Survey of potential participant perspective on ocular gene therapy in Australia

SECTION 2  I  PLEASE RETURN TO RESEARCH TEAM
Survey of potential participant perspective on ocular gene therapy in Australia

Consent form – Version 2 - 5 January 2021

Please sign this second consent form, and return this booklet to the research team using the pre-paid envelope.

I consent to participate in the research study “Survey of potential participant perspective on ocular gene therapy in Australia” and confirm that I have read the plain language statement and understood the following information.

In particular, I have noted that:

☐ Participation in this research is entirely voluntary;

☐ I am free to withdraw from this research at any time, without comment or penalty. If I withdraw I may request withdrawal of any unprocessed data previously supplied;

☐ Providing my contact details to the researchers is entirely voluntary;

☐ Any questions have been answered to my satisfaction and I understand that if I have any additional questions I can contact the research team;

☐ I have been informed that the confidentiality of the information I will provide will be safeguarded, my opinions will be treated as personal information, and my privacy respected;

☐ My de-identified data will be stored and may be utilized for future related research studies on the understanding that consent for further projects will be sought from me at that future time;

☐ I understand that I can contact the Manager, Human Research Ethics, Research Ethics and Integrity, University of Melbourne, VIC 3010. Tel: +61 3 8344 2073 or Email: humanethicscomplaints@unimelb.edu.au.

Name (optional):

Sign:

Date:
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About you

1. Are you completing this questionnaire as a:
   - Adult patient, or
   - Parent or guardian of patient less than 18 years of age?

1a. What is the age of your child/dependent diagnosed with an IRD?

1b. What is the gender of your child/dependent diagnosed with an IRD?
   - Male
   - Female
   - Non-binary
   - I prefer not to say

2. Highest level of education you have completed
   - Primary school
   - Secondary school (Year 10 or above)
   - Trade certificate
   - Bachelor degree
   - Post-graduate degree
   - I prefer not to say
Survey of potential participant perspective on ocular gene therapy in Australia

About you

3. What is your annual household gross (before tax) income?
   - □ Less than $18 200
   - □ $18 201 - $37 000
   - □ $37 001 - $87 000
   - □ $87 001 - $180 000
   - □ More than $180 001
   - □ I’d prefer not to say

4. How many members are in your household?

5. What is your age?

6. What is your gender?
   - □ Male
   - □ Female
   - □ Non-binary
   - □ I prefer not to say
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Diagnosis, symptoms, treatment

7. What is your primary diagnosis as confirmed by your ophthalmologist?
   - □ Achromatopsia
   - □ Bietti Crystalline Dystrophy
   - □ Choroideremia
   - □ Cone Dystrophy
   - □ Cone-rod Dystrophy
   - □ Leber Congenital Amaurosis
   - □ Macular Dystrophy
   - □ Retinitis Pigmentosa
   - □ Stargardt Disease
   - □ X-Linked Retinoschisis
   - □ Other inherited retinal disease (Please specify)
   - □ Not diagnosed with Inherited Retinal Disease (You are not eligible for this study and should not continue)
   - □ I don’t know

8. Have you supplied DNA to any Australian IRD database?
   - □ Yes
   - □ No

9. Have you participated in any medical research in the past?
   - □ Yes
   - □ No
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Diagnosis, symptoms, treatment

10. Have you previously used any of the following treatments for your inherited retinal disease? Select all that apply.
   - Acupuncture
   - Electrical stimulation
   - Human stem cells
   - Vitamin A
   - Herbal remedies
   - None of the above

11. Describe your first symptoms, related to the inherited retinal disease. Select all that apply.
   - Difficulty seeing at night or dusk
   - Bumping into low lying objects
   - Difficulty driving
   - Difficulty adjusting from light to dark and vice versa
   - Missing parts in vision
   - Noticed peripheral or side vision reducing
   - Other
   - No noticeable symptoms
   - Can’t recall

12. What age were you when symptoms first appeared?
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Diagnosis, symptoms, treatment

13. Within which time period, have you experienced your most recent decline in vision?
   - □ Less than 6 months
   - □ 1 Year
   - □ 5 Years
   - □ 10 Years
   - □ No decline, stable vision

14. How likely are you to take up gene therapy if it was available now to you or your family members for their eye condition?
   - □ Very likely
   - □ Likely
   - □ Neutral
   - □ Unlikely
   - □ Very unlikely

