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COMPLETING A PATIENT GROUP SUBMISSION TEMPLATE: GUIDANCE FOR PATIENT ORGANISATIONS

For Health Technology Assessment and Appraisal of Medicines

Prepared by HTAi Patient and Citizen Involvement in HTA Interest Sub-Group, 2014

VERSION 1

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A Note about this Guide

This guide is adapted, with permission, from the pCODR guide-- *A Guide for Patient Advocacy Groups: How to provide patient and caregiver input for a pCODR drug review,* found at: [www.pcodr.ca/idc/groups/pcodr/documents/pcodrdocument/pcodr-patient-guide.pdf]

The pCODR Guide was created through a joint collaboration between pan-Canadian Oncology Drug Review (pCODR) and the Canadian Cancer Action Network (CCAN) that provides a united Canadian voice on patient issues. Development of the guide was led by the Li Ka Shing Knowledge Institute at St. Michael's Hospital in Toronto.

The HTAi Patient and Citizen Involvement in HTA Interest Sub-Group has developed this guide. It worked with Mona Sabharwal (pCODR) to create this generic version of the pCODR guide to support the use of the HTAi Patient Group Submission Template.

The Patient Group Submission Template was developed through a project led by Karen Facey and funded by an unrestricted grant from Eli Lilly.

These documents are intended to be adapted by HTA organisations to suit the needs of their HTA processes and the communities they work with. This may include changing the spelling used in the document, adapting terms or translating the document into another language. We would appreciate you sharing adaptations with us so that we can share these with others who may have needs similar to yours.

INTRODUCTION

What is the purpose of this guide?

This guide is designed to help patient groups like yours complete the Patient Group Submission Template. You will find guidance on what information we are looking for within the template and how to collect and report that input. While this guide provides guidance on collecting and reporting information from surveys and interviews, patient groups should not feel that they must undertake a survey or conduct interviews to successfully complete the submission template. Patient groups may already possess the information required to complete the template.

How to use this guide

Please use the sections that are most relevant to you.

This guide includes four sections:

Part 1. 'The HTA Process and Patient Group Submission Template' gives an overview of the HTA process and how your submission will be used in the assessment of the medicine and to make our recommendations. You may find this section particularly helpful if this is your first submission.

Part 2. 'Planning for Your Submission' helps you think through the steps involved in completing a submission.

Part 3. 'Methods to Collect Patient and Caregiver Input' explains some ways to collect patient and caregiver input; including using surveys and interviews.

Part 4. 'Summarising and Reporting Responses' describes how to write up the input you have received from patients and caregivers, and enter it into the submission template.

Terms used

Further explanations of HTA terms can be found in the HTA Glossary for Consumers and Patients (http://www.htai.org/fileadmin/HTAi_Files/ISG/PatientInvolvement/Glossary/HTAiPatientAndConsumerGlossaryOctober2009_01.pdf)

Comments and questions

If you have any comments or questions about the guide, please contact the HTAi Secretariat: info@htai.org or local HTAi organization (contact details).

PART 1: The HTA Process and Patient Group Submission Template

1.1 What is an HTA?

HTA stands for Health Technology Assessment. This is a systematic process that can involve a review of the clinical evidence compared to existing models of care, cost effectiveness, and social and ethical impacts of a health technology (such as a medicine) on the health care system and the lives of patients. Its main purpose is to inform decision making by health care policy makers. The process advises whether or not a health technology should be used, and if so, how it is best used and which patients will benefit from it. Assessments vary, but most look at the health benefits and risks of using the technology. They can also look at costs and any other wider impacts that the technology may have on a population or on a society.

1.2 What is a Patient Group Submission Template?

To conduct an HTA, the HTA agency [could insert name of agency] considers submissions that include scientific evidence, economic evidence, information on the way services are currently organised, and patient input for a medicine under consideration. The Patient Group Submission Template (Appendix 1) provides an important vehicle for patient organisations to provide suitable patient input for the assessment of a particular medicine. Strong submissions provide clear facts, information and summaries of experiences to give a concise, accurate and balanced overview of a range of patients', and where appropriate caregivers' (carers), perspectives.

The purpose of the patient group submission is to identify the priorities and preferences of patients. Submissions can highlight important aspects that are:

- not identified or well presented in the published literature, or
- not well captured in quality of life measures or other outcome measures that have been used in clinical trials and other research studies, or
- not well understood by experts in HTA.

1.3 How do patient and caregiver experiences add value to the review process?

Understanding the experiences and perspectives of patients and their care givers is key in making recommendations for medicines under review. Patients can provide unique knowledge about what it's like to live with a condition and can explain advantages and disadvantages of therapies that may not be available in the published literature or captured by quality of life or other known measures. Your efforts in collecting patient and caregiver experiences will provide valuable information that could have an impact on the HTA agency's final recommendations on the medicine being assessed. It's important for the HTA agency to understand what matters most to patients (and their caregivers) when they make recommendations about health care.

1.4 What is involved in contributing to the HTA review process?

To be part of the HTA review process, some agencies require that you are registered with them. Check with the agency first by going to its website, or contact the named lead for patient involvement, before you initiate your submission. Your patient input should be provided by completing the template on [provide link to template on HTA agency website]. The specific template for an assessment may also be sent to you by email when your submission is invited. The information you collect and submit will be used by the assessment team along with other information to prepare its reports and by the appraisal committee to develop its recommendations.

The review process is an evaluation of clinical and economic evidence, and is designed to include input from: clinicians/specialists, pharmaceutical manufacturers, and patient groups. As a patient group, your role is to provide patient and caregiver experiences by completing the Patient Group Submission Template. The input you provide is collated and summarised together with other patient submissions to provide a patient/caregiver perspective in the assessment process and committee deliberations. As there are variations in the processes used by HTA agencies, you should contact the named lead for patient involvement, or check the website of your HTA organisation, for a description and requirements for its process.

1.5 What information should you include in your submission?

The HTA organisation and relevant committees want to understand the experiences of those living with or caring for people with the health condition for which the medicine being assessed is used.

To help you provide the most useful information, Table 1 offers suggestions on what to include in your submission and things to consider when presenting your information. It is helpful to look at the Patient Group Submission Template (Appendix 1) while considering the information in this Table.

