Kidney Medicine

Pain and Health-Related Quality of Life in Autosomal Dominant Polycystic Kidney Disease: Results from a National Patient-Powered Registry

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Rationale & Objective: Autosomal dominant polycystic kidney disease (ADPKD) affects health-related quality of life (HRQoL) including pain, discomfort, fatigue, emotional distress, and impaired mobility. Stakeholders prioritized kidney cyst-related pain as an important core outcome domain in clinical trials, leading to the development of disease-specific assessment tools.

Study Design: The ADPKD Registry is hosted online with multiple disease-specific patient-reported outcomes modules to characterize the patient experience in the United States.

Setting & Participants: The ADPKD Registry allows consented participants access to a Core Questionnaire that includes demographics, comorbid conditions, current symptoms, and kidney function. Participants complete subsequent modules on a 3-month schedule, including 2 validated HRQoL tools, the ADPKD-Pain and Discomfort Scale (ADPKD-PDS), the ADPKD Impact Scale (ADPKD-IS) and a Healthcare Access and Utilization module.

Exposures: Patient-reported latest estimated glomerular filtration rate or creatinine used to calculate stage of chronic kidney disease.

Outcomes: Health-related quality of life, measured using validated ADPKD-specific tools; access to polycystic kidney disease-specific health care.

Analytical Approach: For the 2 HRQoL tools, scores were calculated for physical, emotional, and fatigue domains; pain severity; and pain interference (based on the licensed user manuals). Associations to health care access were also assessed.

Results: By July 2022, 1,086 individuals with ADPKD completed at least 1 of the HRQoL modules, and 319 completed 4 over a year. Participants were an average age of 53. In total, 71% were women, and 91% were White, with all chronic kidney disease (CKD) stages represented. In total, 2.5% reported being treated with dialysis, and 23% had a kidney transplant. CKD stage 4/5 participants reported the most dull kidney pain, whereas sharp kidney pain was evenly distributed across early CKD stages. Dull kidney pain had an impact on sleep regardless of CKD stage. There was a strong positive correlation between the ADPKD-PDS and ADPKD-IS. Patients with a neutral or positive HRQoL were less likely to have been denied access to imaging or other care.

Limitations: Currently, all the information collected is patient reported without health record validation of clinical variables.

Conclusions: Use of the HRQoL tools in the ADPKD Registry provided a broad cross-sectional assessment in the United States and provided granular information on the burden of pain across the CKD spectrum in ADPKD. The ADPKD Registry allowed assessment of ADPKD impact in a community that experiences decline in health and kidney function over decades.

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INTRODUCTION

Autosomal dominant polycystic kidney disease (ADPKD) is hereditary and does not discriminate based on sex, race, or ethnicity.[1] However, disparities in care equity and a lack of care guidelines result in inconsistent disease management.[2,3] Continuous growth of kidney cysts leads to tissue damage and fibrosis, an increase in total kidney volume, and a reduction in glomerular filtration rate, along with flank pain, hypertension, or frequent urinary tract infections. Kidney failure occurs in about half of affected individuals by the sixth decade.[1]

Editorial, ● ● ●

Disease-associated pain, discomfort, fatigue, emotional distress, and impaired mobility affect health-related quality of life (HRQoL). Stakeholders prioritized kidney pain as a core outcome domain in both the Standardized Outcomes in Nephrology-Polycystic Kidney Disease (PKD) initiative and the 2021 PKD Regulatory Summit.[4,5] As a result of these and other parallel efforts, ADPKD-specific outcome assessments were designed and validated.[6,7]

In the OVERTURE study, a large international ADPKD cohort was followed with use of disease-specific and generic assessments.[8] Although OVERTURE included an assessment of pain using the commonly used Brief Pain Inventory Short Form as well as questions related to pain in other questionnaires, it lacked the ability to gain a deeper understanding of the ADPKD-pain experience.

In 2019, the Polycystic Kidney Disease (PKD) Foundation launched the ADPKD Registry, a national, longitudinal, patient-reported database to accelerate research and understanding in the clinic.[9] At baseline, both discomfort and physical burden due to pain partially correlated with chronic kidney disease (CKD) stage, although there was a

PLAIN-LANGUAGE SUMMARY

The Autosomal Dominant Polycystic Kidney Disease Registry is a longitudinal, patient-powered research tool created with the goal to better understand the impacts of ADPKD on affected individuals in the United States. Here, we analyze pain and other health-related quality of life outcomes in 1,086 individuals using validated tools and comment on the utility of these tools for future use in clinical trials and observational studies. We found that sharp pain, dull pain, fullness discomfort, and other related impacts affected individuals across the disease spectrum, although some participants reported more dull pain in later stages (CKD stages 4 and 5). Future analysis of these trends over time will be valuable in understanding how to assess and address the burden of pain in autosomal dominant polycystic kidney disease.

moderate increase in higher physical burden scores as disease stages progressed.[10] In this analysis, we provide a deeper examination of participants' self-reported HRQoL and assess ADPKD-HRQoL across different CKD stages as well as in consideration of access to various elements of health care.

