Patient/carer organisation statement template

Thank you for agreeing to give us your views on the technology and the way it should be used in the NHS.

Patients and patient advocates can provide a unique perspective on the technology, which is not typically available from the published literature.

To help you give your views, we have provided a template. The questions are there as prompts to guide you. You do not have to answer every question. Please do not exceed the 8-page limit.

About you

Your name: Jeanette Atkinson

Name of your organisation: ITP Support Association

Are you (tick all that apply):

- a patient with the condition for which NICE is considering this technology?
- a carer of a patient with the condition for which NICE is considering this technology?
- ***an employee of a patient organisation that represents patients with the condition for which NICE is considering the technology? If so, give your position in the organisation where appropriate (e.g. policy officer, trustee, member, etc)***
  Assistant Administrator for The ITP Support Association
- other? (please specify)
- a volunteer of a patient organisation that represents patients with the condition for which NICE is considering the technology

What do patients and/or carers consider to be the advantages and disadvantages of the technology for the condition?

1. Advantages

(a) Please list the specific aspect(s) of the condition that you expect the technology to help with. For each aspect you list please describe, if possible, what difference you expect the technology to make.

ITP Support Association Statement (Shirley Watson)
It will offer another drug to help raise the platelet count in ITP. No one treatment is successful at present, and all of the current treatments (including splenectomy) have serious adverse effects.

Additional information
Many specialists believe that a platelet count of below 30,000 per cubic millimetre increases the risk of a serious and life threatening bleed and thus they consider that treatment should be considered.

The link between a low platelet count and the risk of a bleed was confirmed in a literature-based survey of patients with ITP by Cohen and colleagues (Cohen YC, Djulbegovic B, Shamai-Lubovitz O, Mozes B. The bleeding risk and natural history of idiopathic thrombocytopenic purpura in patients with persistent low platelet counts. Arch Intern Med 2000;160(11):1630-8). Data was taken from 17 case series that included 1,817 patients with persistently low platelet counts. This data set illustrates that the platelet count may be used to estimate the bleeding risk for ITP patients. The bleeding risk of patients with ITP involving persistent low platelet counts (below 30,000 per cubic millimetre) and its impact on prognosis showed that there were 49 cases of fatal hemorrhage over an estimated 1,258 to 3,023 patient-years at risk. Age-adjusted fatal haemorrhage rates were 0.004 cases per patient-year for age groups younger than 40, 0.012 for age groups 40 to 60, and for patients older than 60 years they were 0.130. Predicted 5-year mortality rates ranged from 2.2% for patients younger than 40 years to 47.8% for those older than 60 years. A 30-year-old woman remaining thrombocytopenic due to ITP was predicted to lose 20.4 years (14.9 quality-adjusted life years) of her potential life expectancy. At age 70, predicted loss was 9.4 years or 5.0 quality-adjusted life years

Raising the platelet count should reduce the risk of major bleeds and other distressing symptoms, such as bruising. One important consequence of this will be an improvement in Health-related Quality of Life of patients, which has been confirmed in clinical studies using questionnaires specifically developed for patients with ITP (George JN, Mathias SD, Go RS, Guo M, Henry DH, Lyons R, et al. Improved quality of life for romiplostim-treated patients with chronic immune thrombocytopenic purpura: results from two randomized, placebo-controlled trials. Br J Haematol 2008).

(b) Please list any short-term and/or long-term benefits that patients expect to gain from using the technology. These might include the effect of the technology on:

ITP Support Association Statement (Shirley Watson)
Romiplostim is not expected to change the course of the condition but if it raises the platelet count it will have an enormous effect on improving quality of life.

Many people with ITP live in fear of a major bleed and it causes problems with work, school, various activities, dentistry, surgical procedures, holidays etc.

Our recent lifestyle survey also showed that adults are afraid to expose their bruises in case their partners are suspected of abuse. Women can have such heavy periods that they are house bound. **NB.** This important and debilitating symptom has been omitted from appendix A. I am aware of women having a hysterectomy to prevent this bleeding and girls as young as 12 being prescribed the pill to cope with flooding and extended periods.

Those who have been on steroids for many years with this condition are now finding that they have bone degeneration and yet because of low platelet counts surgery to replace hips cannot always be done.

Additional information
There are some aspects of treatment for ITP or major bleeds that cause the patient the inconvenience of hospitalisation; any reductions in this would be of benefit.

Removal of the need for splenectomy would also be an advantage as asplenic patients live with the added risk of major infection.

2. Disadvantages
Please list any problems with or concerns you have about the technology.

ITP Support Association Statement (Shirley Watson)
Unless there are side effects of this technology, which have not come to light in clinical trials, I don’t believe there are any disadvantages.

Additional information
The adverse effect profile will become clearer when data from longer-term clinical studies are available.

