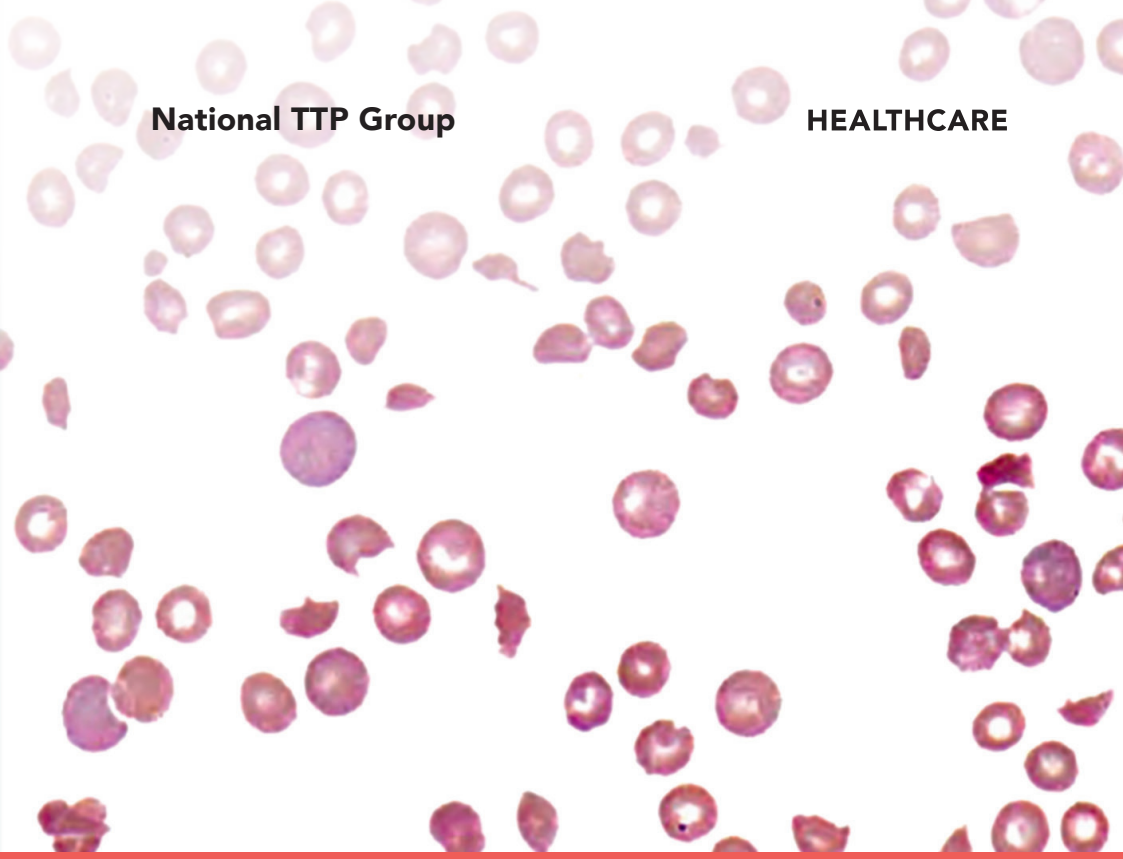
National TTP Group

HEALTHCARE



Fast Facts for Patients

Thrombotic Thrombocytopenic Purpura

United Kingdom TTP Registry

Karger

ttpnetwork

First, the facts...

1. Thrombotic thrombocytopenic purpura (TTP) is a life-threatening condition and needs urgent hospital attention, but can be reversed quickly with intensive treatment.
2. There are two types of TTP: immune TTP (iTTP), which occurs when your immune system mistakenly attacks your own cells, and congenital TTP (cTTP), which is inherited.
3. Emergency treatments for iTTP are plasma exchange (the removal and replacement of part of your blood), steroids and a medication called caplacizumab.
4. Treatment with another medication called rituximab helps to reduce the risk of relapse.
5. Treatment for cTTP is usually different, involving replacement of an enzyme called ADAMTS13, such as by plasma infusion.

This booklet explains what TTP is and the treatments available. It is common to have lots of questions and we've included a list of questions and answers at the back of this booklet (see pages 26 and 27). Contact information can be found on the last page.

My main concerns

Make a note of anything you want to discuss with your doctor here

What is thrombotic thrombocytopenic purpura (TTP)?

TTP is a rare blood disorder that affects around 6 people per 1 million of the UK population every year. It can affect any age group, but is most common in adults, particularly women. It is usually caused by the body's immune system attacking an enzyme (a type of protein that helps with specific tasks in the body) in the blood called ADAMTS13, causing blood clots to form in vital organs.

Acute (severe and/or sudden) TTP is a medical emergency and needs to be treated quickly.

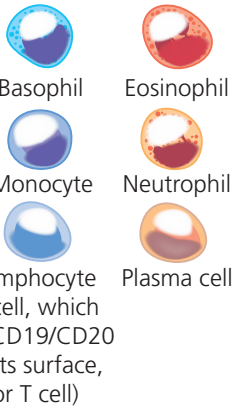
Blood cells

Blood flows around the body in blood vessels called arteries and veins. Blood is made up of three types of blood cell and liquid called plasma. Plasma contains several types of proteins that the body needs to function normally.