15. What are the barriers to you receiving gene therapy for your eye condition? Select all that apply.
   - □ Treatment is still in early phase roll-out and I would prefer to wait
   - □ Treatment may not work
   - □ Fear of side effects
   - □ Out of pocket cost
   - □ Against my religion or personal belief
   - □ Loss of entitlement to government supports
   - □ Other

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Your views on gene therapy

This questionnaire is about your views on gene therapy. Please indicate how much you agree or disagree with the following statements:

1. I have good knowledge about gene therapy for inherited retinal diseases.
   - □ Strongly agree
   - □ Agree
   - □ Neither agree or disagree
   - □ Disagree
   - □ Strongly disagree
Survey of potential participant perspective on ocular gene therapy in Australia

Your views on gene therapy

2. I have obtained information about gene therapy from:

<table>
<thead>
<tr>
<th></th>
<th>Strongly agree</th>
<th>Agree</th>
<th>Neither agree or disagree</th>
<th>Disagree</th>
<th>Strongly disagree</th>
</tr>
</thead>
<tbody>
<tr>
<td>My ophthalmologist</td>
<td>☐</td>
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<td>Registry e.g. Australian Inherited Retinal Disease Register</td>
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</tbody>
</table>
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Your views on gene therapy

3. I understand the difference between an experimental treatment provided in a clinical trial and a treatment that has already been approved by the Australian Government.
   - [ ] Strongly agree
   - [ ] Agree
   - [ ] Neither agree or disagree
   - [ ] Disagree
   - [ ] Strongly disagree

4. Gene therapy for the eye is suitable at any stage of a person’s life.
   - [ ] Strongly agree
   - [ ] Agree
   - [ ] Neither agree or disagree
   - [ ] Disagree
   - [ ] Strongly disagree

5. Generally, gene therapy for inherited retinal disease is delivered to both eyes.
   - [ ] Strongly agree
   - [ ] Agree
   - [ ] Neither agree or disagree
   - [ ] Disagree
   - [ ] Strongly disagree
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Your views on gene therapy

6. Gene therapy for the eye is injected into the blood stream through the arm.
   - Strongly agree
   - Agree
   - Neither agree or disagree
   - Disagree
   - Strongly disagree

7. Gene therapy and stem cell therapy are the same treatment.
   - Strongly agree
   - Agree
   - Neither agree or disagree
   - Disagree
   - Strongly disagree

8. Gene therapy for the eye can restore vision back to normal.
   - Strongly agree
   - Agree
   - Neither agree or disagree
   - Disagree
   - Strongly disagree
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Your views on gene therapy

9. Gene therapy for the eye is a treatment that may slow down the disease.
   - [ ] Strongly agree
   - [ ] Agree
   - [ ] Neither agree or disagree
   - [ ] Disagree
   - [ ] Strongly disagree

10. Treatment complications to my eyes, such as permanent blindness, are possible with an approved gene therapy.
    - [ ] Strongly agree
    - [ ] Agree
    - [ ] Neither agree or disagree
    - [ ] Disagree
    - [ ] Strongly disagree

11. Gene therapy in my eye may have side effects elsewhere in my body.
    - [ ] Strongly agree
    - [ ] Agree
    - [ ] Neither agree or disagree
    - [ ] Disagree
    - [ ] Strongly disagree
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Your views on gene therapy

12. Having gene therapy for their eye condition means a person will not pass on an eye condition to any children they may have in the future.
   - [ ] Strongly agree
   - [ ] Agree
   - [ ] Neither agree or disagree
   - [ ] Disagree
   - [ ] Strongly disagree

13. I may not be eligible for financial or other government benefits if my gene therapy for my eye condition is successful.
   - [ ] Strongly agree
   - [ ] Agree
   - [ ] Neither agree or disagree
   - [ ] Disagree
   - [ ] Strongly disagree

14. Gene therapy for inherited retinal diseases will require many years of follow-up with my eyecare practitioner.
   - [ ] Strongly agree
   - [ ] Agree
   - [ ] Neither agree or disagree
   - [ ] Disagree
   - [ ] Strongly disagree
Survey of potential participant perspective on ocular gene therapy in Australia