This is a treatment requiring self-injection and so all those not capable of injecting themselves will need training to self-inject or assistance to do so. For some, this may mean the need to pay for a carer if romiplostin is licensed for self-injection at home.

Patients must have an ongoing supply of this drug as any sudden withdrawal of treatment may cause a rebound reduction in platelet numbers and, theoretically, an increase in the risk of bleeds.

3. Are there differences in opinion between patients about the usefulness or otherwise of this technology? If so, please describe them.

ITP Support Association Statement (Shirley Watson)
No, correspondence from our members shows that they are desperate to see this technology licensed for general use.

Additional information
It is important, from the patient perspective, to reduce the harm caused by current treatments and improve the effective treatment options in ITP. For example, a patient reported suffering an internal bleed (possibly from a stomach ulcer caused by steroid treatment), which required emergency treatment of a blood transfusion, platelets and immunoglobulin. Months later he tested positive for H. pylori, was treated with antibiotics after which his platelet count went up to 90,000 per cubic millimetre and then he suffered a cold and his platelet count fell to 26,000 per cubic millimetre. Patients are seeking a stable platelet level.

4. Are there any groups of patients who might benefit more from the technology than others? Are there any groups of patients who might benefit less from the technology than others?

ITP Support Association Statement (Shirley Watson)
It is difficult to say who will benefit as ITP is a difficult condition to predict which treatment will work for which patient. Those with the worst symptoms (such as mucosal bleeding) and the lowest platelet counts will benefit the most.

Additional information
Effective treatments are needed for women during menstrual bleeds.

Patients exist who have regular life-threatening bleeds; these patients need an effective means to raise their platelet count and thereby reduce their bleed risk. At present the quality of life for such patients is very poor with some being housebound.

For example, The Association has reports of a patient who lapsed into unconsciousness seven times, vomited blood and had a platelet count of 5,000 per cubic millimere. Manypints of blood were lost in this incident. The patient reported feeling ‘weak, frightened and afraid’ after treatment with 2 grammes of methyl prednisolone and 60 grammes of immunoglobulin. Recently, her situation has worsened as none of the current treatments can maintain a raised platelet count. She is now virtually housebound, living in isolation and her husband stays close to hand in case of emergencies. They are both fearful of social contact in case of contracting colds/influenza that may worsen her daily nose bleeds; so they are socially isolated as well. In addition, this patient has damage to her bones from high doses of steroids over many years. The quality of life of the patient and her partner is severely compromised.

Patients needing surgery who have low platelet counts in whom current treatments are not effective often have important surgery delayed, For example, a patient with renal colic needed surgery to unblock and reshape a ureter, Due to low platelet counts the surgery was delayed for nine years. This resulted in many emergency trips to A&E for pain relief. We have reports of patients needing hip replacements who cannot be operated on. Often these types of patient have to have cocktails of toxic drugs to raise their platelet levels so that surgery can go ahead.

Comparing the technology with alternative available treatments or technologies

NICE is interested in your views on how the technology compares with existing treatments for this condition in the UK.

(i) Please list any current standard practice (alternatives if any) used in the UK.

ITP Support Association Statement (Shirley Watson)
A vast array of treatments are used for ITP as no one treatment is effective for everyone. These include:

-Prednisolone, dexamethosone (steroid)
-IVIG (immunoglobulin)
-Anti-D (immunoglobulin)
-Splenectomy
-Immunosuppressants (danazol, azathioprine, dapsone, cyclophosphamide, vincristine or vinblastine, mycophenolate, cyclosporine)
-Rituximab (not licensed, so not always available)
-Antiobiotic treatment of H. pylori infection (removes stimulus for ITP)
-Platelet transfusion (usually reserved for life threatening bleeds)
-Tranexamic Acid (aids clotting)

Additional information
Approximately two thirds of adult patients do not have an adequate response to steroid treatment. Splenectomy is reported to be ineffective in approximately one third of patients with ITP with complications rates reported to be as high as 12.9% following surgery.

(ii) If you think that the new technology as any advantages for patients over other current standard practice, please describe them.

ITP Support Association Statement (Shirley Watson)
Patients hate steroids (prednisolone, dexamethasone) and their side effects, and they often require very high doses to have any effect.

IVIG is slow to infuse causing difficulty with missing work/school, and both this and Anti-D are not without unpleasant side effects of headache, nausea and joint pain.

Splenectomy and immunosuppressants leave the ITP sufferer wide open to infection and splenectomy is not reversible – many of our asplenic members still have ITP and wish they had not had their spleen removed.

Additional Information
The UK medical advice to asplenic patients is to take prophylactic antibiotics, which many patients are concerned about; they question the long-term effects of such chronic antibiotic treatment.

