



Participant information sheet Rare Disease Quality Statements 2025 Online Survey

General Information

The aim of this project is to develop a set of quality statements for what good care looks like in rare diseases.

We appreciate your interest in participating in this survey.

You may ask any questions before deciding to take part by contacting the quality standard project group – please email info@principleconsulting.org.uk.

As part of this survey, you will be asked to review a series of statements that outline what high-quality care and treatment for rare diseases should look like. These statements are grouped into broad topic areas.

You will need to rate each statement on a scale from 1 to 9, with 9 being the highest level of importance and 1 being the lowest. Your rating should reflect how important you believe each statement is when developing a set of quality standards for rare disease care.

Please note that these statements are not intended to describe the current state of care and treatment but rather to set aspirational standards for good care. If you are unsure about a statement or prefer not to comment, you can select 0.

At the end of the survey, you will be asked to choose your top three statements. The survey should take around 20 minutes, and no prior knowledge is required.

The data from the survey will help us to develop a set of quality statements for rare disease, based on the statements and themes which are most important to the community.

We will also ask for non-identifying information about you, such as your role in the rare disease community, the disease group which is most relevant to you, and the UK nation which is most relevant to you, as well as some questions for equality and diversity monitoring. The purpose of these questions is to ensure that the output from the survey reflects the diverse range of experiences across the rare disease community.

Finally, we will also provide the option to share your email address to express your interest in participating in a consensus workshop on the next draft set of statements. The workshops will be an opportunity for stakeholders across the rare disease community to feed in on the draft statements. This is optional, and if you choose to share your email address, it will be stored separately from your survey responses – they will not be associated. The email addresses will be deleted once we have contacted those who have been selected to participate in the consensus workshop, or after 12 months, whichever is sooner.

Please note that you may only participate in this survey if you are 18 years of age or over.

Do I have to take part?

No. Please note that participation is voluntary. If you do decide to take part, you may withdraw at any point for any reason before submitting your answers by pressing the 'Exit' button/ closing the browser.

We have included a 'Prefer not to say' option for each set of questions should you prefer not to answer a particular question.

How will my data be used?

The data we will collect that could identify you will be email addresses – sharing your email address is optional. If you choose to share your email address, it will be stored separately from your survey responses – they will not be associated. Email addresses will be deleted once we have shared invitations to the consensus workshops, or after 12 months, whichever is sooner.

Your IP address will not be stored. We will take all reasonable measures to ensure that data remain confidential.

Who will have access to my data?

The data is being collected by the Rare Disease Quality Standard Project. All data will be treated in confidence and held securely in line with the Data Protection Act 2018. All data will be stored securely by the Data Controller [Rare Disease Quality Standard Project]. The Data Processor [Principle Consulting] will also have access to all data to conduct the analysis. Nothing you tell us will be used or shared in a way that can identify you personally.

What will happen to the information when this study is over?

The Rare Disease Quality Standard Project will only retain your data in a way that can identify you for as long as is necessary to support the evaluation project and findings. Any personally identifiable data from the survey will be securely deleted from the Rare Disease Quality Standard Project systems once the study and any quality control checks are complete; this is usually carried out within three to six months of the project closing. No personally identifiable data will ever be published or stored for longer than necessary by the Rare Disease Quality Standard Project.

Who has reviewed this research?

This research has been reviewed by, and received ethics clearance through King's College London.

Who do I contact if I have a concern or I wish to complain?

If you have a concern about any aspect of this research, please contact the quality standard project group, by contacting info@principleconsulting.org.uk, and we will do our best to answer your query.

Project sponsors:

Alexion AstraZeneca Rare Disease, Janssen, Pfizer and Roche have sponsored this project and have had no input into the content of the survey or related materials.