

# Understanding Pyruvate Kinase (PK) Deficiency

“ It is a difficult  
disease to  
have, but it  
is manageable.”

Tamara S., 52  
Diagnosed with PK deficiency  
at the age of 6

Managing PK deficiency  
and moving forward



# Setting goals for managing PK deficiency

**With a PK deficiency diagnosis comes some uncertainty about what to do next.**

Whether you've recently been diagnosed or you've been living with the condition for years, learning as much as you can is important. This brochure will help you get started and give you the tools to work closely with your doctor for a more empowered life with PK deficiency.

Creating a treatment journal may also be helpful. Tracking questions and symptoms can make it easier to share changes with your healthcare team and proactively manage your PK deficiency over time.

The **Resources** section at the end of this brochure provides:

- Links to more information
- Ways to find support



At Agios, people living with rare genetic diseases are at the center of everything we do and every decision we make. Agios is a company committed to knowing more about PK deficiency and how it feels for those living with the disease.

## Contents

4

### **About PK deficiency**

The role of red blood cells and what it means to have PK deficiency

6

### **Cause**

The cause of PK deficiency and how the condition is inherited

8

### **Symptoms**

The most common physical symptoms of PK deficiency

10

### **Complications**

Potential short- and long-term complications of PK deficiency

12

### **Management**

Building a PK deficiency management plan with your care team

16

### **Impact**

Understanding the greater impact of PK deficiency

18

### **Moving forward**

Advocating for yourself with PK deficiency

26

### **Resources**

Helpful tools, resources, and support to learn more



*Look out for journal prompts as you're reading. Every note and question is an opportunity to prepare for more productive, meaningful conversations with your hematologist.*

# What is PK deficiency?

PK deficiency is a rare, inherited enzyme deficiency that affects red blood cells (RBCs).

## Key Terms

**Red blood cells (RBCs or erythrocytes):** Cells that carry oxygen throughout the body

**Anemia:** A condition in which the blood has lower-than-normal levels of RBCs or hemoglobin

**Hemoglobin:** A protein that helps RBCs carry the oxygen your body needs

**Hemolytic anemia:** A type of anemia that is caused by the early breakdown of RBCs in the bloodstream or by the spleen

**Hemolysis:** The breakdown of RBCs, which leads to the release of hemoglobin into the blood and production of bilirubin

**Chronic:** Lasting a long time

**Catalyze:** To increase the rate of a chemical reaction

**Enzyme:** A protein that catalyzes chemical reactions that occur inside the body

**Gene mutation:** A permanent change in the DNA sequence of a gene, altering the gene's ability to make a protein and causing the protein to stop working properly

**Deficiency:** A shortage of something

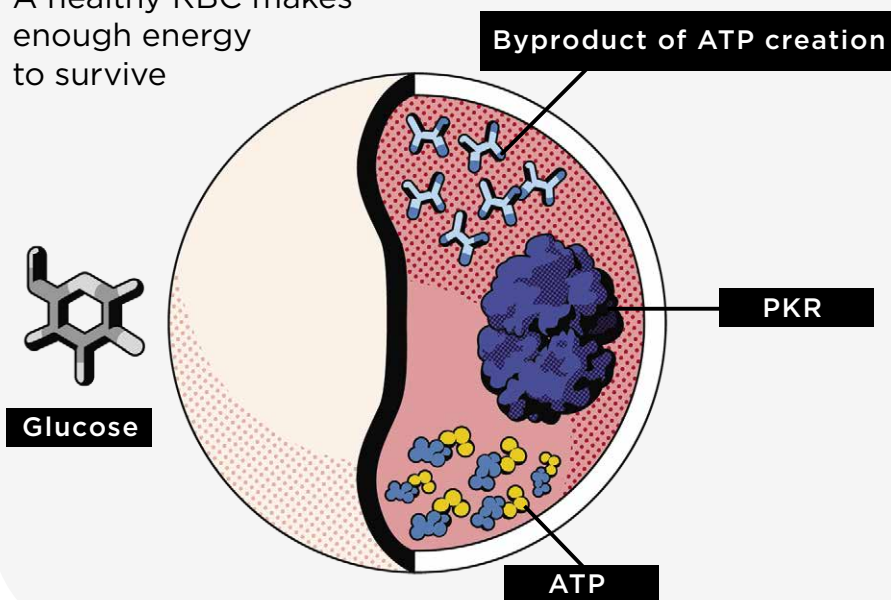
**PKR (pyruvate kinase in RBC):** The pyruvate kinase enzyme

