****

**Improved methods and actionable tools for enhancing HTA**

**Template for Adaptation by HTA Bodies**

**Patient group submission form**

**for re-appraisal of a rare disease treatment**

**after an Outcomes-Based Managed Entry Agreement**

**March 2021**

*This is a template for a patient group submission form for re-appraisal of a rare disease treatment after an Outcomes-Based Managed Entry Agreement (OBMEA). It may also act as a topic guide for HTA bodies who wish to interview patients or run focus groups or workshops with patients in advance of a re-appraisal.*

*As patient groups representing rare diseases have limited capacity, and the focus of the re-appraisal is on patients treated in the OBMEA, patient groups may be encouraged to work together to make a joint submission.*

*This template uses terminology that comes from the* [*IMPACT HTA Template for OBMEA*](https://www.impact-hta.eu/work-package-10) *and should be adapted to suit the healthcare system.*

*This template may be suitable for treatments in more prevalent conditions but some prompts may be less relevant.*

*The basis for the development of this template is shown on the final page.*

*Black text in yellow highlights – explanatory text in the template to be deleted*

*Red text – details to be completed*

*Green text - alter as appropriate*

**This page should be deleted**

**Patient group submission form**

**for re-appraisal of a rare disease treatment**

**after an Outcomes-Based Managed Entry Agreement**

**HTA committee name**

**Treatment X in Disease Y**

**Purpose of this form**

This form has been designed to help patient groups share patients[[1]](#footnote-1)’ and carers[[2]](#footnote-2)’ experiences of receiving rare disease treatment in the Outcomes-Based Managed Entry Agreement (OBMEA)[[3]](#footnote-3). We need information about what difference the treatment has made that will not be captured in the main OBMEA analyses.

This patient group submission will be considered as part of the HTA committee re-appraisal of rare disease treatment. The HTA committee report will document how the information from patients was considered in developing our recommendation/ decision.

The OBMEA has been undertaken to collect additional data in country to resolve important “uncertainties” (gaps in knowledge) about the use or value of the treatment that were identified in the original appraisal <Link to OBMEA data collection document>, <Link to original appraisal report>[[4]](#footnote-4).

This submission form provides a tool for patient groups to add new insights to those already provided in the original appraisal, based on the real-life experiences of patients who received treatment as part of the OBMEA and their carers and family members.

This form has seven sections:

|  |  |
| --- | --- |
| 1 | Background information about the patient group |
| 2 | An overview of how information was gathered from patients and carers |
| 3 | What it was like for patients, carers and families to live with this condition before treatment – focussing on issues not explained well in the original appraisal. |
| 4 | Any issues that arose in accessing treatment or data collection in the OBMEA |
| 5 | What difference did treatment make to patients, carers and families - focussing on aspects that are not addressed in the formal analysis of OBMEA data – such as changes to daily life. |
| 6 | Additional information (e.g. including relevant patient aspects from patient groups in other countries if that is easily available). |
| 7 | Key points for the appraisal committee |

Each section has a series of prompts in a box that are intended to assist you in providing information that will be helpful to the HTA committee. The prompts are just examples of areas you might want to cover. You do not have to address all the prompts; focus on the ones you feel are appropriate and add other issues and information you think is relevant.

Appraisal committees appreciate balanced information that seeks to present all types of experiences with the treatment – benefits and challenges. This helps the committee determine how the treatment can be used optimally in the health service in future. The committee is less convinced by selection of only positive experiences, so try to be as balanced as possible in the information you provide.

