibrous growths) in the bone marrow and also for the platelet count to drop below the pre-treatment count if the treatment is discontinued.

Romiplostim (Nplate®) is a manufactured peptibody (part peptide and part antibody) liquid that is given by subcutaneous injection (under the skin) initially once a week.

Eltrombopag (Promacta™) is a small molecule (pill) taken once daily. Pills must be taken on an empty stomach as food, especially calcium-containing (e.g., milk, yogurt) affects its absorption.

For additional information on all treatments for ITP, visit the PDSA Web site, *www.pdsa.org* .

Q **Do alternative treatments work?**

A Some people report success with herbs, supplements, energy work, diet changes and other alternative treatments. There are many reported cases of their success but few formal studies. Many of the alternative treatments attempt to correct the underlying problem rather than treat the symptoms of the disease. They tend to take a longer time to be effective and have fewer unwanted side effects. Like the more traditional

treatments, the alternative treatments do not have the same results in all who try them.

Q In addition to a low platelet count, I'm tired. Is this a normal part of the disease?

A Fatigue is a common experience for people with ITP. It may be caused by the disease or it could be a response to your medications. Some patients report that dietary changes increase their energy level and reduce fatigue.

Q Is depression a normal part of the disease?

A Many people with ITP report being depressed. There are several possible explanations. One factor might be serotonin, a neurotransmitter that is carried by platelets and delivered to the brain and other parts of the body. Since serotonin helps regulate mood, anything that interferes with serotonin processing could contribute to depression. Another factor is simply that you are dealing with a difficult and potentially chronic illness. This can lead to feelings of isolation, fear, and anger that your body has "turned against you." A third factor is the treatments. Many of them list depression as a potential side effect.

Q Are aches and pains a normal part of the disease?

A Some patients report these symptoms.

Q Will changing my diet or lifestyle make a difference?

A Some patients report that eating a healthier diet, exercising, meditating, avoiding toxins, etc. have a positive effect on their platelet count and how they feel. It is also important to avoid substances such as alcohol that can harm the bone marrow or substances such as aspirin that interfere with platelet function.

Q Can I still do the things I love?

A This is an individual decision based on your platelet count, your symptoms, your current lifestyle and the amount of risk with which you are comfortable. Your doctor can provide guidelines for you. Some people with ITP use the opportunity to enjoy new activities that do not place them at risk of bleeding.

Q Will I die from ITP?

A A very small percentage of people with ITP die from the disease or the treatments. The large majority of people find treatments that raise their platelet counts or successfully live with a low platelet count.

Q Am I eligible to go on disability?

A This varies. Some people with ITP have been successful in getting assistance. Others have found it a challenge. The severity of this disease and the impact on lifestyle varies greatly. Your success in getting disability assistance depends on how your case is presented and on the specific difficulties you are experiencing as a result of the ITP. Contact the Social Security Administration for more information.

"I am one of the lucky ones who, after a five-year struggle (including a brain bleed), have been in remission for two years."
— BARBARA

Q What if I have ITP and want to have a baby?

A Many women with ITP deliver healthy babies, although this is not without some risk. Your decision to have a child and your treatments during pregnancy depend on your count, symptoms, and overall health and should be discussed with your doctor and obstetrician.

Q Can I give ITP to my children?

A If you are a woman with ITP and you become pregnant, some of the anti-platelet antibodies may cross the placenta and your baby may temporarily develop low counts. If this happens, the baby may be treated to prevent bleeding. When the baby's own immune system matures, the platelet count improves.

Q What can family and friends do to help?

A Most people are quite shocked to get this diagnosis. They probably have not heard of the disease before being diagnosed and have no idea what to expect. First reactions may include fear, confusion and stress. It's difficult to assimilate all the new terms and understand the treatment options in a crisis situation. You can ask your family and friends to be extra patient. You can explain that you have so much on your mind. You are learning as fast as possible and dealing with the side effects of some very potent drugs. You can explain that when counts are low, you may feel pretty awful, tired and often sad. Although

you may look just fine, your body is waging an incredible war on the inside and this is exhausting work.

Q **What else should I as the patient do or know?**

A You should learn as much as you can about the disease. Do your homework, learn the benefits and the side effects of the recommended medications, decide how you want to approach the disease and your life, now that it has changed. Keep a copy of every lab report and copies of all blood work. Maintain a log of the medications used, dosages, your platelet count, and how they made you feel. Pay attention to your lifestyle and see if there is any correlation between your platelet count and the food you eat, the places you visit, noxious chemicals in your environment, etc. Often you are the person paying the most attention to these things. Truly, it is up to you to learn and help heal yourself.

"I'm looking forward to completely recovering and getting off the meds."
— WAYNE

Q **Where can I get more information?**

A The Platelet Disorder Support Association (PDSA) has more information on all of the topics in this pamphlet. There are hundreds of pages of information on the PDSA Web site, *www.pdsa.org* . The organization publishes a monthly e-news update, a quarterly newsletter and makes available other publications and articles. Each year, PDSA holds an annual conference and regional meetings. PDSA continues to expand their programs to offer more services and reach more people.

Depending on your circumstances, one of our other booklets may also be helpful:

ITP in Teens — Frequently Asked Questions
ITP in Children — Frequently Asked Questions
ITP and Pregnancy — Frequently Asked Questions
Coping with ITP
PTI en la adultez — Preguntas frecuentes
PTI infantil — Preguntas frecuentes
The Role and Function of Platelets in ITP
Parents Resource Packet
Health Insurance & Assistance Programs for ITP Patients
Living with ITP: Answers to Common Questions

For more information about ITP, additional copies of this booklet, or to become a member of PDSA, please contact us:

Platelet Disorder Support Association
133 Rollins Avenue, Suite 5
Rockville, MD 20852

tel 1-87-PLATELET (1-877-528-3538)
fax 301-770-6638

pdsa@pdsa.org
www.pdsa.org

The Platelet Disorder Support Association is dedicated to enhancing the lives of people with ITP and other platelet disorders through education, advocacy, and research.

Membership benefits include a newsletter, discounts to the ITP Annual Conference, optional participation in the Name Exchange Program, and the good feeling of helping others.

PDSA is a 501(c)3 organization. All contributions are tax deductible.

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Notes

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133 Rollins Avenue, Suite 5
Rockville, MD 20852

tel 1-87-PLATELET (1-877-528-3538)
fax 301-770-6638

pdsa@pdsa.org
www.pdsa.org