**Glycolysis:** The process of RBCs converting glucose (sugar) into pyruvate and ATP

**ATP:** The primary source of energy for cells

## Healthy RBC

A healthy RBC makes enough energy to survive



## Healthy RBCs

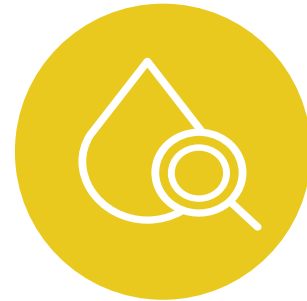
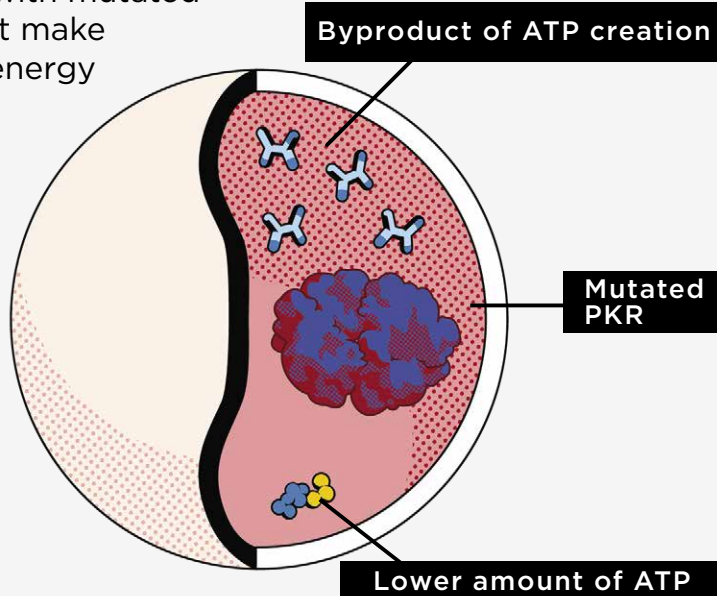
After healthy RBCs are produced, they travel through the lungs, and oxygen binds to a molecule in the cells called hemoglobin. The RBCs then transport oxygen to the rest of the body. Healthy RBCs have enough ATP, or energy, made by the pyruvate kinase R (PKR) enzyme to survive this trip throughout the body.

Each RBC lives for about 120 days before being broken down and removed from circulation.

PK deficiency causes hemolytic anemia. Hemolytic anemia happens when RBCs are broken down faster than they are made.

## PK-deficient RBC

An RBC with mutated PKR can't make enough energy



*The PKR enzyme performs the last step of glycolysis. RBCs convert glucose (a sugar) into pyruvate to make ATP. Deficient PKR leads to less ATP produced, so RBCs have less energy.*

## PK-deficient RBCs

RBCs that do not have enough PK, or that do not have properly working PK, have less ATP, so they die more quickly. The low levels of ATP can cause chronic hemolytic anemia (low RBC counts or low levels of hemoglobin).

### ***DID YOU KNOW?***

While healthy RBCs typically last 120 days, a PK-deficient RBC doesn't have as much energy, so it may only last a few days to weeks.