For examples of helpful submission responses, please see Section 4.3.

1.6 What information is not necessary?

We are aware that your time is valuable and there is limited space in the template, therefore, we want to help you focus on what is most useful to the medicine assessment process. Table 2 lists the information collected from other sources. **You do not need to provide** this input in your submission.

Table 1: What to include in your submission

Section of the input template	Considerations
Information gathering	• Mention how information was obtained (e.g. online surveys, interviews, focus groups, etc.). • Include the number of participants. • HTA agencies are looking for **patient and caregiver experiences** and not references to literature or printed sources (e.g. statistics), since this type of information is already reviewed in other parts of the medicine review process.
Experiences patients have with this condition	• Report on the experiences of **many** of the individuals living with this condition, rather than exceptional cases.
Patients' experience with their current therapies	• Report on the **current** therapies (e.g. medicine therapies, surgery and other procedures, medical devices, radiation, physical therapy, rehabilitation, palliation) to understand whether all aspects of the patients' condition are being managed.
Impact on caregivers	• Report on how the patients' condition and treatment have affected the caregiver and their daily activities. • Report on how the way current treatments are given impacts on caregivers (e.g. hospital visits, tests, etc.)
What are the expectations for the new medicine?	• Keep in mind that this section is designed to be answered by patients who have <u>never used</u> the medicine being assessed. • Comment on the **anticipated** impact of the medicine being assessed and the desired outcomes of using this medicine as compared to their current therapy. • Explore whether patients are willing to live with some side and late effects in return for some benefits of the new medicine and, if so, which side effects. • Comment on patients' unmet needs on current therapies, and what major areas of change they would like addressed.
What experiences have patients had to date with the new medicine?	• This section is only to be completed by patients who <u>have</u> used the medicine being assessed (in the past or are currently on the medicine). • Some patients may currently be on the medicine being assessed. If this is the case, please describe their experiences in this section (not in 'What are the expectations for the new medicine?' of the input template). • The purpose of this section is to get a better understanding of the **advantages and disadvantages** of the specific medicine, and to learn how it has affected patients' quality of life/everyday life, patient reported outcomes.

Table 2: What you do not need to include in your submission

Not necessary	Reason
Scientific evidence	As part of the process for assessing the medicine, the assessment team conducts a thorough and systematic search for the available scientific evidence about the medicine; therefore, you do not need to provide this information. However, if you have views about the interpretation of a paper or a particular clinical trial, we would be happy to hear them.
Summarised or reworded information from sources other than patients or caregivers (such as, clinicians or other healthcare providers, manufacturers)	The purpose of the Patient Group Submission Template is to collect input from both patients and their caregivers. Input and feedback from clinicians and pharmaceutical manufacturers is received separately.
The same messages repeated under different template headings	Sometimes it may be difficult to assign information to only one section of the input template. Please ensure that you are answering the specific question under each section and not repeating information to 'fill up the space'. We want to ensure that only the most relevant input is obtained in order to guarantee the best recommendations possible for the medicine being assessed.

PART 2: Planning for Your Submission

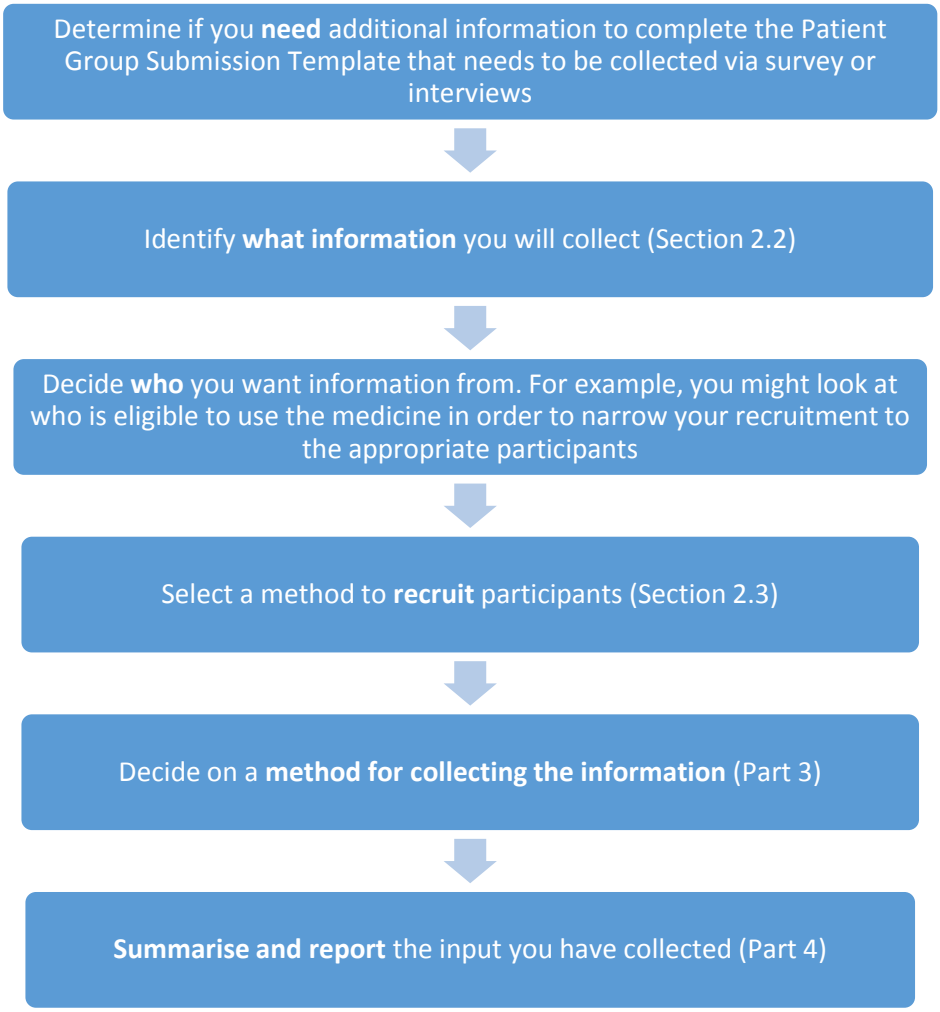
Completing a submission takes some time and effort, but it is an opportunity for you to provide valuable input regarding patient and caregiver experiences. Putting in time to plan your submission can help you be more efficient in collecting input and completing the template. During the planning phase you should determine whether you need to conduct a survey or interviews or whether you have the necessary information to complete the submission template.