Up to one third of patients or more seem to be refractory to 'first-line' treatments. New effective treatments with few adverse effects are urgently needed.

(iii) If you think that the new technology has any disadvantages for patients compared with current standard practice, please describe them.

ITP Support Association Statement (Shirley Watson)
No disadvantages known as this drug is only in clinical studies at the moment and in a limited patient population.

Research evidence on patient or carer views of the technology

If you are familiar with the evidence base for the technology, please comment on whether patients' experience of using the technology as part of their routine NHS care reflects that observed under clinical trial conditions.

ITP Support Association Statement (Shirley Watson)
Not used in routine patient care yet

Are there any adverse effects that were not apparent in the clinical trials but have come to light since, during routine NHS care?

As above

Are you aware of any research carried out on patient or carer views of the condition or existing treatments that is relevant to an appraisal of this technology? If yes, please provide references to the relevant studies.

ITP Support Association Statement (Shirley Watson)
The ITP Support Association carried out its own lifestyle survey last year but results have not yet been published in a medical journal. I can provide a copy of the results if you wish. These show that:

-89% (of the 798 who participated) were prescribed treatment for ITP and 84% were concerned about possible side effects.
-30% had surgery postponed because of a low platelet count.
-30% had difficulty obtaining travel insurance
-44% had varying degrees of difficulty getting dental work done.

The new technology may not be the answer for everyone, but for many of our members it will give the opportunity to raise the platelet count by increasing platelet production rather than dampening the immune system, which is the only approach currently used with the various treatments listed above.

Additional information
A study published this year shows that the HRQoL (assessed using the Short Form 36 Questionnaire) of adult ITP patients is significantly worse than that of the general US population (McMillan R, Bussel JB, George JN, Lalla D, Nichol JL. Self-reported health-related quality of life in adults with chronic immune thrombocytopenic purpura. Am J Hematol 2008;83(2):150-4). ITP patients' HRQoL was worse than that of patients with hypertension, arthritis, or cancer and similar to that of patients with diabetes.

A recent analysis of the literature and views gained from patients with ITP suggest that decreased platelet counts, disease symptoms, and treatment side effects influence multiple domains of HRQoL for ITP patients. Key areas affected by ITP and its treatments include emotional and functional health, work life, social and leisure activities, and reproductive health (Mathias SD, Gao SK, Miller KL, Cella D, Snyder C, Turner R, *et al*. Impact of chronic Immune Thrombocytopenic Purpura (ITP) on health-related quality of life: a conceptual model starting with the patient perspective. Health Qual Life Outcomes 2008;6:13).

Availability of this technology to patients in the NHS

What key differences, if any, would it make to patients and/or carers if this technology were made available on the NHS?

Additional information
New treatments that can raise platelet counts (without suppressing the immune system) and replace existing harmful treatments whilst improving quality of life are urgently needed for chronic ITP patients.

What implications would it have for patients and/or carers if the technology were not made available to patients on the NHS?

Additional information
Untreated low platelet levels may result in a major bleed. Current immunosuppressive treatments may lead to a major infection or other treatment-related adverse effects in the long-term.

Are there groups of patients that have difficulties using the technology?

Additional information
Any patient for whom self-injection is problematic.

Other Issues
Please include here any other issues you would like the Appraisal Committee to consider when appraising this technology.

Additional comments
Rarity of ITP; it is unknown exactly how many adults in the UK are living with chronic ITP (ITP of more than 6 months duration). The ITP Support Association fund a UK-wide ITP registry for adults, which is valuable for obtaining critical information, such as effective treatments for the good management of this rare disease.

A six-year study in the North of England revealed an annual incidence of 1.6 patients per 105 adults in a UK Health Region of 3 million people. The majority of these newly presenting patients achieved complete or partial remission (214 out of 245, or 87%). As a few patients died, this suggests that only approximately 10% of all newly diagnosed adult patients remained as chronic ITP sufferers (Neylon AJ, Saunders PW, Howard MR, Proctor SJ, Taylor PR. Clinically significant newly presenting autoimmune thrombocytopenic purpura in adults: a prospective study of a population-based cohort of 245 patients. Br J Haematol 2003;122(6):966-74).

Life threatening nature/unpredictability of ITP. The course of ITP is highly individual and requires expert management, which involves: individual treatment decisions incorporating bleed history; assessment of bleed indicators (mucosal bleeds), assessment of co-morbidities, patient expectations, patient lifestyle and ability to adhere to treatment regimens. For some, this condition is life threatening and reduces Quality of Life to very low levels.

Harm caused by all of the current treatment options.
At present many patients prefer to remain untreated to reduce the risk of short-, medium- and long-term harm caused by the various treatments available. If major bleeds occur then the costs to the health service can be substantial.