# What causes PK deficiency?

PK deficiency is a genetic disease caused by a mutation in the *PKLR* gene. More specifically, it's an autosomal recessive mutation passed down from parents to their children. Genes are stretches of DNA that carry genetic information. They are found in long strips of DNA called chromosomes.

## Key Terms

**Gene:** Stretches of DNA. Different genes have different jobs, but many genes tell the proteins in our bodies how to work

**Chromosome:** A very long strand of DNA that's stored in the cell's nucleus and contains its genetic information. Each chromosome may contain hundreds to thousands of genes

**DNA:** The genetic material that tells a cell how to grow and what its job is

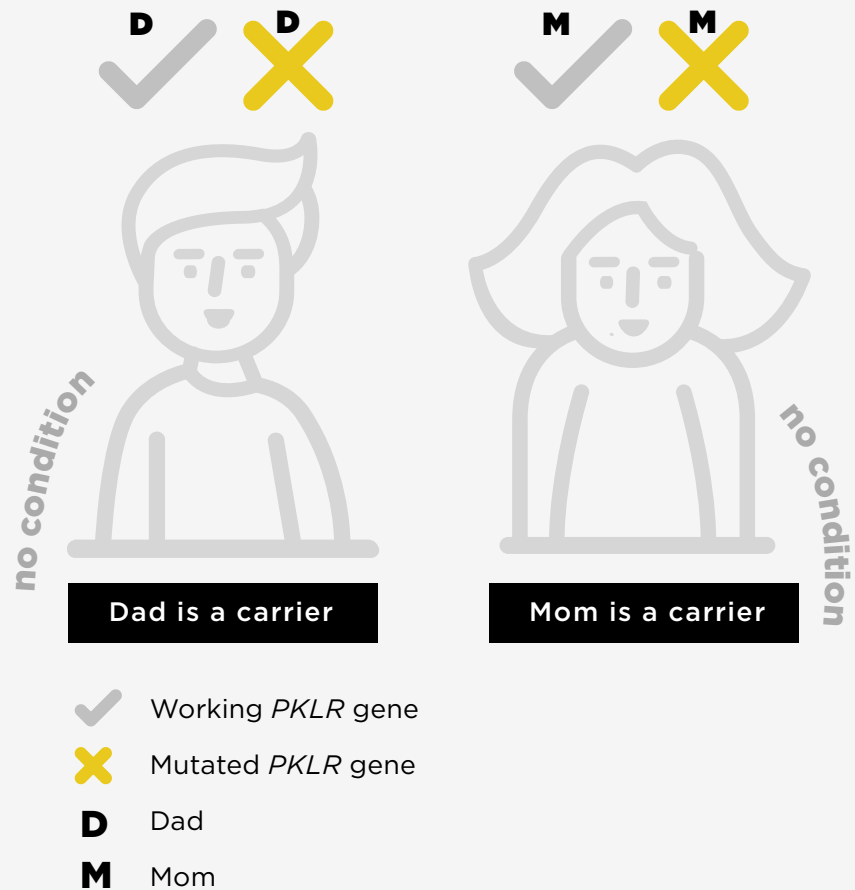
**Autosomal recessive gene inheritance:** A genetic disease that is inherited from receiving 2 nonworking copies of a gene

