Appendix G – patient/carer organisation statement template

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Single Technology Appraisal (STA)

Imatinib for the adjuvant treatment of gastrointestinal stromal tumours (review of TA196)

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: Barbara Doré

Name of your organisation: GIST Support UK (registered Charity 1129219)

Are you (tick all that apply):

YES 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)

I am the Chair of GIST Support UK, a GIST patient who has been taking Aduvant Imatinib on compassionate grounds for the past 6.5 years having had my primary GIST Tumour removed in 2007.
I offer my services to GIST Support UK on a voluntary basis.

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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.

- A significant reduction of the number of patients whose GIST recurs
Where adjuvant therapy is not applied, somewhere between 40/50% of patients who have had a resection experience a recurrence of GIST. Studies have shown that the use of Imatinib in an Adjuvant setting leads to a reduction in the number of patients who have a recurrence.

- A delay of the time to recurrence.
Patients who take Imatinib adjuvantly may experience a recurrence of their GIST at some point but the use of Imatinib adjuvantly delays the recurrence.

- Improved 1 and 5 year recurrence survival rates.
In particular those patients with an exon 11 mutation would benefit based on trial data.(Verweij et al,2004)

Those patients classified as intermediate and high risk would benefit from a reduction of potential risk of recurrence.

There is also evidence shows that GIST patients with KIT exon 9 mutations who are treated with 800mg of Imatinib rather than 400 mg experience an even greater progression free survival.

Mutational analysis can help determine a patients' likelihood of response to Imatinib and should be used for decisions concerning the use of adjuvant Imatinib.

- Imatinib is a GIST treatment that has been called "the wonder drug". It has set the standard that all other cancer drug developers are aiming to achieve for cancer therapy.

(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
Adjuvant use of Imatinib delays recurrence and reduces the number of patients having a recurrence of their GIST. Extending active life further to a cancer diagnosis is of immeasurable benefit.

- **physical symptoms**
oedema, rash and neutropenia are some of the side effects. All are manageable with standard medication and patients are happy to

manage these as Imatinib gives them confidence that their disease is being kept under control.

- **pain**
  Patients do not normally experience pain as a result of using Imatinib.

- **level of disability**
  Disability is minimal when using imatinib. With side effect management patients resume an active life both in employment and socially.

- **mental health**
  There is a reduction in stress and anxiety about recurrence both for the patient and their carer(s) when using Imatinib in adjuvant setting.

- **quality of life** (lifestyle, work, social functioning etc.)
  The ability to resume normal work and social interactions is of immeasurable value when having had a cancer diagnosis. The future had seemed totally without hope and then there is a drug that can shrink your tumour so that it can be surgically removed; after which it will stop the tumour growing back for a long time or maybe even never. How can there be any question that it will not improve quality of life?

- **other quality of life issues not listed above**
  Total improvement of family life and work life and a benefit to carers and patients. Stress reduction for everyone involved.

- **other people** (for example family, friends, employers)
  All as above and employers will be very pleased that their valued employee with many years of skills can be productive in the workplace and resume their normal place within the company.

- **other issues not listed above**

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**
  none

- **difficulties in taking or using the technology**
  none ,this is an oral anti cancer Therapy

- **side effects (please describe which side effects patients might be willing to accept or tolerate and which would be difficult to accept or tolerate)**
  The main side effects are oedema, rash and neutropenia which can all be managed with standard medical treatment and intervention.

- **impact on others** (for example family, friends, employers)
  none, only benefits

- **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)
  This is an oral anti cancer drug and has limited financial impact on the patient and or, their families.

3. Are there differences in opinion between patients about the usefulness or otherwise of this technology? If so, please describe them**.**
The majority of patients have expressed a wish to have adjuvant treatment available routinely. Post operatively patients are concerned about recurrence and the clinical data available indicates a substantial reduction in recurrence in the 5 years post operatively.

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?

- Patients who are at intermediate or high risk of recurrence will benefit and particularly those with an exon 11 mutation.

-There is also evidence shows that GIST patients with KIT exon 9 mutations who are treated with 800mg of Imatinib rather than 400 mg experience an even greater progression free survival than they would on 400 mg.

