

Patient information

Immune Thrombocytopenia (ITP) Treatments

Haematology Liverpool

Treatments for Immune Thrombocytopenia (ITP)

There are a number of different treatment approaches for patients with ITP, including no treatment and a range of different medications that can be used at different points in the patient journey. In this leaflet we will give an overview of the different treatments for ITP which can be used at different times. Not all treatments will be relevant to you at this time – your ITP team will discuss the appropriate treatments with you.

Not all people with ITP need treatment or may only need treatment at certain times (such as prior to a medical procedure).

The treatment of ITP is individual, and your treatment plan will be created with you by your ITP team. ITP treatment should:

- Be tailored to the duration and the severity of your ITP.
- Prevent severe bleeding.
- Improve platelet count to or above 20 30 x109/L.
- Minimise side effects.
- Improve your health-related quality of life.

First line and emergency treatments for ITP

Steroids

If your ITP needs treating, we will normally prescribe steroids as a first line treatment. These reduce the level of antibodies in the blood stream, and stop your immune system from destroying your platelets. Steroids are the usual first line treatment for ITP, and we aim to only use these for a short period (up to six weeks).

Over a short period of time, steroids usually cause no problems. However, steroids can have side effects, especially if you need repeated courses of steroids or take them for a long time. Steroids can increase your chance of getting infections by reducing the production of other antibodies as well as those causing ITP. There are other side effects, including thinning of the bones (osteoporosis), stomach ulcers and high blood sugars (especially if you already have diabetes). They can change your facial appearance and cause thinning and bruising of the skin. People feel they want to eat more when taking steroids and often put on weight. Steroids can also stop you from getting to sleep, cause vivid dreams, or change your mood.

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Steroid side effects usually reverse once the steroids are stopped. If you are taking steroids you should not stop taking them without advice of your ITP team, as over a period of time your body starts to rely on them.

Intravenous Immunoglobulin (IVIg)

This is a medicine containing antibodies (immunoglobulin) which is given into a vein, usually in the arm, through a drip (intravenously). Antibodies are produced by white blood cells to fight infections. IVIg is a human blood product, with the antibodies collected from numerous blood donors.

It is not known exactly how IVIg works to treat ITP, but it is thought that the extra antibodies stop your own white blood cells from destroying the platelets. IVIg works quite quickly, usually during a few days, but the effect doesn't last long (up to a few weeks). It is often given before surgery, or where your platelet count needs to be increased rapidly (for example if you have significant bleeding symptoms).

IVIg is given as an infusion through a drip over several hours.

Other Treatments for ITP

Rituximab

Rituximab is a treatment that was first used to treat cancer, but has been used for over 20 years to treat ITP and other autoimmune conditions. Like steroids it stops your immune system from destroying platelets, but it has fewer side effects than steroids.

Around two in three people given Rituximab for ITP will have an increase in their platelet count. Some people have further episodes of ITP where their platelet count will drop, but usually the platelet count stays at a safe level for over a year.

Thrombopoetin Receptor Agonists (Romiplostim, Eltrombopag and Avatrombopag)

Thrombopoetin (TPO) receptors are on the surface of cells that make platelets in the bone marrow (called megakaryocytes). These drugs use these receptors to tell the cell to make more platelets. The effect of these drugs usually only continues whilst taking the drug, however in some people the medication can be stopped after a period of time and the platelet count remain at a safe level.

Romiplostim is given as an injection under the skin (usually once per week). Eltrombopag and Avatrombopag are tablets.

eight to nine out of ten patients with ITP treated with these drugs to respond (the platelet count improve or have less bleeding). These effects continue in the long term in about five of those patients.

Splenectomy

As your platelets are mainly destroyed in the spleen, removing the spleen can cure the condition. A splenectomy is undertaken whilst you are asleep under a general anaesthetic. It can be done laparoscopically (via keyhole surgery), however in some cases the surgeon will need to carry out open surgery (using a larger cut).

You may need treatment to improve your platelet count prior to surgery to reduce the risk of bleeding during the operation.

Splenectomy has been used for many years to treat ITP, and offers the best chance of curing ITP. One half to two thirds of patients will have a long term response, however it is not possible to predict accurately which patients these will be before the operation. Your ITP team may suggest a special scan which shows where the platelets are cleared, as if this is in the spleen it is thought there is a better chance of splenectomy being successful.

Splenectomy is a surgical procedure and is not without risks. The risks include a reaction to the anaesthetic, bleeding at the time of surgery, damage to other organs during the operation and infection. There is a small risk of death associated with splenectomy.