***PKLR*:** The gene for pyruvate kinase

**Enzyme assay:** A measurement, determined by a blood test, of how active an enzyme is

## Autosomal recessive inheritance

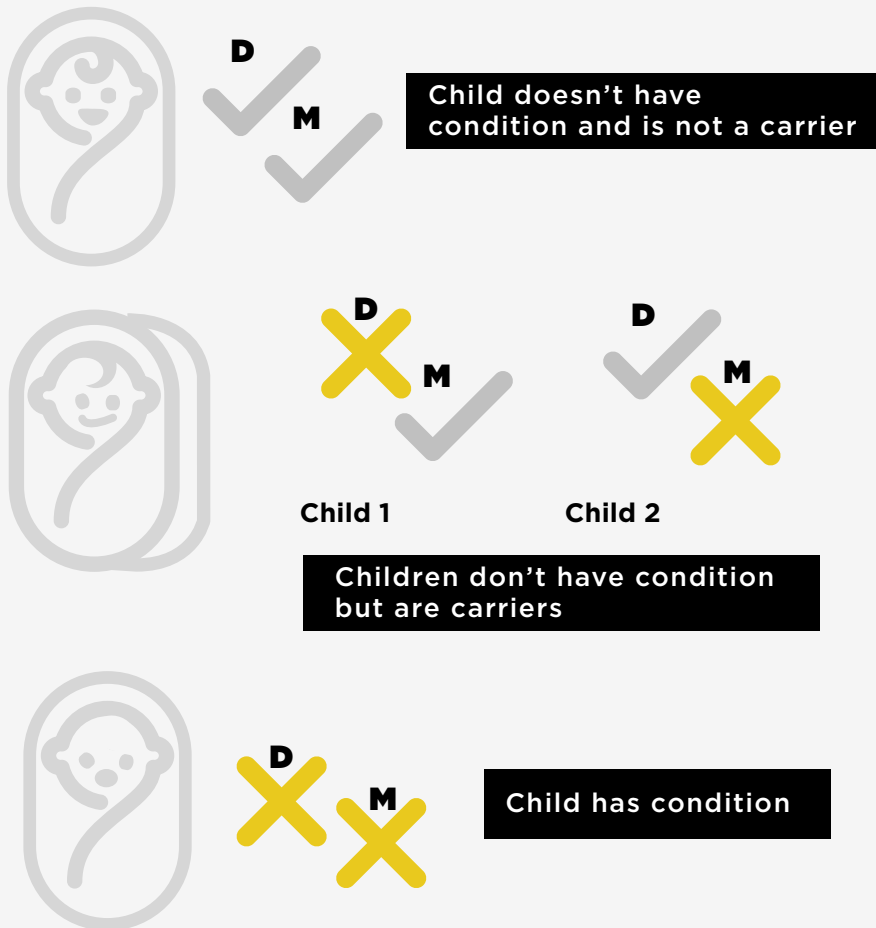
### Carrying mutated *PKLR*



In an autosomal recessive gene inheritance, carriers, or parents, each have one copy of the mutated gene that they pass down to their child. Parents themselves do not usually have PK deficiency. To inherit the condition, a child receives a mutated *PKLR* gene from both parents.

The *PKLR* gene tells the body how to make an enzyme called pyruvate kinase R, or PKR. In PK deficiency, the mutated gene creates PKR that doesn't work properly. As a result, RBCs can't make enough energy and die too soon.

### Inheriting PK deficiency



### Testing to diagnose PK deficiency

Doctors test for PK deficiency in 2 ways. They can test enzyme levels with an enzyme assay, and they can perform genetic tests to identify *PKLR* gene mutations.

*Anemia ID is an Agios-sponsored free testing program. Learn more in the Resources section.*

### DID YOU KNOW?

Over 300 different mutations of the *PKLR* gene have been identified. Approximately 25% of people diagnosed with PK deficiency have versions of the gene mutation that are newly discovered.

# How does PK deficiency affect everyday life?

Signs and symptoms of PK deficiency are different for everyone and can change over time. Even siblings with PK deficiency can have different experiences.

## Key Terms

**Bilirubin:** A substance created from hemoglobin when RBCs break down, which can cause jaundice and scleral icterus

**Jaundice:** Yellowing of the skin caused by high levels of bilirubin in the body

**Scleral icterus:** Yellowing of the whites of the eyes caused by high levels of bilirubin in the body

**Cognitive difficulties:** Problems associated with memory, language, thinking, and judgment

## Common symptoms



- Debilitating fatigue
- Exercise intolerance
- Jaundice
- Scleral icterus

## Some symptoms are cognitive



- Difficulty concentrating
- Brain fog



- Memory loss

“Some days are great and some are horrible and I can't control it.”

