

FOR PEDIATRIC PATIENTS WITH ITP

| | Medication Name: Type/Trade (Brand) | How It Works: | Route of Administration | Common Side Effects: | Typical Response Time | | | |
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| | | FIRST-LINE | | | | | | |
| col ricostel olds: | Prednisone/Prednisolone Brand: Deltasone, Rayos, Prednicot, predniSONE Intensol, Sterapred, Omnipred, and Sterapred DS. | Reduces destruction of antibody- coated platelets in the blood and the bone marrow, thereby increasing effective platelet production. May also reduce ITP bleeding through a direct effect on the blood vessel. | Oral tablet or liquid solution | Mood swings, anger, anxiety, insomnia and other sleep disturbances, weight gain, Puffy and rounder face, stomach irritation, ulcers, elevated blood pressure, elevated blood sugar, and water retention. | Initial response 5-14 days; many see a response within 2-3 days. | | | |
| 50.00 | Dexamethasone Brand: Decadron, Dexamethasone intensol, Dexasone, Solurex, Baycadron, and Zema Pak. | | | | Initial response 3-14 days, Peak response 4-28 days. | | | |
| | Intravenous immunoglobulin (IVIG) | IVIG is a blood product that reduces the destruction of antibody-coated platelets. | Intravenous (IV) infusion | Headache; flu-like symptoms (flushing, nausea, fever, chills, malaise). Rare: Aseptic meningitis, lower than normal blood pressure, hemolysis (breakdown of red blood cells), kidney failure, thrombosis (blood clots), and anaphylaxis (severe allergy). | 24-48 hours | | | |
| | Anti-Rho (D) or RH (D) IMMUNE GLOBULIN Brand: WinRHo | Anti-D immunoglobulin binds to red blood cells to remove antibody-coated platelets. Only used by patients with a positive blood type. | Intravenous (IV) infusion | Headaches, fever, chills, and a slight decrease in hemoglobin level (usually 1-2 g/dL) can occur. In rare situations, life-threatening hemolysis (breakdown of red blood cells) can occur. Other risks include acute kidney failure and disseminated intravascular coagulation (DIC, a serious bleeding and clotting disorder) with multi-organ failure. | 24-72 hours | | | |
| | Watchful Waiting Strategy Aka: ACTIVE OBSERVATION approach | Individuals who have no bleeding or only mild bleeding and are at a low risk of future bleeding are actively followed with observation. This requires close communication between the individual with ITP and their health care provider. | Not applicable. | Patients might feel anxious about not receiving medication to raise their platelet count. Anxiety can be reduced by close communication with the doctor and discussion about concerns. This treatment is not appropriate for patients with moderate-severe bleeding symptoms, who may be at risk of serious bleeding. | Not applicable | | | |
| | SECOND-LINE | | | | | | | |
| כון ומסכו מכנ: | Monoclonal Antibody Brand: Rituximab (Rituxan) | Attaches to and depletes B lymphocytes (immune cells), including the B cells that produce autoantibodies that attach to platelets in patients with ITP. | Intravenous (IV) infusion | Infusion reactions, especially with the first infusion. Rare: serum sickness, late-onset neutropenia, and infection. See black box warning in treatment table in https://www.pdsa.org/treating-itp.html. | 1-7 weeks | | | |
| . A. O | Eltrombopag Brand: Promacta/Revolade | Binds to the thrombopoietin receptor on megakaryocytes, which stimulates platelet production. | Oral tablet | Eltrombopag: Nausea, increased liver enzymes, headache. Platelet count may drop suddenly if drug stopped abruptly. Thrombosis (blood clots), hepatoxicity (liver injury), and bone marrow reticulin (fibrous scarring) have been reported but are rare. | 1-3 weeks | | | |
| | Romiplostium Brand: (NPlate) | | Subcutaneous injection | NPlate: Headache, joint and muscle pain. Platelet count may drop suddenly if drug stopped abruptly. Thrombosis (blood clots) and bone marrow reticulin (fibrous scarring) have been reported, but are rare. | 1-3 weeks | | | |