METHODS

Individuals with a self-reported diagnosis of ADPKD in the United States are invited to participate in the ADPKD Registry. Exclusion criteria include a diagnosis of autosomal recessive PKD or another cystic disorder, or no PKD diagnosis (unless a parent or caregiver is representing a child who is a patient). Consented participants complete a Core Questionnaire (Table S1) with gender (self-reported sex) demographics, diagnostic methods, past participation in clinical research, comorbid conditions including hypertension, current symptoms, and kidney function (creatinine or estimated glomerular filtration rate [eGFR]). Subsequent modules are available every 3 months, including 2 validated HRQoL tools, ADPKD-Pain and Discomfort Scale (ADPKD-PDS) and the ADPKD Impact Scale (ADPKD-IS).[11,12] The ADPKD-PDS tool is limited to individuals who have not undergone nephrectomy. An additional module to collect details of health care access, utilization, and barriers was internally developed and implemented in May 2021. Participation is voluntary with engagement initiatives to encourage longitudinal survey completion. Key opinion leaders and stakeholders (Table S2) inform participant engagement activities and program management. Other processes, protocol, informed consent, schedule of assessments, and module development details were described previously.[10]

The ADPKD-PDS and ADPKD-IS are validated tools for assessing and standardizing measurement of pain related to ADPKD. The 3 pain types within the ADPKD-PDS are acute pain, dull pain, and fullness and discomfort, with further domains to assess severity or interference with routine activities, leisure activities, relationships, and sleep based on recall of a 7-day period. This tool is intended to standardize patient-reported outcomes (PROMs) in future ADPKD research studies. The ADPKD-IS is a validated tool for standardizing measurement of the impact of ADPKD. The ADPKD-IS is divided into the physical, fatigue, and emotional domains with a 14-day recall period and also has 4 items outside of these domains: guilt, sleep, size/shape of abdomen, and urinary urgency/frequency.

The ADPKD Registry, managed by IQVIA, is a web-based application hosted on a secure server.[11,12] This server and the policies of use are compliant with 21 Code of Federal Regulations Part 11, Good Clinical Practice, and the Health Insurance Portability and Accountability Act. The platform is compatible with desktop, tablet, or mobile devices to maximize participant accessibility. The protocol, consent, and participation modules are approved by the New England Institutional Review Board.[13]

Analytical Approach

Deidentified participant data for modules completed by July 12, 2022, were included in this analysis. Data were cleaned by removing extreme values or textual data in a numeric column. Continuous variables are reported using the mean, standard deviation, response range, and number of observations, and categorical variables are reported using frequency tables and bar charts.

For the 2 HRQoL tools, the ADPKD-PDS and the ADPKD-IS, scores were calculated for physical, emotional, and fatigue domains; pain severity; and pain interference (based on the licensed user manuals). Scores are calculated on a Likert scale with responses ranging from 1 ("not at all" or "not difficult at all" or "not bothered at all") to 5 ("completely or "extremely difficult" or "extremely bothered"). For health care access and clinical trial participation sub-analyses, HRQoL was quantified as good, poor, or neutral. For both tools, a score less than 3 on all 3 subscales was equated with good HRQoL, whereas a score 3 or more on at least 2 subscales was equated with poor HRQoL. Similarly, a score 3 or more on only 1 subscale was labeled as neutral. In instances with limited data, neutral responses are collapsed into good (odds are calculated in favor of a good quality of life). A Spearman's ρ correlation coefficient between HRQoL effect and kidney function was calculated between CKD stage (calculated[14] using participant-reported eGFR) and each scale domain score. Those with unknown kidney function or post-transplant were excluded for the CKD stage-level analyses to minimize variability in responses. Analyses were completed using SAS software v9.4 (SAS Institute), and accompanying plots were generated using both SAS and Microsoft Excel.

Table 1. Characteristics of ADPKD Registry Participants who Completed the HRQoL Modules.

