

## Development of the Autosomal Dominant Polycystic Kidney Disease Impact Scale: A New Health-Related Quality-of-Life Instrument

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**Background:** The impact of autosomal dominant polycystic kidney disease (ADPKD) on health-related quality of life (HRQoL) is not well understood due to a lack of instruments specific to the condition.

**Study Design:** Content for a new self-administered patient-reported outcome (PRO) questionnaire to assess ADPKD-related HRQoL was developed through clinical expert and patient focus group discussions. The new PRO instrument was administered to study patients with ADPKD to evaluate its reliability and validity.

**Setting & Participants:** 1,674 adult patients with ADPKD participated in this research: 285 patients in focus groups to generate questionnaire content, 15 patients in debriefing interviews to refine the PRO questionnaire, and 1,374 patients to assess the performance and measurement properties of the PRO questionnaire.

**Outcome:** A new PRO questionnaire.

**Results:** The ADPKD Impact Scale (ADPKD-IS), consisting of 14 items representing 3 conceptual domains (physical, emotional, and fatigue) plus 4 additional questions, was developed. The instrument's reliability (regarding internal consistency and test-retest consistency) and validity (content and construct) were supported.

**Limitations:** Need for more responsiveness testing when more data from clinical use become available over time. Complex concepts such as ADPKD-related pain and impact on a patient's HRQoL need further evaluation.

**Conclusions:** The ADPKD-IS is a new patient-centric tool that reliably and validly provides a standardized method for assessing HRQoL and overall disease burden in patients with ADPKD.

Complete author and article information provided before references.

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Autosomal dominant polycystic kidney disease (ADPKD) is a rare hereditary systemic disease characterized by the progressive development of fluid-filled kidney cysts.<sup>1,2</sup> The expansion of the cysts eventually results in physically displacing and obstructing renal tubules, blood vessels, and lymphatics, as well as promoting apoptosis, atrophy, and fibrosis of the renal parenchyma.<sup>3</sup> ADPKD progresses at varying rates, resulting in loss of kidney function later in disease progression. Many patients with ADPKD present with symptoms such as hypertension, flank pain, urinary tract infection, nephrolithiasis, gross hematuria, or palpable kidneys. In later stages, the disease can have a broad spectrum of additional complications involving multiple organs.<sup>4-6</sup> An estimated 45% to 70% of patients with ADPKD progress to end-stage kidney disease by the age of 65 years,<sup>7</sup> at which point they may have cyst-filled kidneys weighing up to 30 pounds.<sup>8</sup>

ADPKD imposes significant burden on patients due to its complex symptomatology, hereditary nature, and gradual disease progression.<sup>9</sup> ADPKD-related disease burden has been assessed in only a few studies and with instruments that have not been validated to assess the impacts of ADPKD.<sup>10-13</sup>

General health assessment questionnaires, such as the 12-Item Short-Form Health Survey version 2 (SF-12v2),<sup>14</sup> miss aspects of quality of life important to patients with ADPKD. As a result, these questionnaires fail to capture the totality of disease-related physical and mental impacts, making them

less sensitive.<sup>12,13</sup> Instruments specifically designed for kidney disease, such as the Kidney Disease Quality of Life (KDQOL) survey,<sup>15</sup> do not capture burden before end-stage kidney disease in ADPKD, for which fatigue, pain, anxiety, nocturia, polyuria, and disruption of daily activities occur. Although the Brief Pain Inventory–Short Form (BPI-SF),<sup>16</sup> a measure of pain severity and pain interference with daily activities, has been used in kidney disease and end-stage kidney disease,<sup>17,18</sup> it had not been evaluated for use in ADPKD. The ADPKD Impact Scale (ADPKD-IS) was developed to comprehensively assess health-related quality of life (HRQoL) in patients with ADPKD as a patient-centric valid measure.<sup>19</sup>

