

## 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:**

██████████

**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? (please specify)
- ██████████ 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.

It will offer another drug to help raise the platelet count in ITP. No one treatment is successful at present, and many of the current treatments (including splenectomy) have serious side effects.

(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.

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 the 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 housebound. **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 can not always be done.

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

**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 their family (for example cost of travel needed to access the technology, or the cost of paying a carer).

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.

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

No, all correspondence from our members shows that they are desperate to see this technology licensed for general use.

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 say who will benefit as ITP is a difficult condition to predict which treatment will work for which patient. Those with the worst symptoms and lowest platelet counts will benefit the most.

### **Comparing the technology with alternative available treatments or technologies**

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

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

A vast array of treatments are used for ITP as no one treatment is effective for everyone. These include:

Prednisolone

IVIG

Anti-D

Dexamethasone

Splenectomy

Immunosuppressants

Rituximab (not licensed so not always available)

(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 in 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.)

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

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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 undertaken this operation.

(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).

No disadvantages known

### **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.

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?

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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 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..

**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?

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

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

**Other Issues**

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

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