

According to a study from the United Kingdom (UK), people with pyruvate kinase (PK) deficiency may not live as long as the rest of the population

About the UK study*



This study, sponsored by Agios Pharmaceuticals, Inc., compared people with PK deficiency to the rest of the population, matched by characteristics like age and gender, at an average age of 20 years old at the start of the study. Fifteen years after they were matched, both groups have similar probability of survival.

That looks different at 30 years.

*This was a retrospective observational study using patient information from the United Kingdom. The study matched 89 people with PK deficiency to 445 people from the general population with no hemolytic anemia. Entry date comes from the first observed diagnosis of PK deficiency in the database. There are people in the study with PK deficiency whose diagnosis date may not be accurately captured. That means some people may have been diagnosed more than 30 years ago. This study also doesn't report on all medical procedures or complications that may have occurred.

People's records indicated an average age of death for those with PK deficiency

In the study, **8 out of 89** people with PK deficiency died.

Average age of death:



54 YEARS OLD

ranging from 29 to 77 years old

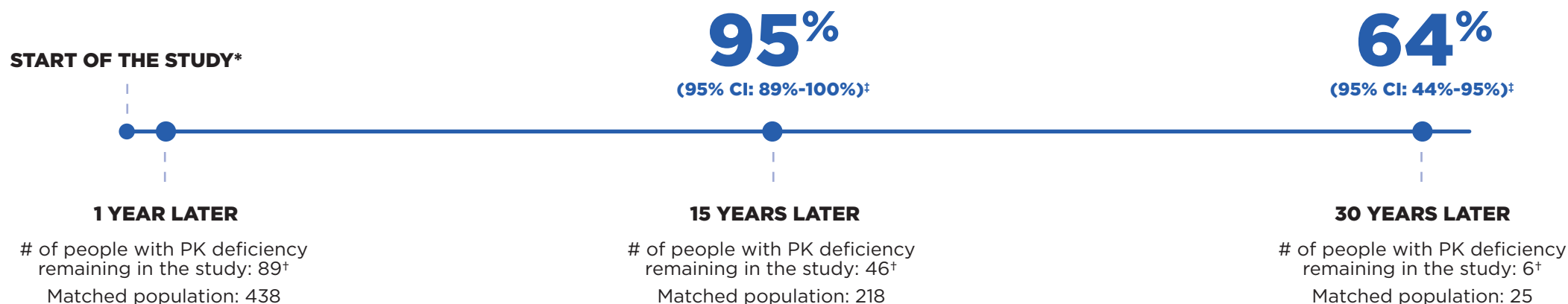
Because not everyone was studied for the full 30 years, it's possible that more people in the study had died. This could change the average age of death.

Due to General Data Protection Regulations and small sample size, causes of death are not reported. This study was unable to confirm the diagnosis of PK deficiency via genetic test results.

People's records also helped determine a probability of survival for those with PK deficiency

Probability of survival can be interpreted as the proportion of patients expected to be alive at a given time point in the study.

Probability of survival for people with PK deficiency:



97% probability of survival (95% CI: 94%-99%)[‡] for the matched population
(30 years from the start of the study)

*Start of the study is the first appearance of diagnosis in medical record.

[†]Over the course of the study, the number of people with available data decreased. In the first year, there were 89 people with PK deficiency and 438 without. By year 15 there were 46 and 218, respectively, and by year 30, there were 6 and 25.

[‡]CI stands for Confidence Interval. For example, 15 years after the start of the study, there is a 95% chance that the probability of survival could be somewhere between 89%-100%.

In a study of United States (US) veterans, people with PK deficiency lived shorter lives than those without PK deficiency

About the US veterans study*



This study, sponsored by Agios Pharmaceuticals, Inc., compared veterans with PK deficiency matched to people with similar characteristics from the general veteran population who did not have hemolytic anemia.

The average age at the start of the study was 59 years.

The life expectancy of the 2 groups were compared.

Because PK deficiency is rare, and women, children, and teens are underrepresented, the results from this study may not reflect what happens with everyone.

*This was a retrospective study that included 18 US veterans with a confirmed PK deficiency diagnosis matched to 90 veterans who did not have hemolytic anemia. The entry date for people with PK deficiency comes from the first medical record of a diagnosis. The entry date for people without PK deficiency was a medical visit during the same year as their match. Over the course of the study, the number of people with available data decreased. In the first year there were 18 US veterans with PK deficiency and 90 without. By year 10, there were 6 US veterans with PK deficiency and 37 without, and by year 20, there were 0 and 2.

People's records helped determine an average number of years until death

Average years until death after the start of the study*



**FOR PEOPLE WITH
PK DEFICIENCY**



**FOR PEOPLE WITHOUT
PK DEFICIENCY**

*We are presenting the median here, which is the midpoint of all calculated years until death in this case. Start of the study refers to when patients were potentially diagnosed with PK deficiency.

People's records also helped determine a probability of survival for those with PK deficiency

Probability of survival can be interpreted as the proportion of patients expected to be alive at a given time point in the study.

Probability of survival for people with PK deficiency:



72% probability of survival (95% CI: 59%-81%)[†] for the matched population
(10 years from the start of the study)

*Start of the study is the first appearance of diagnosis in medical record.