Tamara



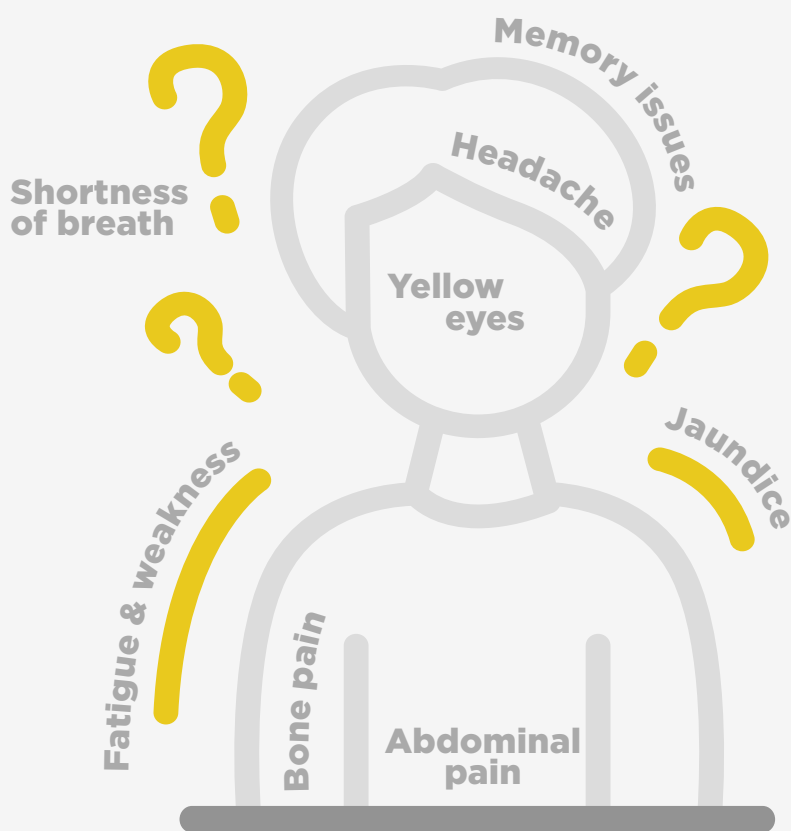


Sometimes it's easy to see the effects of PK deficiency. In other cases, it may be unclear, so it's important to keep track of symptoms over time.

## Additional signs and symptoms

Which of these symptoms do you have and which bother you the most? Are there any you're just starting to notice, or that you didn't think could be related to your PK deficiency?

Circle your symptoms and share with your doctor. If you are experiencing additional symptoms not listed, write them in below.



*Being proactive is key to understanding the different signs and symptoms. It's important to be aware and know what to look for, so you can keep your hematologist informed.*

*Noticing new symptoms or feeling existing symptoms getting worse may signify your condition isn't under control.*

*This could mean you and your doctor need to reevaluate your management plan.*

# What are the complications of PK deficiency?

Different symptoms and complications can arise due to the effect PK deficiency has on RBCs. Complication severity can vary from person to person.

## Key Terms

**Spleen:** An organ that filters blood, helps support the immune system, and removes old or damaged blood cells from the body

**Splenomegaly:** An enlarged spleen

**Aplastic crisis:** When the production of new RBCs temporarily stops

**Gallbladder:** An organ that stores and concentrates bile between meals

**Gallstones:** Small stones that form in the gallbladder

**Iron overload:** An excess of iron in the body

**Ferritin:** A blood protein that contains iron

**Osteopenia:** A decrease in bone mass or bone mineral density. In severe cases, it can progress to osteoporosis

**Osteoporosis:** A disease in which the density and strength of bones are reduced

**Endocrinopathies:** Diseases of the body system that makes hormones

## Complications of chronic hemolysis



### A lack of healthy RBCs

Low amounts of RBCs reduce the amount of oxygen in the body, causing stress on the heart and lungs.