Red blood cells contain hemoglobin, which carries oxygen around your body

Platelets help your blood to clot

White blood cells fight infection; there are several different types

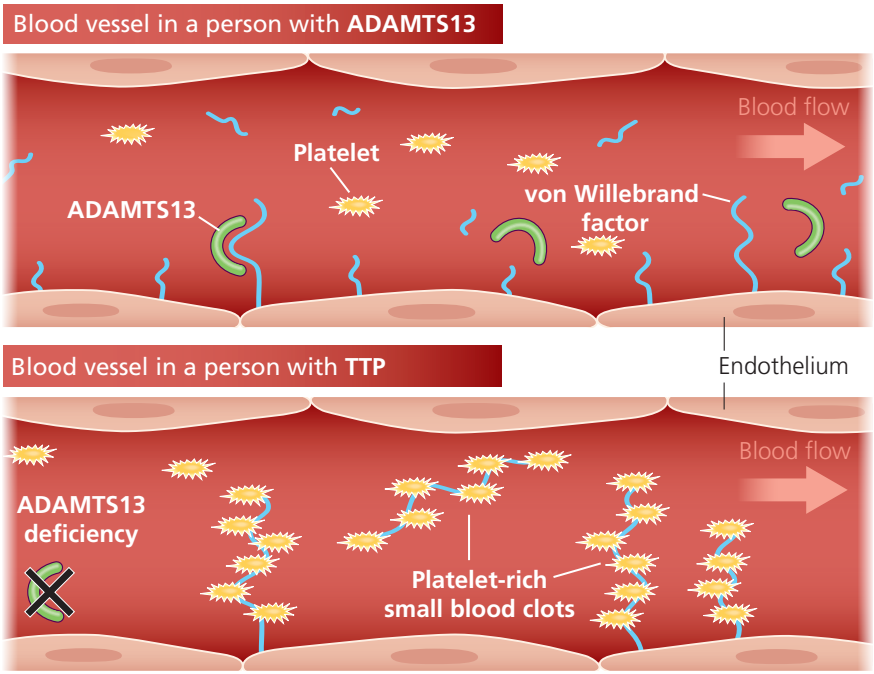


Blood clotting

Blood clotting is the process the body uses to control blood loss and promote healing. When you cut yourself, blood vessels are damaged, and platelets bind to blood-clotting proteins (known as coagulation factors) and clump together to help stop the bleeding. One of the most important of these is called von Willebrand factor, which is made as a very large protein in the body and needs to be cut up smaller to function normally. The ADAMTS13 enzyme in plasma normally does this.

What happens in TTP?

People develop TTP when they don't have the ADAMTS13 enzyme. Without ADAMTS13 to control it, von Willebrand factor can cause platelets to bind to each other, causing blood clots to form in small blood vessels that supply vital organs, usually the brain and heart. Red blood cells can be damaged as they flow past the blood clots and are broken down (by a process called hemolysis) leading to a lack of red blood cells and low levels of hemoglobin (anemia). Because the platelets are making clots, the number of platelets circulating in the blood becomes very low (thrombocytopenia).

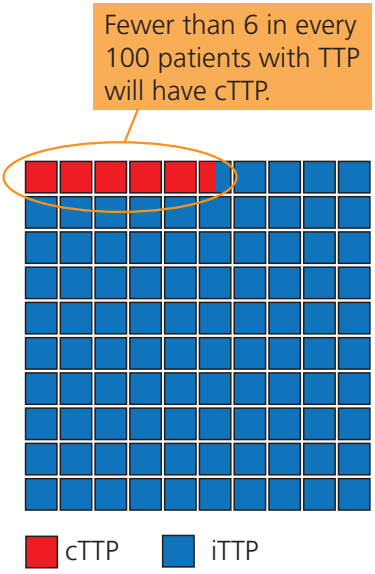


TERMINOLOGY TIP

If you have **thrombocytopenia**, you have low levels of **platelets** in your blood. If you have **anemia**, the number of healthy **red blood cells** and level of hemoglobin in your blood is too low for enough oxygen to be delivered around the body.

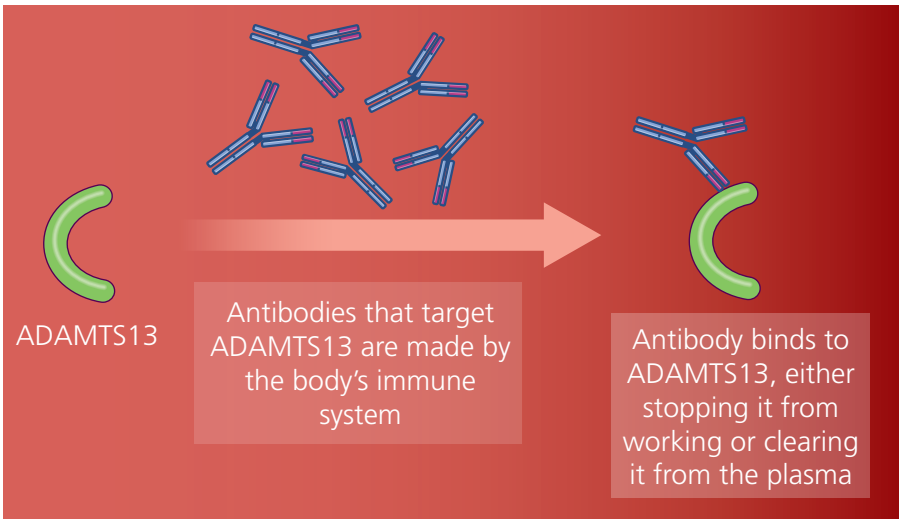
Types of TTP

Although there is often no clear trigger of TTP, it can occur after an infection and also in pregnancy. You cannot 'catch' TTP from someone else who has it. There are two types of TTP, relating to the reason why a person no longer has ADAMTS13. The most common type is iTTP; cTTP is much rarer.



