



# Survey of 275 Patients and Caregivers Affected By Pyruvate Kinase Deficiency: Impact of Communication with Hematologists on Mental Health and Quality of Life

Rachael F. Grace, MD<sup>1</sup> and Wilma Barcellini, MD<sup>2</sup>

<sup>1</sup>Dana-Farber/ Boston Children's Cancer and Blood Disorders Center, Harvard Medical School, Boston, MA, USA;

<sup>2</sup>Hematology Unit, Pathophysiology of Anemias Unit, Foundation IRCCS Ca' Granda Ospedale Maggiore Policlinico, Milan, Italy

Abstract #152326

## Background

- Pyruvate kinase deficiency (PKD) is a rare congenital hemolytic anemia affecting approximately 3 people per million globally<sup>1,2</sup>
- Currently, there are no disease-modifying treatments; management focuses on supportive blood transfusions, iron chelation, and in severe cases splenectomy,<sup>3</sup> but new disease-targeted therapies are in development
- PKD has a wide-ranging impact on quality of life (QoL).<sup>4</sup> Patient advocacy and patient-reported outcomes research has been limited<sup>3</sup>
- The PKD Advocacy Advisory Council (AAC), a group of patients and caregivers, advocates, and physicians, was formed in 2020, by Agios Pharmaceuticals, to improve both timely diagnosis and access to education, support and care for individuals with PKD

## Objective

Collect the opinions of patients and caregivers affected by PKD about communication with their hematologists to raise awareness about living with PKD and improve future practices and outcomes

## Methods

- A global web-based survey was conducted between Jan-Feb 2021 to improve understanding of PKD and its disease burden
- Two versions were developed: one for adult patients and another for caregivers providing unpaid care to a PKD patient
- The survey included respondents from 11 countries, including France, Germany, Italy, the UK and US
- Survey questions were closed-ended, multiple choice, Likert scale and binary choice plus free text
- The survey was carried out according to British Healthcare Business Intelligence Association Legal and Ethical Guidelines, as well as guidelines established by UK Market Research Society
- Participants were recruited via online panels and AAC member channels, including PKD Facebook groups
- The survey covered: (1) information provided at time of diagnosis, (2) treating hematologist's knowledge of the condition, (3) level of communication with the hematologist and (4) the patient's or caregiver's relationship with the hematologist

## Survey Results

- 200 adult PKD patients and 75 adult caregivers (n=275) completed the survey, making it the largest within the community to date (Figure 1)
- Half of the patients had been diagnosed for >10 years

A		B	
Age of respondents	No. of respondents	Age of respondents	No. of respondents
18-30 years	33	18-30 years	11
31-40 years	72	31-40 years	31
41-50 years	56	41-50 years	19
51-60 years	34	51-60 years	11
>60 years	5	>60 years	2

Figure 1. Distribution of study participants by age. (A) Patients; (B) Caregivers

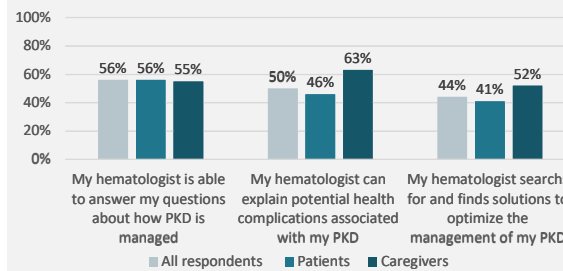


Figure 2. Impression of PKD management among patients & caregivers

## Conclusions

- Survey results highlight a need to adapt clinical approaches to improve outcomes
- Hematologists should seek to improve their PKD understanding and optimize care and support of non-transfused patients
- Care should be holistic and consider emotional and psychosocial health aspects
- Clinicians should consult with other medical specialists, including hematologists specializing in PKD to monitor and manage complications effectively
- Further research is necessary to build on the survey insights, increase awareness, and inform approaches to improve outcomes

### References:

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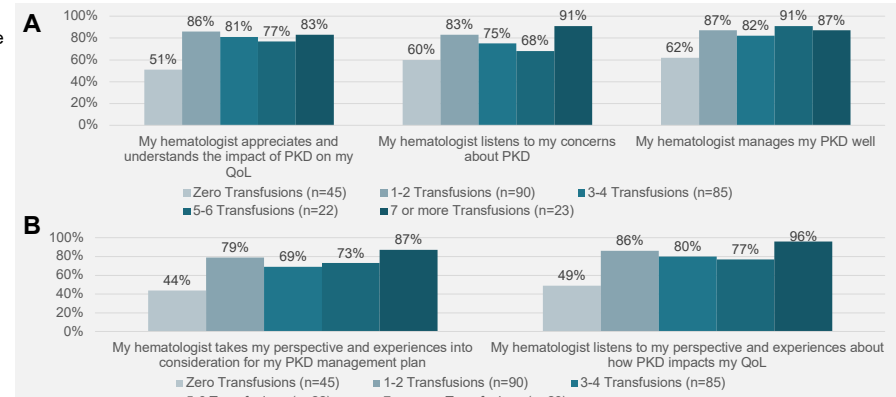


Figure 3. Patient perspective on interactions with hematologist. (A) Management of PKD; (B) Consideration of perspectives.

- 56% reported their hematologist can answer PKD-management questions and 44% reported that their hematologist had profound PKD-related knowledge
- 25% reported feeling neither positive nor negative, or somewhat negative, after interactions
  - 29% reported at least one negative emotion including worried (21%), anxious (17%), and depressed (17%) - the survey reveals an unmet need in emotional and psychosocial support
- Communication was negatively reported amongst non-transfused patients (NTPs) (Figure 3)
  - 62% of NTPs vs. 82% receiving  $\geq 1$  transfusion/year reported that their hematologist manages their condition well ( $P=0.003$ )
  - 51% of NTPs vs. 83% receiving  $\geq 1$  transfusion/year state their hematologist understands the impact of PKD on their QoL ( $P<0.001$ )
  - 44% of NTPs vs. 75% receiving  $\geq 1$  transfusion/year state their hematologist considers their perspective regarding their PKD management plan ( $P<0.001$ )
- Majority of patients have questions on the effects of PKD with age but less than half report that their hematologist discusses this topic

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