

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:

Christopher Allen

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?
Yes
- 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? (please specify)

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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.

The existing treatments do not work for all patients and often have side effects eg steroids. Many other treatments I have previously tried have not only failed to work, but also require administering at hospital taking both my time and valuable hospital resources. This treatment offers the chance for patients to use a non-invasive method to self-administer medication.

Like many ITP sufferers I was advised very early on in my treatment to have a splenectomy once it became apparent steroids were not having a long term impact. I believe that the availability of a long term, easy to prescribe and administer drug would result in fewer invasive procedures that only have a very uncertain outcome.

Tablet forms of treatment are also far easier than injections that are needed for some other drugs.

As this is also a long term solution, once a patient has found a stable dose at which their symptoms are well managed, fewer regular check-ups are needed and the patient is more in control of their own 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

Self-administered drugs require fewer hospital trips and check-ups, freeing up hospital resources and certainly making life much easier for the patient. Patients miss less work time, have to arrange less child care etc and generally can lead a much more 'normal' life.

An increased platelet count not only makes ITP sufferers far less anxious as they go about their normal life, but is also a source of comfort to relatives who are also impacted by the condition. ITP can go from being a constant source of stress to a condition that does not even impact daily life, and indeed does not even enter minds on a daily basis.

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An important additional benefit is that patients are not prevented from enjoying their hobbies. eg five-a-side football, rugby, skiing and other exercise that ITP can stop. This also has the side-effect benefit of increasing fitness.

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)

There are very few disadvantages I can note. Side effects are limited and tablet form drugs are easy to administer

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

When taking a drug for ITP there is always a chance that the drug will not work. My experience is that the chance of success was always discussed in advance and patient expectations well managed.

Due to the hit and miss nature of managing ITP to date patients can feel, post treatment, that a drug has not been as useful as advertised, but is not the case in advance of trying a new drug. In my experience patients are also willing to try new drugs that have a history of relative success

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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?

People who may benefit more from the technology would be:-

- Those for whom conventional ITP medication has not worked
- Those who do not want to self-inject other medication
- People who find hospital based treatment difficult, such as disabled patients, those caring for children during the day, those in full time work or education
- People who cannot use blood products for religious reasons

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.

I have used the following medication over the past decade:-

- Prednisolone steroid
 - Anti-D
 - Danazol
 - Rituximab
- I was also advised to have a splenectomy very early on in my treatment which, after internet research, I declined before asking for a second opinion.

I would also add that only prednisolone had an impact on the platelet count and I had zero reaction to the other drugs that I tried. In the end I was left for many years taking 15mg of Prednisolone a day, keeping my platelet count relatively stable at 15K however, the well-known short and long term side effects had me worried and I really wanted to have a higher count.

(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)

Self-administration saves time for both hospital and patient, and interferes less with daily life than hospital based treatment.

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Tablet form drugs are also easier than self-injections which, despite having done for years myself, is never easy or comfortable.

Side effects are also limited

(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).

I am unaware of any disadvantages

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.

I understand this treatment is not currently widely available on the NHS

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

I understand this treatment is not currently widely available on the NHS

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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.

The only research I am aware of was undertaken by the ITP support association and highlighted the fact many in the medical profession surrounding the treatment for this condition. This certainly rang true given my own experiences

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?

- Easy administration of drug
- Fewer hospital visits.
- As this is also a long term solution, once a patient has found a stable dose at which their symptoms are well managed, fewer regular check-ups are needed and the patient is more in control of their own treatment.

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

- Continued bruising and bleeding and trying to manage symptoms using current available treatment, which some suffers may not have positive reactions with
- Ongoing anxiety for patient and family
- Continued lengthy hospital visits
- The chance that future patients are advised to have a perfectly good spleen removed with only a mixed chance of positive result.

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

Not that I am aware of

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 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.

No comment to add

Other Issues

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

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