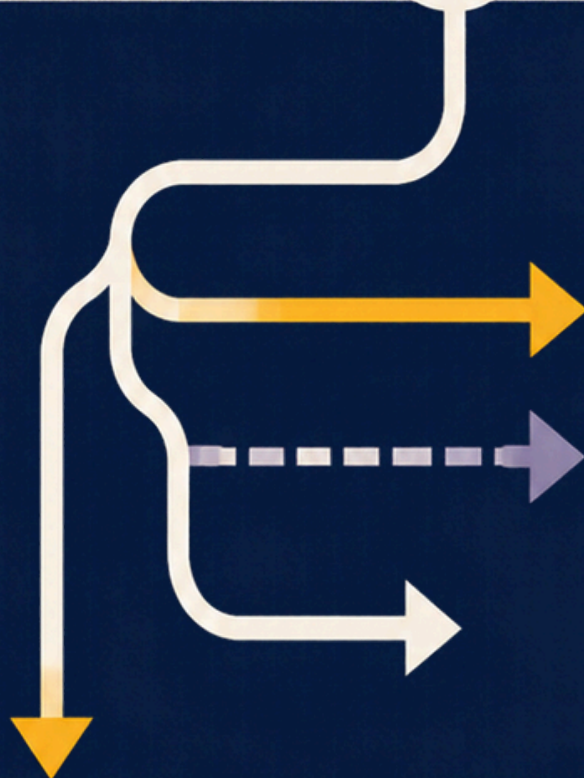


# Validating questions to ask patients in the context of HTA decision-making



This project aimed to identify key questions that are important to ask patients in the context of Health Technology Assessment (HTA) decision-making. The project was conducted in 4 parts.

Part 1: In October 2020, 15 patient organisations participated in an online workshop. Participants were asked to Imagine that nothing exists in relation to community/patient feedback and engagement in health technology assessment (HTA), i.e. that there is no community portal and no mechanism for feedback. This aimed to get participants to think outside of and not be constrained by the current system.

As a starting point, participants were asked 'What questions would you ask patients and families to understand what everyday life is like for them?' (The answers to these questions would be used to educate decision-makers on the impact of the disease/condition).

Part 2: Brainstorming sessions were held in Q2 2021 with stakeholders from industry, HTA agencies, and health economists. Stakeholders came from the UK, USA, Canada, Australia, South Africa, Netherlands, and Switzerland.

Part 3: In part 3 of the project, we refined and selected 10 questions to ask patients with the aim of validating the questions. The validation sought in this process was for understandability and the threshold for acceptability was whether the question had to be repeated more than 10% of the time for the participant to understand the question and provide a response.

Part 4: In part four of this project, we surveyed citizens and patients in three countries (UK, USA and Australia), to see how important engagement in decision-making is for the community in the context of HTA. This is an important step to test the assumption that the community wants to be involved in this process.

# Section 1: National Patient Organisation Network feedback

## Community-Driven evidence for Health Technology Assessment

In October 2020, patient organisations participated in an online workshop. Participants were asked to Imagine that nothing exists in relation to community/patient feedback and engagement in health technology assessment (HTA), i.e. that there is no community portal and no mechanism for feedback. This aimed to get participants to think outside of and not be constrained by the current system.

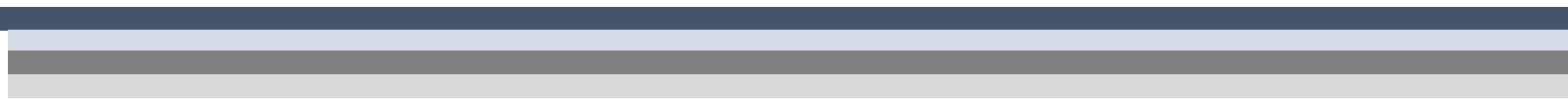
As a starting point, participants were asked 'What questions would you ask patients and families to understand what everyday life is like for them?' (The answers to these questions would be used to educate decision-makers on the impact of the disease/condition).

The second thing participants were asked is to think about their patient community and their everyday life. What are some of the things that would improve their quality of life? (This question aims to be demonstrative of quality of life and also lead us to develop a set of values important to patients and families.)

When responding to the question, we asked for a 'statement' and an 'impact'. For example: If our patients could have better upper arm strength, they would be able to prepare their own meals and do light housework. This would improve their quality of life as they would be able to be more independent.

From the workshop we have a preliminary list of questions that are important to the community in the context of HTA and these are provided below. In part two of the project, we have taken these questions to other stakeholders to test their relevance in the context of HTA and add to the list of questions.

- What needs to change to make life better?
- What do you need to do to get ready every day (Activities of daily living. Insert other aspects of ADLs)?
- What have you had to do to access treatments?
- What is life like with/without a treatment?
- What was life like when you've been on a treatment that starts to lose effectiveness? How did you feel about the opportunities to change to other treatments when this happened(s)?
- How would your life be different if you had access to information....repurposing of drugs.... clinical trials locally or overseas?
- How would my treatment (or treatment satisfaction) change if I was involved in the conversation (including with an MDT)?
- What activities are you excluded from because of your condition/illness?



## Section 2: Stakeholder consultations

In part 1 of this project, patient organisations identified the questions that they felt were important to ask patients in their communities, in the context of HTA decision-making.

In part 2 of this project, brainstorming sessions were held with stakeholders from industry, HTA agencies, and health economists. Stakeholders came from the UK, USA, Canada, Australia, South Africa, Netherlands, and Switzerland.

In the brainstorming sessions, stakeholders were presented with some of the key questions posed by the patient organisations and to review each of these. The review questions and responses are available below and were across the themes of:

- Impact on everyday life
- Treatments + symptoms
- Life with and without treatments (Benefits)
- Non-curative treatments
- Benefits of hindsight
- Limitations caused by their condition

#### Questions raised by APON access interest group

What needs to change to make life better?

What do you need to do to get ready every day (Activities of daily living. Insert other aspects of ADLs)?

#### Brainstorming questions

- What part/parts of everyday life are most important for those making decisions to know about?
- Any other questions within this domain
  - Nutrition: preparing food and what kind of food; preventative measures
  - Sleep: quality of sleep and quantity and impact
  - Fear of the unknown: What do patients do in situations when they are uncertain (Are there certain triggers)
  - What stops you from living (and doing everyday things)?
  - What worries you about going out (immunosuppression, flares, avoiding triggers)?
  - Ability to work
  - Social wellbeing: Impact on social networks, interactions and relationships
  - Identify the 'everyday' things that you need to do just to 'get you out of the door' in the morning

Figure 1: Questions about impact on everyday life

#### Questions raised by APON access interest group

What have you had to do to access treatments?

How do you manage your symptoms?

- Symptoms versus side effects

#### Brainstorming questions

What is important in relation to the pathway to treatment from a decision-making viewpoint?

How should symptoms and side effects be addressed? What's most important (considering it's often hard for a patient to differentiate?)

Any other questions within this domain

- Impact on household budget because of not being able to access a treatment or other sacrifices (Cost impact/ability to work)
  - Were any decisions that you've made in the past based on i) cost or ii) geographic location (inc. travel, parking)?
  - Were any treatments you've accessed been decided because of the clinical setting? (Accessibility)
  - What is your knowledge of current treatments that are available?

Identify different philosophies in different cultures/countries

Mode of administration: How acceptable it would be to patients if there is a new mode of administration?

- What support would be needed to administer and adhere to treatment?
- If there is a change in mode of administration – why it is/is not of 'value' to the patient (what could they do more/less of)
- What is the value of convenience? (Does this then translate to productivity/QoL/adherence + effectiveness?)

Figure 2: Questions about treatments and symptoms

| Questions raised by APON access interest group                                                                                           |
|------------------------------------------------------------------------------------------------------------------------------------------|
| What is life like with/without a treatment?                                                                                              |
| What was life like when you were first diagnosed compared to now?                                                                        |
| <b>Brainstorming questions</b>                                                                                                           |
| What timepoints are important if there are questions that differentiate life with/without treatment (including periods of surveillance)? |
| Any other questions within this domain – what should we explore in relation to benefits of treatment/services?.                          |

What timepoints are important:

- At what time in your experience (from diagnosis to now) have you felt most in control and most vulnerable?
  - Understanding also of patients that have not experienced treatment versus lived experienced
- If no treatment is available or it is not accessible, how do you manage your condition? (Unmet medical need)

Figure 3: Questions about life with and without treatments (Benefits)

| Questions raised by APON access interest group                                                                                                                                                       |
|------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|
| What was life like when you've been on a treatment that starts to lose effectiveness? How did you feel about the opportunities to change to other treatments when this happened(s)?                  |
| Where treatments are not curative, what do you want to see from new treatments?                                                                                                                      |
| <b>Brainstorming questions</b>                                                                                                                                                                       |
| 1. The initial questions focus on effectiveness and non-curative treatments. What other 'grey' areas (non-traditional clinical endpoints) might need to be covered?                                  |
| 2. The question about opportunities to change treatment aims to reduce assumptions about willingness to adapt to new treatments. Are there any other assumptions that need to be tested?             |
| 3. Any other questions within this domain                                                                                                                                                            |
| What is your understanding of the term "progression free survival"?                                                                                                                                  |
| What a person goes through day-by-day and how this relates to the clinical data (technical data):<br>e.g If I had a change in blood pressure; what would I be able to do more/less/how would I feel? |
| Can you describe any adverse events or severe side effects? (impact of treatments experienced)                                                                                                       |
| If treatments for only some or certain populations can be reimbursed, how would you manage your treatment? (age, disease population, stage of disease)?                                              |

Figure 4: Questions about non-curative treatments

| Questions raised by APON access interest group                                                                                      |
|-------------------------------------------------------------------------------------------------------------------------------------|
| How would your life be different if you had access to information.....repurposing of drugs.... clinical trials locally or overseas? |
| How would my treatment (or treatment satisfaction) change if I was involved in the conversation (including with an MDT)?            |
| <b>Brainstorming questions</b>                                                                                                      |
| 1. Are there other aspects of information, knowledge or opportunity that should be included?                                        |
| 2. Any other questions within this domain?                                                                                          |

- How would treatment have differed if the patient was in a different geographic location?
- Has the patient been able to access a specialist?
- How do cultural values and general knowledge impact decisions
  - What the community values in decision-making
  - Are there examples of better knowledge changing final treatment decisions based on values

Figure 5: Questions about benefits of hindsight

| Questions raised by APON access interest group                                                                                                                                                                                                                                                                                                                                                                                                            |
|-----------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|
| What activities are you excluded from because of your condition/illness?                                                                                                                                                                                                                                                                                                                                                                                  |
| <b>Brainstorming questions</b><br>1. The aim is to identify limitations caused by a condition/illness, but then there needs to be a value statement on what it would mean if that limitation is overcome. What questions might be important to demonstrate that value?<br><br>2. Any other questions within this domain                                                                                                                                   |
| <ul style="list-style-type: none"><li>- Questions about values and social determinants:<ul style="list-style-type: none"><li>- Having control over own health</li><li>- Human dignity</li><li>- Confidence and self esteem (mental health)</li><li>- Personal safety</li><li>- Determinants of health/Social value</li><li>- Establishing and describing the patient need for change. Show how the intervention makes an improvement.</li></ul></li></ul> |

Figure 4: Questions about limitations caused by their condition

## Section 3: Testing questions and testing assumptions

In part 3 of the project, we refined and selected 10 questions to ask patients with the aim of validating the questions. The validation sought in this process was for understandability and the threshold for acceptability was whether the question had to be repeated more than 10% of the time for the participant to understand the question and provide a response.

