

Utility Estimates for Vision Loss in Leber Hereditary Optic Neuropathy

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INTRODUCTION

- Leber Hereditary Optic Neuropathy (LHON) is a maternally inherited mitochondrial disease that leads to central vision loss due to optic neuropathy [1,2].
- The condition typically emerges in young adults and is more common among men [3].
- Vision loss starts with a painless blurring and clouding of the central vision, typically in one eye at first, that rapidly progresses to blindness in both eyes [4].
- LHON has a significant impact on many aspects of patients’ lives including daily activities, emotional functioning, relationships, studies, work, recreation, and finances [5].
- GS010 (GenSight Biologics) is a gene therapy for the treatment of LHON in patients with an ND4 mutation.
- Quality of life weights (utilities) can be used to assess the net benefit of treatment.

OBJECTIVE

This study was designed to estimate patient utilities associated with varying levels of visual acuity in LHON.

METHODS

- Previous research combined secondary analysis of trial data, in-depth qualitative interviews with patients, and expert opinion to develop eight LHON health state vignettes describing the wider impact of vision loss on health related quality of life (HRQL).
- The vignettes varied by level of visual acuity as defined by LogMAR score, where a lower LogMAR score equates to better visual acuity. The vignettes ranged from mild vision loss (LogMAR<0.3) through to light perception (LogMAR≥4). An example vignette is presented in **Figure 1**.
- Members of the public from the United Kingdom (UK) (n=301) and the Republic of Ireland (ROI) (n=61) were recruited via an online recruitment platform (Prolific) and completed an online survey (hosted on Qualtrics) in which they rated the vignettes using the Health Utilities Index-3 (HUI-3) and EQ-5D-5L.
- Participants were randomised to value four (out of eight) health state vignettes, which were presented in a random order to each participant.
- HUI-3 data were scored using the associated multiple attribute utility function [6].
- EQ-5D-5L data were scored using the Hernandez et al. (2020) mapping function [7].
- A sub-sample (n=120) also completed a one to one time trade off (TTO) interview (including visual analogue scale [VAS] assessment) to assess all eight vignettes.

Scan the QR code in the bottom right of this poster to see more information about how the health state vignettes were developed.

HS6 – Counting fingers

- You have an eye condition that has left you with almost complete central vision loss. You can count fingers held up in front of you. You have limited use of your peripheral vision. You have been told that your vision may continue to deteriorate.
- You cannot read words on a page and rely on read aloud technology. You can only recognise someone by their voice.
- You rely on adaptations (e.g. read aloud technology) to use electronic devices like mobile phones, tablets and computers.
- You can mostly navigate familiar environments with minimal difficulty. You are unable to navigate unfamiliar environments without the assistance of another person or visual aid.
- You are unable to drive and get limited use of public transport. Your vision loss means that you rely on friends, family members, or taxis to take you where you need to go.
- Your vision loss is devastating, and you find it very difficult to come to terms with. You often feel frustrated and worry about the future. You often feel anxious or depressed.
- You find socialising difficult and feel socially isolated. You find it hard to meet new people because of your vision loss. Social activities such as going to a restaurant can be very logistically and emotionally challenging. This limits the enjoyment you feel from such events and the type of social events you can attend.
- Your visual impairment is not always obvious to other people which adds to the difficulties you experience.
- You rely on others to help you conduct many of your usual activities (e.g. shopping, cooking and paperwork).
- You can physically do some sports, such as gym work, with assistance. Your vision loss limits the types of sports you can take part in.
- Your eye condition does not cause you any pain.
- You rely on support from others and adaptations to participate in work or education. Your condition means that some career paths and roles may not be an option for you.

Figure 1. Example health state vignette – Counting fingers (equivalent to LogMAR 2.0)

RESULTS

Sample characteristics

- The online sample (N=362) included 301 participants from the UK and 61 participants from the ROI.
- Average age 46.5 years, 51% female, 49% employed full-time, and 39% had a diagnosis of a chronic illness.
- The TTO sample (N=120) included 100 participants from the UK and 20 participants from the ROI.
- Average age 46, 52% female, 52% employed full-time, and 63% had a diagnosis of a chronic illness.

Utility estimates

- Generally, utilities were lower for states with lower visual acuity.
- There was minimal ‘misordering’ of health states, where a ‘better’ health state had a lower utility value than a ‘worse’ health state (EQ-5D-5L valuations of health states 4 and 5, TTO valuations of health states 6 and 7). None of these mis-orderings fell outside of the 95%CIs. Results are presented in **Figure 2**.

