NHS
University Hospitals of Liverpool Group

Patient information

Recombinant ADAMTS for Congenital Thrombotic Thrombocytopenic Purpura

Haematology Liverpool

We are specialist service for the diagnosis, treatment, and follow-up of patients with thrombotic thrombocytopenia purpura (TTP).

We aim to provide you with appropriate treatment for your medical condition and to support you with your physical and mental health, social needs, and managing your condition.

Congenital Thrombotic Thrombocytopenic Purpura (cTTP)

People with congenital (also called inherited) TTP are born with the condition. It remains with them throughout their life. There is a fault in the gene that tells the body how to make the enzyme ADAMST13, therefore, the ADAMST13 is very low. It affects males and females equally, and the condition is inherited from parents where each parent has half a faulty gene. The parents will not have the condition themselves as they have one normal and one faulty gene. Congenital TTP is even rarer than the immune form.

Recombinant ADAMTS 13

Recombinant ADAMTS13 is the missing enzyme in TTP but made in a laboratory, not involving blood donations.

It is a powder that is mixed with a small volume of water and given over three to five minutes into a vein. It increases the ADAMST13 levels to a higher level than with plasma infusion. Generally, treatment is required every two weeks on our Haematology Day ward but can also be given more frequently if an acute relapse occurs.

Your bloods tests would be monitored each time you have treatment such as FBC and ADAMTS-13.

The benefit of this new treatment is to reduce acute TTP episode's and improve symptoms in cTTP.

Recombinant ADAMTS13 is currently available in the UK via a patient access scheme. This treatment is not currently approved by NICE, so it is unsure of the long-term availability of the product.

Side effects

As will all medicines, there are side effects associated with recombinant ADAMTS13 treatment. Reported side effects are as follows.

Very common (may affect more than one in ten people):

- nose and throat infection.
- headache.
- feeling dizzy.
- migraine.
- diarrhoea.
- nausea.

Common (may affect up to one in ten people):

- high number of platelets in the blood (thrombocytosis).
- feeling sleepy.
- constipation.
- bloating.
- weakness.
- feeling hot.
- ADAMTS13 activity abnormal.

Some patients may experience allergic reactions to the product. Your treatment will be given in hospital, and you will be closely monitored.

Neutralising antibodies (called inhibitors) may develop in some patients receiving recombinant ADAMTS13. These inhibitors could potentially cause the treatment to stop working properly. You will be closely monitored during your treatment.

The use of Recombinant ADAMTS13 is relatively new so not all side effects may be known. If you start treatment, you will be closely monitored for any adverse reactions, and we will report these via the yellow card scheme.

Feedback

Your feedback is important to us and helps us influence care in the future.

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

TTP Network- https://www.ttpnetwork.org.uk/

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All Trust approved information is available on request in alternative formats, including other languages, easy read, large print, audio, Braille, moon and electronically.

يمكن توفير جميع المعلومات المتعلقة بالمرضى الموافق عليهم من قبل ائتمان المستشفى عند الطلب بصيغ أخرى، بما في ذلك لغات أخرى وبطرق تسهل قراءتها وبالحروف الطباعية الكبيرة وبالصوت وبطريقة برايل للمكفوفين وبطريقة مون والكترونياً.

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در صورت تمایل میتوانید کلیه اطلاعات تصویب شده توسط اتحادیّه در رابطه با بیماران را به اشکال مختلف در دسترس داشته باشید، از جمله به زبانهای دیگر، به زبان ساده، چاپ درشت، صوت، خط مخصوص کوران، مون و بصورت روی خطی موجود است.

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Dhammaan warbixinta bukaanleyda ee Ururka ee la oggol yahay waxaa marka la codsado lagu heli karaa nuskhado kale, sida luqado kale, akhris fudud, far waaweyn, dhegeysi, farta braille ee dadka indhaha la', Moon iyo nidaam eletaroonig ah.