Questions were asked via telephone by a registered nurse or allied health professional who noted whether questions were answered and whether a prompt was needed.

314 people participated in the process. Inclusion criteria included being over the age of 18 and having a chronic condition that had been managed within the Australian health system. 81 participants were male and 233 were female. All participants resided in Australia.

The questions selected for validation were:

1. When you are making decisions about treatment, what are some of things you take into consideration when you are making decisions about treatment?
2. What are your own personal goals of treatment or care?
3. In relation to your condition, beyond being able to access treatments, what would you most like to see from new treatments?
4. Thinking back over your experience, is there anything that you know now that you wished you had of known earlier or at diagnosis?
5. Also thinking back over your experience, is there any aspect of your care or treatment that you would change?
6. Thinking back about your experience, has there been a time or any times that you have felt particularly vulnerable?
7. Has having [INSERT DISEASE] affected your quality of life and the quality of life of your family?
8. When you are using a treatment, what needs to improve for you to feel as though the treatment has worked?
9. If a treatment did [Nurse paraphrases response from above, for example reduce side effects, get rid of nausea] – what would that mean to you in your everyday life, that is, what would you be able to do that you couldn't do if the treatment wasn't doing its job?
10. What are some of the things that you need to do on a regular basis to control the effects of your condition so that you can do all of the activities you need to do every day?

Table 1: % of prompts required for each question

| Question | Asked and answered | Prompt required | % of prompts required |
|----------|--------------------|-----------------|-----------------------|
| 1        | 307                | 7               | 2.23%                 |
| 2        | 291                | 23              | 7.32%                 |
| 3        | 299                | 15              | 4.78%                 |
| 4        | 312                | 2               | 0.64%                 |
| 5        | 311                | 3               | 0.96%                 |
| 6        | 309                | 5               | 1.59%                 |
| 7        | 309                | 5               | 1.59%                 |
| 8        | 304                | 10              | 3.18%                 |
| 9        | 275                | 39              | 12.42%                |
| 10       | 307                | 7               | 2.23%                 |

From this process, the only question that did not pass the test for understandability was 'If a treatment did [Nurse paraphrases response from above, for example reduce side effects, get rid of nausea] – what would that mean to you in your everyday life, that is, what would you be able to do that you couldn't do if the treatment wasn't doing its job?).

## Survey results

## Demographics

In part four of this project, we surveyed citizens and patients in three countries (UK, USA and Australia), to see how important engagement in decision-making is for the community in the context of HTA. This is an important step to test the assumption that the community wants to be involved in this process.

### Age

Participants were aged over 18, most commonly between 18 and 59 (n=273, 90.70%)

### Gender

There were 147 male participants (48.84%), 154 females (51.16%) that completed the questionnaire.

**Table 2.1: Demographics**

| Demographics |         | All participants |                 | Australia |                 | United Kingdom |                 | United States of America |                 |
|--------------|---------|------------------|-----------------|-----------|-----------------|----------------|-----------------|--------------------------|-----------------|
|              |         | Number           | Percent (n=301) | Number    | Percent (n=100) | Number         | Percent (n=101) | Number                   | Percent (n=100) |
| Age          | 18 - 29 | 97               | 32.23           | 36        | 36.00           | 39             | 38.61           | 22                       | 22.00           |
|              | 30 - 44 | 123              | 40.86           | 41        | 41.00           | 46             | 45.54           | 36                       | 36.00           |
|              | 45 - 59 | 53               | 17.61           | 16        | 16.00           | 16             | 15.84           | 21                       | 21.00           |
|              | 60 - 75 | 23               | 7.64            | 7         | 7.00            | 0              | 0.00            | 16                       | 16.00           |
|              | 75 +    | 5                | 1.66            | 0         | 0.00            | 0              | 0.00            | 5                        | 5.00            |
| Gender       | Female  | 154              | 51.16           | 49        | 49.00           | 49             | 48.51           | 56                       | 56.00           |
|              | Male    | 147              | 48.84           | 51        | 51.00           | 52             | 51.49           | 44                       | 44.00           |

## Government making decisions about health

### Questions to ask people about everyday life

Participants were asked, if they or a family member were diagnosed with a serious condition or illness, and the government was making decisions about which treatments should be made available to, what questions would be important to ask you to inform those decisions?

This set of questions aim to help those making decisions understand what your everyday life is like.

For each question, participants rated how important each question was on a scale of 1 to 5, where 1 was not at all important to ask patients and 5 was extremely important to ask patients.

On average, participants thought it was very important to ask about:

- Daily activities to manage condition and maintain health
- Anything people are unable to do or are excluded from because of their condition
- How symptoms are managed
- What life would be like without access to treatment
- What life was life before diagnosis compared to now.

Participants were then asked to rank from the most important to least important questions to ask. The questions were ranked (from most important to least important) in the following order:

- Daily activities to manage condition and maintain health
- Anything people are unable to do or are excluded from because of their condition
- How symptoms are managed
- What life would be like without access to treatment
- What life was life before diagnosis compared to now.

**Table 2.2: Importance of questions about everyday life**

| Every day life                                                                           | Median (n=301) | IQR  |
|------------------------------------------------------------------------------------------|----------------|------|
| What do you need to do every day to manage your condition and maintain your health       | 4.00           | 2.00 |
| Is there anything that you can't do or are excluded from doing because of your condition | 4.00           | 2.00 |
| How do you manage any symptoms that you have                                             | 4.00           | 2.00 |
| What would life be like for you if you weren't able to access treatment                  | 4.00           | 2.00 |
| What was life like before you were diagnosed compared to now                             | 4.00           | 3.00 |