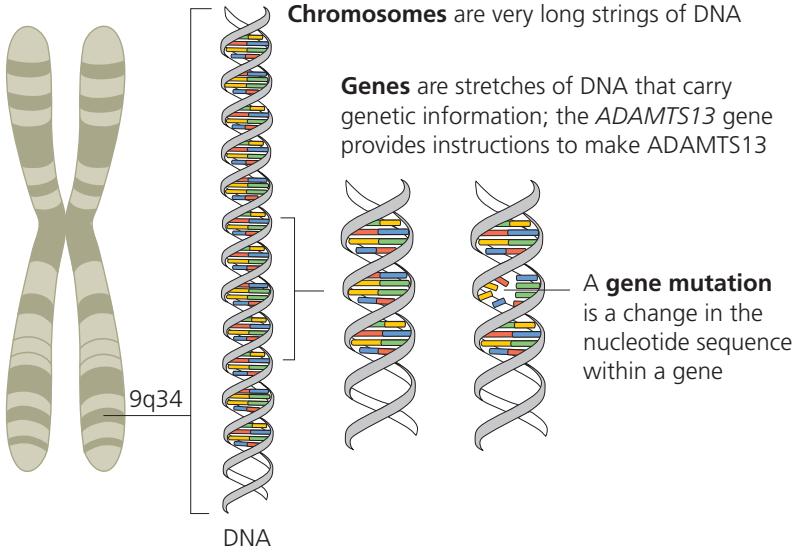
Immune TTP (iTTP)

iTTP is caused when a person's immune system starts to produce antibodies against ADAMTS13 that remove it from the plasma. It cannot be passed onto someone else. Older terms for iTTP that you may come across are 'idiopathic' or 'acquired' TTP.



Congenital TTP (cTTP)

Rarely, TTP is caused by a genetic condition. The production of ADAMTS13 is controlled by a gene called *ADAMTS13*, which is found on chromosome 9. cTTP occurs when mutations (errors in the 'genetic code' – the nucleotide sequence – for ADAMTS13) are inherited, which mean that the body does not make enough ADAMTS13. cTTP can be diagnosed shortly after birth or in childhood, but sometimes is not diagnosed until much later in life, even adulthood (such as during pregnancy).

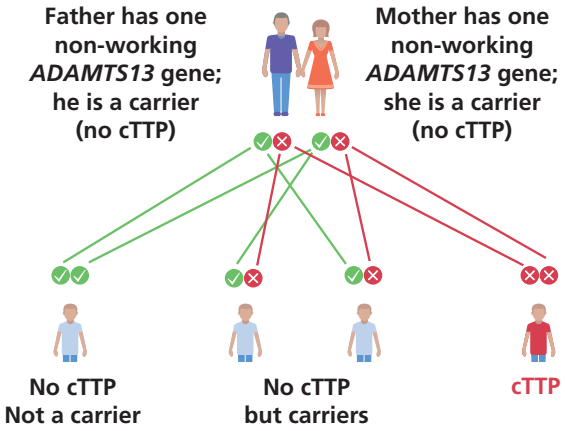


TERMINOLOGY TIP

The sequence of molecules that make up a **protein** depends on the sequence of **nucleotides** in the **gene** that codes for it. If the nucleotide sequence becomes altered (**mutated**), the resulting protein may not work properly or may no longer be made, which can cause problems (diseases).

How is cTTP inherited?

Everyone inherits two copies of the *ADAMTS13* gene, one from each of their parents. To inherit cTTP, you have to inherit two non-working copies of the *ADAMTS13* gene. This is called an **autosomal recessive** disease.



- Working *ADAMTS13* gene (gives the body instructions to produce ADAMTS13)
- Non-working *ADAMTS13* gene

My questions

Note down any questions that you have about how TTP is caused for discussion with your doctor

Symptoms

TTP symptoms can vary from person to person, depending on how the body is affected by the disease. iTTP symptoms usually appear quickly (over a few weeks), whereas cTTP symptoms may appear more slowly.

Common symptoms

Common symptoms include:

- fatigue
- neurological symptoms caused by small blood clots affecting the brain, such as headache, confusion, and weakness/abnormal sensation and/or seizures.

Other symptoms can include:

- abdominal or chest pain
- breathlessness (due to anemia)
- dark urine
- bruising and small bleeds under the skin, which look like small red dots, that are called petechiae (pronounced 'puh-TEE-kee-eye') and caused by low platelet levels.

Long-term symptoms

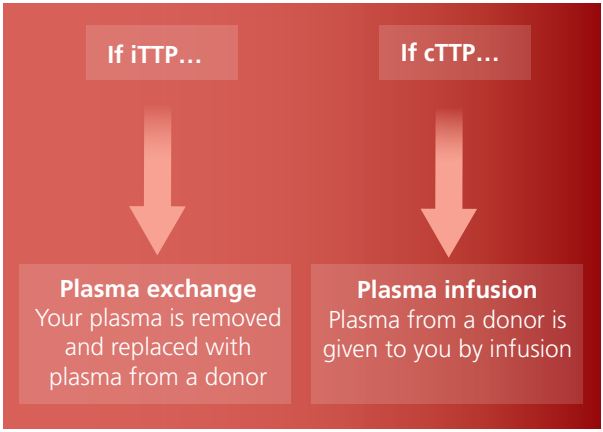
Although symptoms often improve dramatically with treatment, fatigue can persist for several months or longer after an acute TTP episode. Problems with low mood (such as depression) and memory or concentration can occur.

How is TTP treated?

TTP needs urgent hospital treatment; this is important because TTP is life-threatening but can be reversed quickly with intensive treatment.