> **TIP:** You can complete the Patient Group Submission Template based on information held by your patient group. The following sections of this guide are particularly relevant if you elect to conduct a survey or interview to collect information.

2.1 Steps to complete the input template

Start as early as possible. The HTA Agency may provide advance notice, via their website or an email, of when it plans to assess a medicine.

The following steps may help you plan your submission:



2.2. What types of information should you collect?

The type of information you collect will depend on the questions you want to answer. Information can be grouped into two categories: quantitative (numerical information) and qualitative (descriptive information).

Quantitative information is input that is either counted or measured, such as:

- **how much** time do you spend getting to your appointments?
- **how long** does treatment X work for?
- **how many** therapies have you been on?

It is also important to collect the thoughts, opinions, stories, and feelings of patients and caregivers. Usually this would be collected and reported separately. This input is described as descriptive (qualitative) information and answers questions such as:

- What challenges have you encountered while managing the side effects (which may be late in onset) for the person you are caring for (eg it is difficult to get to a hospital or that special equipment or specialists are required that are only available in particular centres)?
- Have you had difficulties in receiving or accessing your current treatment? Describe why.
- Can you describe how treatment X has or has not improved your quality of life?
- What aspects of living with the condition are the most challenging for you to manage?

Ways to collect qualitative information include using interviews or open-ended questions in surveys. These allow participants to explain their experiences in their own voice. More information and examples regarding open-ended questions in surveys and interviews can be found in Section 3.1 and Section 3.2.

TIP: Conserve your time

Many of the sections in the Patient Group Submission Template ask general questions about patient and caregiver experiences with a particular condition. Therefore, you only need to collect this information once and can use it in multiple submissions for medicines indicated for this disease or condition. One helpful strategy is to collect this information in advance then you only need to focus on the questions related to the specific medicine.

2.3 How do you recruit participants?

Recruitment takes time, so plan ahead and start early! Recruiting participants to share their experiences dealing with the condition and specific therapies can be challenging. Patient groups sometimes struggle to connect with patients who have a particular disease or condition, or who have used a specific therapy. It is also important to recruit individuals with different educational background, socio-economic status, geographic location, and age, for example, to see if the issues are common. Because the goal is to get feedback from a range of individuals, you may need to use several approaches to recruit participants.

You can use any combination of the following strategies to recruit participants.

1. Your patient group's database

   Send a survey or interview invitation out through your patient group's database, or put it in a newsletter or on your website.

2. Connect with external groups or partner organizations

   External groups or your partner organizations (such as umbrella groups, or those in other regions of your country) may be in regular contact with patients. Ask if they can help connect with patients who have specific conditions and their caregivers.

3. Use existing events, meetings, or conferences

   You may find a large pool of potential participants at already existing events, meetings, or conferences (e.g. a patient forum or education session). See if you can collect names for future recruitment or conduct interviews at the event.

4. Use social media

   Social media such as Facebook and Twitter can also assist you in reaching participants.

5. Ask the participants

   Once you have recruited some participants, ask them to forward the invitation to others who may be willing to participate. The principle behind this approach is that 'people know people like themselves' (this is termed 'snowballing').

6. Ask clinicians running clinical trials

   Subject to ethical approval and time constraints, clinicians running clinical trials may be willing to provide patients with a printed postcard containing your group's contact information and an invitation to provide their experiences living and dealing with the condition. Note this may be a time consuming approach.

PART 3: Methods to Collect Patient and Caregiver Input

Collecting patient and caregiver experiences involves a series of steps that vary depending on whether you use surveys, interviews, or both. **All information must be kept confidential by you to protect privacy.**

The input you collect may contain very sensitive and private information. Because of this, it may be hard for some participants to discuss their personal experiences. It is important for you to reassure individuals of the importance of their feedback to the input process and explain how exactly it will be used. Take care not to put them in a situation where they are uncomfortable to say no or to stop answering your questions. You should store all information securely, such as password -protected computer files or locking hardcopy documents in a cabinet. Make sure that all names, and other pieces of information that could be used to identify participants, are removed before including the information in the Patient Group Submission Template.

It is important that you respect your respondents, are truthful and fair with the information and how it is presented, and that you are acting to provide benefits to patients and caregivers.

The HTAi Patient and Citizen Involvement in HTA Interest Sub-Group is in the process of working with the HTAi Ethics Interest Sub-Group to develop guidance on these issues.

3.1 How can you collect input from surveys?

Use simple language. It is easier to answer questions that use simple and clear language.

Surveys are a great way to capture responses from a large group of individuals. Given timelines for receiving patient input are often short, web-based surveys (such as Survey Monkey, or Fluid Surveys) are the easiest to create and send out.

3.1.1 Types of question and response options

Survey responses are only as good as the questions asked, so spend some time thinking about the structure of the questions.

Survey questions are typically structured in two types of formats: closed or open-ended. **Closed questions** allow you to receive input/responses that participants select from a set of fixed or pre-determined answers. This type of information is usually collected if you wish to measure or count specific input; such as ‘how many times a year do you travel to receive your current therapy?’. These closed questions allow you to collect quantitative information (see Section 2.2).

If you choose to use closed questions, you will need to select which response options you want for each question. Several possible response examples are provided in Table 3.

Open-ended questions ask participants to provide a response in their own words; such as ‘please describe which side effects you would be willing to tolerate and why’. Open-ended questions allow you to collect qualitative information (see Section 2.2).

A survey can combine closed questions and open questions. For example, a closed question could be followed with ‘Please explain your answer’ to gather quotes and better understand the numerical results from the closed question.