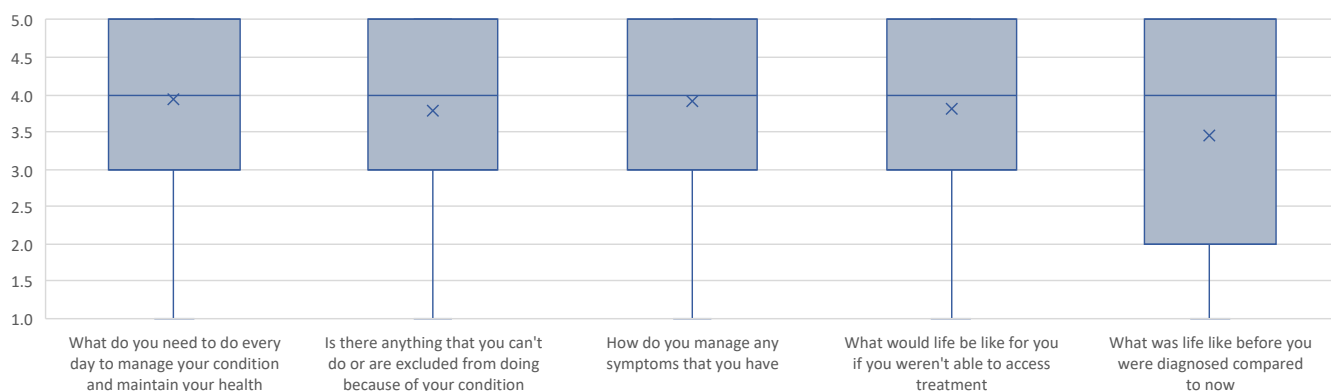


**Figure 2.1: Importance of questions about everyday life**

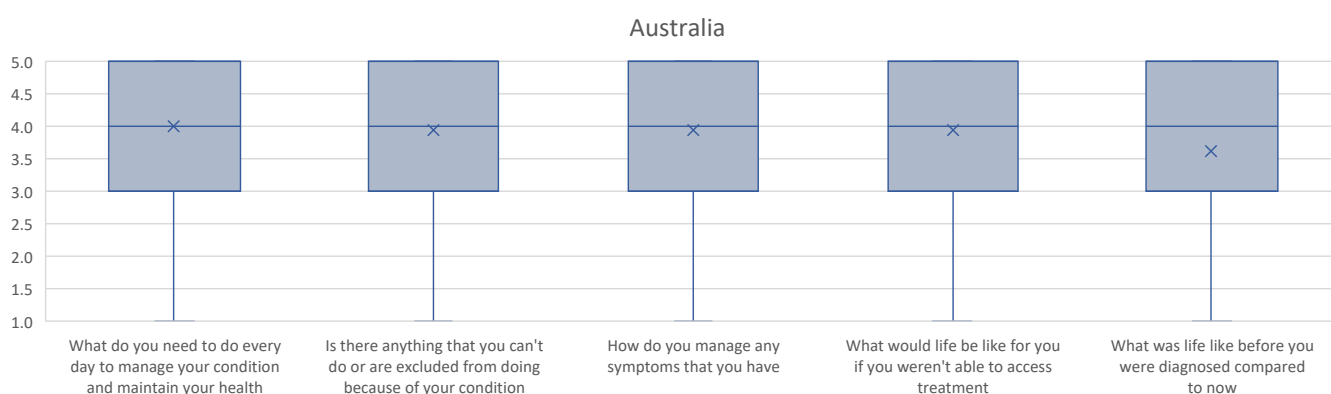


**Figure 2.2: Importance of questions about everyday life – Australian participants**

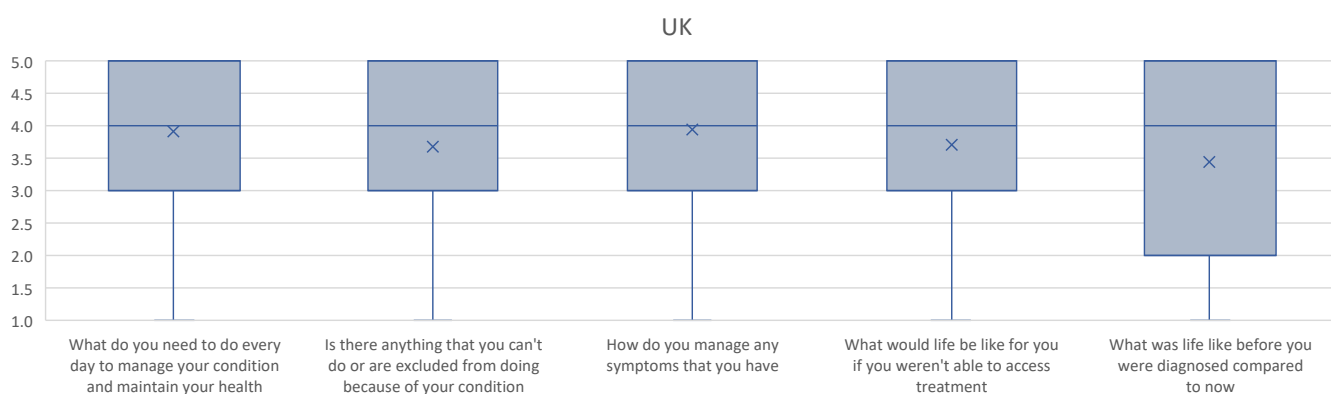


**Figure 2.3: Importance of questions about everyday life – UK participants**

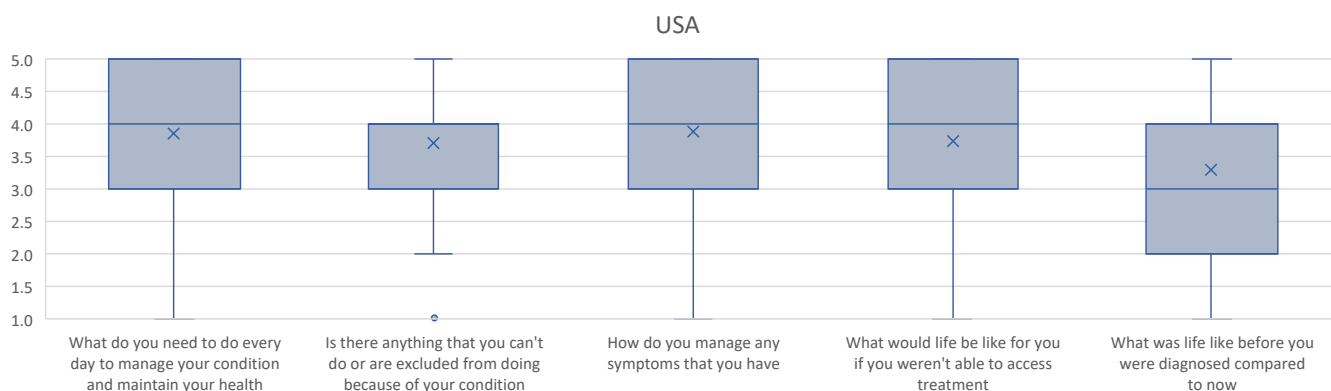


**Figure 2.4: Importance of questions about everyday life – USA participants**

**Table 2.3: Ranked importance of questions about everyday life**

| Every day life - Ranked                                                                  | Weighted average (n=301) |
|------------------------------------------------------------------------------------------|--------------------------|
| What do you need to do every day to manage your condition and maintain your health       | 3.82                     |
| Is there anything that you can't do or are excluded from doing because of your condition | 3.72                     |
| How do you manage any symptoms that you have                                             | 3.35                     |
| What would life be like for you if you weren't able to access treatment                  | 2.48                     |
| What was life like before you were diagnosed compared to now                             | 1.62                     |

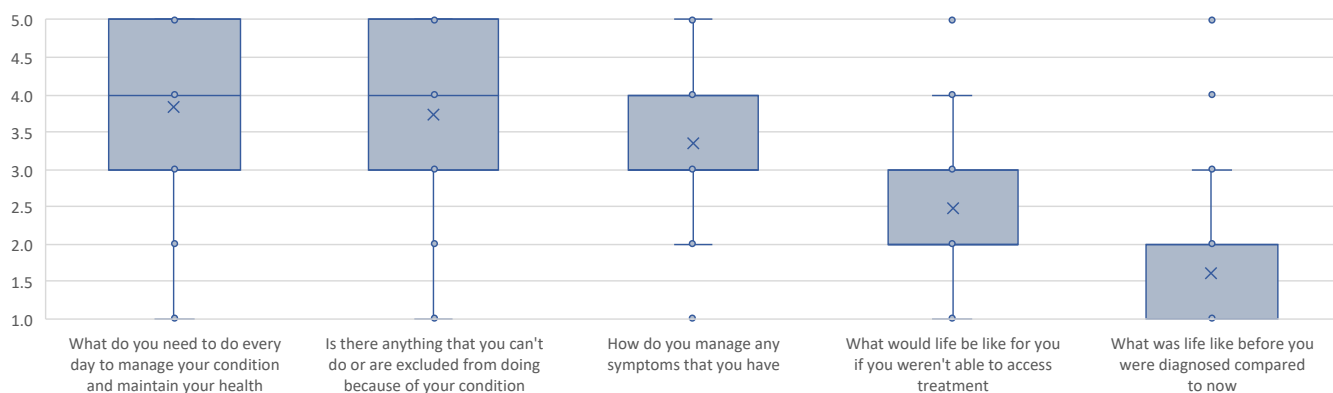


**Figure 2.5: Ranked importance of questions about everyday life**

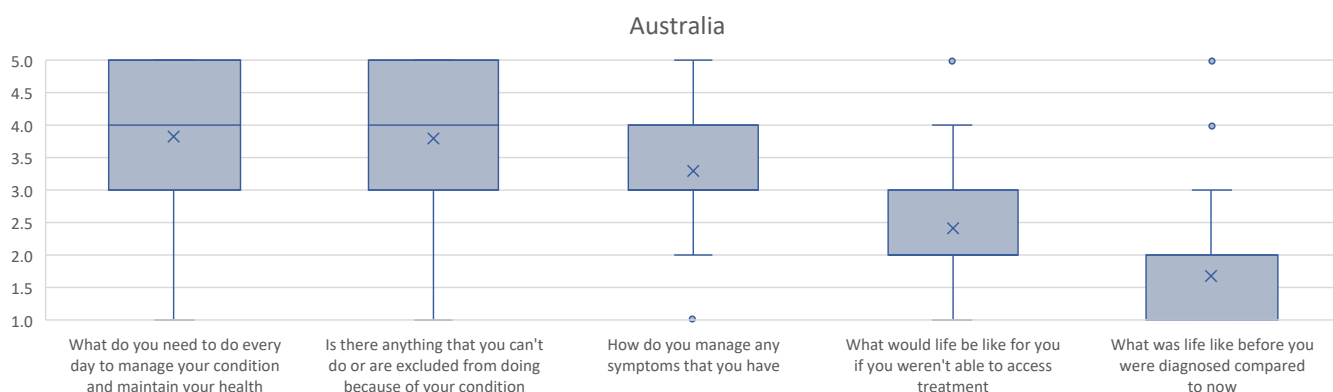


**Figure 2.6: Ranked importance of questions about everyday life – Australian participants**

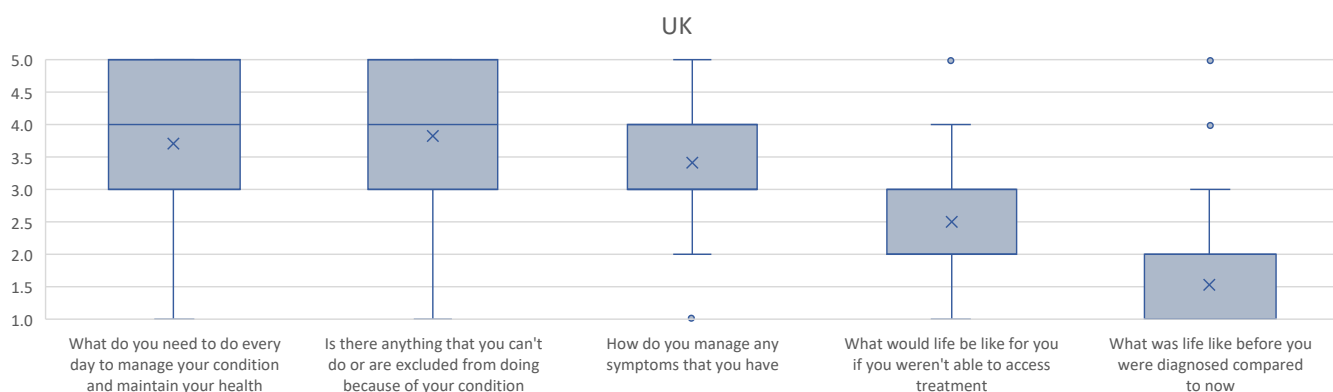


**Figure 2.7: Ranked importance of questions about everyday life – UK participants**

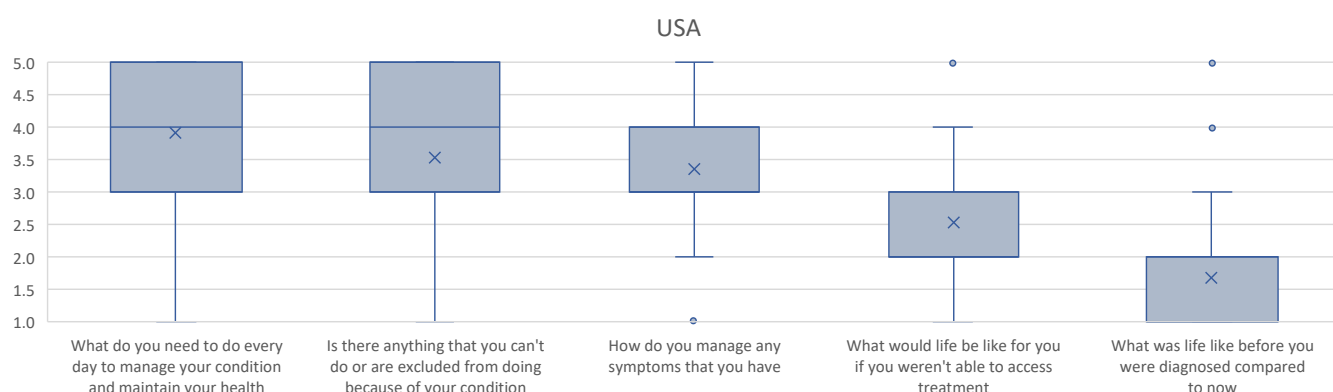


**Figure 2.8: Ranked importance of questions about everyday life – USA participants**

### Questions to ask about treatment access

Participants were asked, if they or a family member were diagnosed with a serious condition or illness, and the government was making decisions about which treatments should be made available to, what questions would be important to ask you to inform those decisions?