†Over the course of the study, the number of people with available data decreased. In the first year, there were 18 US veterans with PK deficiency and 90 without. By year 10, there were 6 and 37, respectively.

†CI stands for Confidence Interval. For example, 10 years after the start of the study, there is a 95% chance that the probability of survival could be somewhere between 28%-79%.

The Peak Registry study explored the wide range of complications that can develop among people with PK deficiency and hemoglobin (Hb) levels over 10 g/dL

About the Peak Registry, an Agios-sponsored study

This study is an ongoing, global observational study of 261 people with PK deficiency (as of March 27, 2023). This part of the study looked at adult patients 18 years of age or older at enrollment as of the data cutoff date of May 13, 2022.

The study compared the number of people with an average hemoglobin level over 10 g/dL or 10 g/dL or lower, who were either regularly transfused or not regularly transfused

People with a hemoglobin level over 10 g/dL
48 people in total

People with a hemoglobin level 10 g/dL or lower
45 people in total

Regularly transfused

(6 or more transfusions in any 12-month period)

8 out of 45 people (or 17.8%)

17 out of 41 people (or 41.5%)

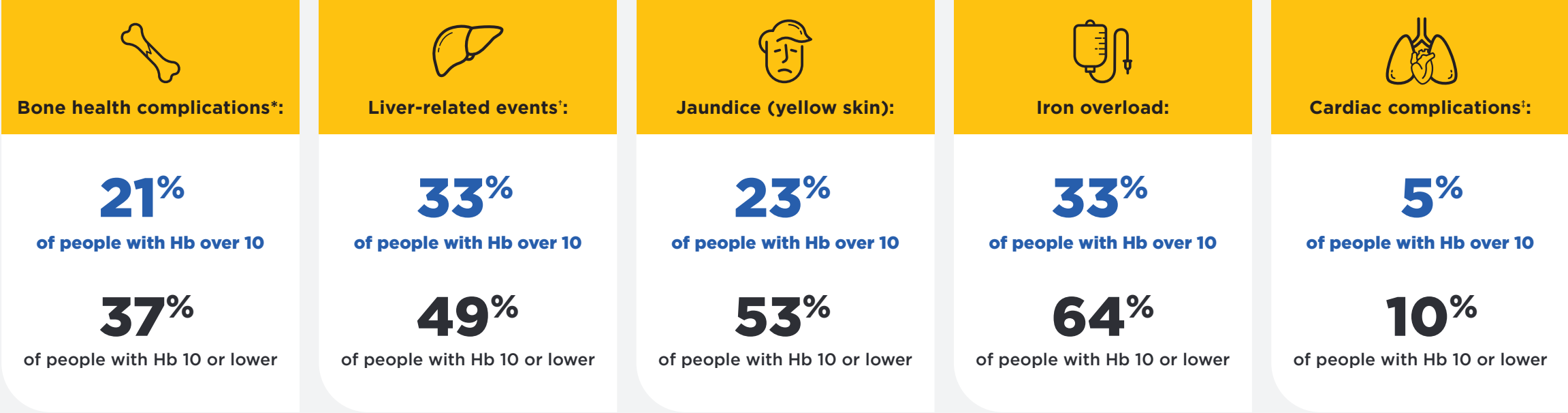
Not regularly transfused

(no more than 5 transfusions in any 12-month period)

37 out of 45 people (or 82.2%)

24 out of 41 people (or 58.5%)

People with PK deficiency may develop lifelong complications, even if they have higher hemoglobin (Hb) levels



Lifelong complications can occur in adults with PK deficiency whether or not their spleen was removed.

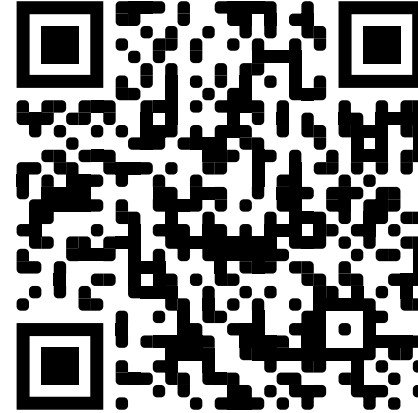
*Bone health complications include fractures, osteopenia, osteoporosis, and bone pain.
†Liver-related events include inflammation of the gallbladder and bile duct, gallstones, and bile duct stones.
‡Cardiac complications include pulmonary hypertension, arrhythmia, left ventricular hypertrophy, and congestive cardiac failure.

Stay on top of complications by carefully monitoring your PK deficiency



Download our monitoring guide by scanning this code

Talk to your doctor about appropriate monitoring now to prevent future complications.



For questions about these studies or for other information about PK deficiency, scan this code

You can also call a Patient Support Manager at **1-800-951-3889** (Mon-Fri, 8 AM to 6 PM ET).

Glossary

CI: Stands for Confidence Interval. For example, if the data says “(95% CI: 89%-100%),” it means that there is a 95% chance that the probability of survival could be somewhere between 89%-100%.

Probability of survival: Can be interpreted as the proportion of patients expected to be alive at a given time point in the study.