Both iTTP and cTTP are treated by replacing the missing ADAMTS13 enzyme.

For iTTP, it is also essential that the immune system is suppressed to stop the production of antibodies that target ADAMTS13. Drug treatment can be given as well. Some patients may also need medicines to stop the body from making blood clots.



Monitoring your treatment

Because acute iTTP is life-threatening, it needs urgent intensive treatment. It is also very important that patients' responses to treatment are monitored. This is done by monitoring symptoms and vital signs (like temperature, heart rate, breathing rate and blood pressure), and also blood markers such as platelet counts and ADAMTS13 enzyme levels.

Platelet counts

- These usually begin to recover from low levels back to normal a few days to 1–2 weeks after starting treatment.

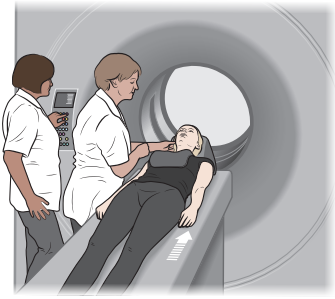
ADAMTS13 levels

- These usually take a few weeks to return to normal.
- It is important that your ADAMTS13 level keeps being monitored, even if you are well and have fully recovered from acute TTP (called remission).
- If your ADAMTS13 level begins to drop, it could indicate that the antibody causing TTP is coming back and there is a risk of another acute TTP episode (called a relapse). This can be treated by giving a medication called rituximab electively (to clear the antibody and prevent a relapse) as an outpatient.

TERMINOLOGY TIP

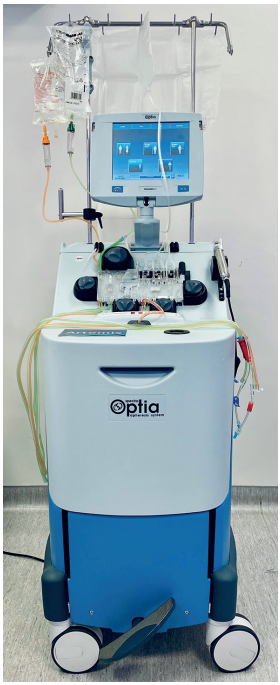
If a treatment is described as being **elective** it means that it is planned, as opposed to being given in an emergency.

Because TTP commonly affects the brain and the heart, you may have a brain scan called an MRI (magnetic resonance imaging) scan and a heart scan (an echocardiogram) to check that they are functioning normally.



It is very important to be followed up for TTP by a specialist.

Immune TTP (iTTP) treatment: plasma exchange



The most important initial treatment for acute iTTP is plasma exchange, which is carried out by a specially trained nurse (apheresis nurse) using a machine called a cell separator. This separates the liquid plasma from the blood cells and replaces it with healthy donor plasma (containing the missing ADAMTS13 enzyme) before the blood passes back into the body.

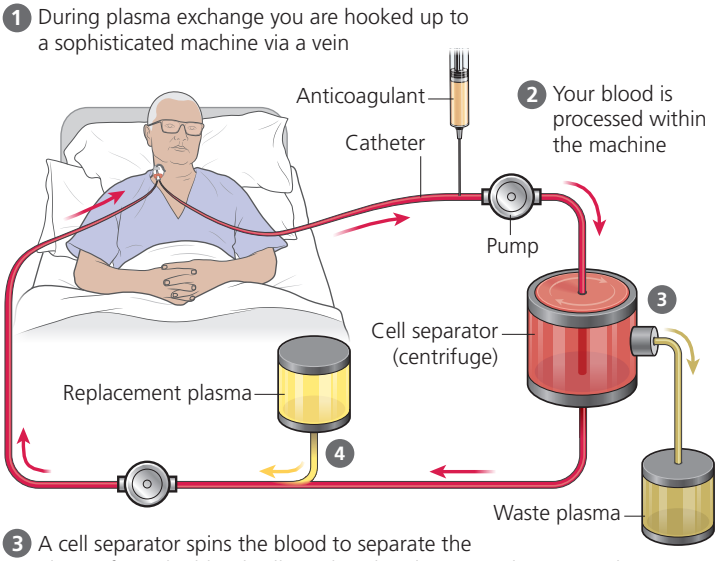
Before the procedure, a small, thin tube is inserted into a large vein, either in your leg (groin) or neck, to allow blood to flow to the cell separator. This is inserted under local anesthetic (to numb the area). To stop blood clotting in the machine, an anticoagulant called ACDA is passed into the plasma and may cause low calcium levels in the body.

Each plasma exchange procedure normally takes 2–5 hours, and one or two procedures per day are usually needed in the first few days after admission to hospital, until the platelet count returns to normal. Plasma exchange can be carried out at your bedside on the ward.

Plasma exchange is very safe. The plasma used is taken from healthy donors and screened to minimize the risk of any infection. It is specially treated to kill viruses, such as hepatitis C virus.

TERMINOLOGY TIP

An **anticoagulant** is something that stops the blood from clotting. The process of blood clot formation is also known as **coagulation**.



3 A cell separator spins the blood to separate the plasma from the blood cells so that the plasma can be removed

4 The blood cells are then mixed with donor plasma and returned to you through the vein in your neck

To stop the blood clotting in the machine, an anticoagulant is added at the time of the exchange. It may temporarily change the calcium level in your blood, but this is monitored and you will be given calcium to correct it if needed. Plasma exchange takes around 2–5 hours.