Characteristics	All Patients n = 1,086	Longitudinal Only[a] n = 319	Longitudinal Excluded n = 767
Age (y), mean (range)	53.45 (2-86)	58.73 (22-84)	51.25 (2-86)
Gender, n (%)			
Female	771 (71.0)	213 (66.8)	558 (72.8)
Male	310 (28.6)	104 (32.6)	206 (26.9)
Nonbinary	1 (0.1)	-	1 (0.1)
Transgender female	2 (0.2)	1 (0.3)	1 (0.1)
Transgender male	2 (0.2)	1 (0.3)	1 (0.1)
Race and ethnicity, n (%)			
White	984 (90.6)	299 (93.7)	685 (89.3)
Hispanic/Latino[b]	46 (4.2)	4 (1.3)	42 (5.5)
Black or African American	19 (1.7)	7 (2.2)	12 (1.6)
Asian	21 (2.0)	4 (1.3)	17 (2.2)
More than one race selected	9 (0.8)	1 (0.3)	8 (1.0)
American Indian or Alaska Native	4 (0.4)	1 (0.3)	3 (0.4)
Prefer not to answer	3 (0.3)	3 (0.9)	-
Genetic test, n (%)			
Yes	173 (15.9)	59 (18.5)	114 (14.9)
PKD1	82 (47.4)	28 (47.5)	54 (47.4)
PKD2	15 (8.7)	7 (11.9)	8 (7.0)
Not sure/do not know	69 (39.9)	19 (32.2)	50 (43.9)
Other	7 (4.1)	5 (8.5)	2 (1.7)
No	913 (84)	260 (81.5)	653 (85.1)
Disease stage,[14] n (%)			
CKD stage 1	94 (8.7)	19 (6.0)	75 (9.8)
CKD stage 2	161 (14.8)	31 (9.7)	130 (16.9)
CKD stage 3a	137 (12.6)	39 (12.2)	98 (12.8)
CKD stage 3b	129 (11.9)	45 (14.1)	84 (11.0)
CKD stage 4	137 (12.6)	47 (14.7)	90 (11.7)
CKD stage 5	50 (4.6)	16 (5.0)	34 (4.4)
Unknown	130 (12.0)	19 (6.0)	111 (14.5)
Postkidney transplant	248 (22.8)	103 (32.3)	145 (18.9)
Dialysis status, n (%)			
Yes	27 (2.5)	9 (2.8)	18 (2.3)
No	1059 (97.5)	310 (97.2)	749 (97.7)
Transplant status, n (%)			
Yes	248 (22.8)	103 (32.3)	145 (18.9)
No	838 (77.2)	216 (67.7)	622 (81.1)
Health-related quality of life score, n (%)			
Poor	191 (17.6)	48 (15)	143 (18.6)
Neutral	150 (13.8)	45 (14.1)	105 (13.7)
Good	745 (68.6)	226 (70.8)	519 (67.7)

Abbreviations: ADPKD, Autosomal Dominant Polycystic Kidney Disease; CKD, chronic kidney disease; HRQoL, health-related quality of life.
[a]Longitudinal only defined as individuals who completed at least HRQoL module at least 4 times. The schedule of assessments releases these modules quarterly; 4 completions represent responses over one year. Statistically significant differences between the general and longitudinal cohorts are bolded.
[b]Hispanic or Latino ethnicity assessed separately from race; overlap exists.

RESULTS

Participant Characteristics

In total, 2,676 individuals were enrolled as of July 2022 with 1,086 completing at least 1 of the HRQoL modules. Characteristics of the HRQoL cohort are described in Table 1. Participants had a mean age of 53. In total, 71% were women, and 91% were White, with 2.5% treated with dialysis and 23% postkidney transplant. All CKD stages are present, including 23.5% in stage 1 or 2, 24.5% in stage 3a or 3b, 13% in stage 4, and 5% in stage 5 (based on self-reported kidney function by eGFR). In total, 12% did not indicate a kidney function. Moreover, 16% reported undergoing genetic testing for ADPKD, of whom 47% reported a *PKD1* mutation and nearly 40% unknown.

Table 2. Distribution of ADPKD-IS and ADPKD-PDS Domain and Severity Scores.

