PDSA
PLATELET DISORDER SUPPORT ASSOCIATION
for People with ITP

ITP in Children

FREQUENTLY ASKED QUESTIONS

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Q **What is ITP?**

A ITP, primary immune thrombocytopenia (also called immune (idiopathic) thrombocytopenic purpura), 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 **Will my child recover?**

A ITP can either be acute (sudden onset, often temporary) or chronic (long lasting). Most children diagnosed with ITP (between 80 and 90 percent) have acute ITP. These children usually recover within a few months whether they receive treatment or not. Recovery is possible even if your child is considered to have chronic ITP.

Q **Why are platelets so important?**

A Platelets are small, sticky components of the blood formed in the bone marrow (the soft, porous tissue found in the long bones of the body). Their job is to maintain the integrity of the blood vessels and seal small cuts and wounds by forming a blood clot. If the blood doesn't have enough platelets, it is unable to clot successfully. The result is excessive bruising and the tendency for people with ITP to bleed for a long time when cut. It is possible, with a very low platelet count, to have spontaneous bleeding, including a cerebral hemorrhage.

Q **Why doesn't my child have enough platelets?**

A The specific cause of ITP is unknown. Some cases of ITP appear after a viral or bacterial infection, immunization, exposure to a toxin, or in association with another illness.

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 under 10,000 are more prone to bleed. The 2010 International Consensus Report defined ITP as an immune disorder characterized by isolated thrombocytopenia with a blood platelet count of less than 100,000 per microliter of blood. The report can be seen at http://bloodjournal.hematologylibrary.org/content/115/2/168.full. 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.

"My granddaughter was diagnosed with ITP when she was less than a year old. It lasted several months, and her count decreased at one point to 5,000. She has been fine ever since. She is now over 4."

— Don

Q **How did my child get ITP?**

A Doctors don't know what causes ITP, but it often occurs in otherwise healthy children a few days or weeks after a viral infection. It's thought that for some unknown reason this infection causes the immune system to lose the ability to distinguish between the body's own cells and those of invaders. There is also some evidence that certain vaccines may trigger ITP, but this occurs in only a small percentage of cases. Why ITP happens in some children and not others who have had the same infection, virus or vaccine is not known.

Q **What are the symptoms of ITP?**

A People with ITP often have bruises or small purple spots on their skin (petechiae) where their blood has escaped from their veins or capillaries. Spontaneous bleeding can also occur in the mucus membranes on the inside of the mouth or in the gastrointestinal tract. It is possible, with a decreased number of platelets, to have a spontaneous cerebral hemorrhage.

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. Commonly doctors will test for the presence of anti-platelet antibodies, perform tests for other diseases such as lupus and do a bone marrow aspiration.

Q **I've heard my child may have to have a bone marrow test. What does this entail?**

A In some cases a pediatric hematologist (blood specialist) will ask to take a sample of bone marrow from the pelvic bone in your child's back (bone marrow test). This is

performed to ensure that the bone marrow is still making platelets and that there is nothing wrong with other blood cells in the marrow. However, most doctors agree that bone marrow examinations are unnecessary in children with ITP unless a different diagnosis is suspected.

Q **Is ITP dangerous to my child?**

A It can be, but the danger is primarily related to your child's platelet count. For example, a platelet count of less than 50,000 may cause your child to bleed or bruise easily. A platelet count lower than 10,000 will increase the risk of serious bleeding. However, life-threatening bleeding, including intracranial hemorrhage (bleeding in the brain) is rare, and occurs in less than 1 percent of children with ITP.

It's important to note that if your child's platelet count is very low your child should not be given aspirin or any medicine containing aspirin (acetylsalicylic acid). Nor should your child be given anti-inflammatory medicines such as ibuprofen or naproxen, or any medicine containing glycerol guaiacolate (Robitussin), as these medicines can prevent the few platelets your child has from working properly.

As a precaution, check with your doctor before giving your child any medicine other than acetaminophen (Tylenol®).

"My daughter Sarah was diagnosed with ITP when she was 2 1/2 years old. I had noticed that she was bruising easily but thought nothing of it because she has two older sisters and goes to day care while I work. "

— STACIE

Q **When is immediate medical attention needed?**

A Contact your doctor **immediately** if your child hits his head or has a serious accident. Also, be on the lookout for lots of bruises or petechiae, as these indicate your child's platelet count is low. The doctor will want to be informed if your child has nosebleeds, bleeding gums, blood in the urine or stools, blood in vomit or during coughing, repeated vomiting, or any other unusual behavior or illness.