This set of questions was about the importance how treatments are access or how they should be accessed in the future.

For each question, participants rated how important each question was on a scale of 1 to 5, where 1 was not at all important to ask patients and 5 was extremely important to ask patients.

On average, participants thought it was very important to ask about:

- How easy or hard it was to access treatment
- When taking a treatment what needs to change to make life better
- What do they want from treatments for symptom management
- Access clinical trials available overseas
- Being involved in treatment discussions

Participants were then asked to rank from the most important to least important questions to ask. The questions were ranked (from most important to least important) in the following order:

- When taking a treatment what needs to change to make life better
- How easy or hard it was to access treatment
- What do they want from treatments for symptom management
- Access clinical trials available overseas
- Being involved in treatment discussions

**Table 2.4: Importance of treatment access**

| Access to treatment                                                                                                             | Median (n=301) | IQR  |
|---------------------------------------------------------------------------------------------------------------------------------|----------------|------|
| How easy or hard has it been for you to access treatments                                                                       | 4.00           | 2.00 |
| When you are taking a treatment, what needs to change to make like better for you                                               | 4.00           | 2.00 |
| Some treatments don't provide a cure, but do help to manage symptoms. What would you like to see from these kinds of treatments | 4.00           | 2.00 |
| Would life be different for you if treatments or clinical trials available from overseas were available to you                  | 4.00           | 2.00 |
| Would the way you feel about treatment options be different if you were involved in the discussions about treatments            | 4.00           | 2.00 |

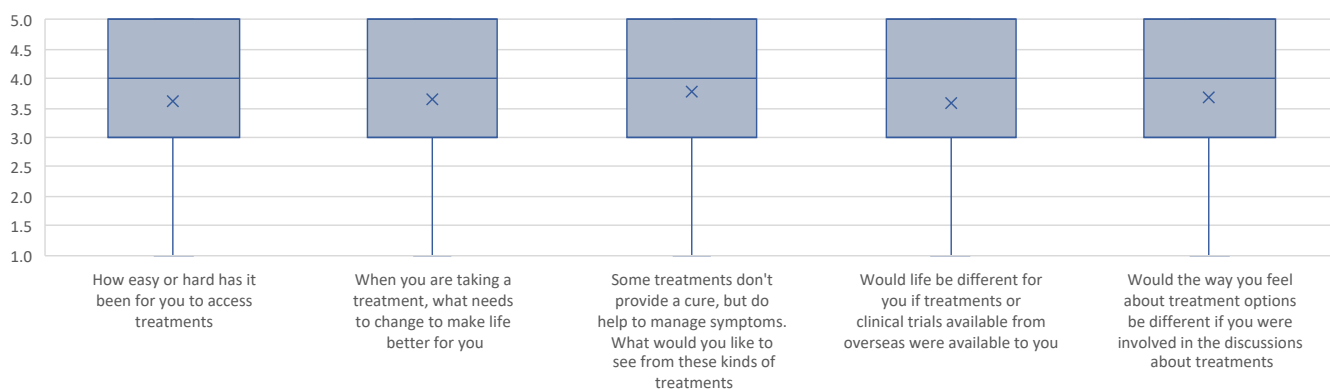


**Figure 2.9: Importance of treatment access**

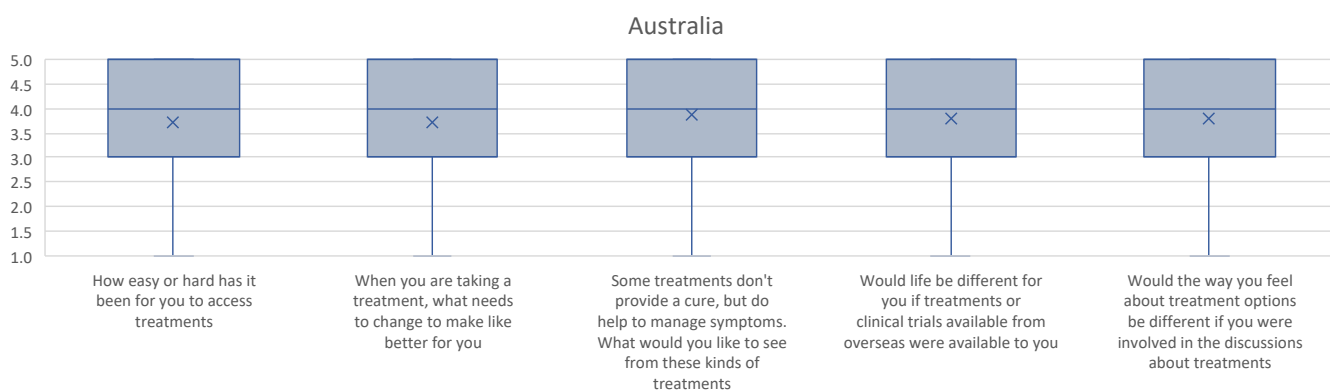


**Figure 2.10: Importance of treatment access – Australian participants**

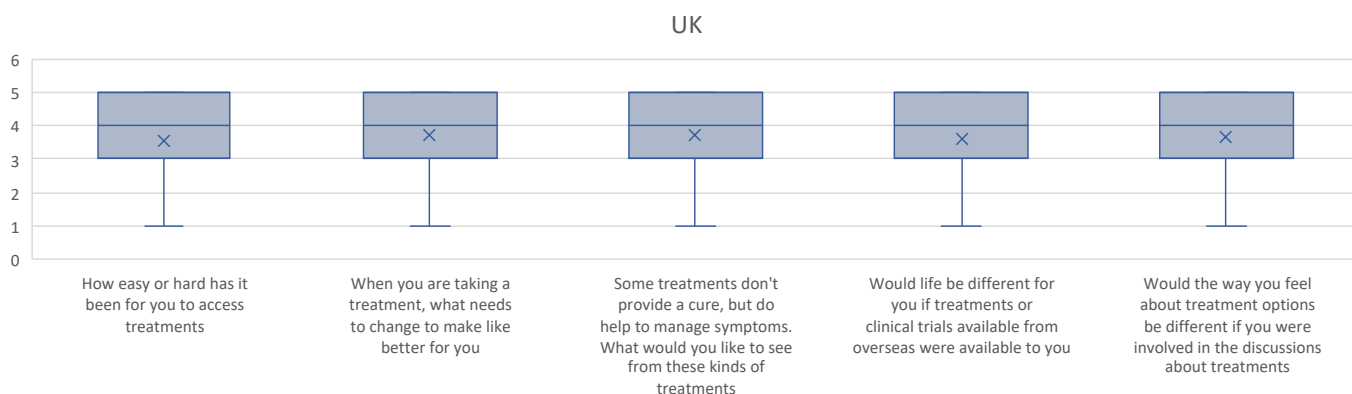


**Figure 2.11: Importance of treatment access – UK participants**

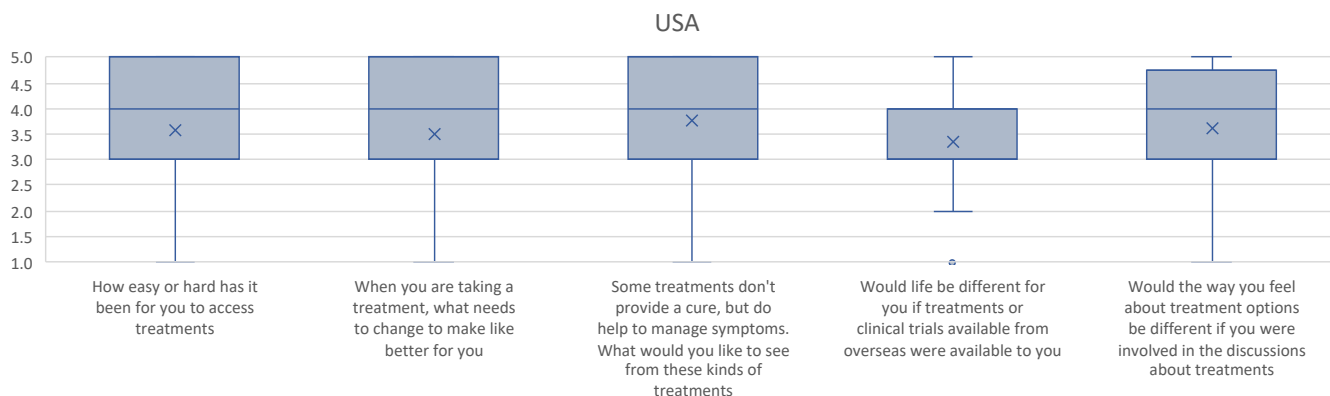


**Figure 2.12: Importance of treatment access – USA participants**

**Table 2.5: Ranked importance of treatment access**

| Access to treatment - ranked                                                                                                    | Weighted average (n=301) |
|---------------------------------------------------------------------------------------------------------------------------------|--------------------------|
| How easy or hard has it been for you to access treatments                                                                       | 3.69                     |
| When you are taking a treatment, what needs to change to make like better for you                                               | 3.85                     |
| Some treatments don't provide a cure, but do help to manage symptoms. What would you like to see from these kinds of treatments | 3.34                     |
| Would life be different for you if treatments or clinical trials available from overseas were available to you                  | 2.28                     |
| Would the way you feel about treatment options be different if you were involved in the discussions about treatments            | 1.84                     |

