

Appendix G – Patient/carer organisation statement template

NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Single Technology Appraisal (STA)

Eltrombopag for the treatment of chronic idiopathic (immune) thrombocytopenic purpura (review of technology appraisal 205)

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:

Shirley Walters MBE

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)
- other?
- **Founder and volunteer of the patient organisation that represents ITP, the condition for which NICE is considering the technology.**
- **My position is Chief Executive & Chair of the Trustees.**
- **Also a mother of a son who has had chronic ITP for 19 years**

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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.

TPOs have been shown to be successful in refractory patients

Eltrombopag will give the availability of a TPO drug in pill form which many patients will prefer to injection.

This TPO will avoid weekly hospital visits (with impact on work/college/travel costs)

Eltrombopag will give access to another licensed drug, and in particular, one that has been through the modern rigours of clinical trials.

Although long term side effects are still being investigated patients report that side effects are few. This compares favourably with steroids and splenectomy which is only succesful in 60% of patients and carries many complications and long term risks plus a lifetime of antibiotic treatment.

(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:

- the course and/or outcome of the condition
- physical symptoms
- pain
- level of disability
- mental health
- quality of life (lifestyle, work, social functioning etc.)
- other quality of life issues not listed above
- other people (for example family, friends, employers)
- other issues not listed above

Although intended as palliative treatment we know of cases where the platelet count has improved and in a couple of cases returned to normal without further treatment.

This treatment will offer another option for patients refractory to treatment.

In ITP a simple blood count will show whether the drug is working or not.

In patients for whom the drug works it will:-

- Reduces the risk of a major bleeding event such as a brain haemorrhage;
- Prevent other bleeding such as nosebleeds and very heavy periods
- Reduce the fear of bleeding
- Reduce the need for hospital visits and admissions

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- Improve QoL (reduced interference with work, leisure activities etc)
- Relieve close family from fear of patient bleeding
- Give availability of ongoing supply when travelling (work or holiday)
- Reduce the problems of getting insurance (life, holiday etc) where hospital admissions and visits increase premiums and lessen the chances of obtaining cover.

2. Disadvantages

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

Disadvantages might include:

- aspects of the condition that the technology cannot help with or might make worse
- difficulties in taking or using the technology
- side effects (please describe which side effects patients might be willing to accept or tolerate and which would be difficult to accept or tolerate)
- impact on others (for example family, friends, employers)
- financial impact on the patient and/or thier family (for example cost of travel needed to access the technology, or the cost of paying a carer)

Side effects from long term use of Eltrombopag are unknown until data from longer term clinical studies are available.

This treatment requires patients to remember to take their medication daily with regularity and to manage their diet so that it is taken in the middle of an 8 hour calcium free period.

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

Because ITP is highly individual patients will have differences in opinion about the treatment which works for them personally. However, our members recognise that as there is no defined patient pathway or disease outcome any additional treatment is welcomed, particularly licensed drugs that have been fully researched.

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?

It is difficult to know who will benefit as clinicians cannot predict which treatment will work for which patient.

It is extremely likely that those with severest symptoms and the lowest platelet counts will reap the most benefit. Patients who have regular life-threatening bleeds have a poor quality of life with some being housebound.

Benefit more:

Patients who have been refractory to other treatments

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Patients who need a TPO drug but don't like injections or have injection site reactions
Patients who need a TPO drug but can't take time off work for weekly hospital visits

Benefit less:

Patients who don't like pills or can't swallow well
Patients who are unlikely to comply with taking a daily oral medication;
Patients who won't like to adjust their diet

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.

As can be seen from the diversity of the following list there is no one treatment that is safe and effective for ITP patients.

The three first line treatments are steroids, immunoglobulin and splenectomy.

Approximately two thirds of adult patients do not have an adequate response to steroid treatment.

There are worries about availability of immunoglobulin as demand is outstripping production.

Splenectomy is only about 60% effective with complications in 13% of cases following surgery, and there is no follow up to track long term complications. Asplenic patients are at lifelong risk of infection and must take daily antibiotics for life.

Surgery

Splenectomy

Licensed treatments for ITP

Prednisolone, dexamethasone (steroid)

IVIg (immunoglobulin)

Romiplostim

Intravenous treatments not licensed for ITP

Anti-D immunoglobulin (licence withdrawn from the European market, Aug 2009)

Cyclophosphamide

Vincristine

Vinblastine

Plasmapheresis

Cyclosporin

Rituximab

Emergency platelet transfusion

Oral treatments not licensed for ITP

Danazol

Azathioprine

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Dapsone

Mycophenolate mofetil

Helicobacter pylori antibiotics

Vitamin C supplements

Tranexamic acid

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

- improvement of the condition overall
- improvement in certain aspects of the condition
- ease of use (for example tablets rather than injection)
- where the technology has to be used (for example at home rather than in hospital)
- side effects (please describe nature and number of problems, frequency, duration, severity etc)

Eltrombopag offers a TPO drug that can be given by pill rather than injection for those who have needlephobia or fear of injections because of poor veins or injection site concerns.

This is the only oral medication other than steroids which is licensed for use in ITP.

Many other treatments (particularly steroids) have recognised awful side effects

An important advantage of eltrombopag will be its ease of use (by mouth) and the ability for this treatment to be essentially home-based. Patients often suffer severe fatigue with ITP which makes regular hospital visits difficult. After initial monitoring the treatment will be available via the GP.

