

Abstract Submission

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

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HEALTHCARE RESOURCE UTILIZATION OF ADULT PATIENTS WITH PYRUVATE KINASE DEFICIENCY: A REAL WORLD STUDY USING US VETERANS HEALTH ADMINISTRATION DATA

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Background: Pyruvate kinase (PK) deficiency is a rare genetic condition caused by autosomal recessive mutations in the *PKLR* gene. Given the rarity of the disorder, limited data are available characterizing this population in the real world.

Aims: This study of healthcare resource use (HCRU) is a sub-analysis of a previously presented US Veterans Health Administration (VHA) study of patients with PK deficiency.

Methods: Patients with ≥ 1 PK deficiency-related diagnosis code (ICD-9: 282.3, 282.9; ICD-10: D55.2, D58.9) and documented diagnosis of PK deficiency from physicians' notes from January 1995–July 2019 were included (PK deficiency cohort). Patients in the PK deficiency cohort were matched 1:5 by age at index date, sex, and index year (± 1 year) to patients from the general US VHA population with no diagnosis codes related to PK deficiency (non-PK deficiency cohort). The index date for the PK deficiency cohort was defined as the date of the first medical record with a diagnosis code related to PK deficiency while the index date for the non-PK deficiency cohort was defined as the date of a random visit during their match's index year. Patient characteristics were compared at index, and HCRU was analyzed for time periods of 12 months pre- and post-index.

Results: A total of 18 patients met inclusion criteria for the PK deficiency cohort (mean [SD] age: 56.8 [13.6] years, 94.4% male, mean [SD] duration of follow-up: 7.3 [5.2] years) and were matched to 90 individuals in the general population who became the non-PK deficiency cohort (mean [SD] age: 56.8 [13.1] years, 94.4% male, mean [SD] duration of follow-up: 9.2 [5.8] years). During the 12-month period preceding the index date, there were no statistically significant differences in the number of procedures, outpatient visits, and inpatient admissions between patients with and without PK deficiency (Table). During the 12-month follow-up period, patients in the PK deficiency cohort had higher rates of red blood cell transfusions and monitoring-related procedures such as echocardiograms and gallbladder ultrasounds compared to the non-PK deficiency cohort. Patients with PK deficiency also had more than twice as many outpatient visits than those in the non-PK deficiency cohort (mean [SD]: 35.2 [23.6] vs 14.1 [17.4] visits, respectively, $p=0.0004$). Approximately 16.7% of patients with PK deficiency had at least one inpatient admission over the 12 months of follow-up compared to 2.2% in the non-PK deficiency cohort ($p=0.0319$). During this time, patients with PK deficiency had an average of 1.8 admissions vs 0.0 admissions in the non-PK deficiency cohort ($p=0.0079$).

Image:

Table. Healthcare resource utilization

| | 12-month pre-index date | | | 12-month follow-up | | |
|--|-------------------------|--------------------------|---------|----------------------|--------------------------|---------|
| | PK deficiency (N=18) | Non-PK deficiency (N=90) | P-value | PK deficiency (N=18) | Non-PK deficiency (N=90) | P-value |
| Procedures, n (%) | | | | | | |
| RBC Transfusion | 1 (5.6) | 0 (0.0) | 0.1667 | 3 (16.7) | 0 (0.0) | 0.0040 |
| Splenectomy | 0 (0.0) | 0 (0.0) | - | 1 (5.6) | 0 (0.0) | 0.1667 |
| Cholecystectomy | 0 (0.0) | 0 (0.0) | - | 0 (0.0) | 0 (0.0) | - |
| Liver biopsy | 0 (0.0) | 0 (0.0) | - | 0 (0.0) | 0 (0.0) | - |
| Monitoring-related procedures* | 2 (11.1) | 4 (4.4) | 0.2612 | 11 (61.1) | 5 (5.6) | <.0001 |
| <i>Liver MRI (liver iron overload)</i> | 0 (0.0) | 0 (0.0) | - | 0 (0.0) | 1 (1.1) | 1.0000 |
| <i>Echocardiogram (pulmonary hypertension)</i> | 0 (0.0) | 0 (0.0) | - | 5 (27.8) | 4 (4.4) | 0.0062 |
| <i>Gallbladder ultrasound (gallstones)</i> | 2 (11.1) | 4 (4.4) | 0.2612 | 7 (38.9) | 1 (1.1) | <.0001 |
| Outpatient visits | | | | | | |
| Number of visits, mean ± SD [median] | 14.2 ± 18.3 [8.0] | 13.4 ± 18.6 [7.0] | 0.6470 | 35.2 ± 23.6 [32.0] | 14.1 ± 17.4 [9.0] | 0.0004 |
| Proportion with at least one visit, n (%) | 17 (94.4) | 72 (80.0) | 0.1879 | 17 (94.4) | 87 (96.7) | 0.5232 |
| Inpatient Admissions | | | | | | |
| Number of admissions, mean ± SD [median] | 1.4 ± 5.9 [0] | 0.0 ± 0.2 [0] | 0.6331 | 1.8 ± 5.9 [0] | 0.0 ± 0.1 [0] | 0.0079 |
| Proportion with at least one admission, n (%) | 1 (5.6) | 3 (3.3) | 0.5232 | 3 (16.7) | 2 (2.2) | 0.0319 |

*Monitoring-related procedures included any liver MRI, echocardiogram, or gallbladder ultrasound.
MRI=magnetic resonance imaging; PK=pyruvate kinase; RBC=red blood cell; SD=standard deviation.

Summary/Conclusion: This study suggests that patients with PK deficiency have higher HCRU compared to the general VHA population in the US. While the sample primarily represents adult males and may not be generalizable to the broader PK deficiency population, the results provide insight regarding the resource use and management of an otherwise understudied population.

Keywords: Anemia, Genetic, Health care, Pyruvate kinase deficiency