# Health State Utilities Associated with X-Linked Retinitis Pigmentosa in the United Kingdom

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## RATIONALE

- X-linked retinitis pigmentosa (XLRP) is a rare inherited retinal disease characterized by progressive impairment in visual field (VF) and visual acuity (VA), typically leading to legal blindness.[1]
- Although no treatments for XLRP are currently available, gene therapies are under investigation. Cost-utility analyses (CUAs) will be needed to examine the value of these treatments in selected countries, and these CUAs will require health state utilities.
- Available utility values representing retinitis pigmentosa in published literature are limited.[2-4] No previously published studies have reported utilities specifically associated with XLRP.
- Although health technology assessment (HTA) agencies typically prefer utilities derived from generic preference-based measures (GPBM) completed by patients in clinical trials,[5-7] alternative methods may be acceptable when these measures are inappropriate or infeasible.[6] The current study used the vignette-based method as an alternative because (1) GPBMs do not assess impact of visual conditions and may have limited sensitivity to differences in XLRP severity, and (2) it may not be possible to administer a generic instrument to a sufficient number of patients in each health state given that XLRP is a rare disease that progresses over multiple decades.

## OBJECTIVE

- The purpose of this study was to estimate health state utilities representing varying levels of visual impairment associated with XLRP so that these values could be used in cost-utility modeling.

## METHODS

### Study Design and Participants

- Utilities were estimated in a vignette-based time trade-off (TTO) study via in-person interviews with general population respondents in three locations in the UK (Edinburgh, London, Newcastle).

### Health State Development

- Health states were developed based on published literature,[8-11] clinical trial data, multiple interviews with four clinical experts, interviews with three patients with XLRP, and an interview with a carer of an adult with XLRP. Draft health states were refined based on a pilot study with a general population sample in the UK (N=20; mean age=52.8 years; 45% female).
- Two images were developed to demonstrate visual impairment in the health states. Photographs taken with a 180-degree lens were altered to simulate the level of VF and VA impairment in each health state by restricting the diameter of the image around a central point and applying Gaussian blur.
- A background description of XLRP was developed to introduce participants to the condition, define VA and VF, and show unaltered versions of the two images representing "normal vision" to provide context for the altered images that appear with each health state (see **Figure 1** for example health state vision images).
- Based on recommendations from clinical experts, eleven health states were included to represent specific combinations of impairment levels in VA and VF (see **Figure 2** for an example health state). Levels of impairment included no impairment, mild, moderate, severe, very severe, near blind, and blind.

**FIGURE 1: Example Health State Images**



**FIGURE 2: Sample Health State D (VA impairment = Mild; VF impairment = Severe)**



### Procedures

- Participants first completed an introductory ranking task (i.e., ranking the 11 health states in order of preference). Then, participants valued the health states in a TTO task with a 10-year time horizon and 6-month trading intervals.
- Participants also completed a demographic and clinical form.

## RESULTS

### Study Participant Description

- A total of 245 participants completed interviews (mean age=41.4 years; 51.0% female), including 80 in Newcastle, 85 in London, and 80 in Edinburgh (**Table 1**). No participants reported having been diagnosed with RP, but three (1.2%) reported knowing someone diagnosed with RP.

**TABLE 1: Demographic and Clinical Characteristics**

| Characteristics | Descriptive Statistics (N=245) |
|---|---|
| Age, Mean years (SD) | 41.4 (15.6) |
| **Gender, n (%)** | |
| Male | 119 (48.6%) |
| Female | 125 (51.0%) |
| Nonbinary | 1 (0.4%) |
| **Ethnic/Racial Background, n (%)** | |
| Asian/Asian British | 18 (7.3%) |
| Black/African/Caribbean/Black British | 5 (2.0%) |
| White | 212 (86.5%) |
| Mixed/multiple ethnic groups | 5 (2.0%) |
| Other | 5 (2.0%) |
| **Marital Status, n (%)** | |
| Single | 99 (40.4%) |
| Married/Cohabitating/Living with a partner | 74 (30.2%) |
| Other[a] | 72 (29.4%) |
| **Employment Status, n (%)** | |
| Full-time work | 111 (45.3%) |
| Part-time work | 48 (19.6%) |
| Other[b] | 86 (35.1%) |
| **Education Level, n (%)** | |
| University degree | 140 (57.1%) |
| No university degree | 105 (42.9%) |