Q **Is ITP contagious?**

A No. ITP is not contagious.

Q **What are the treatments and their side effects?**

A Because ITP in children generally resolves on its own, your hematologist may not recommend any treatment for your child other than a weekly or biweekly blood test to monitor the platelet level. As soon as the platelet count increases, the interval between blood tests may be lengthened; but your child will still be monitored until the platelet level returns to normal and is stable. If treatment is recommended, the following medicines may be used to keep the platelet count within a safe range until your child's body recovers:

"Our Daughter, Ayla, was diagnosed with ITP when she was three. She is now 5 years old. Her journey started with a 7-day hospital stay due to bleeding she was having in her urine that they could not stop. During the next 10 months we did experience short stints of a raise in her platelets, but most of that time the levels were under 5,000."

— Jay

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 many children 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, these side effects will begin to disappear.

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 your child's platelet count, but any improvement is generally short-lived. Treatment may be repeated until the platelet count improves permanently.

If IVIg is the recommended treatment for your child, it will be given directly into a vein in the arm (intravenous infusion) for several hours a day over a period of 1 to 5 days. The IVIg experience can be improved if the treatment is given more slowly, at room temperature, and the child receives pretreatment with diphenhydramine (Benadryl®) and acetaminophen (Tylenol®).

Possible side effects: Some children 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®). WinRho 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 WinRho 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 recommends treating your child with WinRho, it will be given by intravenous infusion. The procedure takes less than a half hour and can be done

* 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.

during an outpatient visit. WinRho will generally **not** work in children who are Rh-negative or who have had a splenectomy (removal of the spleen).

Possible side effects: Temporary side effects from WinRho include fever, headache, chills, nausea and vomiting and anemia, and rarely, kidney failure. A very small number of people receiving anti-D experience intravascular hemolysis, the destruction of red blood cells in circulation, which can cause anemia, multi-system organ failure, difficulty breathing, and even death. December 2009, the FDA revised the WinRho SDF package insert to highlight these warnings and suggest additional tests and patient monitoring to be better able to identify and treat those patients at risk.

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 77% 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 mega-

"When I explain ITP to other kids at school, I ask if they know what a platelet is. If they don't know, I say it is like a bandage in your body that covers up scrapes and bruises. If they do know, I say that I do not have a lot of platelets."

— EMILY

karyocytes 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. There is ongoing research in the use of these treatments for children younger than 18.

"My 1-year-old son has been diagnosed with ITP. We first found out about his ITP 2 months ago, and since then we have been struggling with the health system in order to get his IVIg treatment."

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 pretreatment 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**.

Other treatments. Your doctor may suggest other treatments for your child. You can contact our organization or Web site (**www.pdsa.org**) for information about these.

Please note that side effects from all treatments will vary from one child to another. Your child may experience all, some, or no side effects at all.

Q **What about alternative/complementary treatments?**

A Although few scientific studies have been conducted to assess how effective alternative and complementary therapies are in the treatment of ITP, some patients report success with herbal therapies, vitamin C, antioxidants and mind/body interventions. Be sure to your let your child's doctor know if you are considering alternative and complementary treatments.

Q **Can my child's ITP return?**

A A small number of children with acute ITP who appear to have recovered will later have a recurrence of ITP. A recurrence of ITP may indicate that chronic ITP is

developing and should be carefully monitored. Consult your doctor for more information.

Q **What if my child doesn't get better?**

A If your child's ITP persists beyond 6 months from the time of diagnosis, your child will be classified as having chronic ITP. The treatment, as with acute ITP, will focus on minimizing the risk of bleeding. A small percentage of children with chronic ITP, who are older than 5 years, and who suffer from persistent bleeding, may be assessed for the risks/benefits of a splenectomy.

Q **Will my child get other illnesses because of ITP?**

A If your child is otherwise healthy she should be no more susceptible to contracting illnesses or viruses than children who do not have ITP. However, if your child is being treated with steroids (prednisone) or a drug that suppresses the immune system, or has had a splenectomy, your child's ability to fight infections will be lowered.

"My 6-year-old son has chronic ITP, and I'm a very scared mother. I ask myself why. Please help me to understand this. "

— CARMEN

Q **If I have more children, will they get ITP?**

A As ITP is not hereditary, it is unlikely to affect other members of your family.

Q **How will my other children feel?**

A Other family members, including children, may feel confused, guilty, angry and jealous.

Confused — because they don't understand what's going on.

Guilty — because they think something they did may have caused their brother or sister to get ITP.