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

Disadvantages might include:

- worsening of the condition overall
- worsening of specific aspects of the condition
- difficulty in use (for example injection rather than tablets)
- where the technology has to be used (for example in hospital rather than at home)
- side effects (for example nature or number of problems, how often, for how long, how severe).

Patients (particularly elderly or forgetful people) may find it difficult to adjust their diet (although the ITP Support Association will offer practical advice on meal timings, menu alternatives and time of dosage options if Eltrombopag is approved)

Platelet counts could rise too high while initial dosage is being adjusted

Ongoing medication required as sudden withdrawal is not advised.

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We are aware that animal trials raised a possibility of cataracts as a side effect. This would be a minor concern to ITP patients compared with the risk of bleeding, and in any case cataracts are a known side effect of the most common ITP treatment, steroids.

Patients who have inappropriate platelet aggregation may not be suitable subjects for this treatment.

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.

We have heard good reports (platelet level increases and few adverse events) from patients in other countries and from those on patient programmes in the UK.

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

None known

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.

Studies relating to QoL :

Health-related lifestyle in adults and children with primary immune thrombocytopenia (ITP) British Journal of Haematology
doi:10.1111/j.1365-2141.2010.08322.x

Self-reported health-related quality of life in adults with chronic immune thrombocytopenic purpura. Am J Hematol 2008;83(2):150-4).

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).

Studies showing the risks of treatments dampening immune system

Side effects of steroids hated by patients

Corticosteroid side effects and risk of bleeding in immune thrombocytopenic purpura; patient and hematologist perspectives. Eur J Haematol 2009; 83:175-182).

GSK conducted a qualitative patient preference study in which 203 of our membership participated. The study asked about burden of disease and preference of treatments. The study has not been published but I am aware that there was a substantial preference for pills over injections.

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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 was made available on the NHS?

It would make available:

- A TPO drug in an oral form
- An oral drug that can raise platelet counts without the dangers of dampening the immune system
- An oral drug that can replace harmful drugs (steroids & chemo) or surgery (splenectomy)
- Another licensed and fully researched drug

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

It would limit the number of licensed drugs available, which in ITP are few.

Patients refractory to treatment need every available option

It would deny a TPO drug to those who cannot have injections or to those who develop an injection site problem with injected TPO

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

Those who have difficulty swallowing pills or remembering to take a daily tablet.

Those who may be unable to successfully re-organise their diet to avoid foods containing calcium 4 hours before and 4 hours after taking the medication.

Although compliance may be more of an issue in self administration versus injection, marked blister packs will help, and many younger people use their mobile phone for treatment reminders

Equality

NICE is committed to promoting equality of opportunity, eliminating unlawful discrimination and fostering good relations between people with particular protected characteristics and others. Please let us know if you think that this appraisal:

- could exclude from full consideration any people protected by the equality legislation who fall within the patient population for which [the treatment(s)] is/are/will be licensed;
- could lead to recommendations that have a different impact on people protected by the equality legislation than on the wider population, e.g. by

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making it more difficult in practice for a specific group to access the technology;

- could lead to recommendations that have any adverse impact on people with a particular disability or disabilities.

Please tell us what evidence should be obtained to enable the Committee to identify and consider such impacts.

Any patient with a movement disorder may benefit from a TPO that can be easily taken in a home setting. This applies to patients who have joint damage from steroids, extreme fatigue, regular active bleeding or find travelling difficult, and patients who are disabled from an additional condition.

People with ITP having regular blood sampling can become needlephobic, and then the oral TPO would be most appropriate.

Other Issues

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

Although ITP is classed as a trivial disease for some ITP patients, for many it adversely affects their QoL with a constant fear of bleeding is often associated with fatigue. For a small percentage <10% ITP carries a real risk of a major bleed.

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 people of all ages die. The Association knows of two people in their twenties who have died this year and during the last month we have heard from relatives of 3 patients who survived intracranial haemorrhage (a male in his fifties, a mother in her thirties and a child aged 2. To us it is very important that treating physicians have the choice of as many effective drugs as is possible.

Patients hate steroids and the side effects of bone degeneration are very difficult to treat. For example, unpleasant cocktails of immunosuppressant drugs are sometimes given to raise a patient's platelet count enough for hip replacement surgery, which leaves the patient susceptible to hospital acquired infection.

Although some ITP patients are able to manage without treatment, TPOs could be used instead of immunosuppressants to raise the platelet count before surgical or dental procedures.

In the past few months we have received reports from a number of patients that they are being forced into a splenectomy before being allowed to try TPO treatment. We would like NICE guidance to approve TPOs without the patient having to be asplenic. Most patients do not want to lose a healthy spleen.

Splenectomy is only 60% successful and carries a lifelong risk of infection

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Eltrombopag is not like some cancer drugs that extend life by a few months. It will be a life-changing drug for people who suffer regular bleeding episodes and live with the constant fear of a major bleed and opens up the possibility that they will be able to return to a near normal lifestyle.

We would like NICE to encourage clinicians to register their patients on the ITP Registry (based at the Royal London Hospital) so that independent long term ITP patient data can be gathered. Such data could improve ITP management, giving an indication of which patients are at risk and require more aggressive treatment and show which treatments are most successful. This could save lives and save the NHS expense!