Possible side effects

- The shift of fluid when plasma is being removed and then replaced can sometimes cause low blood pressure, leading to dizziness and/or feeling faint. It is important to rest for at least 20 minutes after the procedure, and to avoid moving too quickly.
- Some people are very sensitive to ACDA and their calcium levels drop very quickly. Calcium is given to correct this, either as tablets or intravenously (through a cannula, which is a small plastic tube, into a vein in the arm).
- Because plasma contains other proteins apart from ADAMTS13, it is possible to have allergic reactions. These can include an itchy rash or, very rarely, a feeling of swelling in the tongue, irritation of the nasal passages, wheezing, cough or breathlessness. If you have an allergic reaction during the exchange, we will give you medication to treat it.

Practical tips

- It is important that you rest for at least 20 minutes after the plasma exchange procedure and avoid moving too quickly.
- Let the apheresis nurse know if you have pins and needles in your nose, face, hands or body, or you can feel a vibration in your chest. Your calcium level may have dropped and will need to be checked.

My concerns and questions

Make a note here of anything you want to ask your doctor about plasma exchange

Immune TTP (iTTP) treatment: medications that suppress the immune system

Steroids

Steroids (given intravenously or as tablets) are usually given in the first few days or weeks after acute iTTP is diagnosed. They suppress the immune system quickly, which is useful in the short-term because other therapies can take time to work.

Possible side effects

- Changes in mood, indigestion and stomach pains: these are the most common side effects.
- Other side effects can be thinning of the bones (osteoporosis), weight gain, a 'round face' and easy bruising, but these are normally seen in patients who are on steroids longer term (many months or years).
- Your blood sugar can be affected, so this will be monitored; if you are diabetic, your medication/insulin dosage may need to be altered while you are on steroids.

Practical tips

- Where possible you should take steroids in the morning, and you will be given another tablet to protect your stomach while you are taking them (see page 18).

Rituximab/anti-CD20 therapy

Rituximab is given during an acute iTTP episode, but you can also have it electively as an outpatient (where you come to hospital for a short appointment rather than being 'admitted') to prevent another acute TTP episode occurring (known as an acute relapse).

Rituximab is an antibody medication that binds to a type of lymphocyte (a type of white blood cell) called a B cell at a receptor called CD20. B cells can produce antibodies, including the antibody

that causes TTP. When rituximab binds to the CD20 receptor, it causes the B cells to break down and clears the TTP-causing antibodies from the body. Other so-called anti-CD20 therapies are sometimes used instead of rituximab.

Rituximab is given as a drip (infusion) through a cannula inserted into a vein. Normally, 4–8 weekly infusions are given after an acute TTP episode, depending on how long it takes to get a response.

It is quite common to get a mild reaction during the rituximab infusion, so the first infusion is usually given slowly over 8–12 hours, during which time you will be closely monitored by the TTP team. You will be given an antihistamine (such as piriton) and paracetamol beforehand to help reduce the risk of a reaction.

Possible side effects

- Flu-like symptoms, including a high temperature, chills, weakness, muscle aches, tiredness, dizziness and headaches, can occur during the infusion, but do not usually last long.
- You may have low blood pressure (hypotension) during the infusion, so your blood pressure will be checked regularly.
- If you feel sick and/or have occasional vomiting, you will be given antisickness tablets.
- You may have a slight allergic reaction (see Practical tips on page 15).
- Rituximab can reduce your body's production of antibodies, making you more likely to get an infection.
- Sometimes the number of neutrophils (a type of white blood cell) can drop after treatment, making you more likely to get a bacterial infection.
- You may experience warmth in your face (flushing) as well as some redness or darkening for a short time during the infusion.
- Very rarely, patients can complain of joint pains, especially in the knees.

- Progressive multifocal leukoencephalopathy is a very rare complication of rituximab. It is usually seen in patients receiving combination chemotherapy. If you have more questions, please discuss these with your doctor.

Practical tips

- If you still feel sick after taking antisickness tablets, tell the nurses, who will get a stronger antisickness medication prescribed for you.
- Let the nurses know if you have any of the following allergic reaction symptoms: skin rashes or itching, a feeling of swelling in the tongue, irritation of the nasal passages, wheezing, cough or breathlessness.

Mycophenolate mofetil (MMF)

MMF is taken as a tablet; it is usually started when ADAMTS13 levels stay low despite administration of rituximab. MMF works by inhibiting lymphocytes (a type of white blood cell) called B cells and T cells. It can take up to 4–6 weeks for MMF to start working, so there will not be an immediate response. The dose may be increased depending on the response. Side effects are generally mild and usually only occur in the first few weeks of treatment.

Possible side effects

- Headache is common, but not experienced by all patients.
- Abdominal distension (swelling) can happen in some patients; others may experience abdominal pain.
- Feeling sick and occasional vomiting can occur.
- Some patients experience changes in their sense of taste.
- You may be more likely to get an infection, so you will be closely monitored in the clinic by the TTP team.

Practical tip

- If you do feel sick, let your doctor/nurse know and antisickness tablets can be prescribed if needed.

Velcade® (bortezomib)

Velcade® (bortezomib) is sometimes given together with other treatments for TTP. It works by reducing the number of B cells. Velcade® can be given either as a subcutaneous injection (given under the skin) or intravenously. Most people do not experience any side effects, and any experienced are normally mild. This is because the doses of Velcade® used to treat TTP are quite low.

Possible side effects

- Low blood pressure (hypotension) can occur, which may make you feel dizzy or faint. If this happens, it is important to make sure you drink more fluids and stay hydrated.
- Nerve problems (peripheral neuropathy) cause a tingling sensation in the fingers and toes. Patients with TTP do not usually get this because only a few injections of low-dose Velcade® are given.
- Abdominal problems like diarrhea or constipation.
- There is an increased risk of infection. You will be closely monitored by the TTP team in the clinic.