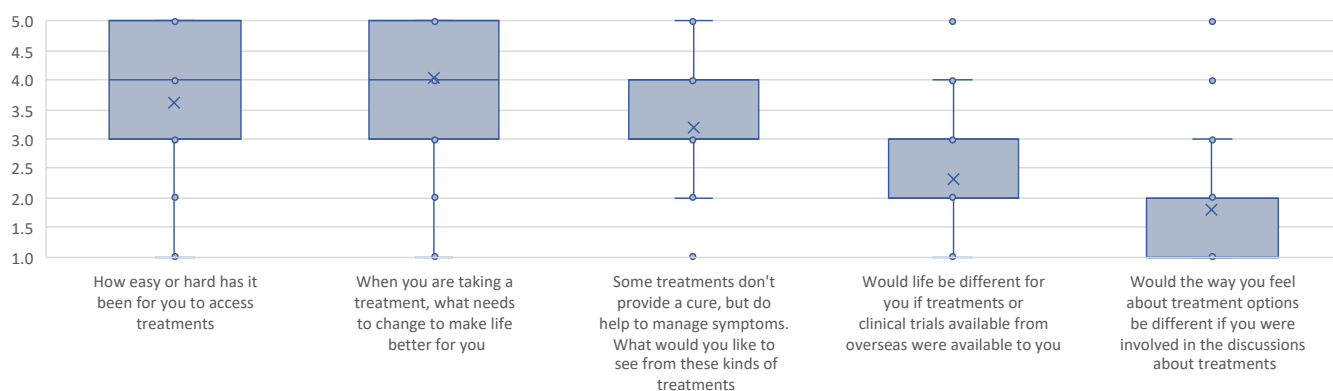


**Figure 2.13: Ranked importance of treatment access**

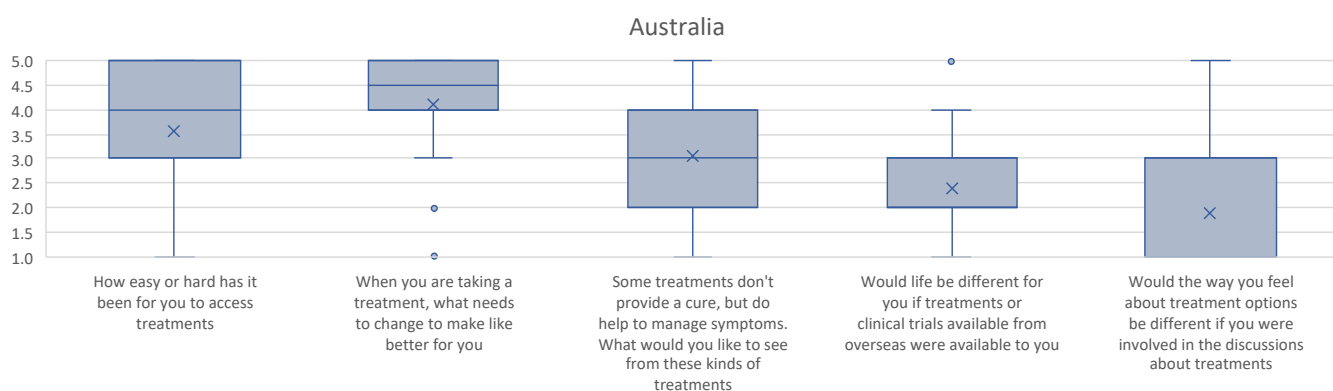


**Figure 2.14: Ranked importance of treatment access – Australian participants**

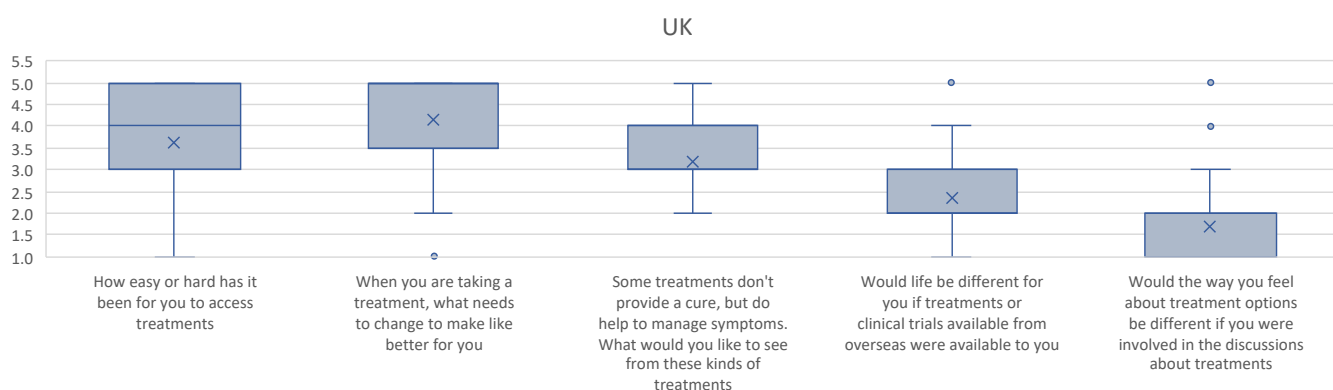


**Figure 2.15: Ranked importance of treatment access – UK participants**

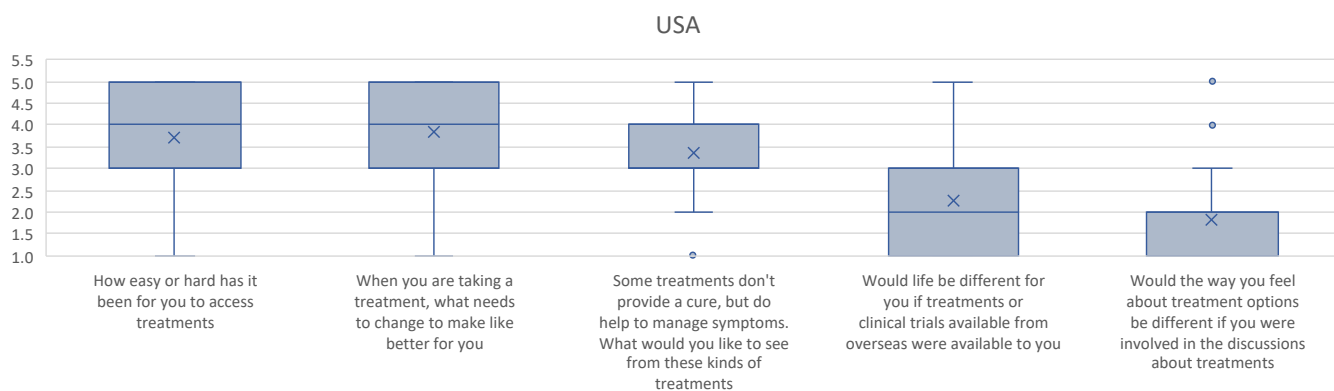


**Figure 2.16: Ranked importance of treatment access – USA participants**

## Patient involvement in decisions at different timepoints during treatment development

### Patient involvement in decisions at different timepoints during treatment development

Participants were asked to rate how important they think it is for patients to be involved in decisions at the various points in time during treatment developments?

For each question, participants rated how important each question was on a scale of 1 to 5, where 1 was not at all important to ask patients and 5 was extremely important to ask patients.

On average, participants thought it was **very important** for patients to be involved:

- When treatments are being reviewed for safety and use within a country
- When governments and pharmaceutical companies are discussing which treatments should be subsidized within a country.

And that it was **important** for patients to be involved:

- When research is being developed in the laboratory before it gets to clinical trials
- When clinical trials are being developed and designed

Participants were then asked to rank from the most important to least important times for patients to be involved. The timepoints were ranked (from most important to least important) in the following order:

- When clinical trials are being developed and designed
- When research is being developed in the laboratory before it gets to clinical trials
- When treatments are being reviewed for safety and use within a country
- When governments and pharmaceutical companies are discussing which treatments should be subsidized within a country.

**Table 2.6: Importance of patient involvement in decisions at different timepoints during treatment development**

| Time points for patients to inform the way new treatments are developed                                              | Mean (n=301) | IQR  |
|----------------------------------------------------------------------------------------------------------------------|--------------|------|
| When research is being developed in the laboratory before it gets to clinical trials                                 | 3.00         | 2.00 |
| When clinical trials are being developed and designed                                                                | 3.00         | 1.00 |
| When treatments are being reviewed for safety and use within a country                                               | 4.00         | 2.00 |
| When governments and pharmaceutical companies are discussing which treatments should be subsidized within a country. | 4.00         | 2.00 |