If you have any questions when completing this form, please contact

<NAME, PHONE, EMAIL – Contact person from HTA organisation>

**1. Information about your group**

**Name of group:**

**Key contact name:**

**Role:**

**Email:**

**Phone:**

**Postal address (if applicable):**

**Website address (if applicable):**

**Type of group (tick all that apply):**

Registered charity

Fellowship

Informal self-help group

Unincorporated organisation

Other, please specify\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_

**Purposes of group (tick all that apply):**

Advocacy

Education

Campaigning

Service provision

Research

Improving patient care

Other, please specify\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_

**Describe the conditions/diseases covered by your group, number and range of people represented, regions covered, demographics etc.**

**Was your group involved in the original HTA committee appraisal for Treatment X?**

**YES/NO**

**How has your group been involved in the OBMEA for Treatment X (tick all that apply)?**

Not involved

Design of the data collection agreement

Member of committee to regularly monitor progress

Scoping/agreeing the terms of re-appraisal

As a contributor of data

Other, please state\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_

**In line with how we treat other stakeholders, we ask you to complete our declaration of interests.**

**Are you willing for this submission to be shared on our website after removal of financial information and personal information that could identify patients?**

**YES / NO**

**We may invite you to meetings that contribute to the re-appraisal of this treatment. Would a member of your group be willing to participate in such meetings?**

**YES / NO**

**2. Sources of information provided**

**2a. How did you gather information about the experiences of patients and carers to include in this submission?**

|  |
| --- |
| 300 words maximum  *How did you gather information from patients, carers and families in the OBMEA?*  *If you used any of the following methods (or something else) briefly describe what you did, when and how many patients were involved for each activity:*   * *individual discussions* * *group workshop* * *web-based survey* * *focus group* * *interviews* * *patient-report outcomes in the OBMEA* * *review of patient diaries* * *patient stories* * *patient data about clinic visits* * *a discussion in a closed social media group with or without polls*   *Provide links to any reports.*  *There is no right or wrong answer, this information just helps the committee understand the basis for what you present.*  ***Refer to these information sources when you make statements in the following sections to indicate where your information comes from.***  *If you got information from other international sources, briefly describe them:*   * *a national group from a different country that has information about patient experiences with the treatment* * *social media* |

**2b. Did anyone help you prepare this submission? YES / NO**

**If yes – who helped you and in what way?**

(e.g. survey commissioned and analysed by a contractor/pharmaceutical company/academic institution)

**3. Impact of condition**

3a. How does the condition affect patients?

Answer any prompts you think are relevant, or provide other information not related to these prompts.

Provide clear facts, information and summaries to give a concise and balanced overview of a range of patients’ and carers’ experiences.

State the source of your information.

|  |
| --- |
| (500 words maximum)  *This is your opportunity to highlight the important aspects of the illness for patients.*  *Questions to consider in your response*  Compared to the description of the nature of the condition in the original appraisal report[[5]](#footnote-5), is there anything you would like to add or clarify about the patients who entered the OBMEA and what their life was like before treatment?  For example:   * Were different body systems affected (mobility, cognition, gastrointestinal, senses etc)? * Which aspects of the condition were most challenging? * What activities did patients find difficult, or impossible to do? * What symptoms did patients most want to control? * What functionality did they want to maintain (ability to use hands, ability to transfer from bed to wheelchair, vision/hearing level, etc) * What life-decisions had patients made as a result of their condition (schooling, work, family planning etc.)? |

**3b. How does the condition affect carers and families?**

|  |
| --- |
| (500 words maximum)  This is your opportunity to highlight the important aspects of the illness for carers and families.  Questions to consider in your response  Compared to the description of the impacts of the condition on carers and families in the original appraisal report[[6]](#footnote-6), is there anything you would like to add?  For example:   * Have any new insights from carers and families of patients treated in the OBMEA about living with the condition before treatment come to light, if so what? * What do carers have to do for the patient(s) on a daily basis (such as washing, lifting, avoidance of harm, treatment administration etc)? * What are the most challenging aspects for carers (e.g. physical challenges, emotional effects, anxiety, guilt, sleep deprivation, stress, depression, need to give up work, costs of caring, need to strictly adhere to treatment timetable limiting other activities, impact on social life)? * Who was involved in the care and support of the patient before they had treatment in the OBMEA? (Consider impacts on extended family, friends etc). * What are the hard or demanding things carers/families don’t complain about? * What are the challenges faced by the family (e.g. impacts on family life for siblings; genetic implications – observing a family member with a condition you know you have, decision whether to have further children; effects of living with a loved one who has a limited lifespan) |