Your views on gene therapy

15. Receiving gene therapy for my inherited retinal disease means I won’t be eligible for future genetic treatments.
   □ Strongly agree
   □ Agree
   □ Neither agree or disagree
   □ Disagree
   □ Strongly disagree

16. I will lose my privacy if I undergo gene therapy, and my data will be in the public domain.
   □ Strongly agree
   □ Agree
   □ Neither agree or disagree
   □ Disagree
   □ Strongly disagree

17. If I undergo gene therapy, it will affect my eligibility or terms of conditions in life, disability or health insurance in the future.
   □ Strongly agree
   □ Agree
   □ Neither agree or disagree
   □ Disagree
   □ Strongly disagree
Survey of potential participant perspective on ocular gene therapy in Australia

Your views on gene therapy

18. The government should pay all costs of my gene therapy.
   - [ ] Strongly agree
   - [ ] Agree
   - [ ] Neither agree or disagree
   - [ ] Disagree
   - [ ] Strongly disagree

19. Government subsidy of my treatment would be an effective use of taxpayer money.
   - [ ] Strongly agree
   - [ ] Agree
   - [ ] Neither agree or disagree
   - [ ] Disagree
   - [ ] Strongly disagree

20. If gene therapy for my condition was not available in my state I would consider travelling interstate to access it.
   - [ ] Strongly agree
   - [ ] Agree
   - [ ] Neither agree or disagree
   - [ ] Disagree
   - [ ] Strongly disagree
Survey of potential participant perspective on ocular gene therapy in Australia

Your views on gene therapy

21. My private health insurance should pay all out of pocket costs for my gene therapy.
   - [ ] Strongly agree
   - [ ] Agree
   - [ ] Neither agree or disagree
   - [ ] Disagree
   - [ ] Strongly disagree

22. I would consider a payment plan for my gene therapy.
   - [ ] Strongly agree
   - [ ] Agree
   - [ ] Neither agree or disagree
   - [ ] Disagree
   - [ ] Strongly disagree
Health Questionnaire

English version for Australia
Under each heading, please tick the ONE box that best describes your health TODAY.

**MOBILITY**
- I have no problems with walking around
- I have slight problems with walking around
- I have moderate problems with walking around
- I have severe problems with walking around
- I am unable to walk around

**PERSONAL CARE**
- I have no problems with washing or dressing myself
- I have slight problems with washing or dressing myself
- I have moderate problems with washing or dressing myself
- I have severe problems with washing or dressing myself
- I am unable to wash or dress myself

**USUAL ACTIVITIES** *(e.g. work, study, housework, family or leisure activities)*
- I have no problems doing my usual activities
- I have slight problems doing my usual activities
- I have moderate problems doing my usual activities
- I have severe problems doing my usual activities
- I am unable to do my usual activities

**PAIN / DISCOMFORT**
- I have no pain or discomfort
- I have slight pain or discomfort
- I have moderate pain or discomfort
- I have severe pain or discomfort
- I have extreme pain or discomfort

**ANXIETY / DEPRESSION**
- I am not anxious or depressed
- I am slightly anxious or depressed
- I am moderately anxious or depressed
- I am severely anxious or depressed
- I am extremely anxious or depressed
• We would like to know how good or bad your health is TODAY.

• This scale is numbered from 0 to 100.

• 100 means the **best health** you can imagine.
  0 means the **worst health** you can imagine.

• Mark an X on the scale to indicate how your health is TODAY.

• Now, please write the number you marked on the scale in the box below.

YOUR HEALTH TODAY = [ ]
National Eye Institute
Visual Functioning Questionnaire - 25 (VFQ-25)
version 2000

(SELF-ADMINISTERED FORMAT)

January 2000

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7/29/96

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version 2000

The following is a survey with statements about problems which involve your vision or feelings that you have about your vision condition. After each question please choose the response that best describes your situation.

Please answer all the questions as if you were wearing your glasses or contact lenses (if any).

Please take as much time as you need to answer each question. All your answers are confidential. In order for this survey to improve our knowledge about vision problems and how they affect your quality of life, your answers must be as accurate as possible. Remember, if you wear glasses or contact lenses, please answer all of the following questions as though you were wearing them.

INSTRUCTIONS:

1. In general we would like to have people try to complete these forms on their own. If you find that you need assistance, please feel free to ask the project staff and they will assist you.

