

# Immune Thrombocytopenic Purpura (ITP) Fact Sheet



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## About Immune Thrombocytopenic Purpura (ITP)

In adults, immune (or idiopathic) thrombocytopenic purpura (ITP) is a rare, serious and often chronic autoimmune disorder characterized by low platelet counts in the blood (a condition known as thrombocytopenia).

Platelets, also called thrombocytes, are specialized blood cells needed to prevent bleeding. Low platelet counts leave adult patients with ITP at risk for bleeding events. The risk of a serious bleeding event increases when platelet counts drop to less than 30,000 platelets per microliter of blood. In extreme cases, death can occur due to an intracerebral hemorrhage (bleeding into the brain).<sup>1</sup>

ITP occurs when immune system cells (specialized lymphocytes) produce antibodies that cause the destruction of platelets in the spleen and other organs. As such, ITP has historically been considered a disease of platelet destruction. However, recent data also suggest that low platelet counts in the blood may be caused by the inability of the body's natural processes to produce platelets. Therefore, increasing the rate of platelet production may help address low platelet counts associated with ITP.<sup>2</sup>

## Causes

The specific cause of ITP is unknown. In some cases, ITP has appeared following a viral or bacterial infection, immunizations, exposure to a toxin, or in association with another illness such as lupus or the human immunodeficiency virus (HIV). ITP is not contagious.<sup>3</sup>

## Symptoms of ITP

The main symptom of ITP is uncontrolled bleeding, which can produce bruising (purpura) and tiny red dots on the skin (petechiae). In some instances, bleeding from the nose, gums, and digestive or urinary tracts as well as the formation of blisters (such as in the lining of the mouth) may occur. Bleeding within the brain or other organs may occur, but is rare.<sup>4</sup>

## Incidence and Occurrence

There are an estimated 60,000 adult patients with chronic ITP in the United States. ITP affects about two times as many adult women as men.<sup>5</sup> ITP is recognized as an orphan disease by U.S. Food and Drug Administration (FDA). An orphan disease is defined as a condition that affects fewer than 200,000 people nationwide.<sup>6</sup>

## Diagnosis

In most adults, ITP is a chronic condition. The diagnosis of ITP is based principally on the history, physical examination, complete blood count, and examination of the peripheral smear, which should exclude other causes of thrombocytopenia. ITP is often considered a diagnosis of exclusion, as other potential causes must be eliminated before a diagnosis of ITP can be made.<sup>7</sup>

## Traditional Treatment Options

Chronic treatment of thrombocytopenia in adults with ITP is considered an unmet medical need by the U.S. Food And Drug Administration (FDA). Traditional treatments for ITP have included:<sup>8</sup>

### Corticosteroids

- Corticosteroids such as prednisone or dexamethasone work to alter or suppress the immune system to decrease the antibody-mediated platelet destruction.
- Corticosteroids are associated with side effects such as weight gain, increased susceptibility to infections, GI bleeding, hypertension, psychosis, and osteoporosis which may limit long-term use for most patients.
- Approximately 80 percent of adult ITP patients require additional therapy once initial corticosteroids are stopped.

### Intravenous Immunoglobulin (IVIG)

- IVIG is a pooled solution of globulins, which are simple proteins normally present in adult human blood that provide immunity against disease. A protein is made up of several amino acids, which are the microscopic building blocks that make up all cells.
- IVIG is typically used as a short-term treatment to rapidly elevate platelet counts by coating platelets to slow their destruction by the immune system.
- The side effects include allergic reactions and heart palpitations.

### Splenectomy

- A splenectomy is the surgical procedure to remove the spleen.
- The spleen is the primary site where platelets are produced and are targeted for destruction.
- Splenectomy has potential short- and long-term complications such as compromising the immune system and putting patients at risk for serious infections. This procedure may not be appropriate for some ITP patients, and in others, ITP may recur in days or years after the procedure.

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<sup>1</sup> Platelet Disorder Support Association. About ITP Fact Sheet. <http://www.pdsa.org/itp-information/index.html>. Accessed March 5, 2008.

<sup>2</sup> Kuter, D., Bussel, J. et al, Efficacy of Romiplostim in Patients with Chronic Immune Thrombocytopenic Purpura: A Double-Blind Randomised Controlled Trial. *Lancet*, 2008; 371. 395-396.

<sup>3</sup> ITP Foundation. About ITP. <http://www.itpfoundation.org/newinfo/facts.htm>. Accessed March 5, 2008.

<sup>4</sup> ITP Foundation. About ITP. <http://www.itpfoundation.org/newinfo/facts.htm>. Accessed March 5, 2008.

<sup>5</sup> McMillan R. Therapy for Adults with Refractory Chronic Immune Thrombocytopenic Purpura. *Ann Intern Med*. 1997;126:307-314.

<sup>6</sup> U.S. Food and Drug Administration. Definition of Disease Prevalence for Therapies Qualifying Under the Orphan Drug Act. <http://www.fda.gov/orphan/designat/prevalence.html>. Accessed March 5, 2008.

<sup>7</sup> ITP Foundation. About ITP. <http://www.itpfoundation.org/newinfo/facts.htm>. Accessed March 5, 2008.

<sup>8</sup> Platelet Disorder Support Association. ITP Treatments Fact Sheet. <http://www.pdsa.org/itp-treatments/treatment-synopsis.html>. Accessed March 5, 2008.