Diagnosing and Treating Immune Thrombocytopenia

NATIONWIDE CHILDREN'S

When your child needs a hospital, everything matters.℠

Immune Thrombocytopenia (ITP)

Immune thrombocytopenia (ITP) is an autoimmune disorder where the immune system destroys platelets, reducing the body's ability to form blood clots. It is marked by the sudden onset of bruising and petechiae. It is the most common acquired pediatric bleeding disorder, usually affecting previously healthy children between 2 and 5 years of age. Nosebleeds and other mucosal bleeding such as oral bleeding and heavy menstrual bleeding may also occur.

National guidelines currently recommend non-intervention as the standard of care, as ITP is typically a benign illness in children with rare recurrence. Symptoms usually begin to improve within 3 to 10 days. However, ITP can present as a symptom of other disorders especially in adolescents and should always be brought to the attention of a Hematologist.

Diagnosis

Pediatric ITP is diagnosed by physical examination, history as well as blood tests. Recommended tests include:

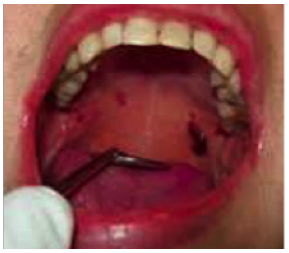
- CBC
- Peripheral blood smear. Affected patients will have few large to normal-sized platelets
- An immature platelet fraction which measures new platelet formation may also be helpful

In two thirds of cases, pediatric ITP is preceded by a viral infection, such as an upper respiratory infection. ITP in children does not present:

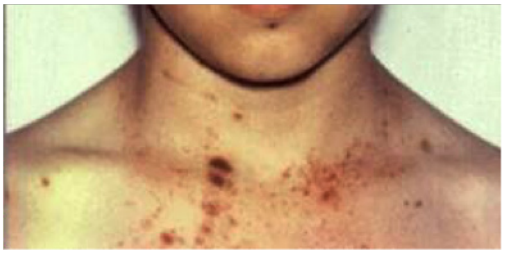
- with an enlarged liver, spleen or lymph nodes
- with retinal hemorrhage
- dysmorphic features such as skeletal anomalies

Thrombocytopenia can occur in isolation or present as part of various autoimmune diseases.

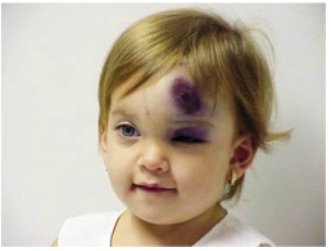
Symptoms



petechiae in the mouth



chest bruising/petechiae



easy bruising

Treating Cases of ITP

Patients with mild symptoms such as bruising and petechiae can be treated with observation alone regardless of the platelet count, and hospital admission is usually unnecessary. Oral bleeding, gastrointestinal tract bleeding, or cases of hematuria are risk factors for more significant bleeding and should prompt discussion of treatment options.

Every pediatric ITP case should be referred for outpatient treatment. It is rare that a patient needs to be admitted to the hospital or emergency department. At Nationwide Children's, physicians and nurse practitioners with unique expertise in this area are equipped to see ITP patients the next day.

Call Nationwide Children's Hospital PCTC at (614) 355-0221 or 877-335-0221 to arrange a next-day follow-up in our Hematology clinic. There is a "doctor of the day" that will be available to see the patient.

When evaluating children and adolescents with suspected acute ITP, it is unnecessary to test for antinuclear antibodies or to perform a bone marrow examination. Bone marrow examination is also unnecessary prior to initiation of treatment with corticosteroids or before a splenectomy in cases of chronic ITP.

Chronic ITP

Chronic ITP is ITP that lasts longer than 12 months and tends to have an insidious onset with no preceding acute illness, vaccination or mucosal bleeding. Patients may present later, in the second decade of life or older. Females are at higher risk for chronic ITP, and patients who are at least 10 years old with an initial platelet count over 20,000 are at very highest risk for chronic ITP.

Self-Care

Most cases of pediatric ITP can be managed with self-care: wearing helmets, padding cribs or toddler beds, eating soft foods to prevent mouth or gum bleeding and avoiding aspirin or other NSAIDs that increase the chance of bleeding. Avoiding contact sports such as football, rugby, wrestling or other high-injury activities can also reduce bleeding risk until the platelet count normalizes.

If the Patient Requires Further Treatment

The goal of every treatment strategy for ITP is to achieve a platelet count that allows for adequate hemostasis rather than a "normal" platelet count. If treatment is required due to bleeding:

First-line Treatment Options	Second-line Therapies
• Oral corticosteroids: prednisone, dexamethasone • Intravenous immunoglobulin (IVIg) • Anti-D immunoglobulin	• Splenectomy • Rituximab • Thrombopoeitin mimetic agents-RAs: romiplostim, eltrombopag • Immunosuppressive agents

Clinical Trials

As leaders in care, clinicians in the Division of Hematology/Oncology/BMT at Nationwide Children's Hospital are participating in clinical trials of Romiplostim for pediatric patients with chronic ITP. Trials include:

- A recently completed phase 3 randomized, double-blind, placebo-controlled study to determine the safety and efficacy of Romiplostim in thrombocytopenic pediatric subjects with chronic ITP
- An extension study evaluating the safety and efficacy of long-term dosing of Romiplostim in thrombocytopenic pediatric subjects with ITP
- A newly opened single-arm open-label long-term efficacy and safety study of Romiplostim in pediatric subjects with ITP

For more information about these and other Hematology, Oncology and Blood and Marrow Transplant clinical trials at Nationwide Children's, visit **NationwideChildrens.org/cancer-clinical-studies**.

Referrals and Consultations Online

NationwideChildrens.org/Hematology-Oncology-BMT

Phone: **(614) 355-1272** | Fax: **(614) 722-3369**

Physician Direct Connect Line for 24-hour urgent physician consultations:

(614) 355-0221 or **(877) 355-0221**.