Angry and jealous — because everyone's attention is focused on ITP, and normal family life has been disrupted.

ITP is a family issue and, as such, your other children and family members should be kept as informed as possible about what's going on — although this can be difficult for young children who may find it hard to understand why the family routine has suddenly changed. Many parents of children with ITP find that encouraging children to express their emotions and setting aside some time alone with each of them at least once a week can help.

Q **What should I tell people?**

A What you tell people about your child's ITP will primarily depend on their role in your child's life, and how much your family wants other people to know. Below is a guide that will help you tell other people about ITP.

For acquaintances you may want to say…

"ITP is a blood clotting disorder. She/he bruises and bleeds very easily. But it's not contagious."

For teachers, scout leaders, day-care staff, and coaches you may want to say…

"ITP puts her at risk of injury. If she bleeds, this is how to stop it, and this is how to reach me. If trauma occurs that causes loss of consciousness, call 911 immediately, then contact me."

If your child is on medication, you may want to add…

"The medicine makes her feel hungry/tired/irritable, but the disorder itself doesn't make her feel bad."

Please feel free to distribute this booklet among caregivers, friends and family members. The information it contains will not only increase their understanding of ITP, but minimize their fear and anxiety.

Q **Are their special problems for older or adolescent children who are coping with ITP?**

A Children and adolescents may experience many of the same problems living with chronic illness that adults do (pain, physical symptoms, or medication side effects), but certain problems are more exclusive for adolescents.

Denial — Adolescents, more than other age groups, have a common, non-verbalized (sometimes loudly verbalized) belief about their medical status: "I'm fine!" They don't want to feel sick; they don't want to be sick; and they don't want to be different.

School — Unless a chronic illness is affecting the young person to such a degree that he/she is literally unable to function, in many cases the young person's attitude will continue to be, "I'm fine." This can be frustrating for other family members, who are trying to be protective and helpful, and for healthcare professionals, who have more difficulty treating a young person if the answers to questions about symptoms are evasive or denying.

The adolescent's primary "job" is to go to school. For adolescents with chronic illness, the attitude about school varies with some who are upset and frustrated if their illness interferes with consistent school attendance and schoolwork performance. Others see school as less important and may have few or no qualms about missing excessive time in school.

Peer Pressure — Adolescents with chronic illness may have a more difficult time in school if they have to deal with hostility or criticism from classmates or

friends ("Why can't you go out with us tonight?") or the ignorance of teachers ("You've missed too much school work; either get with the program or you're going to fail."). Because of chronic illness, adolescents may feel ostracized and even excluded from activities that were once within their physical capabilities.

Peer pressure may affect young people with chronic illness. The need to fit in — at its strongest during adolescence — can be devastating to someone with a chronic illness with noticeable physical effects (e.g., rashes, bloating, bleeding, hair loss, disfigurement, etc.) and behavioral effects (slower, more painful movements, tired, etc.). It is heartwarming to hear stories of young people with chronic illness whose friendships continue despite their illness. Yet it is sad to hear of stories in which the adolescent with chronic illness is ridiculed and even abandoned by former friends.

Parents — Adolescents with chronic illness may have parents who are overprotective ("Stay inside, the sun is out."), not protective enough ("You want to go to the beach? Do whatever you want."), or insensitive to their needs ("Stop complaining about your pain already. Get up and finish your school work."). Any of these parental reactions may be difficult for adolescents to understand.

Parents may be concerned about the effects their child's chronic illness will have on the family, such as financial issues, problems with or neglect of other children, or even feeling as though their independence is being inhibited. These concerns can likewise affect the young person with chronic illness.

Already unhappy because of having chronic illness, but feeling responsible for problems within the family or with the parents, an adolescent may feel guilt to the extent that it interferes with physical — and emotional — health. For example, the young person may not tell parents about a serious physical symptom, knowing that it might mean another trip to the doctor or even the possibility of hospitalization.

Siblings — Brothers and sisters of an adolescent with chronic illness may be very resentful. Being less able to understand the physical impact of the disease, they may dislike the added "attention" being directed at their sick sibling. They also may not like getting less attention and may act out in an attempt to regain their "share" of parental interactions. Resentment toward their sick sibling may be manifested in hurtful ways, such as anger, ignoring instructions, spiteful behavior, or concealing important information from their parents.

Q What are ways to help an adolescent deal with having a chronic illness like ITP?

A Here are some guidelines offered by *Robert H. Phillips, Ph.D., Founder & Director, Center for Coping, Long Island, NY:*

For adolescent children coping with ITP, here are some helpful suggestions...