2. Please answer every question (unless you are asked to skip questions because they don’t apply to you).

3. Answer the questions by circling the appropriate number.

4. If you are unsure of how to answer a question, please give the best answer you can and make a comment in the left margin.

5. Please complete the questionnaire before leaving the center and give it to a member of the project staff. Do not take it home.

6. If you have any questions, please feel free to ask a member of the project staff, and they will be glad to help you.

STATEMENT OF CONFIDENTIALITY:

All information that would permit identification of any person who completed this questionnaire will be regarded as strictly confidential. Such information will be used only for the purposes of this study and will not be disclosed or released for any other purposes without prior consent, except as required by law.

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Visual Functioning Questionnaire - 25

PART 1 - GENERAL HEALTH AND VISION

1. **In general,** would you say your overall **health** is:
   
   (Circle One)
   
   Excellent ...................... 1
   Very Good ...................... 2
   Good ............................ 3
   Fair ............................. 4
   Poor ............................ 5

2. At the present time, would you say your eyesight using both eyes 
   (with glasses or contact lenses, if you wear them) is **excellent**, **good**, 
   **fair**, **poor**, or **very poor** or are you **completely blind**?
   
   (Circle One)
   
   Excellent ...................... 1
   Good ............................. 2
   Fair ............................. 3
   Poor ............................. 4
   Very Poor ....................... 5
   Completely Blind .............. 6

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3. How much of the time do you worry about your eyesight?

(Circle One)

None of the time .......................... 1
A little of the time .......................... 2
Some of the time .......................... 3
Most of the time .......................... 4
All of the time? .......................... 5

4. How much pain or discomfort have you had in and around your eyes (for example, burning, itching, or aching)? Would you say it is:

(Circle One)

None .......................... 1
Mild .......................... 2
Moderate .......................... 3
Severe, or .......................... 4
Very severe? .......................... 5

PART 2 - DIFFICULTY WITH ACTIVITIES

The next questions are about how much difficulty, if any, you have doing certain activities wearing your glasses or contact lenses if you use them for that activity.

5. How much difficulty do you have reading ordinary print in newspapers? Would you say you have:

(Circle One)

No difficulty at all .......................... 1
A little difficulty .......................... 2
Moderate difficulty .......................... 3
Extreme difficulty .......................... 4
Stopped doing this because of your eyesight .... 5
Stopped doing this for other reasons or not interested in doing this .......................... 6

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6. How much difficulty do you have doing work or hobbies that require you to **see well up close**, such as cooking, sewing, fixing things around the house, or using hand tools? Would you say:

(Circle One)

- No difficulty at all................................. 1
- A little difficulty.................................... 2
- Moderate difficulty............................... 3
- Extreme difficulty............................... 4
- Stopped doing this because of your eyesight.... 5
- Stopped doing this for other reasons or not interested in doing this .................................. 6

7. Because of your eyesight, how much difficulty do you have finding something on a crowded shelf?

(Circle One)

- No difficulty at all................................. 1
- A little difficulty.................................... 2
- Moderate difficulty............................... 3
- Extreme difficulty.................................. 4
- Stopped doing this because of your eyesight.... 5
- Stopped doing this for other reasons or not interested in doing this .................................. 6

8. How much difficulty do you have **reading street signs or the names of stores**?

(Circle One)

- No difficulty at all................................. 1
- A little difficulty.................................... 2
- Moderate difficulty............................... 3
- Extreme difficulty............................... 4
- Stopped doing this because of your eyesight.... 5
- Stopped doing this for other reasons or not interested in doing this .................................. 6

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9. Because of your eyesight, how much difficulty do you have **going down steps, stairs, or curbs in dim light or at night**?

   (Circle One)
   
   No difficulty at all............................................. 1
   A little difficulty............................................. 2
   Moderate difficulty........................................... 3
   Extreme difficulty............................................. 4
   Stopped doing this because of your eyesight.... 5
   Stopped doing this for other reasons or not interested in doing this .................................6

10. Because of your eyesight, how much difficulty do you have **noticing objects off to the side while you are walking along**?

