Participant Information Statement

Consumers

Preferences for high upfront cost gene therapies: a survey for patients, their families/carers and the general public

Professor Kirsten Howard (Responsible Researcher)
The Leeder Centre for Health Policy, Economics and Data, University of Sydney
Phone: +61 2 9351 2587| Email: kirsten.howard@sydney.edu.au

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1. What is this study about?

We're conducting a research study to understand the preferences that patients, their families/carers and the general public, have for different aspects of high-upfront cost gene therapies. Gene therapy involves the use of genetic material to prevent and treat disease and is emerging as a treatment option for a range of health conditions including for blood disorders such as haemophilia, beta-thalassemia, and sickle cell disease. This study is part of a larger body of work which is seeking to develop tools to support funding decision-making for these therapies by national committees including the Pharmaceutical Benefits Advisory Committee (PBAC) and the Medical Services Advisory Committee (MSAC). Taking part in this study is optional. Please read this sheet and ask about things that aren't clear, or you want to know more about.

2. Who is running the study?

The researchers conducting this study are:

- Professor Kirsten Howard, Co-Director, The Leeder Centre for Health Policy, Economics and Data, University of Sydney
- Professor Richard De Abreu Lourenco, Health Economist
  Centre for Health Economics Research and Evaluation (CHERE), University of Technology Sydney
- Professor Rosalie Viney, Director, Centre for Health Economics Research and Evaluation
  Centre for Health Economics Research and Evaluation (CHERE), University of Technology Sydney
- Ms Jo Watson, Chair, HTA Consumer Consultative Committee
- Dr Rakhee Raghunandan, Health Economist
  The Leeder Centre for Health Policy, Economics and Data, University of Sydney
- Dr Andrea Natsky, Research Fellow
  The Leeder Centre for Health Policy, Economics and Data, University of Sydney
- Associate Professor Martin Howell, Health Economist
  The Leeder Centre for Health Policy, Economics and Data, University of Sydney
- Dr Alice Yu, Research Fellow
  Centre for Health Economics Research and Evaluation (CHERE), University of Technology Sydney

This study is funded by a grant from the Australian Government's Medical Research Future Fund (MRFF).

3. Who can take part in the study?

We are seeking:

1. Individuals who have the following disorders: haemophilia A or B (Factor VIII or IX deficiency), beta-thalassemia, or sickle cell disease.
2. Have anyone within their family with the following disorders: haemophilia A or B (Factor VIII or IX deficiency), beta-thalassemia, or sickle cell disease.
3. A carer of an individual who has any of the following disorders: haemophilia A or B (Factor VIII or IX deficiency), beta-thalassemia or sickle cell disease.

For this survey, people with the blood disorder and/or their family member/carer are welcome to participate.

To be eligible to participate in this study you must be at least 18 years of age.

4. What will the study involve for me?

If you decide to take part in this study, you will be asked to complete an online survey. The survey is designed to seek your views on what are the important aspects for government decision makers to consider in relation to funding and provision of high-upfront cost gene therapies. You will have three weeks to complete the survey, and you can complete the survey at any time within this period. We estimate that it will take approximately 15-20 minutes to complete the survey.

5. Can I withdraw once I've started?

Participating in this study is optional and you do not have to take part. Your decision will have no impact on your current or future relationship with the researchers, anyone else at The University of Sydney, or anyone at any of the involved partner organisations through whom you may have been invited to participate.

By submitting your survey, you are consenting to take part in the study. You can withdraw any time before submitting the survey. You will not be able to withdraw your data once you have completed the survey as we won't be able to link your specific responses to you as an individual (survey completion is anonymous).

6. Are there any risks or costs?

The questions in the online surveys will not collect any information that is sensitive in nature. However, there is a small chance that you may experience some discomfort if the topic involves you thinking about your own experience with illness or other family members experience with illness. Similarly, some discomfort or anxiety may be generated through developing an understanding of healthcare decision making which you may not have previously considered. We expect the chance of this risk occurring is small. If you experience any feelings of psychological distress, we encourage you to contact:

- Lifeline's 24-hour crisis support team (Ph: 131114)
- Beyond Blue's 24-hour support service on (1300 224 636).

7. Are there any benefits?

We hope that the information that you share will allow Government decision makers to make more informed decisions on high-upfront cost gene therapy, ultimately benefitting Australian society. In recognition of the time you spend on the survey, you will receive a $25 gift voucher as a thank you for your participation once you complete the survey.

8. What will happen to information that is collected?

Your information will be securely stored, and results may be published. No personal information will be used in the data analysis and reporting.

9. Will I be told the results of the study?

You have the right to hear the results of this study. In the online survey, you will be asked to indicate whether you would like to receive a summary of the study results via email.

10. What if I would like further information?

After reading this information, the researcher/s will be available to have further discussions with you and answer any questions you may have:

- Professor Kirsten Howard, Co-Director, The Leeder Centre for Health Policy, Economics and Data, University of Sydney, email: kirsten.howard@sydney.edu.au, phone: +61 2 9351 2587
- Ms Maria Gomez, Project Manager, The Leeder Centre for Health Policy, Economics and Data, University of Sydney, email: maria.gomez@sydney.edu.au

11. What if I have a complaint or any concerns?

The ethical aspects of this study have been approved by the Human Research Ethics Committee (HREC) of The University of Sydney [HREC Approval No.: 2025/HE000195] in accordance with the *National Statement on Ethical Conduct in Human Research (2007).*

If you have any concerns about the study's procedures or would like to make a complaint to someone not involved in the study, please contact the University:

Human Ethics Manager
human.ethics@sydney.edu.au
+61 2 8627 8176

This information is for you to keep