# Estimating Health State Utilities in Primary Hyperoxaluria Type 1: A Valuation Study

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#### Conclusions

- · Published data on the HRQoL burden of PH1, particularly quantitative health state utility data, are scarce; our study using 3 different utility assessment techniques presents data on how the humanistic burden of PH1 varies across CKD stages and post-CLKT.
- Quantitative health state utility data from this study demonstrate the HRQoL impact of PH1, with its unique clinical features relative to non-PH1-related CKD (eg. need for earlier and more intensive hemodialysis, potential need of CLKT); this impact becomes especially pronounced once hemodialysis is required and systemic disease mainfestations occur The data obtained in this vignette-based health state valuation study will facilitate health economic evaluation of future treatments for PH1

### Introduction

- Primary hyperoxaluria type 1 (PH1) is a rare genetic disease characterized by overproduction of oxalate
- retina, heart, blood vessels, bone, skin, and nervous system<sup>5-7</sup> Because oxalate is cleared by the kidneys, patients with PH1 may experience kidney stones, becomes impaired and oxalate accumulates in other parts of the body, damage may also occur to the nephrocalcinosis, progressive kidney failure, and ESKD due to renal oxalate deposition; as renal clearance
- including intensive (eg, daily) hemodialysis and CLKT for later stages of disease<sup>6,6</sup> Management options for PH1 include various pharmacologic and nonpharmacologic interventions
- Quantitative utility data to inform cost-effectiveness analysis in PH1 are limited; to address this gap, the present valuation study was conducted to estimate health state utilities for PH1 in adults and children

Health state vignettes for adult patients and pediatric patients were developed based on literature review and expert opinion to describe different stages of CKD (defined by GFR) related to PH1, and a post-CLKT health state at >1 year following transplantation (Table 1)

## Table 1. PH1 Health States for Valuation

CKD Stage/Kidney Function/Health State	Adult Health State	Child Health State (~6 Years)	_
CKD Stage 1 (GFR ≥90) Normal or high kidney function	2		
CKD Stage 2 (GFR 60-89) Mildly decreased kidney function	2	2	
CKD Stage 3a (GFR 45-59) Mildly to moderately decreased kidney function	A2	<u>-</u>	
CKD Stage 3b (GFR 30-44) Moderately to severely decreased kidney function	A3		
CKD Stage 4 (dialysis) (GFR 15–29) Severely decreased kidney function	A4	C2	
CKD Stage 5 (GFR <15) End-stage kidney disease	A5	СЗ	
CLKT ≥12 months post-transplant	A6	C4	

The utility associated with living in each PH1 health state, as described by the vignettes, was valuated by members of the UK general public using 3 standard utility assessment techniques (EQ-5D-5L, VAS, and TTO assessments) (Figure 1)

## igure 1. Study Objective and Methodology Overview



own EQ-5D-5L responses (ie, responses relating to their own health, separate from the PH1 vignettes). EQ-5D-5L, VAS, and TTO data for the PH1 health state vignettes were also summarized descriptively Descriptive statistics were calculated for participants' sociodemographic information and participants'

# Overall/any dimension problems %Ind et al.13

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	Mean (SD)	Range	95% CI
2-5D Index	0.89 (0.11)	0.56-1.00	0.87-0.91
2-5D VAS	88.61 (9.80)	45-100	86.69-90.53

 The mean EQ-5D-5L utility scores for PH1 health states ranged from 0.68 (CKD stage 1-2 health states) to 0.02 (CKD stage 5 health state) for adult patients and from 0.80 (post-CLKT health state) to -0.05 (CKD stage 5 health state) for pediatric patients (Figure 2)

#### Results

# Participant Demographics and Baseline Characteristics

UK general population according to census data (Table 2) Demographics of the UK respondent sample were generally similar to those of the



In their responses on the EQ-5D-5L regarding their own health status (**Tables 3** and **4**), participants reported higher levels of anxiety/depression and fewer problems in mobility, self-care, and usual activities compared with the UK general

# Table 3. Vignette Study Participants' Self-reported EQ-5D-5L Responses on

THEIR OWN REGILT (N=100) Compared with Normative Data	Compared with the	יווומנועט במנמ
EQ-5D-5L Dimension	N, %	UK Population, %*
Mobility problems	12	18
Self-care problems	_	4
Usual activity problems	00	16
Pain problems	34	33
Anxiety/depression problems	38	21

# Table 4. Study Participants' Mean Self-reported EQ-5D-5L Index Score and EQ-5D VAS Rating of Their Own Health (N=100)

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	Mean (SD)	Range	95% CI
Index	0.89(0.11)	0.56-1.00	0.87-0.91
VAS	88.61 (9.80)	45-100	86.69-90.53

#### EQ-5D-5L

# Figure 2. Adult and Child PH1 Health State Vignette EQ-5D-5L Index Scores (N=100)



- substantially worse by participants when compared with CKD stage 1-3b The CKD stage 4-5 health state utilities for adults and children were rated
- The adult patient post-CLKT health state EQ-5D-5L mean utility score (0.68) was the same as that for the CKD stage 1–2 health states, whereas the pediatric patient post-CLKT health state EQ-5D-5L mean utility score (0.80) was higher than that for the CKD stage 1-3b health states (0.59)

## Visual Analog Scale

The mean VAS scores for adult PH1 health states ranged from 75.02 (CKD stage 1-2 health states) to 38.38 (CKD stage 5 health state) (Figure 3)

# Figure 3. Adult and Child Health State Vignette VAS Scores (N=100)



- The mean VAS scores for pediatric PH1 health states ranged from 73.35 (post-CLKT health state) to 35.97 (CKD stage 5 health state)
- VAS scores showed that the utility associated with the CKD stage 1–2 health states in adults was similar to that for the CKD stage 3a-3b health states
- The CKD stage 4–5 health state utilities for adults and children were rated worse by participants when compared with CKD stage 1–3b, for the post-CLKT health state, the utility scores were higher than for the CKD stage 4–5 health states and generally more comparable to those for the CKD stage 1–3b health states

## The mean adult patient TTO utility scores ranged from 0.88 (CKD stage 1–2 health Time Trade-off



- state) to 0.33 (CKD stage 5 health state)
- The TTO utility scores (Figure 4) showed a pattern similar to the VAS results to CKD stage 4-5 and were generally more comparable to those seen in CKD to the VAS ratings, the TTO scores increased in the post-CLKT health state relative with the lowest utility scores observed in the CKD stage 4-5 health states; similar

# Figure 4. Adult and Child Health State Vignette TTO Weights (N=100)



#### Discussion

- This study provides results from a valuation exercise to estimate utilities for health states describing various stages of CKD as well as the post-transplantation health state in PH1
- Utility values were roughly constant from CKD stage 1-3b and then dropped daily) dialysis to slow progression of systemic oxalosis. systemic oxalosis, with its painful and/or debilitating impacts, in CKD stage 4–5, and because patients in CKD stage 4 often must start intensive (eg. sharply moving to CKD stage 4, possibly because of the emergence of
- · Study limitations include the lack of a caregiver perspective; inability of UK general population (eg, higher prevalence of anxiety in the study has a high degree of symptom variability; lack of direct patient input in health state descriptions to fully capture the burden of living with PH1 which sample) vignette development; and differences between the study sample and the

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