

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

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Introduction

- X-linked retinitis pigmentosa (XLRP) is a rare inherited retinal disease characterized by impairment in visual field (VF) and visual acuity (VA) with gradual loss of vision leading to blindness.¹
- Gene therapies for XLRP are currently under investigation, and health state utilities will be needed for use in cost-utility analyses to examine the value of these treatments.
- Available utility values representing retinitis pigmentosa in published literature are limited.²⁻⁴ A previous study estimating XLRP utilities in the UK found that less severe visual impairment was associated with higher mean utilities.⁵
- Although utilities derived from generic preference-based measures (GPBM) like the EQ-5D are useful for maximizing comparability across studies,⁶⁻⁸ alternative methods are often used when generic measures are inappropriate or infeasible.⁷ The current study used the vignette-based method as an alternative because (1) GPBMs may have limited sensitivity to visual impairment associated with XLRP, and (2) it may not be possible to administer a generic instrument to sufficient numbers of patients given the rarity of XLRP.

Objective

- The purpose of this study was to estimate health state utilities representing varying levels of visual impairment associated with XLRP.
- This study was a replication of a study previously conducted in the UK.⁵

Methods

Study Design and Participants





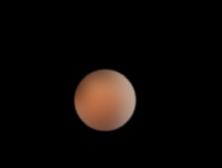
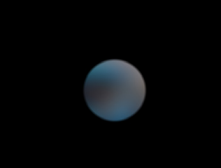
- Utilities were estimated in a vignette-based time trade-off (TTO) study via in-person interviews with adult general population respondents in two locations in the US (Bethesda, Maryland and Seattle, Washington).

Health State Development

- Health states were developed based on published literature,⁹⁻¹² clinical trial data, multiple interviews with four clinical experts, interviews with three patients with XLRP, and an interview with a caregiver of an adult with XLRP. Draft health states were refined based on a pilot study with a sample of general population respondents in the US (N=22; mean age=49.0 years; 63.6% female).
- Two images were included in the health states to demonstrate visual impairment. To simulate the level of VF and VA impairment in each health state, photographs taken with a 180-degree lens were altered 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).

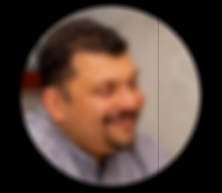

Methods (continued)

Figure 1. Example Health State Images

Health State	Visual Field Impairment	Visual Acuity Impairment	Image of Faces	Image of Street Scene
	Normal Vision without Impairment (shown with background information)			
	None	None		
	Moderate	Mild		
	Very Severe	Severe		

- Based on recommendations from clinical experts, 11 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 2. Sample Health State D (VA impairment = Mild; VF impairment = Severe)

Visual Acuity: Mild	
<ul style="list-style-type: none">• You have mild visual acuity impairment.• Your vision is somewhat blurred.• Objects do not appear perfectly clear and sharp.• In most situations, you can see well enough to read and recognize faces.	
Visual Field: Severe	
<ul style="list-style-type: none">• You have severe visual field impairment.• Your visual field is constricted. You only see objects that are in the middle of your visual field.• You notice this in all situations.• When walking, you often trip or bump into objects.	
Night Blindness	
<ul style="list-style-type: none">• It is very difficult for you to see in the dark.	
Impact	
<ul style="list-style-type: none">• You have difficulty with daily activities such as finding things in your home, shopping, and getting around when you leave home.• You are concerned about the future. Sometimes, your visual condition has an impact on your emotions.• You cannot drive.	
Images Representing Impairment	
 	

Procedures

- Participants were introduced to the health state descriptions and completed a ranking task ordering the 11 health states according to preference. Participants then valued the health states in a TTO task with a 10-year time horizon and 5% trading increments.
- Participants also completed a demographic and clinical form.

Results & Discussion

Sample Description

- A total of 107 participants completed interviews (mean age=47.3 years; 47.7% female), including 53 in Bethesda and 54 in Seattle (**Table 1**). One participant (0.9%) reported having been diagnosed with retinitis pigmentosa, and two (1.9%) reported knowing someone diagnosed with retinitis pigmentosa.

Results & Discussion (continued)

Table 1. Demographic and Clinical Characteristics

Characteristics	Descriptive Statistics (N=107)
Age, Mean years (SD)	47.3 (15.5)
Gender, n (%)	
Male	53 (49.5%)
Female	51 (47.7%)
Nonbinary	3 (2.8%)
Ethnicity, n (%)	
Hispanic or Latino	8 (7.5%)
Not Hispanic or Latino	99 (92.5%)
Ethnic/Racial Background, n (%)	
Asian	11 (10.3%)
Black or African American	14 (13.1%)
White	74 (69.2%)
Mixed/Multiple racial groups ^a	6 (5.6%)
Other ^b	2 (1.9%)
Marital Status, n (%)	
Single	46 (43.0%)
Married	28 (26.2%)
Other ^b	33 (30.8%)
Employment Status, n (%)	
Full-time work	49 (45.8%)
Part-time work	17 (15.9%)
Other ^c	41 (38.3%)
Education Level, n (%)	
University degree	76 (71.0%)
No university degree	31 (29.0%)

^a Mixed/multiple racial groups include 'American Indian or Alaska Native and Black or African American' (n=1), 'American Indian or Alaska Native and White' (n=2), 'Asian and White' (n=1), 'Black or African American and White' (n=1), and 'White and Other Race' (n=1); ^b Other marital status includes divorced (n=13), separated (n=4), widowed (n=7), cohabitating/living with a partner (n=7), and other (n=2); ^c Other employment status (grouped) includes homemaker (n=2), student (n=3), unemployed (n=11), retired (n=17), and other (n=8).