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| THIRD-LINE THIRD-LINE | | | | | | | | |
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| Azathioprine Brand: Imuran, Azasan | Reduces the immune response. Used to treat diverse autoimmune disorders and some forms of lymphoma. | Oral tablet | Hepatotoxicity (liver damage) can occur. Rare: neutropenia (low white blood cells) which increases the risk for serious bacterial infections, and hair loss. | 3-6 months | | | | |
| Cyclosporine A Brand: Neoral and Sandimmune | Reduces the immune response. | Oral tablet or Intravenous (IV) injection | Elevated blood pressure, tremor, muscle pain, overgrowth of gum tissue, headache, stomach irritation, neuropathy (nerve pain), and decreased magnesium levels. Rare: Severe hypertension (high blood pressure) and vascular damage causing hemolysis, thrombocytopenia and renal failure. Infections may occur. | 3-4 weeks | | | | |
| Cyclophosphamide Brand: Cytoxan, Neosar | Interferes with the proliferation of cells involved in (auto)immune responses. | Oral tablet or intravenous (IV) infusion | Bone marrow suppression. Mouth sores and ulcers can occur if severe neutropenia (low white blood cell counts) develops. Hemorrhagic cystitis (irritation and bleeding from the bladder), hair loss, nausea and vomiting have also been reported. May cause infertility. Serious infection and bleeding. Secondary malignancy (e.g. bladder cancer and secondary leukemia) have been reported with long-term use. | 2-10 weeks | | | | |
| Danazol Brand: Danocrine | A modified testosterone. Mechanism of action not fully established. May reduce destruction of antibodycoated platelets initially and act as an immunosuppressant with long-term use. | Oral tablet | Weight gain, fluid retention, muscle pain, rash, and hair loss. This drug disrupts the production of estrogen and can have a masculinizing effect on women leading to hirsutism, deeper voice, and menstrual irregularities. In men it can affect sperm production. Liver damage and hypoglycemia (low blood sugar). | 1-6 months | | | | |
| Dapsone (diphenylsulfone) Brand: Avlosulfon | Mechanism of action is not established. May work by inducing "mild" hemolysis and thereby divert splenic macrophages from clearing antibody-coated platelets. | Oral tablet | Mild hemolysis (destruction of red blood cells) causing anemia and itching. Severe hemolytic anemia, methemoglobinemia (an abnormal type of hemoglobin), Stevens-Johnson syndrome (severe skin rash). | 1-3 months | | | | |
| Mycophenolate mofetil therapy (MMF) Brand: Cellcept | Interferes with the proliferation of cells involved in (auto)immune responses. | Oral tablet or liquid suspension | Cytopenias (low platelets, low red and/or white cells), headaches, gastrointestinal, liver damage, high blood pressure, and increased susceptibility to infection. A rare but possible risk of lymphoma/cancer. | 1-3 months | | | | |
| Sirolimus Brand: Rapamune/Rapamycin | Interferes with the function of cells involved in (auto)immune responses. | Oral tablet or liquid suspension | Susceptibility to infection, hypertension (high blood pressure), angioedema (swelling to the lips), peripheral edema (swelling to the arms and legs), impaired wound healing, hyperlipidemia, impaired renal function with proteinuria. There is a 'black box' warning for infections in both liver and lung transplant patients. Possible increased risk of lymphoma or other malignancies. | 2-4 weeks | | | | |
| Vincristine Brand: Oncovin, Vincasar | Thought to increase platelet count by decreasing destruction of antibody-coated platelets. | Intravenous (IV) push | Peripheral neuropathy (nerve pain), constipation, hair loss. Serious skin reactions have been reported if the medication gets under the skin when infusing instead of staying in the vein. | 1 week | | | | |
| Splenectomy (rarely used in pregnancy; often used as a last resort). | A surgical procedure in which the spleen (organ) is removed. The spleen plays a major role in platelet clearance. | Surgical procedure | Immediate surgical discomfort. Hospitalization for 2-3 days if there are no complications. Most patients can return to their normal activity level by 6 weeks. While rare, there is a life-long increased risk of blood infection (sepsis), and thrombosis (blood clots). | 1-3 days | | | | |