TIP: Use simple language

It is easy to answer questions that use simple and clear language. Strategies to simplify your language are shown in Appendix 4 of the pCODR Guide for Patient Advocacy Groups, available for download from http://www.pcodr.ca/idc/groups/pcodr/documents/pcodrdocument/pcodr-patient-guide.pdf**.**

Table 3: Considerations for choosing response options for closed questions

Type of Response Options	Considerations	Examples
Named categories (e.g. gender, medical diagnosis)	• Do not overlap categories (i.e. do not state age categories as 20-30, 30-40; instead include 21-30, 31-40) • Include a 'choose all that apply' option where applicable. • Cover all possible categories or include an 'other' category (which you can ask people to detail, or not)	Under 21; 21-30; 31-40; 41-50; 51-60; 61-70; 71-80; 81-90; over 90 years of age
Scaled categories (e.g. frequency or intensity of side effects)	• Balance response sets (i.e. the middle option should be neutral) • It is advisable to include an 'I don't know' or 'not applicable' response option	1-Strongly disagree; 2-Disagree; 3-Neither disagree nor agree; 4-Agree; 5-Strongly agree Completely dissatisfied; very dissatisfied; somewhat dissatisfied; somewhat satisfied; very satisfied; completely satisfied

Some points you may want to consider when deciding whether to use open-ended questions are:

- Do you wish to collect quotes?
- How will you analyse responses? (Section 4.2 will describe analysis methods in more detail)
- Is the answer to any of your questions currently unknown? (Open-ended information allows you to collect information that you did not expect.)

3.1.2 Survey layout

Not only is it important to pay attention to the types of questions you ask, the answer options and wording, but the order of the questions affects how people respond as well. Consider the following:

- Begin with simple and relevant questions
- Group together questions of a similar type
- Provide instructions at the beginning of the survey and at any point where the survey format changes (e.g., from closed to open-ended questions)

- Always include the option to not answer a question or to stop answering the questions
- Provide broader questions first, followed by detailed questions
- Do not make the survey too long as participants may not complete the full survey
- Participants should be able to complete the survey in a reasonable time limit (15-20 minutes)
- If you are including questions such as participants' age or gender, these should be asked at the end of the survey
- Thank the participants for their time

Further help with surveys
See Appendix 2 and 3 of the pCODR Guide for Patient Advocacy Groups for sample surveys of patients and caregivers. Available for download from http://www.pcodr.ca/idc/groups/pcodr/documents/pcodrdocument/pcodr-patient-guide.pdf

3.2 How can you collect input from interviews?

3.2.1 How many interviews to conduct

Interviews allow you to get more detail from a small number of participants. There is no rule on the ideal number of interviews to conduct. Generally, you will find that after a certain number of interviews, similar information is being obtained from participants (i.e., participants are not providing new information). Typically this happens after about 6 to 8 people in a similar grouping have been interviewed. Your aim in interviewing is to explore issues in as much depth as possible. For information on recruitment see Section 2.3.

3.2.2 How to develop an interview guide

An interview guide is a great way for you to prepare broad, open-ended questions relating to topics or issues of interest for an interview (for information on open-ended questions, see Section 3.1). You can use this guide to capture each participant's unique experiences of the condition and therapies. Table 4 highlights some of the key considerations for developing an interview guide.

Table 4: Considerations for developing an interview guide

Length	**Avoid making the interview guide too long**. Do not plan to collect in-depth information on more than 12 issues in a single interview.
Order	**Sequence your questions in a logical order**. The order in which the questions are asked will affect responses. Begin with broad questions and move to more specific questions.
Sensitivity	Try to manage sensitivity by informing participants of areas to be discussed before the interview, and being clear about their right to not answer a question or to discontinue the interview. You should have a **strategy** in place to ensure participants are supported when discussing potentially sensitive information. If sensitive topics are addressed, they should be asked later in the interview when participants are usually more comfortable with the interviewer.
Prompts	**Prompts are used to clarify a point or to obtain more information**. If there is something you need more clarification on, don't hesitate to ask a participant to

	expand on their thoughts.
Open-ended questions	**Use open-ended questions to reveal what participants are thinking** and to encourage a detailed response.
Specific experiences	**Ask participants to think about specific experiences** to encourage concrete, specific responses about participants' feelings, attitudes, and opinions rather than popular opinion.
Avoid asking 'why?'	**Consider alternatives such as 'What are the reasons (why) you think that way?'** Using 'why' often puts participants on the defensive.
Avoid giving examples	**These may limit responses**, as participants may focus on the examples provided rather than their own ideas.
Self-awareness	**Be aware of your own assumptions and prejudices** and really listen to and take note of the responses.

3.2.3 Conducting the interview

When talking to people in an interview to gain insight into their experiences, it is important to gather as much information as possible from the participant. The interviewer helps to steer the direction of the discussion while giving the participant room to share his or her experiences in detail.

A checklist with the particular questions you are collecting information on can keep you on track. It is also helpful to audio record your interviews, in addition to having a note-taker present. This allows you to go back and recall what participants have discussed. It is very time consuming if you rely on the recording without taking notes. You will need to ask permission to record the interview and you will need to store the recordings securely.

> **TIP: Take notes during an interview**
>
> It is helpful for the interviewer to jot down notes as the participant speaks. Recording points that require clarification or possible probes (for expansion) will help the interviewer gather meaningful information and to capture direct quotes.

Depending on where you are in the interview (beginning, middle, or end), certain types of questions can help you obtain better quality responses. Table 5 outlines the different types of questions and offers examples of each.

Table 5: Types of questions to incorporate into your interview guide.

When to use the question	Example
At the beginning of an interview introduce the topic.	"Have you ever heard of treatment X?" "What sorts of comments have you heard about the treatment, either positive or negative?"
In the middle of an interview dive more deeply into the topic.	"In what ways do you think the treatment under review has improved your quality of life?" "Has the treatment lived up to your expectations?"
At the end of an interview see if anything was missed.	"Is there anything else you would like to say about your experience with treatment X?"
Throughout	Prompts, eg can you tell me more about that?

The following agenda may help you structure the interview.

1. **Introduction:** begin by introducing yourself and assure the participant that all comments will be confidential and that they have the right to not answer questions or end the interview.
2. **Purpose:** review the purpose of the interview and why you are interviewing the participant ('We are looking for a better understanding of…').
3. **Findings:** provide a brief overview of how the findings will be used (i.e., to inform the content of the submission template).
4. **Overview:** indicate how long the interview will last and the order in which questions will be presented (e.g., first we will ask about your experience with your current treatment and then about treatment x).
5. **Questions:** ask the questions in the interview guide.
6. **Conclusion:** thank the participants for their time and let them know how and when they can expect to receive feedback from their input.