The spleen has a role in fighting infection. To reduce the long term risk of infection after splenectomy, you will be given vaccinations prior to the surgery and antibiotics after the surgery. You will need to take antibiotics long term at low doses, and have scheduled vaccinations including the influenza and pneumococcal vaccines throughout your life.

Immunosuppressants

These medications work by supressing the immune system, preventing the production of antibodies to destroy platelets. They are sometimes described as "steroid sparing agents" as they replace the effect of steroids without the same side effects.

There are a number of drugs in this group, but the two most commonly used in ITP are Mycophenolate Mofetil (MMF) and Azathioprine. There are other drugs including Cyclophosphamide, Vincristine and Ciclosporin. They are all given as tablets, apart form Vincristine which is given as an injection into a vein.

Fostamatinib

Fostamatinib is a drug that belongs to a group of drugs called spleen tyrosine kinase inhibitors. It works by blocking the effect of an enzyme which is involved in stimulating the immune system. It is used for patients who have not responded to or are unsuitable for other ITP treatments including a Thrombopoetin receptor agonist. One in two patients respond to Fostamatinib.

Fostamatinib is a tablet treatment, usually taken twice a day. It can cause high blood pressure, diarrhoea, abdominal pain and abnormal liver blood tests. These side effects do not affect all patients, and are often worse at higher doses.

Dapsone

Dapsone is an antibiotic, but it can also be used to treat ITP. It is not clear exactly how it works, but it appears to dampen down the immune process, which can stop your body from attacking your platelets. It is taken as a tablet once a day, and has few side effects. About one in two people who have already tried steroids and Rituximab will respond to Dapsone, however less than half of those people will still have a good platelet count after six months.

Dapsone cannot be given to people with a blood condition called G6PD deficiency (a rare condition which affects your blood cells), and your ITP team will check you don't have this if they are going to suggest dapsone treatment. If dapsone is given to people with G6PD deficiency it can cause severe anaemia due to damage of the red blood cells.

Anti-D

Anti-D is a collection of antibodies from blood donors (like IVIg). It is more commonly used for some pregnant women to prevent them from creating antibodies that can affect the pregnancy. It can only be used for people who are "rhesus D positive" and have not had a splenectomy. It is given as a drip over a few minutes as a one off dose.

Seven out of ten people given Anti-D will have a response in their platelets, but in half of these this lasts less than three weeks. It can be repeated at a later date if effective. Anti-D is not commonly used in the UK as some people treated with it experienced a breakdown of red blood cells resulting in kidney damage. Anti-D may however be an option if other treatments have not worked for you.

Trial Treatments

Treatments for ITP are being developed all the time, and your ITP team may discuss treatments with you that are in development, or where their effectiveness is being assessed. This can give you an opportunity to access new treatments before they are widely available. If your ITP team feel that a trial treatment may be suitable for you they will discuss this with you.

Can I have platelet transfusions to treat my ITP?

In ITP your bone marrow is healthy, and produces platelets as normal. The low platelet count is due to your platelets being destroyed in the circulation. If you are transfused platelets these would also be destroyed by your immune system, and would only hast a few minutes or hours before being destroyed.

Platelet transfusions can be used as an emergency treatment for severe bleeding as they help you form a clot, but are not useful for long term prevention of bleeding. When used in ITP platelet transfusions are usually given with IVIg.

What other treatments might I receive?

Tranexamic Acid

Tranexamic acid is a medication which helps blood clots last longer once they have been formed. The blood clots are then more stable than normal, and more resistant to being broken down. Tranexamic Acid does not treat ITP, but can be useful if you have bleeding when your platelets are low.

Tranexamic acid is usually taken as a tablet three or four times a day, but can also be used as an infusion (drip) or a mouthwash. It should not be used if you have blood in your urine.

Other supportive treatments

You may be offered other treatments, which are often to reduce the risk of side effects from your treatments. These include:

- Stomach protectors (omeprazole or lansoprazole) to reduce the risk of stomach side effects from steroids.
- Calcium and Vitamin D to reduce the risk of osteoporosis from steroids.

- Antibiotics to reduce the risk of infection after rituximab treatment.
- Vaccinations after splenectomy or if taking immunosuppressive medication.

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Following your discharge from hospital or attendance at your outpatient appointment you will receive a text asking if you would recommend our service to others. Please take the time to text back, you will not be charged for the text and can opt out at any point. Your co-operation is greatly appreciated.

Further information

If you have any questions or queries, please contact Haematology Liverpool – 0151 706 3397 Text phone number: 18001 0151 706 3397

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