    (Circle One)
    
    No difficulty at all............................................. 1
    A little difficulty............................................. 2
    Moderate difficulty........................................... 3
    Extreme difficulty............................................. 4
    Stopped doing this because of your eyesight.... 5
    Stopped doing this for other reasons or not interested in doing this .................................6

11. Because of your eyesight, how much difficulty do you have **seeing how people react to things you say**?

    (Circle One)
    
    No difficulty at all............................................. 1
    A little difficulty............................................. 2
    Moderate difficulty........................................... 3
    Extreme difficulty............................................. 4
    Stopped doing this because of your eyesight.... 5
    Stopped doing this for other reasons or not interested in doing this .................................6

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12. Because of your eyesight, how much difficulty do you have picking out and matching your own clothes?

(Circle One)

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<tr>
<th>Difficulty</th>
<th>Score</th>
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<td>3</td>
</tr>
<tr>
<td>Extreme difficulty</td>
<td>4</td>
</tr>
<tr>
<td>Stopped doing this because of your eyesight....</td>
<td>5</td>
</tr>
<tr>
<td>Stopped doing this for other reasons or not interested in doing this</td>
<td>6</td>
</tr>
</tbody>
</table>

13. Because of your eyesight, how much difficulty do you have visiting with people in their homes, at parties, or in restaurants?

(Circle One)

<table>
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<tr>
<th>Difficulty</th>
<th>Score</th>
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<tbody>
<tr>
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<td>Extreme difficulty</td>
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<td>Stopped doing this because of your eyesight....</td>
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<tr>
<td>Stopped doing this for other reasons or not interested in doing this</td>
<td>6</td>
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</table>

14. Because of your eyesight, how much difficulty do you have going out to see movies, plays, or sports events?

(Circle One)

<table>
<thead>
<tr>
<th>Difficulty</th>
<th>Score</th>
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<tbody>
<tr>
<td>No difficulty at all</td>
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<td>5</td>
</tr>
<tr>
<td>Stopped doing this for other reasons or not interested in doing this</td>
<td>6</td>
</tr>
</tbody>
</table>

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15. Are you currently driving, at least once in a while?
   (Circle One)
   Yes ....................  1  Skip To Q 15c
   No ......................  2

15a. IF NO: Have you never driven a car or have you given up driving?
   (Circle One)
   Never drove ......  1  Skip To Part 3, Q 17
   Gave up.............  2

15b. IF YOU GAVE UP DRIVING: Was that mainly because of your eyesight, mainly for some other reason, or because of both your eyesight and other reasons?
   (Circle One)
   Mainly eyesight .........................  1  Skip To Part 3, Q 17
   Mainly other reasons .....................  2  Skip To Part 3, Q 17
   Both eyesight and other reasons ...  3  Skip To Part 3, Q 17

15c. IF CURRENTLY DRIVING: How much difficulty do you have driving during the daytime in familiar places? Would you say you have:
   (Circle One)
   No difficulty at all ......................  1
   A little difficulty .......................  2
   Moderate difficulty .....................  3
   Extreme difficulty .....................  4

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16. How much difficulty do you have **driving at night**? Would you say you have:

(Circle One)

- No difficulty at all.......................... 1
- A little difficulty............................. 2
- Moderate difficulty........................... 3
- Extreme difficulty............................ 4
- Have you stopped doing this because of your eyesight......................... 5
- Have you stopped doing this for other reasons or are you not interested in doing this ......................... 6

16A. How much difficulty do you have **driving in difficult conditions**, such as in bad weather, during rush hour, on the freeway, or in city traffic? Would you say you have:

(Circle One)

- No difficulty at all.......................... 1
- A little difficulty............................. 2
- Moderate difficulty........................... 3
- Extreme difficulty............................ 4
- Have you stopped doing this because of your eyesight......................... 5
- Have you stopped doing this for other reasons or are you not interested in doing this ......................... 6

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PART 3: RESPONSES TO VISION PROBLEMS

The next questions are about how things you do may be affected by your vision. For each one, please circle the number to indicate whether for you the statement is true for you all, most, some, a little, or none of the time.

<table>
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<tr>
<th>READ CATEGORIES:</th>
<th>All of the time</th>
<th>Most of the time</th>
<th>Some of the time</th>
<th>A little of the time</th>
<th>None of the time</th>
</tr>
</thead>
<tbody>
<tr>
<td>17. Do you accomplish less than you would like because of your vision?</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
<tr>
<td>18. Are you limited in how long you can work or do other activities because of your vision?</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
<tr>
<td>19. How much does pain or discomfort in or around your eyes, for example, burning, itching, or aching, keep you from doing what you'd like to be doing? Would you say:</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
</tbody>
</table>

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For each of the following statements, please circle the number to indicate whether for you the statement is **definitely true**, **mostly true**, **mostly false**, or **definitely false** for you or you are **not sure**.