Further help
You will also find a sample interview guide in Appendix 5 of pCODR Guide for Patient Advocacy Groups, available for download from http://www.pcodr.ca/idc/groups/pcodr/documents/pcodrdocument/pcodr-patient-guide.pdf

PART 4: Summarising and Reporting Experiences

Now that you have collected patient and caregiver experiences, the next step is to summarise and report these responses in the Patient Group Submission Template. The way you present this information will depend on the types of questions that you asked. Remember that the HTA assessment team and committee are looking for an overview of experiences or themes.

4.1 How do you summarise the information that can be presented in numbers?

This is termed quantitative information.

The quantitative information you collect will mostly come from closed questions used in your survey. To summarise data, it is helpful to combine responses as averages, frequencies or counts (i.e. number of people), or proportions (i.e. percentages). It is best to keep the statistics simple. Quantitative information can be presented in sentences or as a table. Depending on how much data you have, it may be easier to use a table which allows you to present a large amount of content in a small amount of space. These examples present both methods.

Example 1: Summarising quantitative information in text

Those who completed the survey ranked 'infections' as the most important, with 71.8% (total number participants = 22) rating it as 10, a 'very important' aspect of controlling xxx cancer. 'Infections' were followed by 'kidney problems', 'pain', 'mobility', 'neuropathy', 'shortness of breath', and 'fatigue'. In all cases, more than 50% of respondents rates these aspects as a 10, 'very important' to control. In all cases the rating average was greater than 8, which meant that all listed symptoms were considered important.

Example 2: Using tables to report quantitative information

Symptom or problem related to xxx cancer	% of respondents who rated a '10'	Number of respondents	Rating average
Infections	71.8%	22	9.17
Kidney problems	68.2%	21	9.06
Pain	64.3%	22	9.03
Mobility	59.7%	22	8.95
Neuropathy	56.7%	22	8.75
Shortness of breath	51.0%	20	8.42
Fatigue	50.9%	22	8.69

TIP: Use tables to present numerical information

Remember that when reporting experiences you are trying to capture the majority of input collected. Findings presented in a table are easy to read and allow a lot of content to be incorporated into a smaller space. (It is easier for people who are used to reading tables.)

4.2 How do you summarise the qualitative (descriptive) information (i.e. open-ended questions in surveys, and interviews)?

Regardless of how you collected input, patient and caregiver experiences need to be summarised. A great way to present descriptive information is to include quotes from participants. Before you choose quotes, it is important to analyse all of your qualitative information as a whole. If you begin by selecting random quotes you may not realise that there are specific themes that a majority of participants collectively discussed. Qualitative information can come either from:

- Responses collected through interviews; or
- Open-ended questions asked in your surveys.

TIP: Use the voice of the participant

Remember that findings should be in the voice of the participant e.g. what participants expressed, reported, said, described, etc. It should be made apparent that they were taken directly from the participants' experiences and are not the opinion of the interviewer.

4.2.1 Summary of the descriptive or qualitative analysis process

1. Do an initial read through of all documents (e.g. notes) to become familiar with the information.
2. During the second reading, highlight sections of text with a label that you think is relevant and representative of what is being said.
3. Once you have labelled your documents, look for common themes.
4. Review your themes. If some themes do not seem to fit on a second review, consider either reassigning the response or creating a new theme. Alternatively, it may become clear that several themes can be combined into a single idea (i.e. if they are ultimately getting to the same topic or point).

Example 3: Using quotes to support themes

For an overall theme called ‘accessibility’, with codes ‘distance to place of treatment’ and ‘financial burden’, a response may look like this:

Patients reported difficulties with respect to access to treatments. The most widely discussed factor that affected access to treatment was financial burden, given that the treatment was not covered by some private health plans. Some patients reported that the particular treatment was difficult to access because it was only available at a centre far from their home, which made distance an important factor that limited accessibility. As described by one patient:

> “*It’s frustrating that my therapy isn’t easier to access because I find that it is working well. I just get so tired having to drive so far to be able to receive my IV medicines at the hospital. This is costing me a lot of time and money, especially as we have to pay for some of my medications out of pocket.”*

4.3 Examples of how to report findings

To increase the amount of space you have to report responses, remove any instructions and examples provided in the Patient Group Submission Template.

You are now ready to present the patient and caregiver experiences in the template. Remember there is no right or wrong way to report your responses. Just remember to highlight important experiences from a group of participants, rather than exceptional cases. A good way to do this is to describe general trends and then present a quote to support the finding. This section provides sample responses from previous submissions organized in the order of the questions in the template:

- Information gathering
- Experiences patients have with this type of health condition
- Patients’ experience with current treatment
- Impact on caregivers
- What experiences have patients had to date with the new treatment?
- What are the expectations for the new medicine?

TIP: Be concise yet descriptive

It may feel like there is limited space to report responses, but this reporting structure ensures that you are being concise when providing meaningful and descriptive information. Also, you can shorten lengthy quotes by using ellipses (…) to omit phrases that may be less relevant.

4.3.1 Information Gathering

Example 4: Helpful response

Below is an example of a useful way to describe how you collected the information.

[Patient Group] conducted a participant [anonymous] online survey, which was sent by e-mail to xxx patients and caregivers across xxx who were on the [Patient Group] database. Respondents of the survey were from across xxx [xxx respondents were from outside of xxx.].

There were a total of xxx respondents; of this total, xxx were individuals living with the condition, and xxx were caregivers. A total of xxx respondents indicated that either they, or the person they provide care for, used the medicine under review for their condition.

This section can be short and concise. The most important information to get across is:

- The method used to collect patient and caregiver experiences; and
- The number of participants (divided into patients and caregivers) who were recruited (e.g. who were sent the survey), who participated, and who are on the treatment under review.

4.3.2. Experiences patients have with this type of health condition

Example 5: Helpful response

Below is an example of how you can use the information you have gathered from a survey to provide the assessment with an understanding of some specific impacts of the condition on patients' lives.

According to the survey, xx% of patients are negatively impacted by their [condition] in their day-to-day life. Only xx% indicated no major change. The respondents indicated that the biggest impact has been on their ability to work or volunteer (xx%). In many cases, individuals retired early or went on extended leave due to the increased fatigue and pain experienced living with the disease.