This can lead to:

- Tiredness and fatigue
- Headaches
- Shortness of breath
- An inability to exercise
- Cognitive difficulties
- Aplastic crisis
- Osteopenia/osteoporosis



### The breakdown of RBCs

RBCs break down and release bilirubin into the bloodstream, causing:

- Jaundice and scleral icterus
- Bilirubin to build up in the gallbladder, creating gallstones

### The removal of RBCs

As the spleen removes old or damaged RBCs, they may collect in the organ, causing splenomegaly. Working RBCs may also be removed, leading to worsening anemia.

## DID YOU KNOW?

Some complications of PK deficiency appear later on in life, and may not have any signs. It's important to regularly monitor to prevent future risk.

PK deficiency can cause iron overload in the blood. Iron can collect in the tissues of the body and damage the liver and heart. It may also contribute to other symptoms, such as fatigue and abdominal pain.

## Complications of iron overload

Everyone with PK deficiency is at risk for iron overload. While iron overload can be caused by frequent blood transfusions, many people with PK deficiency who don't get regular transfusions can also develop it—it can occur at any age, with any hemoglobin level.



- **Liver cirrhosis:**  
Scarring of the liver



- **Heart issues and pulmonary hypertension:**  
High blood pressure that affects the arteries in the lungs and right side of the heart



- **Osteopenia/osteoporosis:**  
In addition to iron overload and treatments for it, bone disease and/or bone fragility may be caused by the bone marrow expanding while trying to create new blood cells at a high rate



- **Endocrine/hormone problems:**  
This includes testing for diabetes



*It's important to have a regular monitoring schedule for iron overload. Most hematologists recommend testing ferritin levels once or twice a year.*

*By testing the blood for ferritin (Fe), doctors can see how much iron is building up in the body. If ferritin levels exceed a certain amount (for example, ferritin greater than 500 nanograms per milliliter), be prepared to take action and ask about potential follow-up assessments.*

“ Iron overload is something to monitor and proactively treat because otherwise you would run into all the complications of iron overload itself, including liver disease, cardiac disease, and endocrinopathies. ”

S.S., PK deficiency specialist

# How is PK deficiency managed?

There is no cure for PK deficiency. Your doctor may recommend some of the following options to manage symptoms and complications. Talk to your doctor to find out more.

## Key Terms

**Transfusion:** The process of putting blood into the bloodstream by intravenous (IV, meaning through the veins) infusion into the arm

**Splenectomy:** Surgical removal of the spleen

**Cholecystectomy:** Surgical removal of the gallbladder

## Managing hemolytic anemia



### RBC transfusions

To boost RBC levels, donated blood cells can be added to the bloodstream. Some people with PK deficiency may never have transfusions; some may have them occasionally; others may have them on a regular basis. The degree of anemia and symptoms that come with it are evaluated before adding transfusions to a management plan.



### Splenectomy

The spleen may become enlarged due to RBCs breaking down. The spleen also sometimes removes RBCs that still work. A splenectomy may be considered to increase RBC counts or prevent further complications. But people who have their spleen removed may be at higher risk for certain bacterial infections. If you undergo a splenectomy, talk with your hematologist about ways to minimize your risk.

“ Find somebody willing to listen to you...to treat you how you feel. Every person is different. ”

Molly



Some treatments can have different effects on your health or other aspects of your life. How you choose to manage your PK deficiency is up to you and your hematologist to decide, so it's important to meet with them regularly.

## Managing complications



### Cholecystectomy

To prevent the ongoing risk of gallstones due to continued hemolysis, the removal of the gallbladder may be considered. Gallstones can cause nausea, stomach pain, or other forms of gallbladder disease.



### Chelation therapy

To manage iron overload, a type of medicine called a chelation agent may be prescribed. Chelation agents bind with iron in the bloodstream to form a substance the body can remove more easily.