	Mean (Standard Deviation)	Median
ADPKD-IS (n= 953)[a]		
Physical domain score	1.9 (0.9)	1.7
Emotional domain score	2.1 (0.9)	2.0
Fatigue domain score	2.2 (1.1)	2.0
ADPKD-PDS (n=944)[a]		
Dull pain severity score	2.1 (1.0)	2.0
Sharp pain severity score	1.7 (1.0)	1.0
Discomfort severity score	2.3 (1.1)	2.3
Overall pain and discomfort severity score	2.1 (0.9)	1.9

Abbreviations: ADPKD-IS, Autosomal Dominant Polycystic Kidney Disease Impact Scale; ADPKD-PDS, Pain and Discomfort Scale.
[a]The ADPKD-PDS and the ADPKD-IS scores were calculated for physical, emotional, and fatigue domains; pain severity; and pain interference (based on the licensed user manuals). Scores are calculated on a Likert scale with responses ranging from 1 ("not at all" or "not difficult at all" or "not bothered at all") to 5 ("completely or "extremely difficult" or extremely bothered").

Additionally, a subcohort of 319 individuals participated longitudinally (defined as completing at least one HRQoL tool at least quarterly over 1 year). Compared with the broader HRQoL cohort, the longitudinal cohort consisted of a larger percentage of males (33% compared with 29%), had more genetic testing (19%-16%), and more stage 3b and 4 (14%-12% and 15%-13%, respectively). Kidney transplant patients were also more prevalent (32% compared with 23%).

Impact of Pain on Quality of Life

Acute or sharp pain in ADPKD may be due to cyst hemorrhage, urinary tract infections, or nephrolithiasis. In contrast, chronic or dull pain is more elusive. Liver cysts may also cause pain.[15] We examined distribution of various domain and severity scores between the 2 tools (Table 2), as well as reviewed specific elements stratified by disease stage. When investigating pain type in the pretransplant cohort (Fig1), we found that, on average, CKD stage 4 and 5 participants reported the most dull kidney pain ($P < 0.0001$). Sharp kidney pain was reported more evenly across disease stages but with increased average frequency in stage 5. Reports of pain type and frequency burden were similar but were lower in the longitudinal compared with the general cohort, especially in later disease stages.

To understand chronic/dull pain's relationship to sleep-related fatigue, we separated responses into CKD stages (Fig 2). Dull kidney pain had an impact on sleep ("somewhat" or "very much") throughout the cohort regardless of CKD stage, although stage 5 participants were more likely to report an impact of "completely" than other pretransplant

