THE UNIVERSITY OF SYDNEY

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

---

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. Ultimately, this information will help to inform government decision makers in their assessments of new gene therapies.

We are seeking adults with haemophilia A and B (Factor VIII or Factor IX deficiency), beta-thalassemia or sickle cell disease and/or their immediate family members/carers from across Australia. Participants will need to be 18 years or older to participate in the study.

Taking part in this study will involve completing an online survey. We estimate that it will take approximately 15-20 minutes to complete the survey. If you would like more information, please see participant information sheet below.

Please contact Professor Kirsten Howard, The Leeder Centre for Health Policy, Economics, and Data Kirsten.howard@sydney.edu.au, 02 9351 2587 if you have any questions about this study.

To participate in this study, please click the following link or scan the QR Code: https://url.au.m.mimecastprotect.com/s/HPveCVARKgC5EEY7ZiGf4fE4oZs?domain=surveydau.pureprofile.com

This study has been approved by the Human Research Ethics Committee (HREC) of The University of Sydney [HREC Approval No. 2025/HE000195].