[a] Other marital status includes divorced (n=21), separated (n=7), widowed (n=2), cohabitating/living with a partner (n=38), and other [not specified] (n=4).
[b] Other employment status includes homemaker (n=11), student (n=24), unemployed (n=12), retired (n=26), and other (not specified) (n=13).
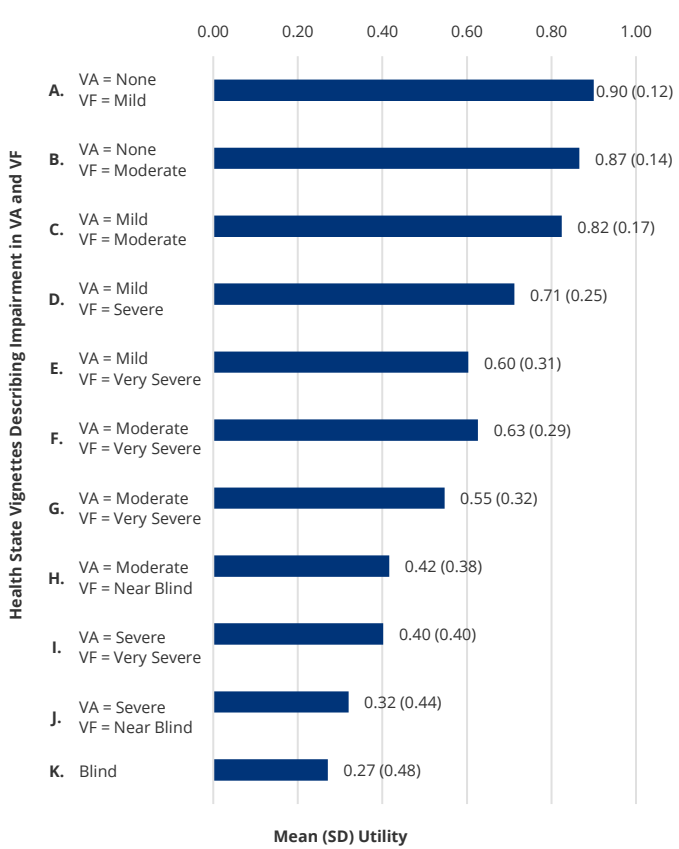Abbreviation: SD = standard deviation

### Health State Rankings

- In the ranking task, participants tended to prefer health states with less impairment in VA and VF over health states with more severe impairment.
- Health state K (blind) was ranked as the least preferred health state by nearly all participants (91.8%). The 20 (8.2%) participants who preferred health state K (blind) over at least one other health state thought that being blind would be less "irritating," "distracting," "frustrating," or "stressful" than having impaired vision.

### Health State Utilities

- Mean (SD) utilities are presented in **Figure 3**.
- Mean utilities followed the expected pattern with utility decreasing with greater impairment in VA and VF. Health state A, with no VA impairment and mild VF impairment, had the highest utility (0.900). The blind health state had the lowest utility (0.271). This pattern of mean utilities was consistent across the three interview locations.
- The utility estimate of 0.27 for the health state representing blindness is similar to values for blindness reported in previous research.[12]

**FIGURE 3: Mean Health State Utilities**



| | Health State | Utility |
|---|---|---|
| A. | VA = None, VF = Mild | 0.90 (0.12) |
| B. | VA = None, VF = Moderate | 0.87 (0.14) |
| C. | VA = Mild, VF = Moderate | 0.82 (0.17) |
| D. | VA = Mild, VF = Severe | 0.71 (0.25) |
| E. | VA = Mild, VF = Very Severe | 0.60 (0.31) |
| F. | VA = Moderate, VF = Very Severe | 0.63 (0.29) |
| G. | VA = Moderate, VF = Very Severe | 0.55 (0.32) |
| H. | VA = Moderate, VF = Near Blind | 0.42 (0.38) |
| I. | VA = Severe, VF = Very Severe | 0.40 (0.40) |
| J. | VA = Severe, VF = Near Blind | 0.32 (0.44) |
| K. | Blind | 0.27 (0.48) |

Abbreviation: SD = standard deviation; VA = visual acuity; VF = visual field

## KEY TAKEAWAY



The health state utilities estimated in this study reflect the substantial impact of VF and VA impairment associated with XLRP. These utilities may be useful in CUAs assessing the value of treatments for XLRP.

## CONCLUSIONS

- In general, utilities followed reasonable patterns, with lower utilities associated with more severe impairment in VA and VF.

- The relatively low utilities for the more severely impaired health states highlight the substantial impact of XLRP on health-related quality of life.

- Utilities in this study are based on preferences for vignette-based health state descriptions rather than the real-world experience of patients. To mitigate this limitation as much as possible, health states in the current study were based on input from a broad range of clinicians and patients to ensure that they accurately represent XLRP.

- The health state utilities estimated in this study may be useful in CUAs assessing the value of treatments for XLRP. In addition, these utilities may be applicable in models examining treatments for other visual conditions where reduced VF and VA are the primary symptoms.

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