Other treatments

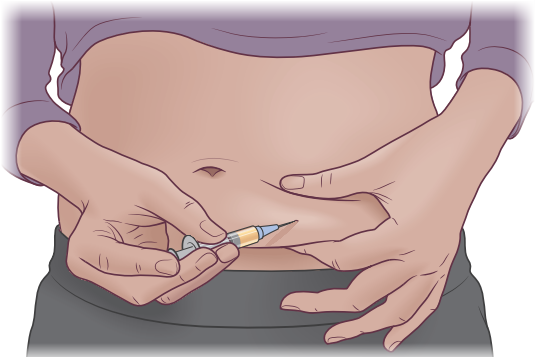
Although not common, TTP is sometimes associated with an infection, including with the human immunodeficiency virus (HIV). This is rare, but if it is identified you will be started on antiviral medications after discussion with a specialist.

Immune TTP (iTTP) treatment: medications that prevent blood clots (thrombosis)

Caplacizumab

Caplacizumab is given during an acute iTTP episode. It is an antibody medication that stops von Willebrand factor binding to platelets; it is given to speed up recovery from and reduce the risk of small blood clots (microvascular thrombosis).

It is usually given as a daily subcutaneous injection for around 4 weeks during an acute iTTP episode, but sometimes for longer. Most people receiving caplacizumab injections have very few side effects (if any).



Possible side effects

- Increased risk of bleeding, including nosebleeds and heavy periods.

Aspirin and low-molecular-weight heparin

Aspirin and low-molecular-weight heparin are medications given to thin the blood in acute iTTP episodes. Low-molecular-weight heparin is usually only given in hospital, but you may be given aspirin for several months (sometimes longer). It is important to let your doctor/nurse specialist know if you have any bleeding symptoms.

Immune TTP (iTTP) treatment: other supporting medications/treatments

Folic acid

Folic acid is a vitamin that is needed to make new red blood cells. It is given after an acute iTTP episode to speed up their production.

Omeprazole, lansoprazole and ranitidine

These medications protect the stomach against other medications that can irritate it (such as steroids).

Blood transfusion

Because red blood cells can become damaged and broken down in TTP (hemolysis), it is normal to have a low hemoglobin level (anemia) and require a blood transfusion. The transfusion is given intravenously, either through a cannula or a vascath (both tubes that go into a vein).

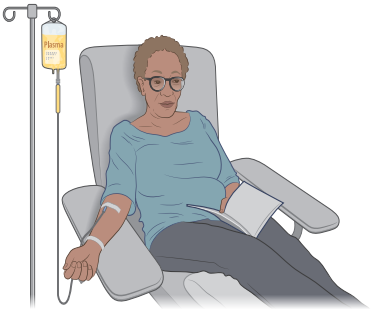
My concerns and questions

Make a note here of anything you want to ask your doctor about medications used to treat iTTP and other supporting medications/treatments

Treatment for congenital TTP (cTTP)

Treatment of cTTP focuses on replacing the missing ADAMTS13 enzyme.

Plasma infusion



Infusions of plasma are given via a cannula inserted into a vein. The amount of plasma infused and the frequency of the infusions are based on symptoms (such as headaches) and blood test results, but infusions are normally 1–2 weeks apart. In some circumstances, recombinant ADAMTS13 enzyme may be given if you decide to take part in a clinical trial. See the next section for more information.

During plasma infusion, patients with cTTP sometimes need to take medications that thin the blood, such as aspirin (see page 17). Folic acid may also be taken to speed up the production of red blood cells (see page 18).

Response to plasma infusions in patients with cTTP is assessed by monitoring symptoms and blood test results. How much plasma you are given and how many infusions you have are based on this.

My concerns and questions

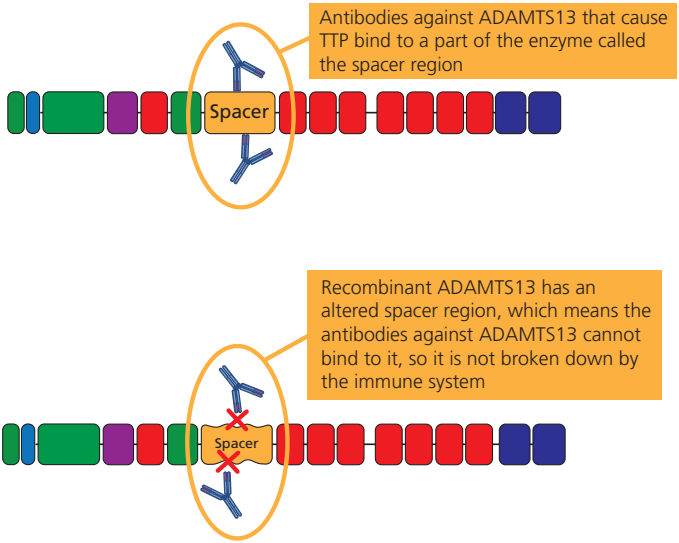
Make a note here of anything you want to ask your doctor about plasma infusion

Clinical trials

Clinical trials are very important. They test new treatments for both iTTP and cTTP and compare them with existing treatments. Several important TTP clinical trials have been carried out in recent years, and others are ongoing; they include trials involving caplacizumab for acute iTTP, and recombinant ADAMTS13 for cTTP and acute iTTP. If there are clinical trials that you could join, your doctor will discuss this with you.