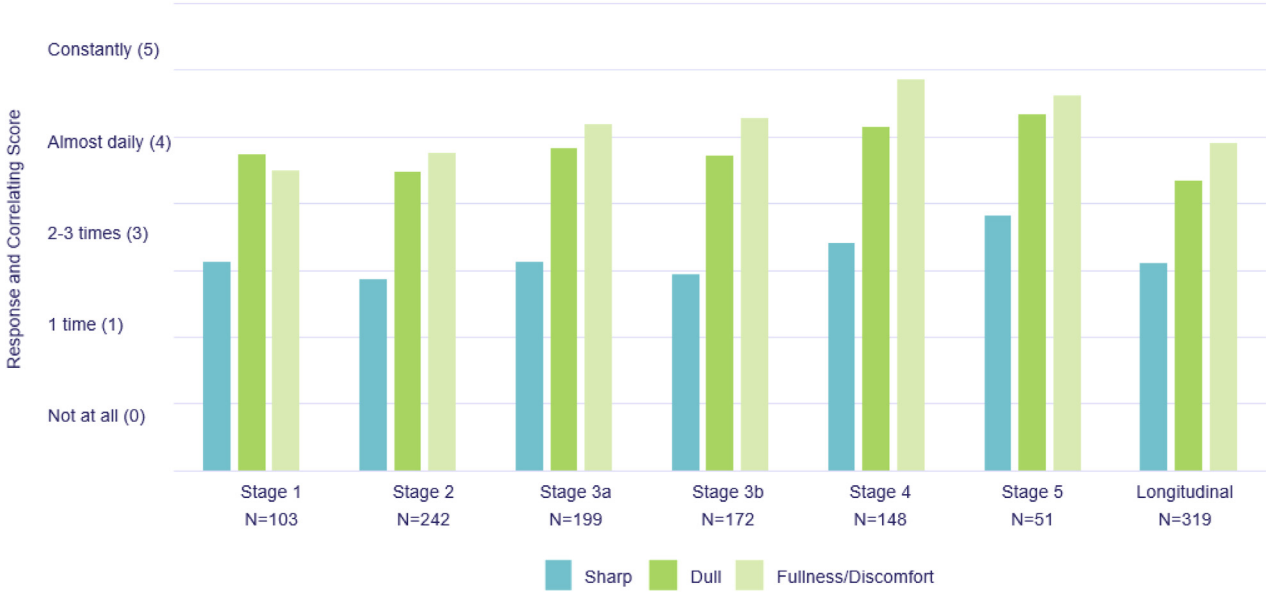

Figure 1. Average response to pain frequency over 1 week by eGFR stage.
Pain type and frequency were collected using the ADPKD-Pain and Discomfort Scale HRQoL outcome assessment. Respondents reported, independently from other pain types, how often they experienced each type of pain believed to be due to their kidney disease. Those with unknown kidney function or postkidney transplant were excluded. The longitudinal cohort is also distributed throughout stage categories. There was a statistically significant association between the individual pain scores (dull, sharp, and discomfort) and CKD stage with eta-squared values of 0.015, 0.021, and 0.030, respectively, indicating small effects, with corresponding p-values using one-way analysis of variance of 0.0259, 0.0047, and 0.0002, respectively. In general, CKD stages 4 and 5 had higher average scores than CKD stages 1-3 for all 3 pain severity scores (dull, sharp, and discomfort) with P value < 0.0001. ADPKD, Autosomal Dominant Polycystic Kidney Disease Impact Scale; CKD, chronic kidney disease; eGFR, estimated glomerular filtration rate; HRQoL, health-related quality of life.