**(Circle One On Each Line)**

<table>
<thead>
<tr>
<th></th>
<th>Definitely True</th>
<th>Mostly True</th>
<th>Not Sure</th>
<th>Mostly False</th>
<th>Definitely False</th>
</tr>
</thead>
<tbody>
<tr>
<td>20.</td>
<td>I stay home most of the time because of my eyesight.....</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
</tr>
<tr>
<td>21.</td>
<td>I feel frustrated a lot of the time because of my eyesight..........................</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
</tr>
<tr>
<td>22.</td>
<td>I have much less control over what I do, because of my eyesight. .......................</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
</tr>
<tr>
<td>23.</td>
<td>Because of my eyesight, I have to rely too much on what other people tell me.</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
</tr>
<tr>
<td>24.</td>
<td>I need a lot of help from others because of my eyesight..............................</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
</tr>
<tr>
<td>25.</td>
<td>I worry about doing things that will embarrass myself or others, because of my eyesight........................</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
</tr>
</tbody>
</table>
Survey of potential participant perspective on ocular gene therapy in Australia

Patient attitudes to clinical trials

We are seeking your opinion on future clinical trials for gene therapy. Please indicate how much you agree or disagree with the following statements.

1. **New and better treatments can only be produced if patients agree to take part in clinical trials.**
   - □ Strongly agree
   - □ Agree
   - □ Neither agree or disagree
   - □ Disagree
   - □ Strongly disagree

2. **Without the results from clinical trials, doctors would be less able to select the best treatment.**
   - □ Strongly agree
   - □ Agree
   - □ Neither agree or disagree
   - □ Disagree
   - □ Strongly disagree

3. **Pharmaceutical companies should ensure that valid clinical trials are conducted on every drug treatment before it is generally available.**
   - □ Strongly agree
   - □ Agree
   - □ Neither agree or disagree
   - □ Disagree
   - □ Strongly disagree
Survey of potential participant perspective on ocular gene therapy in Australia

Patient attitudes to clinical trials

4. If most patients refused to take part in clinical trials, important developments in medicine would be seriously delayed.
   - [ ] Strongly agree
   - [ ] Agree
   - [ ] Neither agree or disagree
   - [ ] Disagree
   - [ ] Strongly disagree

5. Clinical trials are carried out according to strict rules to safeguard the interests of patients.
   - [ ] Strongly agree
   - [ ] Agree
   - [ ] Neither agree or disagree
   - [ ] Disagree
   - [ ] Strongly disagree

6. I assume that drug treatments that have been prescribed for me have already been thoroughly tested in clinical trials.
   - [ ] Strongly agree
   - [ ] Agree
   - [ ] Neither agree or disagree
   - [ ] Disagree
   - [ ] Strongly disagree
Survey of potential participant perspective on ocular gene therapy in Australia

Patient attitudes to clinical trials

7. **Clinical trials are only conducted on drugs/treatments for which there is already evidence to show that they are likely to be effective.**
   - [ ] Strongly agree
   - [ ] Agree
   - [ ] Neither agree or disagree
   - [ ] Disagree
   - [ ] Strongly disagree

8. **The conduct of all clinical trials is carefully regulated to ensure that the results are valid.**
   - [ ] Strongly agree
   - [ ] Agree
   - [ ] Neither agree or disagree
   - [ ] Disagree
   - [ ] Strongly disagree

9. **I would want as much written information as possible about a clinical trial before I agreed to take part.**
   - [ ] Strongly agree
   - [ ] Agree
   - [ ] Neither agree or disagree
   - [ ] Disagree
   - [ ] Strongly disagree
Survey of potential participant perspective on ocular gene therapy in Australia

Patient attitudes to clinical trials

10. I would want to know before agreeing to take part that I would be free to withdraw from the clinical trial at any time.

☐ Strongly agree
☐ Agree
☐ Neither agree or disagree
☐ Disagree
☐ Strongly disagree

11. I would want to know if I would be likely to get side effects by taking part in a clinical trial before I agreed to take part.