Recombinant ADAMTS13

Recombinant ADAMTS13 is an artificial version of the ADAMTS13 enzyme naturally found in human plasma, which has a slightly different structure so antibodies that target ADAMTS13 cannot bind to it. It has the advantage that it is not a blood product (derived from donor blood), so there is no risk of infection.



Recombinant ADAMTS13 has the potential to revolutionize the way TTP is treated. Recombinant ADAMTS13 is currently being given to patients with cTTP and iTTP in clinical trials.

UK TTP registry

The UK TTP registry is important for improving our understanding of TTP. The UK TTP registry is used to collect information and blood test results (from when you are admitted to hospital until the episode has resolved) to help improve TTP care and treatment. Your TTP team will be able to discuss this with you at your appointments. You may also be asked to take part in other studies.

Psychological support and neuropsychological testing

TTP can have important effects on both mood and brain function (cognition). Problems such as low mood or anxiety, or issues with memory and concentration, are fairly common after a TTP episode, and can present in many ways. They may happen early after diagnosis or several weeks later, and can be temporary or, occasionally, last longer.

Whenever they emerge, if you are experiencing any of these symptoms, it is important that you discuss them with your TTP team.

Support is available and your team can organize it for you. Sometimes a referral is made for assessment by a clinical psychologist/counsellor if needed. The psychology team is experienced in dealing with patients with TTP so has a good understanding of the impact it can have. Your TTP team can also refer you to specialists who can test your memory/cognitive function (neuropsychological testing) and help you improve it.

Going home after TTP treatment

You will be able to go home when you are feeling better, your platelet count is within normal limits and plasma exchanges have stopped. Your general practitioner (GP) will be informed of your condition and the treatments that you have received, and we will establish links with your local hospital if you live some distance from your TTP centre.

Practical tips

- When you do get home, you will need to relax. You will feel very tired because you will have likely been less mobile than normal for a while and your body will still be recovering from the acute TTP episode. In addition, you will probably still be anemic (low red blood cell count/hemoglobin), which can add to feeling fatigued. Do not try to go straight back into your old routine, and accept offers of help from friends and family.
- You will need to consider a phased return to work or working from home where possible. We can provide certificates/letters for your employer.

Medications after discharge

If you have started caplacizumab treatment, you will continue this at home for a few weeks and will need to self-inject (or have someone else administer it for you). Your nurse specialist will teach you how to do this.

Having the caplacizumab injection every day is very important; it is stopping the little blood clots from forming and preventing TTP from relapsing.

You will probably also go home with folic acid and a medication to protect your stomach (see page 18).

DOs	DON'Ts
• **DO** check with your TTP team before you travel abroad; we can give you a list of specialist insurance companies that will insure you at a reasonable rate. • **DO** speak to your Consultant Hematologist if you are considering starting a family.	• **DON'T** start any medicine without contacting us first, including the contraceptive pill and other tablets. • **DON'T** believe all you read on the internet about TTP or the treatments used. Not all of the information on the internet is from reliable sources or up to date. • **DON'T** feel that you are being silly if you have any questions, contact your TTP nurse specialist; remember, their telephone number is not just for emergencies.

What to do if you feel unwell

When you first go home you should expect to feel tired, but there are some things to look out for:

- headaches not relieved by paracetamol
- blurred vision, dizziness or confusion
- easy bruising without obvious cause
- dark urine
- feeling unwell and worried.

It is important that you call your TTP nurse specialist (or emergency hematology contact number, if outside normal working hours) if you have any of the symptoms above. They will arrange for you to have a blood test.

In most cases, the blood counts will be steady, but occasionally a patient may have an early relapse and will need to be readmitted for further treatment. If you do not live near your TTP centre we may be able to arrange for you to have a blood test locally.

TTP Team contact information

Follow-up appointments

You can expect to be seen weekly as an outpatient for at least 4 weeks for further treatment/clinic appointments with your Consultant Hematologist or Senior Registrar and a TTP nurse specialist. They will be able to monitor your recovery and give you further treatment where needed, or refer you to other services that may be helpful. As your ADAMTS13 level returns to normal/your baseline, these visits will increase from weekly to monthly, and then to every 3–6 months. However, this will depend on how clinically well you are and your ADAMTS13 level. For example, if your ADAMTS13 level is repeatedly normal, you may have fewer initial visits. Similarly, if you are showing signs of needing elective rituximab treatment, extra visits may be needed.

If you have been diagnosed with cTTP you will need to attend clinic regularly for your plasma infusion treatments.

It is important that you attend all your clinic visits so that we can see how you are responding to treatment. They are also the best way for you to access services that you need.

TTP in the long term

It is important to remember that TTP (both iTTP and cTTP) will need lifelong follow-up in the clinic. This will allow appropriate monitoring and any need for treatment to be identified.

If you have iTTP, treatment may be needed intermittently to prevent a relapse; your ADAMTS13 level will be checked at regular intervals to see if this is needed. Patients with cTTP often need regular plasma infusion treatments to control their symptoms.

Frequently asked questions about TTP

Q: How do you get TTP?

People with cTTP are unable to make ADAMTS13 because of a genetic condition. iTTP occurs when the immune system mistakenly attacks ADAMTS13 with an antibody, stopping it from working. For most people, the reason why this happens is unknown. Low levels of ADAMTS13 result in small blood clots, and low red blood cell and platelet levels.

Q: Can it be passed on and can I catch it from someone else?

No, iTTP cannot be passed on to or caught from someone else. People with cTTP have inherited it from their parents (if their parents are carriers). There is a chance that they might pass it on to their children (see page 6).

Q: Can I get it again?