**4. Practical issues in the OBMEA**

4a. Were there any issues related to patients entering the OBMEA?

|  |
| --- |
| (300 words maximum)  *Questions to consider in your response*   * *Were there any challenges for patients doing the assessments required for eligibility?* * *Could all eligible patients across the country access treatment via the OBMEA? If not, who couldn’t and why?* * *Do you know why some patients decided not to enter the OBMEA (and not have the treatment)?* * *Were there any delays in obtaining treatment?* * *Did patients understand the consent agreements they had to sign, including what would happen after the re-appraisal?* |

4b. Were there any issues during the conduct of the OBMEA?

|  |
| --- |
| (300 words maximum)  *Questions to consider in your response*   * *Were there any challenges doing the assessments required to determine if treatment should continue?* * *Did patients miss clinic visits? If so, for what reasons?* * *Were there any challenges with data collection (e.g. late development of patient reported outcome measure, challenges with use of data collection app, measurement of endpoints that didn’t seem relevant for certain patient groups)?* * *Did you feel that patients’ experiences (both physical and psychological) were captured adequately in the OBMEA data collection? If not, what was missed?* * *Were any major changes made during the OBMEA as a result of patient feedback?* |

**5. Experiences with the treatment in the OBMEA**

**5a. What were patients’ experiences of the treatment?**

Focus on areas where you think the data collection in the OBMEA may have been weak or missed aspects that are important to patients.

See if you can address any of the “uncertainties” (gaps) in the original appraisal.

|  |
| --- |
| (500 words maximum)  *This is your opportunity to highlight the important aspects of the treatment for patients, both good and bad.*  *Questions to consider in your response*   * *What were patients’ expectations of treatment?* * *Did the treatment outcomes meet patients’ expectations?* * *What tangible impacts did it have (e.g. improvement of a disabling symptom, stabilisation of the condition, maintenance of mobility, reduction in other medications, fewer emergency hospital visits, more days in school/work etc)?* * *What less tangible impacts did it have, e.g. on mental health or social aspects?* * *Were there unexpected benefits that were not reported/discussed in the original appraisal[[7]](#footnote-7)?* * *Were there side effects that were not listed on the patient information leaflet?* * *How were side effects managed?* * *Were side effects acceptable or did they lead to treatment discontinuation?* * *What were patients’ views of the side effects?* * *Were there any challenges taking this treatment alongside the other care and management necessary for the condition?* * *Were there differences in benefits or side effects across different types of patients?* * *Did patients stop treatment for reasons other than not meeting the continuation criteria?* |

**5b. What difference did the treatment make to carers and families?**

|  |
| --- |
| (500 words maximum)  *This is your opportunity to highlight how carers’ and families’ lives changed after the patient received treatment, both good and bad.*  *Questions to consider in your response*   * *What were carers’ and families’ expectations of treatment?* * *Did the treatment meet carers’ and families’ expectations?* * *Did the patient’s treatment impact their carer(s)? (time taken to provide care and accompany patient to clinic visits, altered need for physical support/lifting, sleep pattern, ability to work, engage in social activities, engage with other family members)* * *What less tangible effects did it have on the emotional aspects of carers everyday lives?* * *Were there unexpected impacts that had not been discussed/considered at the original appraisal[[8]](#footnote-8)?* * *Were side effects manageable?* * *Were costs to the family for managing the patient’s condition altered (e.g. transportation costs, care costs, supportive therapy costs)* * *How did life change for the extended family (impact on siblings, grandparents etc)?* |

