

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 for systemic oxalosis, potential need for CLKT); this impact becomes especially pronounced once hemodialysis is required and systemic disease manifestations 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 by the liver¹⁻⁴
- Because oxalate is cleared by the kidneys, patients with PH1 may experience kidney stones, nephrocalcinosis, progressive kidney failure, and ESKD due to renal oxalate deposition; as renal clearance becomes impaired and oxalate accumulates in other parts of the body, damage may also occur to the retina, heart, blood vessels, bone, skin, and nervous system¹⁻⁷
- Management options for PH1 include various pharmacologic and nonpharmacologic interventions, including intensive (eg, daily) hemodialysis and CLKT for later stages of disease^{8,9}
- 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

Methods

- 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)

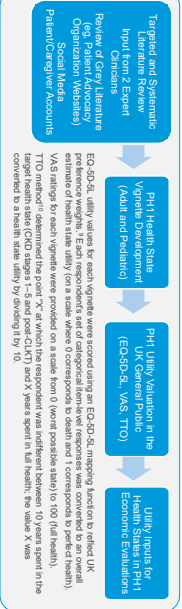
Table 1. PH1 Health States for Valuation

CKD Stage/Utility Function/Health State	Adult Health State	Child Health State (<6 Years)
CKD Stage 1 (GFR ≥90)	Normal or high kidney function	A1
CKD Stage 2 (GFR 60–89)	Mildly decreased kidney function	A2
CKD Stage 3 (GFR 45–59)	Mildly to moderately decreased kidney function	A3
CKD Stage 4 (GFR 30–44)	Moderately to severely decreased kidney function	A4
CKD Stage 5 (dialysis) (GFR 15–29)	Severely decreased kidney function	A5
CKD Stage 6 (dialysis) (GFR <15)	End-stage kidney disease	A6
CLKT 312 months post-transplant		C4

GFR expressed in mL/min/1.73 m²

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)

Figure 1. Study Objective and Methodology Overview



Targeted Literature Review
Review of Key Literature
Organizational Review
Social Media
Patient/Caregiver Accounts

PH1 Health State Vignette Development
PH1 Health State Vignettes
(EQ-5D-5L, VAS, TTO)

PH1 Utility Valuation in the UK General Public
EQ-5D-5L, VAS, and TTO data for the PH1 health state vignettes were also summarized descriptively

Utility Results for PH1 Health States and Economic Evaluations

Abbreviations: CLKT, combined liver and kidney transplant; CKD, chronic kidney disease; ESKD, end-stage kidney disease; GFR, glomerular filtration rate; HRQoL, health-related quality of life; PH1, primary hyperoxaluria type 1; SD, standard deviation; TTO, time-to-state-of-VAS, visual analog scale.

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