

# Abstract Submission

35. *Quality of life, palliative care, ethics and health economics*

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## ADAPTING PATIENT EDUCATIONAL APPROACHES IN PYRUVATE KINASE DEFICIENCY: EUROPE AND NORTH AMERICA FINDINGS FROM THE AAC PATIENT AND CAREGIVER SURVEY

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**Background:** Pyruvate kinase (PK) deficiency is a rare congenital hemolytic anemia affecting approximately 3 people per million globally. As little is known about the burden of this disease and the patient perspective on its management, a patient and caregiver survey was conducted by the PK Deficiency Advocacy Advisory Council (AAC), a group of patients, advocates and physicians focused on supporting and improving care for people with PK deficiency.

While survey respondents were overall satisfied with their hematologist interactions, certain responses indicated underlying differences between subgroups.

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**Aims:** The aims of this study were to understand the perception of patient-hematologist communications among people with PK deficiency and their caregivers and investigate the influence of culture and location on care.

**Methods:** A global web-based survey was conducted in 2021 to assess the (1) information provided at time of diagnosis, (2) treating hematologist's knowledge of the condition, (3) level of communication with the physician and (4) patient's or caregiver's relationship with their hematologist.

Two versions of a questionnaire were developed: one for adult patients and another for caregivers providing unpaid care to a patient. Survey questions were closed-ended, multiple choice, Likert scale and binary choice plus free text. Data were analyzed according to statistical hypothesis testing to determine the significance of results. Test statistics (standard scores) were calculated, and p-values determined using a normal distribution Z-score table.

Participants were recruited via online panels and AAC member channels, including PK deficiency Facebook groups.

**Results:** Of the 199 adult PK deficiency patients and 74 caregiver respondents (N=273), 95 (35%) were from North America (NAM) and 178 (65%) from Europe (Eu). Most reported hematologists manage the condition well (82%). In terms of what drives positive feelings, NAM respondents were most likely to cite their clinician's listening skills (52%), while Europeans most often pointed to their hematologist explaining health complications clearly (55%). After interactions, Americans more often reported feeling 'optimistic' than Europeans (33% vs 24%, p=0.89) while Americans less often reported feeling reassured than Europeans (8% vs 25%, p=0.97).

However, negative emotions were also expressed, with Europeans (31%) being somewhat more likely to experience a negative emotion after interactions than Americans (24%). Europeans were less likely to report that their hematologist takes their perspective and experience into consideration when creating their disease management plan (81% NAM vs 65% Eu, p=0.08).

Among those who reported suboptimal disease management, Eu respondents were twice as likely to point to a lack of listening from their hematologist, while Americans were more likely to report a lack of proactive management of their condition (54% vs 36%,  $p=0.82$ ).

While referrals to specialists, patient groups and counseling services were not common, they were less so in NAm than Eu (18% vs 32%,  $p=0.05$ ). Sharing information about patient support groups is similar in NAm and Eu (36% and 30%, respectively).

**Summary/Conclusion:** To assess potential regional influence on respondent answers, results were separately described by geography. Findings provide potential indicators on how to tailor communications and approaches in Eu and NAm to optimize PK deficiency care.

**Keywords:** Caregiver, Hemolytic anemia, Patient, Pyruvate kinase deficiency