*There are trade-offs in choosing management methods. For example, transfusions can help with fatigue but they can be time-consuming and have additional risks.*

*To learn more about management options, talk to your doctor or download the Fast Facts brochure from [KnowPKDeficiency.com](http://KnowPKDeficiency.com).*

## DID YOU KNOW?

While some doctors do provide RBC transfusions at their offices, many times they are administered at a transfusion center in a hospital or at a stand-alone location. One transfusion typically takes 1 to 4 hours.

# Build a management plan with the right team

Studies show that patients who communicate well with their healthcare team are happier with their treatment and receive better care. Many different healthcare professionals will play an important role in managing PK deficiency.

## Key Terms

**Extramedullary hematopoiesis:** Blood cell production occurring outside of the bone marrow, in organs such as the liver or spleen

**DXA (or DEXA) scan:** An X-ray performed to assess bone strength

**MRI:** A scan performed to look for iron overload in the liver and heart

**Abdominal ultrasound:** A test performed to look for gallstones or other complications involving the gallbladder

**Echocardiogram (echo):** A test assessing heart function and signs of pulmonary hypertension

## Your healthcare team



### Hematologist

A doctor who specializes in blood disorders

### Family doctor or general practitioner

The doctor you see for checkups and yearly flu shots or other wellness visits

### Registered nurse

In addition to nurses at your doctor's office, there are nurses who specialize in giving transfusions

### Counselor or psychologist

Living with PK deficiency can cause stress and anxiety. It may help to find a support group or a mental health professional to talk to

## DID YOU KNOW?

The need and timing of tests varies for everyone. Download the monitoring tool from [KnowPKDeficiency.com](https://www.knowpkdeficiency.com) and use it to help guide conversations about how often assessments should be done.

Regular monitoring of PK deficiency can help make sure you're getting the right care. Tests, and how often your doctor performs them, can vary depending on treatment history.

Visit **KnowPKDeficiency.com** to download our monitoring tools to help you discuss which tests you may need with your doctor.

## Know the tests for monitoring your complications



### Annual blood tests for:

- Degree of anemia (hemoglobin levels)
- Reticulocyte count (number of newly developing RBCs)
- Iron overload (ferritin levels)
- Vitamin D levels (to help assess bone health)
- Hormone changes (to check for diabetes, thyroid problems, or sex hormone levels)
- Viruses, such as HIV, and hepatitis A, B, and C (for people who receive transfusions)



### Gallstones

Monitored by ultrasound if there is new or worsening abdominal pain, or if bilirubin levels are consistently high.



### Iron damage to the heart or liver

Monitored by a yearly T2\* MRI scan. Patients who receive regular transfusions, or who need chelation therapy, may need to be assessed more frequently.



### Osteopenia and osteoporosis

A DXA should be done in early adulthood. Results of the scan determine how often the test should be repeated.



### Pulmonary hypertension

An echocardiogram should be done after age 30. Doctors determine if the test needs to be repeated based on what the picture shows.



### Extramedullary hematopoiesis

A visual exam is performed regularly, with further testing if there is unexplained swelling or symptoms that indicate signs of nerve damage, such as numbness, tingling, burning, or shooting pain.

# PK deficiency affects more than just the body

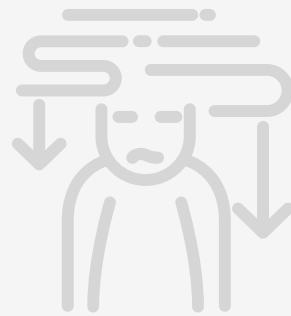
When navigating a chronic condition, it's normal to feel overwhelmed sometimes. Symptoms and treatments of PK deficiency may have effects on mental health, but there are ways to find support.