It is difficult enough for anyone to live with chronic illness; but the adolescent with a chronic illness has added, age-related problems. Increased awareness of the potential impact of chronic illness can pave the way to a better understanding of the unique needs of adolescents, and it can lead to methods for better alleviating the problems that may occur.

- **Be sensitive to the adolescent's unique needs.** Chronic illness can be difficult to live with, especially for an adolescent who has less "life experience" and consequential coping strategies in place. Don't assume that the young person has the emotional strength or the social support network to handle chronic illness-related problems successfully.
- **Communicate appropriately.** Try to view any chronic illness-related issues through the eyes of the adolescent. See what the young person sees. Feel what he/she feels. Issuing commands or using anger and aggressiveness in forcing issues is rarely productive. Calm, constructive discussion is a much more positive way to address chronic illness-related issues.
- **Try to treat the adolescent as an adult.** Plan together the appropriate ways to treat, and live with, chronic illness. The more you treat the adolescent like an adult, the more likely it is that this will generate adult-like behavior in return.
- **Educate significant others.** Any individuals who are not familiar with living with medical problems, including family members, friends, and teachers, can be obstacles to successful living with chronic illness. This is especially important in school since the adolescent is going to spend a good number of hours there each day. Provide pamphlets and other information to teachers, guidance counselors, and even classmates, so that school can truly be a "home away from home."

Q In which sports and activities can my child participate?

A Having ITP shouldn't stop your child from having fun, but if the platelet count is low certain activities will need to be restricted in order to lower the risk of bleeding.

Your child's doctor will help you assess which sports and activities are safe for your child.

Remember to make sure your child wears the recommended safety equipment such as helmets, kneepads, elbow pads and wrist pads appropriate to the sport or activity.

Q What will happen when my daughter starts menstruating?

A Young girls with ITP may experience heavy bleeding (menorrhagia) and prolonged menstruation with their first or all of their periods. If this becomes a problem, oral contraceptives can be used to decrease the severity of menstruation. In cases where oral contraceptives fail to control bleeding, Depo-Provera (a form of progesterone that inhibits ovulation) can be given via injection every 3 months or so to completely stop menstruation until the ITP has resolved, or at least been controlled. In some cases tranexamic acid, a non-steroidal medication (such as Lysteda®) that be given. It helps prevent clots from breaking down.

Q Where can I meet other parents of children with ITP?

A The Platelet Disorder Support Association (PDSA) offers several ways for parents to meet other families of children with ITP. These include a discussion group, a name exchange program, an annual conference, and regional meetings during the year. PDSA offers the ITP Parents Teleconference Local Support Group, which meets by teleconference every 2 to 3 months. For details see http://www.pdsa.org/join-the-community/local-groups/item/313.html.

Q What else can I do to help my child?

A Try to find other children with ITP or join a family support group in your area.

Learn as much about ITP as possible, and be there to listen when your child is not feeling well.

Keep your child active. Just because they can't play football doesn't mean they can't play tennis.

Use "maybe" instead of "no" if you feel an activity or outing is uncertain.

Purchase a medical alert bracelet for the child to wear.

Maintain academic expectations (if your child is of school age) and continue household responsibilities because that is life, too.

Concentrate on what they can do, not what they can't.

"Lily is almost 3 years old. She doesn't understand ITP and the effects it may have for her future. She loves wearing her helmet but doesn't know why it is an integral part of her daily wardrobe."

— LEAH

For younger kids, provide soft surfaces for them to play on and choose exciting activities that don't involve rough and tumble.

For older kids, encourage them to be involved when visiting the doctor and discussing treatment options.

Discovering your child has ITP can be a frightening experience for any family. But remember, most children get better within six months or less and serious consequences are extremely rare.

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

ITP in Teens — Frequently Asked Questions
ITP in Adults — Frequently Asked Questions
ITP and the Female Lifecycle: Bleeding Issues in the Stages of a Woman's Life
PTI infantile – preguntas frequentes
PTI in la adultez – preguntas frecuentes
PTI chez l'enfant – Questions Fréquemment Posées
PTI chez l'adult – Questions Fréquemment Posées
Coping with ITP – Frequently Asked Questions The Role and Function of Platelets in ITP Parents Resource Packet
Health Insurance and 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 Ave., Suite 5
Potomac, 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.

PDSA would like to thank **Amgen** for their assistance in printing this booklet.

The information in this guide is for educational purposes only. For your child's unique medical condition, please consult a doctor.

PDSA

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SUPPORT ASSOCIATION

for People with ITP

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Potomac, MD 20852

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