> *"Symptoms and problems at this time impact my day-to-day life and quality of life to a great extent. In the past xxx months I have found that I needed to build up stamina to cook and many times I overexert myself with any day-to-day housekeeping activities. I still need to rest for a minimum of 1-2 hours each afternoon and go to bed between 8 and 9 each evening. The limitations of this disease are frustrating and can bring about fits of depression."*

As a patient organization, you know how important it is to understand how patients are dealing with their condition on a daily basis. Your goal is to highlight how the diagnosis of the [condition] impacts patients' lives by emphasizing general trends and providing quotes, like in the example above. The focus is not to present information you would find in a textbook or scientific article. You are being asked to provide patient and caregiver experiences on a personal level.

The following is an example of what the HTA agency is **not** looking for:

Example 6: Unhelpful response

This [condition] is very common. The most recent statistics for [condition] are from xxx, where almost xxx people were diagnosed. Patients are generally diagnosed in their xxx's and xxx's—it is usually a disease of an older population though [condition] had been diagnosed in younger people. Children do not get this disease. This is a chronic, slow-progressing condition for many who are diagnosed; however, there are cases where the disease does progress quickly. People may live with disease for years before requiring treatment. While there are treatment options available that are life-extending for the patient, they do not cure the disease and relapse will happen. There are many genetic variables that help doctors predict how the disease will progress and how patients will respond to treatment.

4.3.3 Patients' experience with current therapies

This section should focus on experiences from patients who have never used the medicine under review.

Example 7: Helpful response

xx% (xxx respondents) of individuals living with [condition] and their caregivers indicated that they did experience some hardship in accessing treatment for [condition]. Hardships included:

- *the need to pay out-of-pocket for treatments*
- *the need to travel long distances to receive treatment*
- *the need to meet significant criteria to qualify for the treatment*
- *discontinuation of the treatment when the funding ran out*
- *lack of access through the hospital or private health plan to necessary treatment.*

Patients may be on one of many treatments, yet they may describe similar experiences across treatments.

4.3.4 Impact on caregivers

You should not report patients' perspectives of their caregivers' experiences

Example 8: Unhelpful response

Based on responses from xxx patients, the biggest impact on their caregivers' lives was daily routine or lifestyles (xx%). Often patients expressed that their caregiver put their life and needs on hold to be able to provide care for them. In many cases, the amount of time caregivers could spend with their children or other family members and friends. Patients expressed disruption to their caregivers' daily routine as their sole focus had been on them and helping them to manage their appointments, treatments, meals, and other personal care matters.

Caregivers' experiences are an essential component to understanding the impact of the therapies for the condition on the daily routines, quality of life, relationship with family/friends, and stress and mental health of those dealing with the condition and themselves. Being able to discuss these challenges is the key goal of this section. Usually caregivers put on a strong face in front of patients, in order to ensure that they are a stable form of support, so patients may not have an accurate description of their true feelings. It is best to ask these questions directly to caregivers rather than through the patient survey.

4.3.5 What experiences have patients had to date with the new medicine?

It is important to always provide the specified response under the correct section. Many patient groups tend to combine the expectations of the treatment under review with the experiences of those currently on the treatment.

Example 9: Helpful response

A total of xxx respondents had direct experience with the medicine under review, in which xx% were accessing it in a late line of treatment. xxx respondents were receiving it in the second line. When asked about the side effects experienced with the treatment, respondents mentioned fatigue, nausea, diarrhoea, and high blood pressure. In rating the side effects of the treatment, xx% of xxx respondents assigned a score of low to moderate (respondents in the range of: 1, no side effects at all, to 4) and xx% indicated that the side effects were debilitating (respondents in the range of 8 to 10, with one of these respondents (xx%) rating the side effects at 10, debilitating side effects that impact daily life). Of the side effects experienced, respondents indicated xx% were willing to accept them, xx% felt some were acceptable and others were not, one person (x%) had an adverse event and discontinued usage; xxx respondents did not answer directly.

The above example incorporates a number of points into a small paragraph. This response highlights:

- The number of respondents who had direct experience with the treatment under review
- The key side effects experienced by patients
- The percentage of respondents who were in each section of the rating scale

Example 10: Unhelpful response

Positive and negative effects are described above. The treatment manages to control tumour growth better than existing treatments thereby preventing potential blockages in the body. The treatment can cause reactions to develop in some patients. Hand and foot problems are acceptable, and grey hair is acceptable and a certain amount of fatigue is acceptable, and pain up to 5 or 6 out of 10 is acceptable. The treatment is easier than with some other treatments, and the patient can remain at home. The treatment can extend life for each patient and, for some, quite significantly.

The above example discusses experiences that were highlighted in previous sections, which is not useful for the current question. In addition, it is not clear how many participants felt a specific way since scales are not defined. As always, it is helpful to present rating scale definitions alongside scale values to ensure readers understand which extreme of the scale respondents are on (i.e. 1: no side effects versus 10: debilitating side effects).

4.3.6 What are the expectations for the new medicine?

If patients have no experience using the new medicine, you should report what their expectations are for it.

Example 11: Helpful response

- *In considering new treatments, xx% of the respondents (n=xxx) indicated that it is 'extremely important' to see an improvement in their condition (symptoms and signs).*
- *xx% of the respondents (n = xxx) indicated that it was 'extremely important' to realise an improved quality of life when considering a new treatment.*
- *When asked about whether it was important to evaluate the average period of the expected benefit, again, the respondents (xx%) (n = xxx) indicated an extremely high degree of importance to this decision.*
- *In considering a new treatment, xx% of the respondents (n = xxx) indicated that they were willing to tolerate a moderate to high severity of side effects (xxx respondents in the range of 5 to 10, where 10 = significant side effects).*

Bullet points are a quick and easy way to discuss a number of topics in a small amount of space. In the example, the patient group was able to describe the key expectations of the treatment under review, in combination with numerical information (e.g. percentages). It is helpful to include rating scales when reporting numerical information to help the reader understand the response.

Example 12: Unhelpful response

Input from patients without direct experience with the treatment under review indicated that patients are seeking treatments which can extend life expectancy, even if only for short periods of time. In addition, treatments with manageable side effect profiles that would not affect a patient's daily life would also be considered favourable to patients.