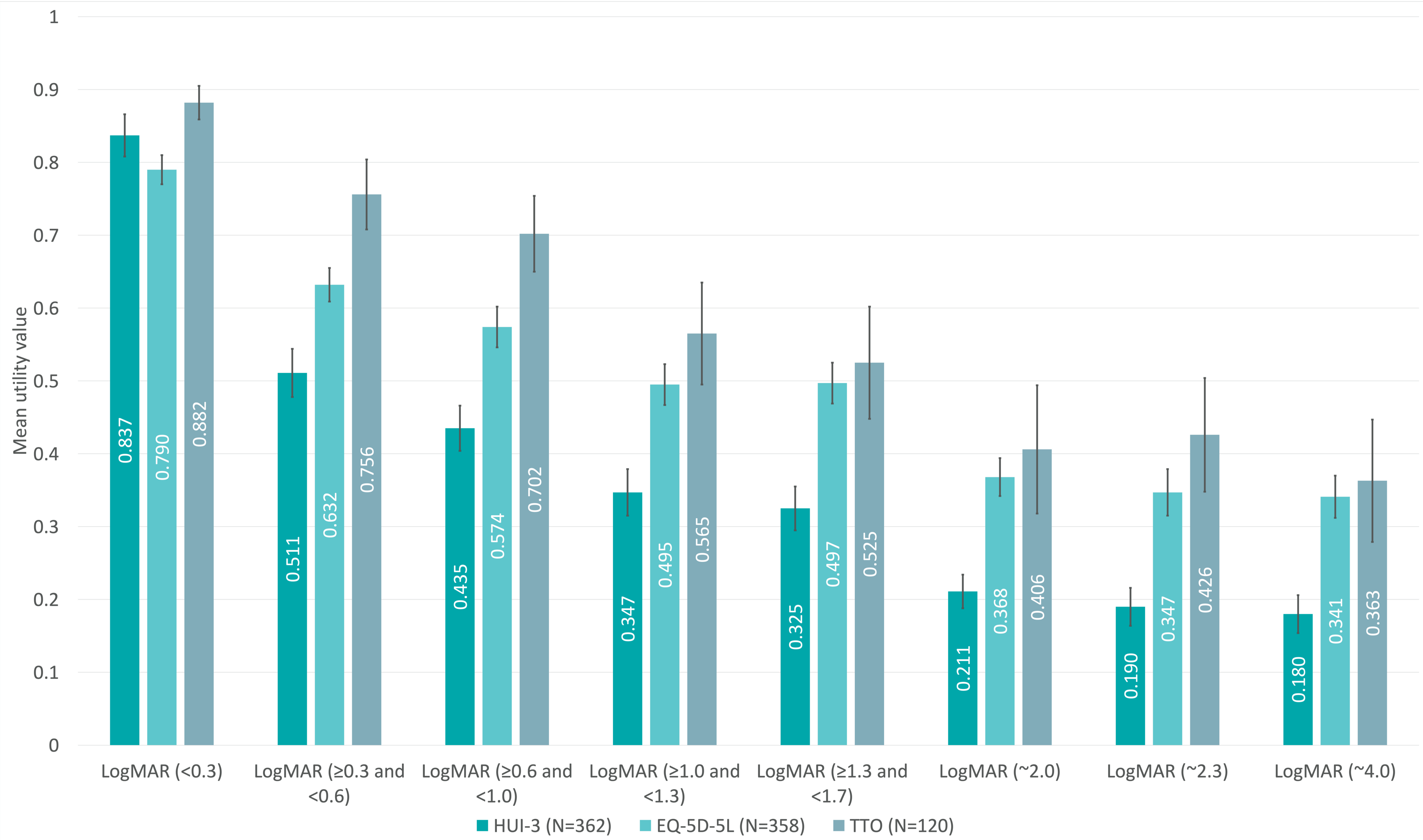


Figure 2. Health state utilities based on HUI-3, EQ-5D-5L and TTO valuations of health state vignettes. Error bars show confidence intervals 95%.

To see the results for UK and ROI separately, scan the QR code on the bottom right of this poster.

DISCUSSION

- This study describes utilities for eight health states describing different levels of vision loss in LHON patients.
- The utility results varied by the methods used to generate them. HUI-3 results showed the greatest level of difference by visual acuity.
- The HUI-3 scores were lower than the EQ-5D-5L scores which is likely due to the inclusion of a question specifically related to vision in the HUI-3.
- The utility results are dependent on the accuracy of the vignettes. Trial data and qualitative interview data were combined to develop vignettes. Iterative rounds of review were undertaken to establish content validity. However, trial data were not available for all health states.
- In vignette development interviews, clinical experts discussed the similarity and potential overlap between health states. Clinical experts were unable to differentiate the HRQL impacts in similar health states. This could explain the small amount of ‘misordering’ between health states.
- The vignettes did not allow for the description of other potential factors influencing HRQL such as time since diagnosis.
- As members of the general public were required to imagine living in the health state described when making valuations, there is uncertainty around how feasible it was for people to imagine living with a visual impairment.

CONCLUSION

The data suggest that early prevention of decline in vision loss through therapeutic intervention could lead to substantial net gains in HRQL.

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Scan the QR code to view supplementary results for this study and related research exploring patient and carer burden in LHON