☐ Strongly agree
☐ Agree
☐ Neither agree or disagree
☐ Disagree
☐ Strongly disagree

12. I would only take part in a clinical trial if I thought I understood everything about it.

☐ Strongly agree
☐ Agree
☐ Neither agree or disagree
☐ Disagree
☐ Strongly disagree
Survey of potential participant perspective on ocular gene therapy in Australia

Patient attitudes to clinical trials

13. I think I would find being in a clinical trial frightening.
   - □ Strongly agree
   - □ Agree
   - □ Neither agree or disagree
   - □ Disagree
   - □ Strongly disagree

14. I would only take part in the clinical trial if I thought that my own health would benefit.
   - □ Strongly agree
   - □ Agree
   - □ Neither agree or disagree
   - □ Disagree
   - □ Strongly disagree

15. I would only take part in a clinical trial if I thought that I would not be inconvenienced by doing so.
   - □ Strongly agree
   - □ Agree
   - □ Neither agree or disagree
   - □ Disagree
   - □ Strongly disagree
Survey of potential participant perspective on ocular gene therapy in Australia

Patient attitudes to clinical trials

16. I would only take part in a clinical trial if I know which treatment I was going to receive.
   - [ ] Strongly agree
   - [ ] Agree
   - [ ] Neither agree or disagree
   - [ ] Disagree
   - [ ] Strongly disagree

17. I would only take part in a clinical trial if I was sure that the doctor treating me knew which treatment I was getting.
   - [ ] Strongly agree
   - [ ] Agree
   - [ ] Neither agree or disagree
   - [ ] Disagree
   - [ ] Strongly disagree

18. If I was satisfied with my current treatment, I would probably refuse to take a different treatment in a clinical trial.
   - [ ] Strongly agree
   - [ ] Agree
   - [ ] Neither agree or disagree
   - [ ] Disagree
   - [ ] Strongly disagree
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Patient attitudes to clinical trials

19. It is important for people like me to take part in clinical trials to confirm the value of new treatments and or medical techniques.
   ☐ Strongly agree
   ☐ Agree
   ☐ Neither agree or disagree
   ☐ Disagree
   ☐ Strongly disagree

20. I would take part in a clinical trial because the results should benefit patients like me in the future.
   ☐ Strongly agree
   ☐ Agree
   ☐ Neither agree or disagree
   ☐ Disagree
   ☐ Strongly disagree

21. I think all patients who are eligible should be asked to take part in clinical trials.
   ☐ Strongly agree
   ☐ Agree
   ☐ Neither agree or disagree
   ☐ Disagree
   ☐ Strongly disagree
Survey of potential participant perspective on ocular gene therapy in Australia

Patient attitudes to clinical trials

22. Unless advised by their doctor to withdraw from a trial, all patients should cooperate fully until the trial is finished.

☐ Strongly agree
☐ Agree
☐ Neither agree or disagree
☐ Disagree
☐ Strongly disagree
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Future contact

We intend to publish the results of this study. These reports of de-identified aggregated results will be provided to patient support groups which have assisted in participant recruitment. Study results will be disseminated to the scientific community through scientific publications in peer-reviewed journals and presentations in relevant national and international conferences.

Would like to receive a de-identified copy of the aggregated results at the completion of the study (anticipated early 2022) via email?

☐ Yes
☐ No

Do you consent to your data being retained for possible future longitudinal research, subject to further ethics approval?

☐ Yes
☐ No

Would you like to be contacted for possible testing to see if you have the mutation in RPE65 or any other identified mutations associated with inherited retinal dystrophy?

☐ Yes
☐ No
Survey of potential participant perspective on ocular gene therapy in Australia

Future contact

If you answer yes to any of these questions, please provide your contact details. This is entirely optional and any information will be kept confidential.

Title

First name

Last name

Email address

Phone

Thank you for your time. You are helping researchers understand patient perspectives in treating inherited retinal disease.

Yours sincerely,

Heather Mack (principal researcher), Lauren Ayton (responsible researcher), Fred Chen, John Grigg, Thomas Edwards, Fleur O'Hare, Ceecee Zhang, Keith Martin

Please return this document in the enclosed reply paid envelope.

If the envelope is missing mail to

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VIC 3002

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Royal Victorian Eye and Ear Hospital
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