Patient input highlighted that the currently available treatments provide time-limited relief or that treatments are inconsistent in maintaining relief. Patients note that currently available treatments prolong life at best, however, provide very little or no increase in quality of life. Moreover, patients expressed concerns about secondary infections, risk of death, and other complications that may arise from invasive interventions.

At first, this example may seem appropriate, but it does not present information on the patients who responded (e.g. the number of patients) or how strongly they felt about the topics discussed. Without this information, it is difficult to understand the impact of the statement.

Appendix 1: Patient Group Submission Template (HTAi)

1. Purpose of this form

We recognise that patients can provide unique knowledge about what it's like to live with a condition and can explain advantages and disadvantages of therapies that may not be apparent in the published literature or quality of life measures. The submission process/form has been created to help patient groups provide information to the assessment of a particular medicine.

In a patient [group] submission it is important to provide clear facts, information and summaries of experiences to give a concise, accurate and balanced overview of a range of patients' and where appropriate care-givers' (carers') perspectives.

State the source of your information (e.g. web survey, helpline analysis, social networking, focus group, patients' records, one-to-one conversations, patient stories, etc) and provide clear references where they are available and any assistance you have received in preparing this submission.

For any of the sections, if there are issues that should have special consideration for a particular group, please indicate the specific needs/issues of this group (e.g. children, women/men, ethnic groups, those living in a particular location, those with other disabilities, disease sub-types, etc).

The term 'patient' refers to anyone living with, or who has lived with, the condition for which the new therapy/intervention is indicated. Caregivers have different needs and perspectives than the patients themselves.

2. How to complete this form

In the main sections of this form, you are asked to describe the challenges patients face in living with the condition being studied, experiences of using current therapies, expectations from the new medicine and, if you are aware, the potential benefit or drawbacks from the new medicine being assessed.

Each question has a series of prompts in a box that are intended to assist you in providing the information that will be helpful to HTA reviewers and committees in understanding the impact of the condition and its treatment. Please address any of the prompts that your group feels is important and describe any other relevant issues that are not captured in the list of prompts.

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 medicine is indicated.

Please provide clear facts, information and summaries of experiences to give a concise, accurate and balanced overview of a range of patients' and care-givers' (carers') perspectives/views. State the source of your information (e.g. web survey, helpline analysis, social networking, focus group, patients' records, one-to-one conversations with those in clinical trials, patient stories) and provide clear references where they are available.

There is no need to send us published scientific papers, as we already have access to those. However, if you have views about the interpretation of a paper about a particular clinical trial, we would be happy to hear them.

For any of the sections in the form, if there are groups that should have special consideration, please indicate the specific needs/issues of that group (e.g. children, women/men, ethnic groups, those living in a particular location, those with other disabilities, disease sub-types).

If you require help in understanding HTA related terms, please refer to the HTAi glossary for patients or visit the training resources on the HTAi website.

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

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

3. Information about your group

Name of group:

Key contact name:

Role:

Email:

Phone:

Postal address:

Type of group (tick all that apply):

- Registered charity ☐
- Fellowship ☐
- Informal self-help group ☐
- Unincorporated organisation ☐
- Other
  - Please state_____

Purposes of group (tick all that apply):

- Advocacy ☐
- Education ☐
- Campaigning ☐
- Service ☐
- Research ☐
- Other
  - Please specify_____

Describe your membership (number and type of members, region that your group represents, demographics, etc)?

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

Did anyone help you prepare this submission? YES / NO
If yes – who helped you and in what way?

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 where this HTA is to be discussed. Would a member of your group be willing to attend such meetings? YES / NO

4. Impact of condition

How does the condition or disease for which the medicine is being assessed, affect patients' quality of life?

Issues to consider in your response

- *Aspects of the condition that are most challenging (e.g. symptoms, loss of ability to work, loss of confidence to go out, inability to drive, social exclusion).*
- *Activities that patients find difficult or are unable to do.*
- *Aspects of the condition that are the most important to control (e.g. symptoms that limit social interaction or ability to work such as difficulty breathing, pain, fatigue, incontinence, anxiety).*
- *Support required for daily living (physical or emotional).*
- *Types of patients that are most affected by the condition (e.g. men/women, children, ethnic groups).*
- *Challenges in managing this condition when patients also have other medical conditions.*
- *What patients would most like to see from a new treatment (e.g. halting of disease progression, improvement in a particular symptom).*

How does the condition or disease affect carers/unpaid care-givers?

Issues to consider in your response

- *Challenges faced by family and friends who support a patient to manage the condition.*
- *Pressures on carers/care-givers daily life (e.g. emotional/psychological effects, fatigue, stress, depression, physical challenges).*

5. Experience with current therapies

How well are patients managing their condition with currently available therapies?

(Currently available therapies may include any form of medical intervention such as medicines, rehabilitation, counselling, hospital interventions etc. If no specific therapy is available that should be stated.)

Issues to consider in your response

- *Main therapies currently used by patients for this condition and how they are given (tablet,*

injection, physiotherapy, hospital check-ups, etc, at home, in hospital; dose and frequency, ease of access)

- *Extent to which current therapies control or reduce the most challenging aspects of the condition.*
- *The most important benefits of current therapies.*
- *The burden of therapy on daily life (e.g. impact at different stages of disease, interruption to work, stigma, clinic visits to receive infused medicines, need for weekly blood tests or describe a typical episode of therapy over a week or period of treatment).*
- *Side effects from the therapies that are difficult to tolerate.*
- *Concerns about long-term use of current therapy.*
- *If the current therapy is a medicine:*
  - *Challenges in taking it as prescribed (e.g. swallowing the pill, self-injecting, use of a device to deliver the medicine, taking after food, not being able to lie down for 30 minutes after taking medicine).*
  - *Ways in which the dosing is modified compared to what is prescribed (e.g. dividing the dose to avoid unwanted side effects, missing doses to fit into daily life).*

6. Experiences with new medicine being assessed

For those <u>with</u> experience using the new medicine, what difference did it make to their lives?