**6. Additional information**

Additional information you believe would be helpful to the HTA body reviewers and committee**.**

|  |
| --- |
| (500 words maximum)  *Provide any reflections about the OBMEA that you think are important.*   * *Was the information gathered in a robust manner that should allow a fair re-appraisal of the treatment?* * *Were the right outcomes collected?* * *What gaps in evidence might still remain?* * *From the experiences of patients in the OBMEA, do you have any thoughts on how the treatment might be best used in routine practice if it is approved?*   *Other international patient insights on the treatment*   * *Information from other patient groups internationally about experience with the treatment*   + *Do your insights tally with these?*   + *Was there a difference in the way the treatment was given (dosing, type of patients etc)?* |

**7. Key messages**

Section moved to top when presented to HTA Committee

List the most important points for the committee to consider.

|  |
| --- |
| Maximum 5 statements  *For example*   * *Was the OBMEA setup to capture outcomes that matter to patients?* * *Were patients/carers happy with the way the OBMEA was run?* * *What were that biggest challenges faced by patients entering the OBMEA?*      * *What impacts did treatment in the OBMEA have on patients’ daily life – (provide a balanced view)?* * *Were there unexpected benefits to patients?* * *Were there benefits for carers?* * *What did patients, carers and their families find difficult about the treatment?* |

**Acknowledgements**

This form has been adapted from a template developed in the IMPACT HTA research project funded by a European Commission H2020 grant.

**Background to the development of this template**

Karen Facey is a Co-Principal Investigator for IMPACT HTA Work Package 10: Appraisal of Rare Disease Treatments ([karen.facey@ed.ac.uk](mailto:karen.facey@ed.ac.uk)). She developed this template by adapting the:

* HTAi Patient Organisation Submission for Medicines (2014)
* NICE Patient Organisation Submission for Elosulfase Alfa re-appraisal (February 2020).

Changes were made to take account of experiences shared by rare disease patients and their carers, which were provided in the IMPACT HTA ethnographic study of appraisals and OBMEA meetings at the National Institute for Health and Care Excellence and the Scottish Medicines Consortium.

Major adaptations were made to a draft document following feedback from the following experts who have experience of patient involvement in HTA, but KF takes responsibility for the final version.

Ivett Jakab, Health Economist, Head of Patient Policy Research, Syreon Research Institute, Hungary

Jonathan Kingsley, Parliamentary and Policy Manager, Muscular Dystrophy, UK

Heidi Livingstone, Public Involvement Adviser, National Institute for Health and Care Excellence, UK

Eric Low, Owner, Eric Low Consulting Ltd, UK

Alex Morrison, Head of Research and Medical Communications, Rare Disease Research Partners, UK

Jean Mossman, IMPACT HTA Advisory Board, International Consultant on Patient Involvement in HTA, UK

Liz Ryburn, Support Services Manager, Spinal Muscular Atrophy, UK

Ann Single, Patient Voice Initiative Coordinator, Australia and Chair of HTAi Patient and Citizen Involvement Interest Group

Mandy Tonkinson, Public Involvement Adviser, National Institute for Health and Care Excellence, UK

1. In all parts of this form the term “patient” refers to anyone living with, or who has lived with, the condition for which the new treatment is indicated. [↑](#footnote-ref-1)
2. Carers are sometimes called informal/unpaid care-givers, they are often family members. [↑](#footnote-ref-2)
3. A range of different terms exist for this nationally such as Coverage with Evidence Development, Performance-Based Risk Sharing Agreement, Managed Access Agreement. [↑](#footnote-ref-3)
4. If you require help understanding terms relating to the appraisal, please refer to the [HTAi glossary](https://htai.org/wp-content/uploads/2020/08/HTAi-Patient-and-Consumer-Glossary-Aug-2020.pdf) for patients or visit the training resources on the HTA body website or [EUPATI](https://toolbox.eupati.eu/search-toolbox/category/health-technology-assessment/). [↑](#footnote-ref-4)
5. Link to specific report section [↑](#footnote-ref-5)
6. Link to specific report section [↑](#footnote-ref-6)
7. Link to specific report section [↑](#footnote-ref-7)
8. Link to specific report section [↑](#footnote-ref-8)