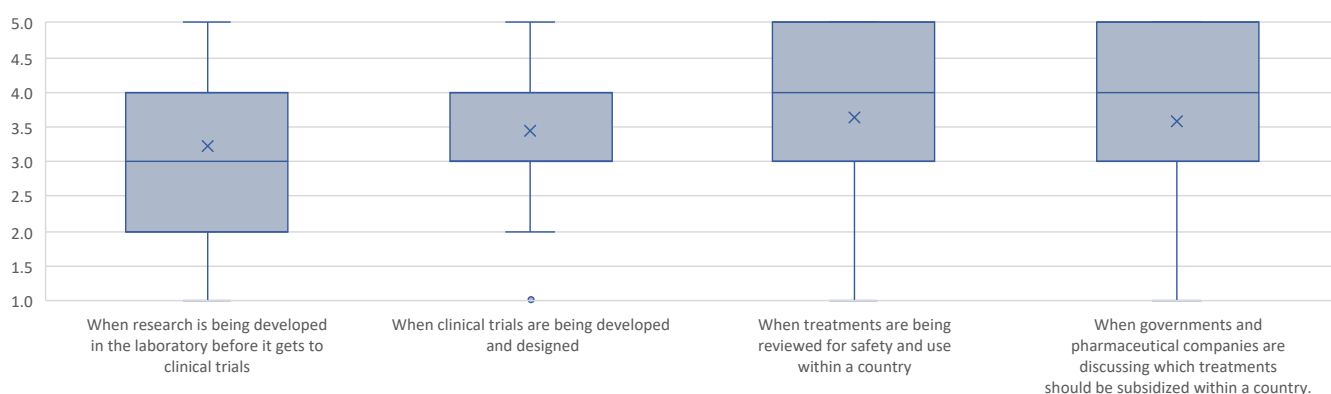


**Figure 2.17: Importance of patient involvement in decisions at different timepoints during treatment development**

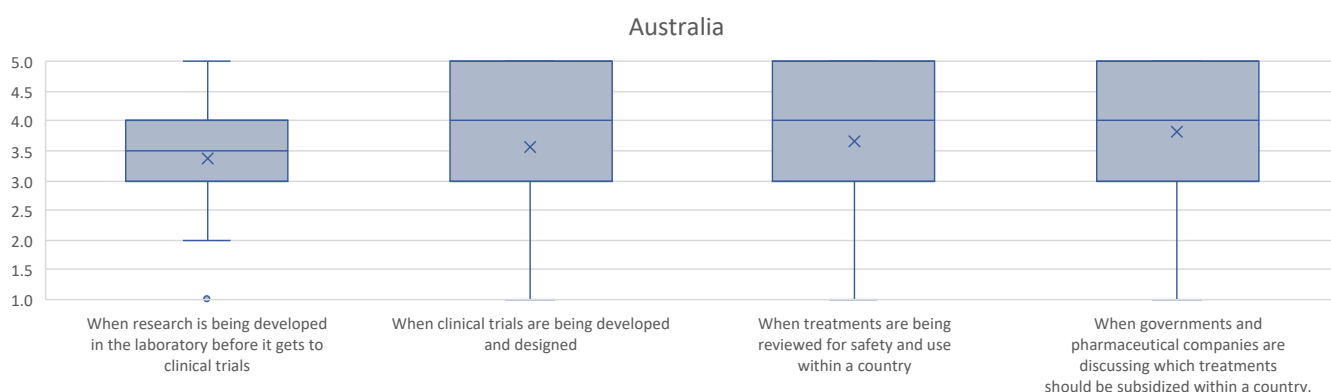


**Figure 2.18: Importance of patient involvement in decisions at different timepoints during treatment development – Australian participants**

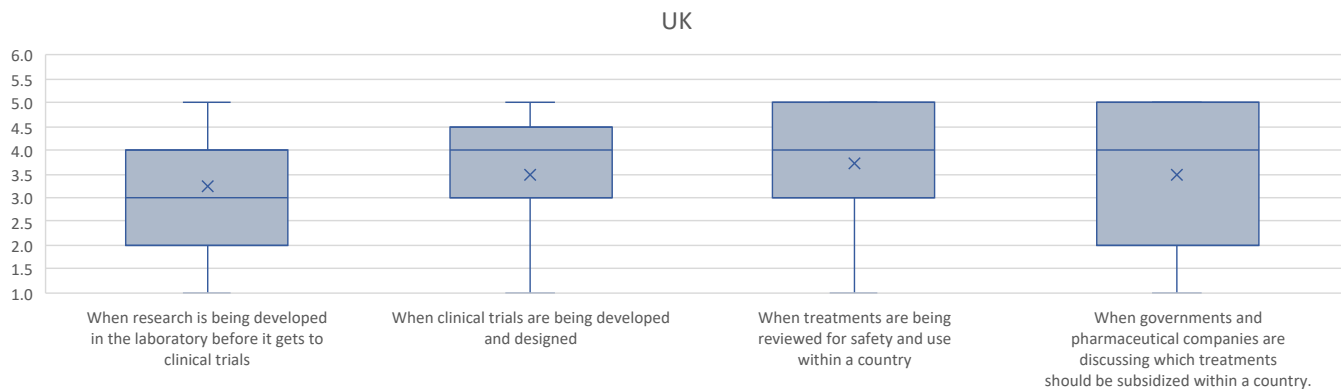


**Figure 2.19: Importance of patient involvement in decisions at different timepoints during treatment development – UK participants**

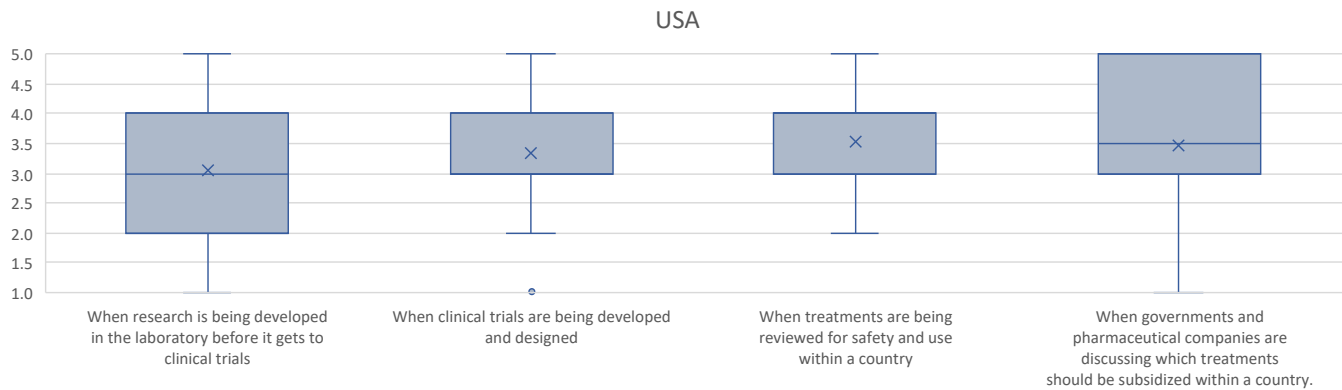


**Figure 2.20: Importance of patient involvement in decisions at different timepoints during treatment development – USA participants**

**Table 2.7: Ranked importance of patient involvement in decisions at different timepoints during treatment development**

| Time points for patients to inform the way new treatments are developed - ranked                                     | Weighted average (n=301) |
|----------------------------------------------------------------------------------------------------------------------|--------------------------|
| When research is being developed in the laboratory before it gets to clinical trials                                 | 2.64                     |
| When clinical trials are being developed and designed                                                                | 3.07                     |
| When treatments are being reviewed for safety and use within a country                                               | 2.41                     |
| When governments and pharmaceutical companies are discussing which treatments should be subsidized within a country. | 1.88                     |

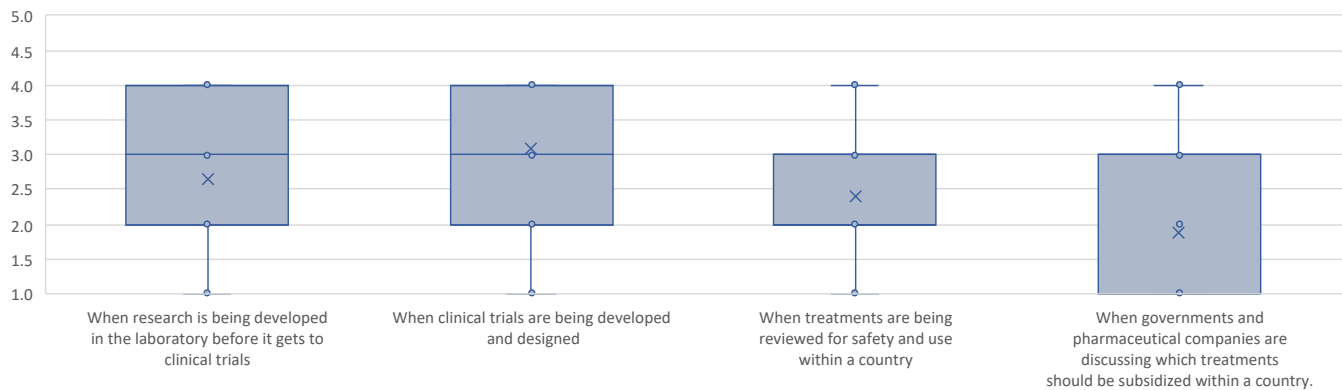


**Figure 2.21: Ranked importance of patient involvement in decisions at different timepoints during treatment development**

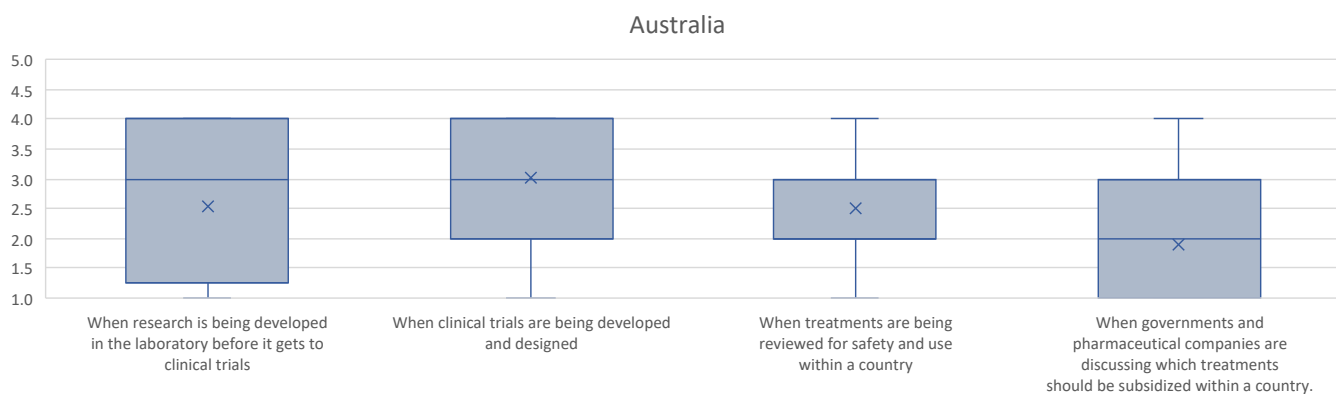


**Figure 2.22: Ranked importance of patient involvement in decisions at different timepoints during treatment development – Australian participants**

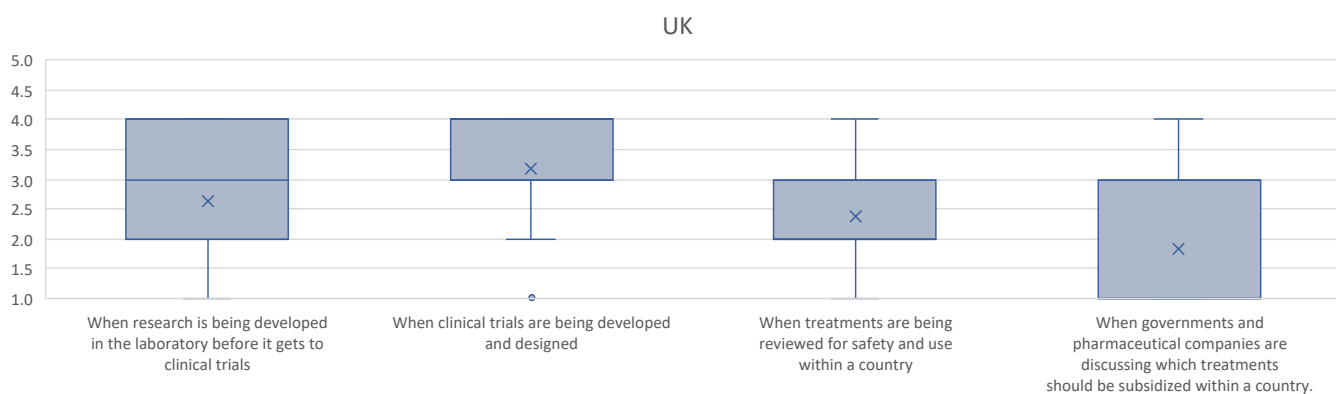


**Figure 2.23: Ranked importance of patient involvement in decisions at different timepoints during treatment development – UK participants**