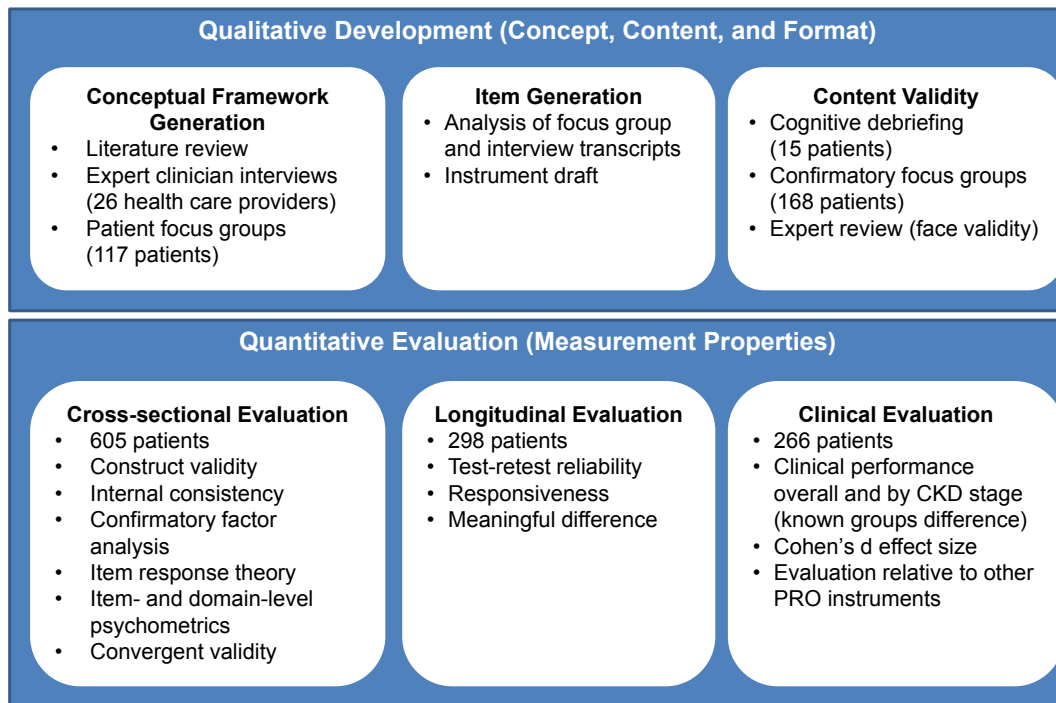
### Methods

#### Overview

In developing the ADPKD-IS instrument, we followed standard guidelines for development of a new patient-reported outcome (PRO) instrument.<sup>20-22</sup> Figure 1 provides an overview of the development process, which consisted of a series of individual studies. The New England Institutional Review Board (Needham, MA) served as the central review body for all studies included in this research.

#### Recruitment

Men and women 18 years or older with ADPKD were recruited through physician and family referrals, advocacy



**Figure 1.** Development of the Autosomal Dominant Polycystic Kidney Disease Impact Scale (ADPKD-IS). Abbreviations: CKD, chronic kidney disease; PRO, patient-reported outcome.

groups, and print advertising. Participating patients provided informed consent before any study-related activities.

### Development of a Conceptual Framework and Questionnaire

A literature review that focused on disease-related unmet need and burden, PRO instruments, and key outcome gaps related to ADPKD was conducted. A list of categories (concepts) to be measured was compiled for further evaluation. Additional detail on the literature review is available in [Item S1](#).

Twenty-six ADPKD clinical experts from North America ( $n = 16$ ), Europe ( $n = 8$ ), and Japan ( $n = 2$ ) were interviewed regarding: (1) the relevance of identified PRO instruments from the literature review, (2) issues likely considered burdensome to patients with ADPKD, (3) aspects of patients' lives likely to improve following successful ADPKD treatment, and (4) potential concepts and questions (items) for an ADPKD-specific PRO instrument. The expert feedback was reviewed to identify issues that, from the perspective of the clinician, affected the HRQoL of patients with ADPKD.

The burden of ADPKD on patients' daily lives was explored in focus groups with 117 adult patients with ADPKD in chronic kidney disease (CKD) stages 1 to 5 from the United States, Turkey, Germany, United Kingdom, and Japan. Groups of up to 8 participants were single sex when possible due to urologic and body image topics. Patients discussed how ADPKD affected their general health, daily activities, physical or social activities, pain experience,

urinary issues (urgency, frequency, and nocturia), and emotional well-being. Transcripts were coded, and concepts or themes mentioned by at least 2 participants in a group were considered relevant. Data saturation (the point at which additional sampling provides no new information) was achieved when no new concepts or themes were identified in subsequent groups.

Based on information from patients and clinical experts, an ADPKD-specific conceptual framework (a model representing concepts/themes to be measured and their relationships) and an initial questionnaire were created. Fifteen cognitive debriefing interviews with US-based patients assessed the level of comprehension, understanding, and interpretation of all instructions and questioned wording and response options by the target audience (content validity), and the instrument was refined (question wording, format, and structure) via an iterative process. Additional content validation focus groups with 168 patients in Australia, Argentina, Brazil, Canada, China, Czech Republic, Hungary, Japan, Poland, Romania, South Korea, Spain, and the United States were conducted to further explore ADPKD-related pain and ensure global applicability of the measured concepts. Finally, the ADPKD-IS was reviewed by clinical experts, including physicians and nurses, to ensure that the instrument was a good reflection of the concepts to be measured (face validity).

### Quantitative Evaluation

The ADPKD-IS was administered in an observational study ([ClinicalTrials.gov](#) study number NCT01430494) to obtain

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