Issues to consider in your response

- *Advantages and disadvantages of the new medicine compared with current therapy.*
- *Ease of use.*
- *Symptoms that were improved with the new medicine and impact on quality of life.*
- *Impact of the new medicine on the use of healthcare services (e.g. fewer visits to hospital).*
- *Financial implications (e.g. cost of medicine, travelling costs, medicine administration supplies, days off work).*
- *Explain whether the full prescribed dose of the new medicine is generally taken and what factors have led patients to miss doses.*
- *Side effects from the new medicine that are difficult to tolerate.*
- *Extent to which the new medicine addresses patients' needs.*

For those <u>without</u> experience using the new medicine, but who are aware of the results of clinical trials about the new medicine, what are the expectations/limitations of it?

Issues to consider in your response

- *Perceived advantages and disadvantages of the new medicine.*
- *Whether the clinical studies have reported outcomes that are important to patients.*
- *Level of improvement patients would like to see.*
- *Impact the new medicine might have on use of healthcare services (e.g. fewer visits to hospital).*
- *Financial implications (e.g. cost of medicine, travelling costs, medicine administration supplies, days off work).*
- *The level of side effects that patients would tolerate for a given benefit.*
- *Groups of patients who might particularly benefit or who might benefit less from the new*

medicine than others.

- *Aspects of patients' needs or expectations that it is hoped the new medicine will address (explaining specific issues for particular stages of disease).*

7. Additional information

Please include any additional information you believe would be helpful to the HTA reviewers and committee (e.g. ethical or social issues).

8. Key messages

Section moved to top when presented to HTA Committee

In no more than five statements, please list the most important points in of your submission.

For example:

- *The biggest challenges of living with this condition are…*
- *Current therapies are inadequate because…*
- *This new medicine will be important for patients because…*

Appendix 2: Checklist for Confirming Completeness of Patient Group Submission Template

This checklist can be used to guide you in completing the Patient Group Submission Template and grouping the patient responses. Refer to the template for questions.

The purpose of the patient submission is to identify important aspects that are not well presented in the published literature and are not well captured in quality of life measures or other outcome measures that have been used in clinical studies; and to identify the priorities and preferences of patients. You may not be able to address all of the questions in this Checklist as the questions may not be relevant or you may not have the information.

Please provide the following information.

1.1. General information required for completing the submission

☐ Have you described your background (the person completing the form)?

☐ Have you described the membership of the group, if a group response (number and types of members, purpose, geographical region covers)?

☐ Have you described how you obtained the information for your submission, and over what time period?

☐ If required, have you provided a 'declaration of interests' form with regard to funding or assistance you receive from corporate bodies, government or other?

☐ If you received assistance in any way with completing your submission, have you stated this?

1.2. Patients' experiences with the health condition (Q4 of template)

☐ What are the signs and symptoms of the condition that are most challenging to patients (and how and why)?

☐ What are the activities that patients find most difficult or are unable to do?

☐ What are the aspects of the condition that are most important to control or manage?

☐ How does the condition affect patients' psychological/emotional well-being?

☐ What support is needed for daily living?

☐ What is the financial impact?

☐ What are the limitations in relief from the condition?

☐ Are there any unmet needs?

☐ Are some groups of patients affected more than others (and who)?

☐ How are patients affected by other conditions they may also have?

1.3. Patients' experiences with current therapies (Q5 of template)

☐ What are the main therapies for this condition, and how are they given?

☐ To what extent do the current therapies manage the condition?

☐ What are the most important benefits of the current therapies?

☐ What are the limitations of the current therapies, do they have unwanted effects?

☐ How easy are the current therapies to access and utilise, do they provide a burden on life?

☐ Do patients have concerns about long term use of the current therapies?

☐ Do the current therapies improve well-being?

☐ What is the financial impact of the current therapies?

☐ What are the unmet needs with the current therapies?

1.4. Impact on caregivers

☐ Do caregivers feel able to provide care?

☐ Are there any identifiable limitations in the care provided?

☐ What are the pressures on caregivers in providing this care?

☐ Other…

2.1. What are the expectations for the new medicine?

☐ What would patients most like to see from a new therapy?

☐ Have the clinical trials addressed the outcomes most important to patients?

☐ What are the perceived advantages?

☐ What are the levels of improvement patients are looking for?

☐ Is the new therapy easier to access and use?

☐ What are the perceived disadvantages (what unwanted effects would patients be prepared to accept)?

☐ What impact will the new therapy have on use of healthcare services?

☐ What will the financial impact of the new therapy be on patients?

☐ Will the new therapy benefit any particular patients?

2.2. What experiences have patients had to date with the new medicine? (Q6 of template)

☐ What signs and symptoms are improved by the new therapy?

☐ What is the level of improvement with the new therapy, are unmet needs met?

☐ Does the new therapy change disease progression or 'cure' the condition?

☐ What are the gains in health and well-being?

☐ What are the disadvantages of the new therapy (unwanted effects or harms)?

☐ Are these tolerable?

☐ How easy is the new therapy to access and to use?

☐ What effect does the new therapy have on use of health services?

☐ Are there any patients who would be expected to benefit more than others (and who)?

☐ What is the financial impact of the new therapy for patients?

Overall:

☐ Are there any ethical or social issues that you can see with the new therapy (and what are they)?

What are the 5 key messages in your submission?

1. _____
2. _____
3. _____
4. _____
5. _____

Appendix 3: Helpful resources

The following resources have been created to help patients and the public to be involved in HTA. All can be freely accessed at HTA Resources (http://www.htai.org/index.php?id=744)

Videos

- ***Introducing HTA to Patients & Patient Organizations***
  A 20 minute video presentation for patients, patient organizations, self-help groups, citizens' groups, filmed in Seoul June 2013
- ***eMEET***
  A training resource for patient advocacy groups giving an overview of medicines development, evaluation and assessment.

Publications

- ***Health Equality Europe: A Guide to Understanding HTA for Patients and the Public***
  Written for patient organizations who want to represent the views of patients but may not clearly understand what HTA is and how they can contribute (available in English, Spanish, Mandarin, Italian, Polish, Greek and Swedish)
- ***Guide for Patient Advocacy Groups to Provide Submissions for Oncology Drugs Undergoing HTA Review***
  Created by pan-Canadian Oncology Drug Review (pCODR)
- ***HTA Glossary for Consumers and Patients***
  HTA terms explained in plain language
- ***Values and Quality Standards for Patient Involvement in HTA***
  Values and quality standards to inform and guide patient involvement in HTA processes