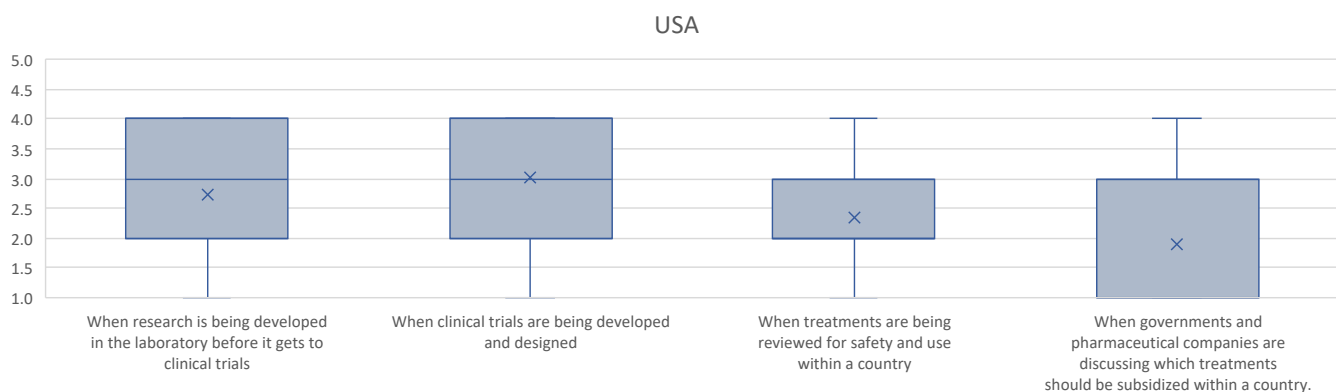


**Figure 2.24: Ranked importance of patient involvement in decisions at different timepoints during treatment development – USA participants**

## Stakeholder involvement in patient input and feedback

### Stakeholder involvement in patient input and feedback

Participants were asked when governments are making decisions about treatments and asking patients for input and feedback, who should run this process, hold the data collected and advocate on behalf of patients and families?

There was very little difference in the involvement of each type of stakeholders (pharmaceutical companies, government, and non-profit, independent patient organisations). About 15% thought that each type of

stakeholder should not be involved at all, just under 50% thought that each type of stakeholder should only be able to read the results of the feedback but not be involved, and about 35% thought that the initiative should be led by each of the different stakeholders.

Participants were then asked to rank stakeholders in relation to who should lead processes to collate patient feedback. There was very little difference in ranking between each of the different organisation types, with slightly more ranking patient organisations most appropriate, and slightly more participants ranking the government least appropriate.

**Table 2.8: Stakeholder involvement in patient input and feedback**

| Stakeholder involvement                                                                                        | Pharmaceutical companies |       | Government |       | Non-profit, independent patient groups |       |
|----------------------------------------------------------------------------------------------------------------|--------------------------|-------|------------|-------|----------------------------------------|-------|
| 1: This stakeholder should not be involved at all                                                              | 43                       | 14.29 | 50         | 16.61 | 43                                     | 14.29 |
| 2: This stakeholder should only be able to read the results of the feedback but not be involved in the process | 149                      | 49.50 | 146        | 48.50 | 145                                    | 48.17 |
| 3: This stakeholder should lead the initiative                                                                 | 109                      | 36.21 | 105        | 34.88 | 113                                    | 37.54 |

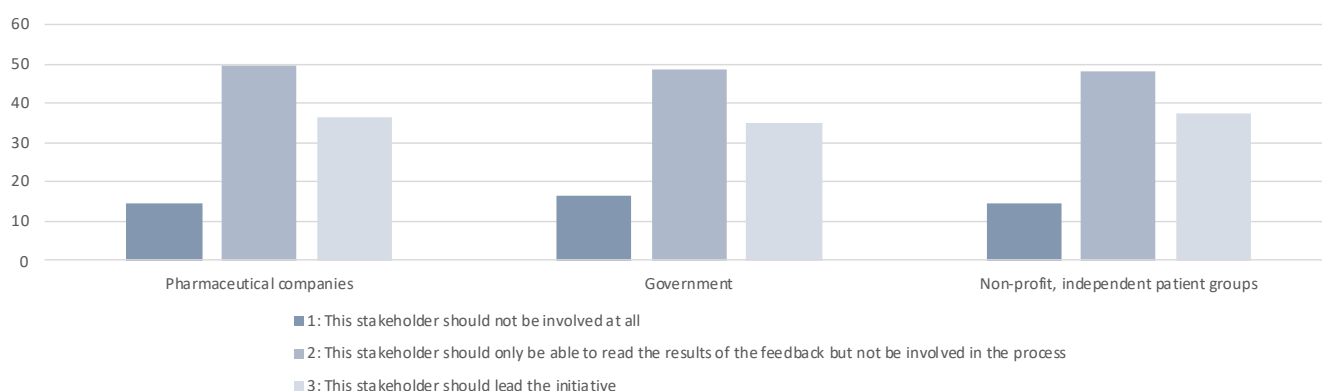


**Figure 2.25: Stakeholder involvement in patient input and feedback**

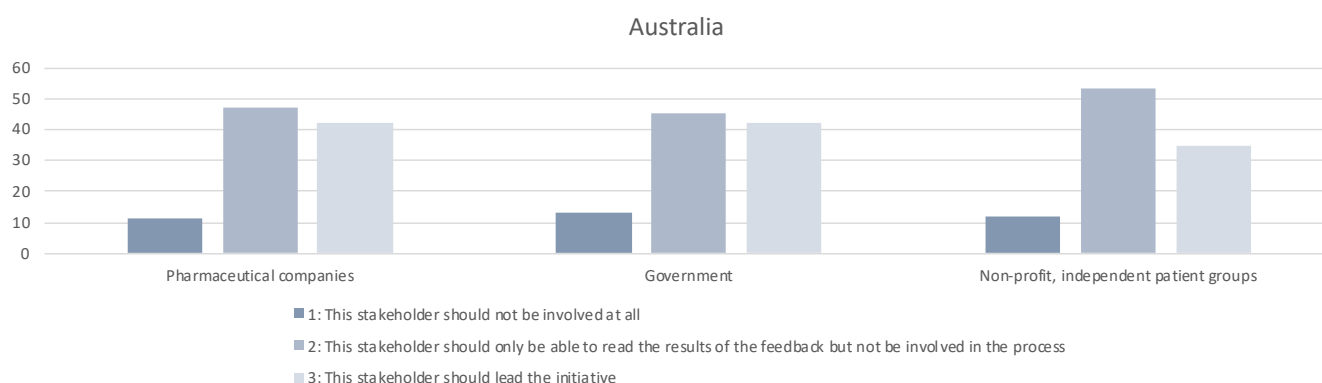


**Figure 2.26: Stakeholder involvement in patient input and feedback – Australian participants**

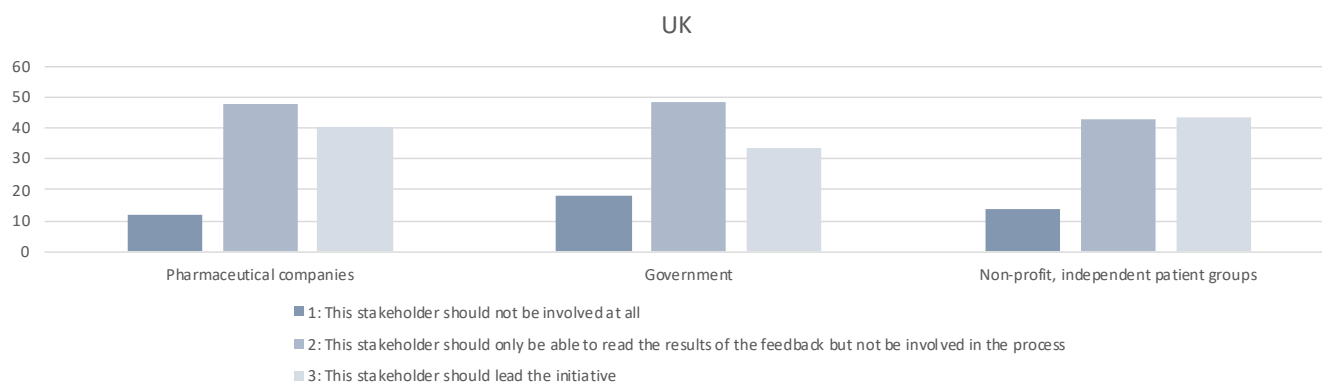


**Figure 2.27: Stakeholder involvement in patient input and feedback – UK participants**

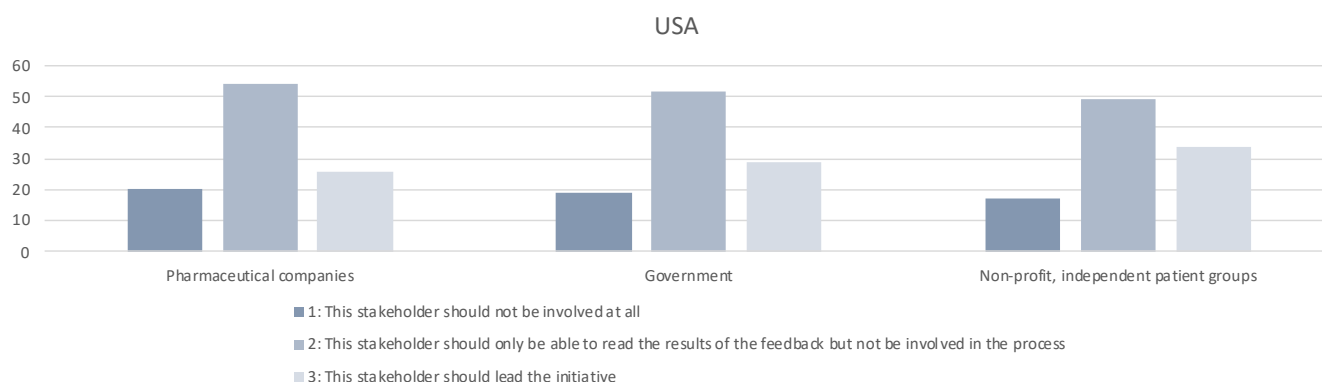


**Figure 2.28: Stakeholder involvement in patient input and feedback – USA participants**

**Table 2.9: Ranked stakeholder involvement in patient input and feedback**

| Stakeholder involvement - rank | Pharmaceutical companies |       | Government |       | Non-profit, independent patient groups |       |
|--------------------------------|--------------------------|-------|------------|-------|----------------------------------------|-------|
| Ranked most important          | 93                       | 30.79 | 87         | 28.81 | 121                                    | 40.07 |
| Ranked second most important   | 107                      | 35.43 | 103        | 34.11 | 91                                     | 30.13 |
| Ranked least important         | 101                      | 33.44 | 111        | 36.75 | 89                                     | 29.47 |