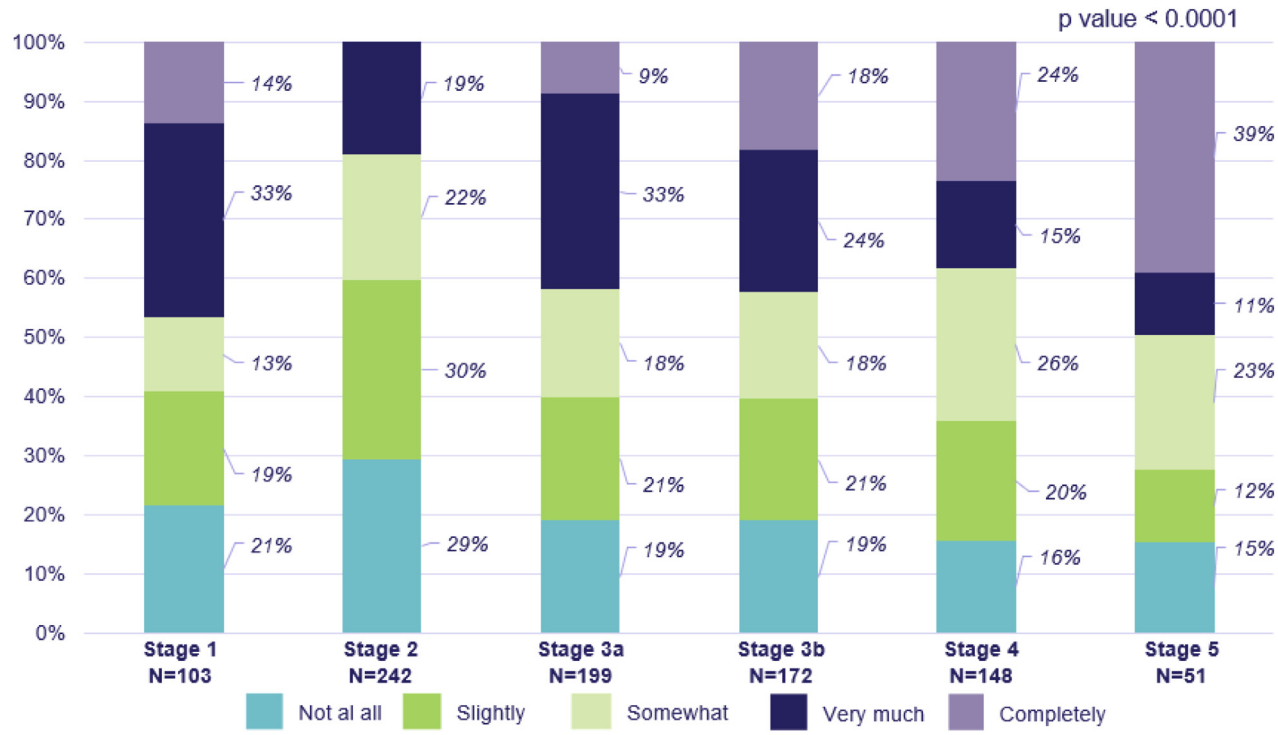

Figure 2. Impact of dull kidney pain on sleep, stratified by CKD stage.
Interference of dull kidney pain on sleep over the past 7 days was collected using the ADPKD-Pain and Discomfort Scale HRQoL outcome assessment. Dull kidney pain was defined as chronic uncomfortable ache or discomfort, often felt in the lower to middle back, abdomen, or sides. Those with unknown kidney function or postkidney transplant were excluded. Distribution of dull kidney pain scores varies significantly by CKD stage (non-zero correlation $\chi^2 = 28.07$, *P* value < 0.0001). ADPKD, Autosomal Dominant Polycystic Kidney Disease Impact Scale; CKD, chronic kidney disease; HRQoL, health-related quality of life.

participants (39.2% vs 11.5%, P < 0.0001). Interestingly, a higher burden of "very much" was most reported in CKD stage 1 and 3 (29.8% average across stage 1, 3a, and 3b) compared with stage 2 (19.0%) (P = 0.0018).

To understand the reliability of HRQoL reported impacts across the 2 scales, we compared the pain response on the ADPKD-IS to the average pain and discomfort severity scores on the ADPKD-PDS. A Jonckheere–Terpstra trend test indicated strong evidence for ordered differences in median ADPKD-PDS scores across the PKD-related pain (ADPKD-IS) categories ranging from "not bothered at all" to "extremely bothered" (P < 0.0001). Participants who report being bothered by PKD-related pain also report higher pain severity and vice versa. This strong positive correlation is also displayed in Figure 3. All pairwise comparisons of mean ADPKD-PDS scores across the various PKD-related pain (ADPKD-IS) levels were statistically significant (P < 0.0001).

Clinical Trial Participation

The Registry asks participants about their prior clinical study experience, motivations, or barriers to participation and notifies them of potential eligibility for actively recruiting studies. Of those who answered the HRQoL modules, 342 (31.6%) indicated that they had previously participated in a clinical study. Those who reported prior participation were 70% less likely to report a poor quality of life than those who did not (odds ratio [OR] = 0.69, P = 0.0536).

Health Care Access And Utilization

A subset of 579 participants also completed the Healthcare Access and Utilization module. We analyzed whether those categorized as having poor HRQoL were more likely to report experiencing barriers accessing care (Table 3). We found no association between lower HRQoL scores and reported barriers to accessing health insurance, specialists, genetic testing, or medications. We also did not see a relationship to higher reported out-of-pocket costs or time to referral to a PKD specialist. However, those with a neutral or positive HRQoL were less likely to report having been denied access to PKD-related imaging procedures (OR = 0.46, P = 0.0418) and had nearly an 80% decrease in the odds of reporting being denied access to other kinds of care because their insurance would not cover the care plan or the copay was too expensive (OR = 0.24, P < 0.0001).

DISCUSSION

Utilization of the ADPKD Registry to assess PROMs in a cohort across the United States provided insights into both the feasibility of the outcome tools used and response