Yes, it is possible to get iTTP again. The best way to prevent this is to monitor ADAMTS13 levels in the blood. If they are low, treatment with rituximab can be started to prevent a TTP relapse. In cTTP, ADAMTS13 levels are always low, so you will need regular plasma infusions to keep you well.

Q: What treatment will I need?

If you have iTTP, you will need treatment during an acute TTP episode with plasma exchange and medication to suppress the immune system. You may also need outpatient treatment to suppress the immune system to prevent a relapse. If you have cTTP you will need treatment with infusions of plasma.

Q: Will I need treatment for the rest of my life?

This depends on the type of TTP that you have. Patients with iTTP will need treatment for acute episodes or to prevent an

acute episode. If you have cTTP, you are likely to need long-term treatment with infusions of plasma.

Q: What are the side effects of treatment?

Treatment for TTP is generally very safe, but there are potential side effects, depending on the treatment given. These are listed in this booklet (see pages 10 to 17).

Q: Can I exercise with TTP?

Yes, it is safe to exercise, but it is normal to feel tired early on after an acute TTP episode. Regular exercise is good for you, but do not push yourself too hard.

Q: When can I go back to work?

Your medical team will discuss this with you; it will depend on the TTP treatment you are receiving and, most importantly, how you are feeling and the type of job you have. This may mean you cannot return to work for at least a few weeks after an acute iTTP episode, and sometimes it can take longer.
We recommend a gradual, phased return to work and, where possible, you should discuss this with your employer and/or your occupational health department.

Q: Can I take or eat anything to stop another TTP episode?

What you eat will not cause or prevent a TTP episode, but it is important that you eat a healthy balanced diet for your general health.

Q: I want to go on holiday, will TTP affect my travel insurance?

Having TTP can mean your travel insurance premium will be higher; however, some insurance companies generally offer insurance for patients with TTP, and it is important that you take insurance out before you travel.

Useful resources

UK TTP Network

www.ttpnetwork.org.uk

UCLH TTP website

www.uclh.nhs.uk/our-services/find-service/cancer-services/blood-diseases-clinical-haematology/blood-diseases-types-and-services/red-cell-diseases/TTP

My notes

Make a note here of any other questions that you have or issues that you would like to discuss with your TTP team

Glossary

ADAMTS13: an enzyme that is present in the body at low levels in patients with TTP

Anemia: a low level of red blood cells/ hemoglobin

Antibody: a protein produced by the body's immune system that binds to things that the body considers to be 'foreign', like viruses and toxic molecules

Apheresis: the process of separating blood components in a machine called a cell separator

Autosomal recessive: you need to inherit a mutated copy of a gene from both parents to develop the genetic condition that is linked to the mutation

Cannula: a small plastic tube that is inserted into a vein to give medicine

Catheter: a small, thin plastic tube that carries fluids into or out of your body

Coagulation factors: proteins that help the blood to clot (coagulate)

cTTP (congenital TTP): also known as hereditary TTP (hTTP) or Upshaw-Shulman syndrome; the type of TTP that can be inherited

Echocardiogram: a type of heart scan

Electively: optional

Enzyme: a type of protein that helps with specific tasks in the body, usually by speeding something up

Gene: a stretch of DNA that carries genetic information that codes for a protein

Hemoglobin: is the protein in red blood cells that transports oxygen around the body

Hemolysis: the process by which red blood cells are broken down

Intravenous: into a vein

iTTP (immune TTP): also known as acquired TTP (aTTP), and some older information may call it Moschowitz syndrome; the type of TTP that is caused when the body's immune system starts to produce antibodies against ADAMTS13

Lymphocyte: a type of white blood cell that plays a key role in the body's immune system; lymphocytes include B and T cells

Magnetic resonance imaging (MRI) scan: a type of brain scan

Mutation: a change in the nucleotide sequence of a gene, which may mean that the protein that the gene codes for is no longer made or may not work properly

Neuropsychological testing: testing that checks your memory and cognitive (brain) function

Peripheral neuropathy: nerve problems

Petechiae/purpura: small bleeds under the skin that can look like a rash or little red/purple dots (petechiae is pronounced 'puh-TEE-kee-eye')

Plasma: the fluid component of blood

Plasma exchange: separates plasma from the blood cells and replaces it with healthy donor plasma (containing the missing ADAMTS13 enzyme), before the blood passes back into the body

Plasma infusion: plasma from a donor given via a cannula inserted into a vein

Platelets: a type of blood cell that helps blood clots form

Proteins: large molecules that do most of the work in cells, and are needed for the structure, function and regulation of the body's tissues and organs

Recombinant ADAMTS13: an artificial version of the ADAMTS13 enzyme

Red blood cell: type of blood cell that contains hemoglobin, which carries oxygen around the body

Subcutaneous injection: given when a drug needs to be injected into the fatty tissue just under the skin

Thrombocytopenia: low levels of platelets in the blood

Thrombotic: forming a blood clot in a blood vessel

UCLH: University College London Hospitals

von Willebrand factor: a protein that helps blood clots form

White blood cell: type of blood cell that helps fight infection

By Professor Marie Scully – lead consultant
Dr John-Paul Westwood – consultant
Dr Mari Thomas – consultant
Dr Matthew Stubbs – consultant

Please contact us if you have any questions or are worried.
For contact details please see page 24

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Questions for the Editor

How has this booklet helped you? Is there anything you didn't understand? Do you have any unanswered questions? Please send your questions, or any other comments, to fastfacts@karger.com and help readers of future editions.

Thank you!

With sincere thanks to those who have reviewed this publication for all their help and guidance.

HEALTHCARE

Hematology

Fast Facts for Patients

Thrombotic Thrombocytopenic Purpura

United Kingdom TTP Registry

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