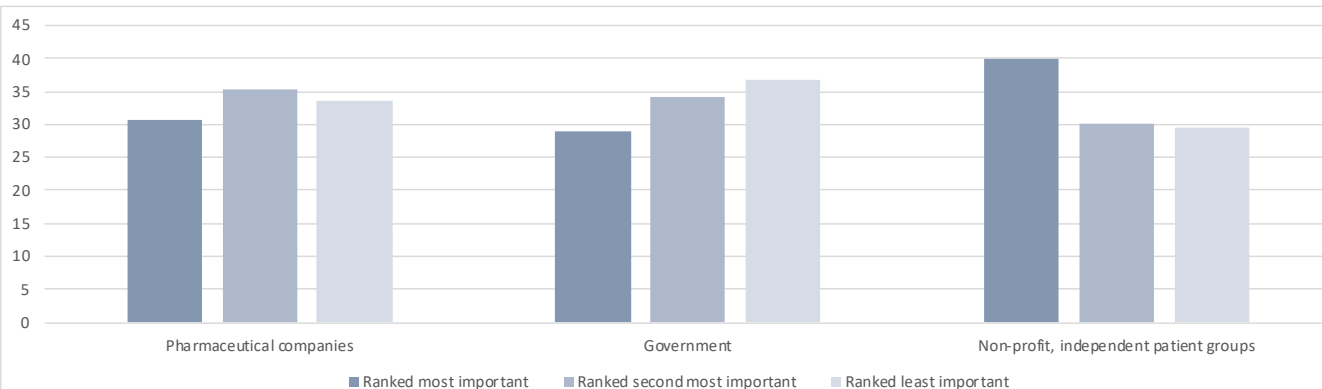


**Figure 2.29: Ranked stakeholder involvement in patient input and feedback**

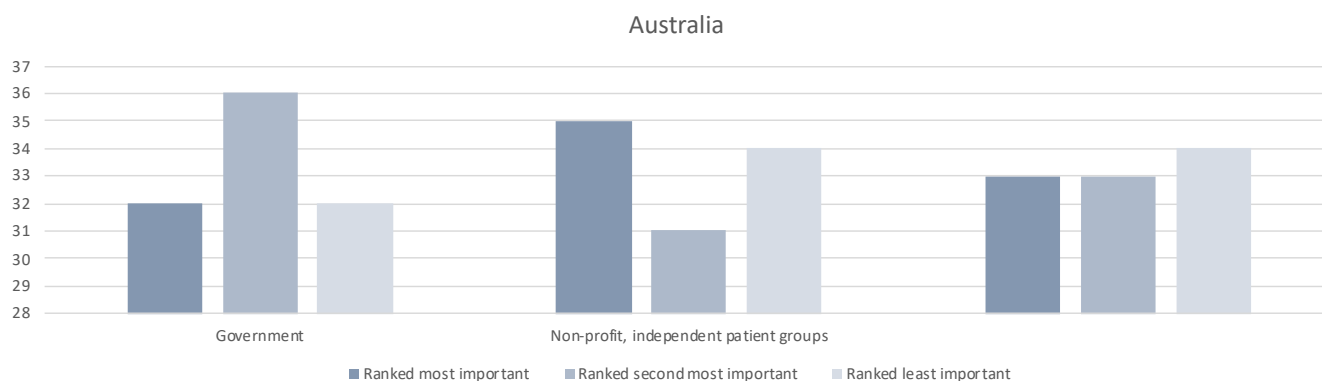


**Figure 2.30: Ranked stakeholder involvement in patient input and feedback – Australian participants**

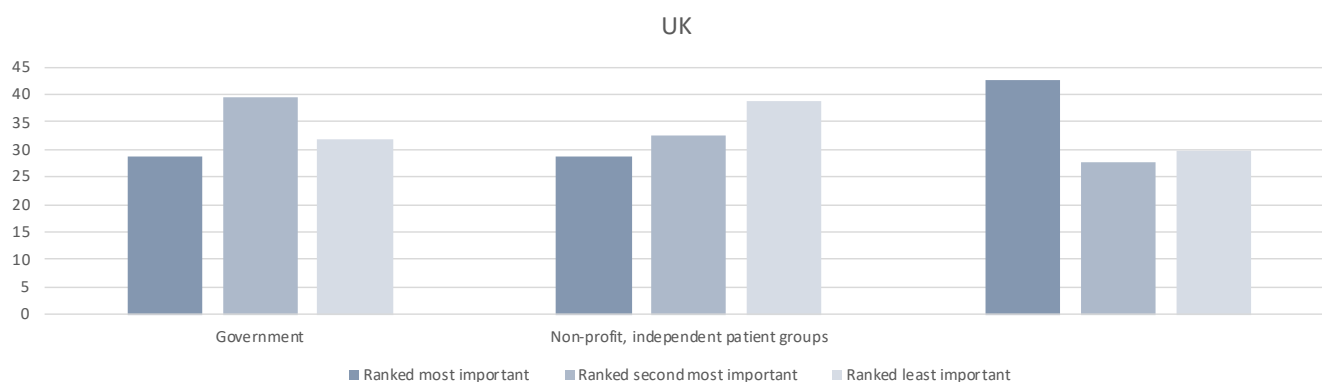


**Figure 2.31: Ranked stakeholder involvement in patient input and feedback – UK participants**

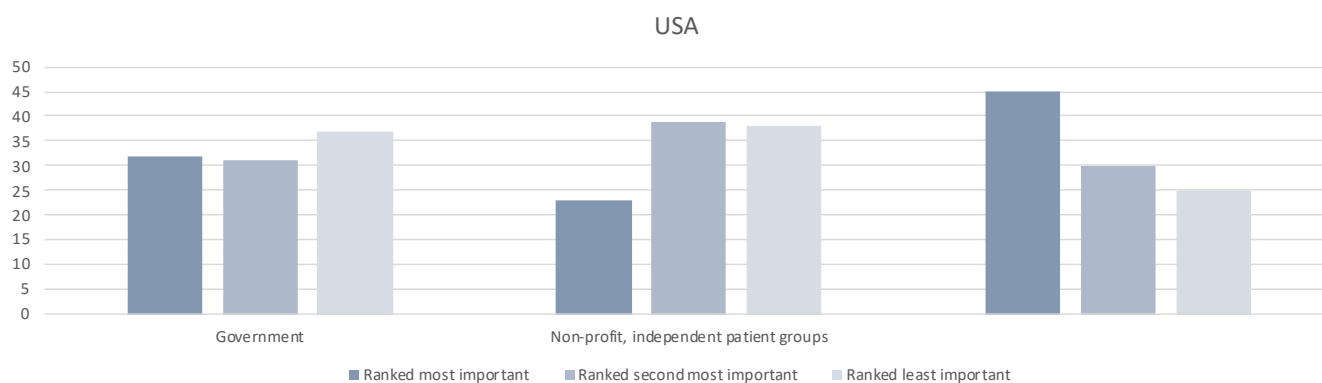


**Figure 2.32: Ranked stakeholder involvement in patient input and feedback – USA participants**

## Section 4: Key points and recommendations

- In part one of this project, participants from the National Patient Organisation Network Australia were asked ‘What questions would you ask patients and families to understand what everyday life is like for them?’ The answers to these questions would be used to educate decision-makers on the impact of the disease/condition. While not all of the questions identified were carried through to the validation process, these remain important questions to consider in various disease contexts. Likewise in part two of this project, the consultation extended to stakeholders from industry, HTA agencies, and health economists, and their feedback may be useful in disease-specific contexts.
  - In part four of this project, we surveyed citizens and patients in three countries (UK, USA and Australia), to see how important engagement in decision-making is for the community in the context of HTA. This is an important step to test the assumption that the community wants to be involved in this process and we recommend that decision-makers review these results to ensure that the questions they are asking of the community are important to them.
  - The following questions all passed the threshold for understandability and acceptability (the only question that did not was If a treatment did [Nurse paraphrases response from above, for example reduce side effects, get rid of nausea] – what would that mean to you in your everyday life, that is, what would you be able to do that you couldn’t do if the treatment wasn’t doing its job?). It is recommended that these be the first questions listed on a common repository of questions that are suitable to ask patients in the context of HTA decision-making. Future work would however need to test these questions in various languages.
1. When you are making decisions about treatment, what are some of things you take into consideration when you are making decisions about treatment?
  2. What are your own personal goals of treatment or care?
  3. In relation to your condition, beyond being able to access treatments, what would you most like to see from new treatments?
  4. Thinking back over your experience, is there anything that you know now that you wished you had of known earlier or at diagnosis?
  5. Also thinking back over your experience, is there any aspect of your care or treatment that you would change?
  6. Thinking back about your experience, has there been a time or any times that you have felt particularly vulnerable?
  7. Has having [INSERT DISEASE] affected your quality of life and the quality of life of your family?
  8. When you are using a treatment, what needs to improve for you to feel as though the treatment has worked?
  9. What are some of the things that you need to do on a regular basis to control the effects of your condition so that you can do all of the activities you need to do every day?