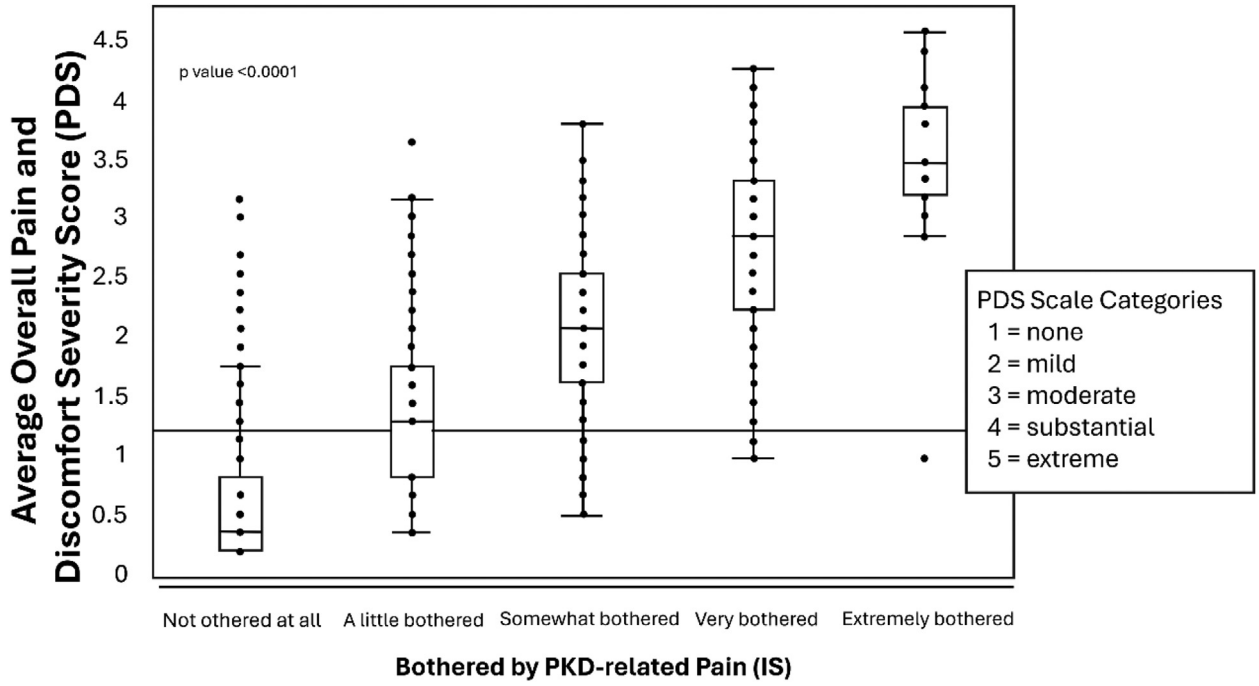

Figure 3. Correlation between pain-related responses on the 2 outcomes assessments. The ADPKD-IS asked "As it relates to your PKD, over the past 2 weeks how bothered were you by your PKD-related pain?". The effect size estimates (and corresponding 95% confidence intervals) comparing successive PKD-related pain categories were 0.71 [0.46, 0.96] for "extremely bothered" vs "very bothered", 1.29 [1.06, 1.52] for "extremely bothered" vs "somewhat bothered", 2.01 [1.79, 2.23] for "extremely bothered" vs "a little bothered", and 2.73 [2.52, 2.94] for "extremely bothered" vs "not bothered at all" (P value < 0.0001). ADPKD-IS, Autosomal Dominant Polycystic Kidney Disease Impact Scale.

patterns across various disease stages. We also tested several assumptions about how HRQoL may affect, or be affected by, elements, such as health care access and clinical trial participation. As a genetic, chronic disease with a progression that spans decades, PKD-specific nuances are important to understanding when and how to measure HRQoL incorporating PROMs in future clinical and research settings.

Prior studies have used existing tools to study HRQoL in the ADPKD population, such as the 36-Item Short Form Health Survey and the Wisconsin Brief Pain Survey.[16] Based on weaknesses identified in a recent review, future studies will benefit from use of the standardized tools specific to the ADPKD population with various types of experienced pain and discomfort.[17] El-Damanawi et al[18] also developed and used an ADPKD-specific outcomes tool for measuring PROMs in ADPKD; however, its use was cumbersome for both patients and test administrators.[19] We chose to use ADPKD-PDS and ADPKD-IS because both are relatively short and were easily adapted to an online format to increase patient accessibility. The ADPKD-PDS also has strong patient endorsement.[20]

We found that pain was common in all levels of pre-transplant kidney function, but most respondents with CKD stage 3b or higher had nearly daily dull pain and fullness/discomfort (Fig 1). In addition, dull pain had a significant impact on sleep (Fig 2) even when kidney function was relatively well preserved. The analysis by Miskulin et al[16] of 1,043 patients with ADPKD in a cross-sectional study using different pain scales also found that pain was observed across different CKD stages and not only in late-stage disease when enlarged kidneys may contribute to discomfort.

ADPKD-PDS and ADPKD-IS were compared at 2 time points over 1 month in a previous study, during which patient burden was observed to start early in disease with differentiation between CKD stages.[21] We found similar results in our analysis. However, to our knowledge, ADPKD-PDS and ADPKD-IS have not been directly compared in the same patient population over a longer timeframe. We show that there is good correlation between the 2 surveys in our study cohort over 1 year. Given that both tools were developed with reiterative testing and validation in ADPKD patients, this is an expected, and reassuring, finding. The strong correlation between the pain-related question on the ADPKD-IS and the pain burden score on the ADPKD-PDS is also notable.