The effect of adjuvant Imatinib on KIT/PDGFRA Wild-type GIST may be variable so clinical decision making should be individualised according to the patients molecular data.

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**.**
There are no other adjuvant treatments available for GIST patients in the UK.

(i) Please list any current standard practice (alternatives if any) used in the UK. -
There is no standard adjuvant treatment currently available in the UK (except through the cancer drug fund). In the absence of drug treatment patients in the UK have no alternative but to watch and wait with the continual fear of recurrence of their GIST. Currently the Cancer Drug Fund funds 8-10 new

patients per month. We have not heard of any relapses of patients while on adjuvant therapy in our group. We have approximately 23 members on therapy.

(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**
  A delay in the number of patients having a recurrence and a subsequent reduction in utilising NHS resources.

  A reduction in the number of patients having a recurrence and a subsequent reduction in utilising NHS resources.

- **improvement in certain aspects of the condition**
  The alleviation of stress regarding the potential for recurrence together with possibility of resuming normal day to day activities would be a substantial improvement over the current situation.

- **ease of use** (for example tablets rather than injection)
  as this is an oral treatment it is very patient friendly

- **where the technology has to be used** (for example at home rather than in hospital)
  Imatinib can be taken wherever the patient is, making it both patient friendly and cost effective.

- **side effects** (please describe nature and number of problems, frequency, duration, severity etc)
  As with most TKI's there are some side effects but most of them are well known and manageable by health care professionals.

(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**
  not applicable

- **worsening of specific aspects of the condition**
  not applicable

- **difficulty in use** (for example injection rather than tablets)
  not applicable

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- **where the technology has to be used** (for example in hospital rather than at home)
  not applicable

- **side effects** (for example nature or number of problems, how often, for how long, how severe**).**
  Side effects are largely unpredictable and vary hugely from patient to patient. All are manageable with standard treatments and medication.

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.

- Many UK patients have been involved in both clinical trials and access via the cancer drugs fund. Their experience is that the treatment is hugely beneficial. It is also recommended by the European Sarcoma Group guidelines for GIST. Here are the ESMO Guidelines as last published in 2012: http://www.esmo.org/Guidelines-Practice/Clinical-Practice-Guidelines/Sarcoma-and-GIST/Gastrointestinal-Stromal-Tumours

I was invited as a patient representative to the most recent ESMO meeting to discuss the next edition of these guidelines in December 2013. The discussions did not centre on whether to use adjuvant therapy, but rather, adjuvant therapy is accepted Europe wide as standard treatment for high and intermediate risk patients, and the discussions centre on which of these patients would benefit most. To this end mutation analysis is recommended as essential in determining the use of adjuvant therapy – some mutations being insensitive and not requiring any.

Are there any adverse effects that were not apparent in the clinical trials but have come to light since, during routine NHS care? –
None

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. **YES, please see below**

www.esmo.org/Guidelines-Practice/Cli...ma-and-GIST/Gastrointestinal-Stromal-Tumours

Published in 2012 – Ann Oncol 2012; 23 (Suppl 7): vii49-vii55. Authors: The ESMO / European Sarcoma Network Working Group

Adjuvant imatinib mesylate after resection of localised, primary gastrointestinal stromal tumour: a randomised, double-blind, placebo
Dematteo et al
Ann Oncol. 2012 Nov;23(11):2776-81. doi: 10.1093/annonc/mds198. Epub 2012 Jul 25.

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Adjuvant therapy in primary GIST: state-of-the-art. Ann Oncol. 2012 Nov;23(11):2776-81. doi: 10.1093/annonc/mds198. Epub 2012 Jul 25.

Reichardt P, Blay JY, Boukovinas I, Brodowicz T, Broto JM, Casali PG, Decatris M, Eriksson M, Gelderblom H, Kosmidis P, Le Cesne A, Pousa AL, Schlemmer M, Verweij J, Joensuu H.

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?

- Improved quality of life.
-Ability to conduct life relatively "normally"
-Reduction in mental stress post resective surgery.
-Prolonged recurrence free survival.
-The ability to plan for the future

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

-Ongoing worry and fear about their health and the risk of recurrence.
-likey recurrence of their GIST
-reduced life expectancy

Are there groups of patients that have difficulties using the technology?
None

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;
None

- 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;
None

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

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None

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

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