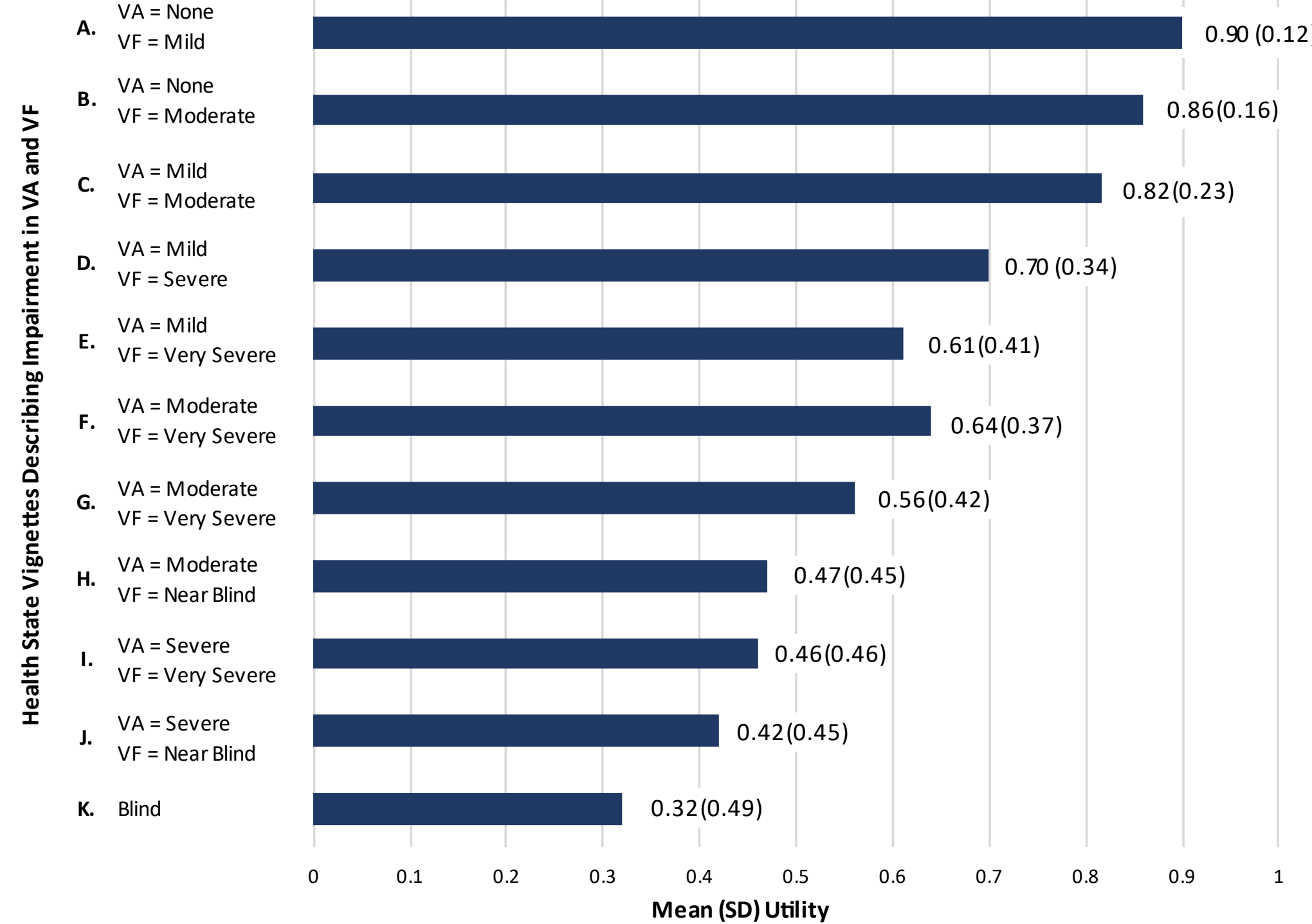
Health State Rankings

- In the ranking task, participants preferred health states with less impairment in VA and VF over those with more severe impairment.
- The blind health state (K) was ranked as the least preferred by nearly all participants (97.2%). The three (2.8%) participants who preferred the blind health state over at least one other health state thought that it would be easier to adjust to or cope with being completely blind.

Health State Utilities

- Mean (SD) utilities are presented in **Figure 3**.
- Mean utilities decreased with greater impairment in VA and VF. Health state A, with no VA impairment and mild VF impairment, had the highest utility (0.904). The blind health state (K) had the lowest utility (0.323). This pattern of mean utilities was consistent across both interview locations.
- There were no significant differences in mean health state utilities between the US and UK studies.
- The utility estimate of 0.323 for the health state representing blindness is similar to values for blindness reported in previous research.¹³

Figure 3. Mean Health State Utilities



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

Conclusions

- In general, utilities followed expected patterns, with lower utilities associated with more severe impairment in VA and VF.
- The relatively low utilities for the more severely impaired health states reflect the substantial impact of VA and VF impairment associated with XLRP on health-related quality of life.
- The utilities derived in this study represent preferences for health state vignettes rather than the real-world experiences of actual patients. To minimize this limitation and ensure the health states accurately represent XLRP, they were developed with input from clinicians, patients, and a caregiver.
- Utilities estimated in this study may be useful in US-based cost-utility analyses assessing the value of treatments for XLRP or other visual conditions where reduced VF and VA are the primary symptoms.

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Disclosures

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