Participants in the registry come from different geographic areas and therefore likely have different experiences with access and utilization of health care. Although we assessed for impact of care with survey questions targeting various health services, we found that only

Table 3. Likelihood of Reporting a Positive Quality of Life Associated With Health Care Access and Utilization[a]

	OR (95% CI)	*P* Value
Do you have health insurance?	4.09 (1.08-15.50)	0.0256
Have you ever been denied access to a **specialist** for PKD-related care because your insurance would not cover it, or had to opt out because the copay was too expensive?	0.56 (0.21-1.44)	0.2217
Have you ever been denied access to a **PKD-related imaging procedure (MRI, CT, MRA or ultrasound)** because your insurance would not cover it, or had to opt out because the copay was too expensive?	0.46 (0.21-0.99)	0.0418
Have you ever been denied access to a **genetic test** related to your PKD diagnosis because your insurance would not cover it, or had to opt out because the copay was too expensive?	0.69 (0.30-1.56)	0.3646
Have you been unable to access a **PKD-related medication (or evaluation for a medication)** because your insurance would not cover the cost of prescription?	1.40 (0.84-2.29)	0.1979
Have you ever been denied access to **any other PKD-related care** because your insurance would not cover it, or had to opt out because the copay was too expensive?	0.24 (0.11-0.50)	<0.0001

Abbreviations: CI, confidence interval; CT, computed tomography; MRA, magnetic resonance angiography; MRI, magnetic resonance imaging; PKD, Polycystic Kidney Disease.
[a]Two analyses were not included due to complexity of categorical variables. For "On average, how much do you have to pay in total each month out-of-pocket for your PKD-related prescription medications?", we did not find that proportion of neutral/positive QoL responses varied significantly ($P = 0.486$). For "If you see a nephrologist, when did you start seeing one?", we also did not find that proportion of neutral/positive QoL responses varied significantly depending on their awareness levels to kidney diagnosis based on the following responses: "before PKD diagnosis", "within a year of PKD diagnosis", "more than a year after PKD diagnosis", or no reported visits to the nephrologist ($P = 0.430$).

insurance denial of an imaging test or nonspecified PKD-related care correlated with a negative HRQoL.

We found that participants who had participated in a clinical trial were more likely to report a neutral or positive HRQoL. Additionally, research on patient motivations to join clinical trials suggests that the 2 biggest motivators are helping others and improving personal treatment, whereas the biggest reason to not join a trial (or being excluded) is because of being too sick.[22,23] If poor HRQoL influences participation in trials, it may also lead to underrepresentation in registries like ours. We are hopeful that virtual participation, not requiring travel to a clinical site, may alleviate part of that risk.

The ADPKD Registry is valuable in many respects. Currently limitations include that all the information collected is patient-reported without health record validation of important variables (such as eGFR). In the future, patients will be able to share their electronic medical record with the ADPKD registry, allowing for more granular correlation of disease state with PROMs. Additionally, most participants report adequate access to health care and insurance coverage, which suggests potential overrepresentation of those with insurance or with good HRQoL who may have a greater capacity for survey participation. Patients with chronic disease who are uninsured may have worse clinical outcomes than those with insurance.[24] Patients who are denied access to imaging or other PKD care may have downstream effects on clinical outcomes because they are underinsured. Other limitations include overrepresentation of females, Whites, patients in CKD stages 2-4, and varied stages of module completion across the cohort. Future efforts are planned to increase representation from underrepresented groups, including returned participant value and enrollment promotion.

Because data entry is patient driven, we can administer surveys, such as the ADPKD-PDS and the ADPKD-IS (with short 1-2 week recalls), more than once and independent of clinic visits. We can thus start to assess changes in response over time and observe if trends in HRQoL can predict disease progression or worse outcomes. The participants in the longitudinal cohort (who had completed at least 1 survey at least 4 times), varied from the broader cohort in representative gender and CKD stage, among others, and also had less reported pain (Fig 1) compared to the broader cohort. Although future analyses on the trends in HRQoL responses over time will be valuable, future interpretation of Registry data should include acknowledgement that participants who participate in a single survey may have different characteristics than those who participate longitudinally.

CONCLUSIONS

Self-reporting on pain, discomfort, fatigue, sleep quality, mobility, health care access, and other ADPKD-related impacts allows understanding of disease effect over time in a community that experiences decline in health and kidney function over decades. Use of the ADPKD-PDS and ADPKD-IS provided a broad cross-sectional assessment of ADPKD-HRQoL in the United States and increased our understanding of the burden of pain across the CKD spectrum in ADPKD. Through online participation opportunities, the Registry seeks to make research contributions more accessible and representative of individuals affected by the disease.

SUPPLEMENTARY MATERIAL

Supplementary File (PDF)

Table S1: Data Dictionaries for Core Questionnaire.

Table S2: Advisory Group Members.

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