*Often, physical symptoms of PK deficiency can affect emotional and social health. When talking about physical symptoms with your support system and care team, you should also feel comfortable having open discussions about feelings.*

## Common mental health challenges

People with PK deficiency and their caregivers participated in a poll\* and reported feeling:



**Anxiety**

**84%**

**Depression**

**55%**

**Low self-esteem**

**61%**

**Social isolation**

**58%**

If you no longer take enjoyment in activities, or everyday tasks seem too much, or if you feel sad, empty, or guilty about your condition, it could be a sign of depression. It's important to talk to your healthcare provider about these feelings.

\*Poll results found in "Voice of the Patient Report: Pyruvate Kinase Deficiency" from the National Organization for Rare Disorders.

## DID YOU KNOW?

When asked about PK deficiency management methods, only 11% of participants responded that their current methods work very well. Continue gathering information and advocating for yourself to find the right plan and team that works for you.



Because the impact of PK deficiency goes beyond physical symptoms, it's also important to keep track of your feelings and your ability to cope with everyday activities. This will help you paint a full picture when talking to your healthcare team.

“**My parents got me into horses when I was 10. I couldn't do the swimming and the tennis and all the stuff that my sister did. The horses I could do, so I excelled at them. It gave me an outlet to not think about the bullying, the disease, and I was able to compete in an athletic event like other kids and do as well as them.**”

Robin

“**There are different points in your life where it's harder versus other times where it's easier. That's important to know.**”

Tamara

“**I ultimately found a physician willing to work with me; one who saw me as a person and who read the resources I shared with them. If you don't get that, it's okay to walk away and seek a second opinion. Ultimately, it was my body, my choices, and my life.**”

Molly



*Think about the obstacles you are facing or potentially may face. Keep those in mind as you keep track of symptoms.*



# Taking the next steps



Being prepared can be a source of strength as you manage PK deficiency. Keeping track of goals, obstacles, and questions can help make sure you and your healthcare team come up with the right plan.

## STEP 1: Get organized

**If you haven't been keeping track of your condition in a journal, a good place to start is to gather any information you may have, such as:**

- Surgeries
- Transfusion history
- Blood or other lab tests
- Any other medical conditions
- Medicines or supplements you take on a regular basis

Consider keeping track of your symptoms on a weekly basis. These data can help your healthcare team understand which symptoms you have and how they affect you. If you undergo regular transfusions, for example, it may help to show what kind of effect they have, and how long those effects last.

## STEP 2: Talk about daily life

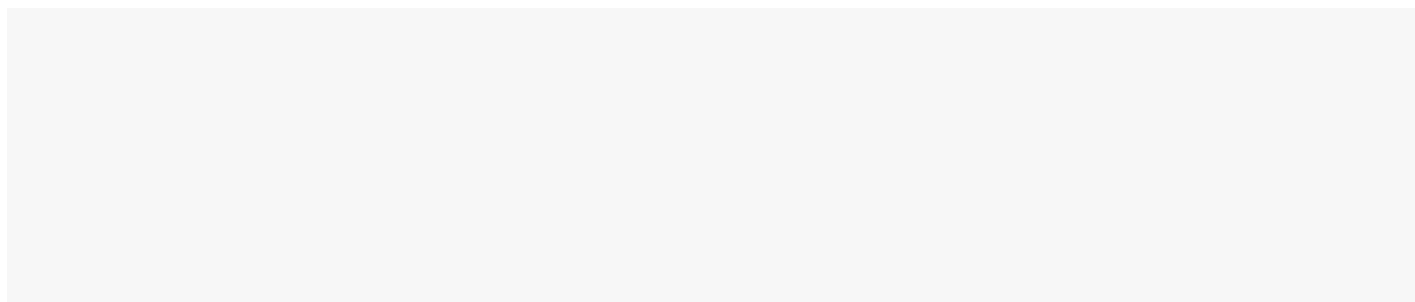
**Talking in detail about your life with PK deficiency can help your healthcare team get a better idea of how you feel.**

To help you be specific at your next appointment, try completing these prompts.

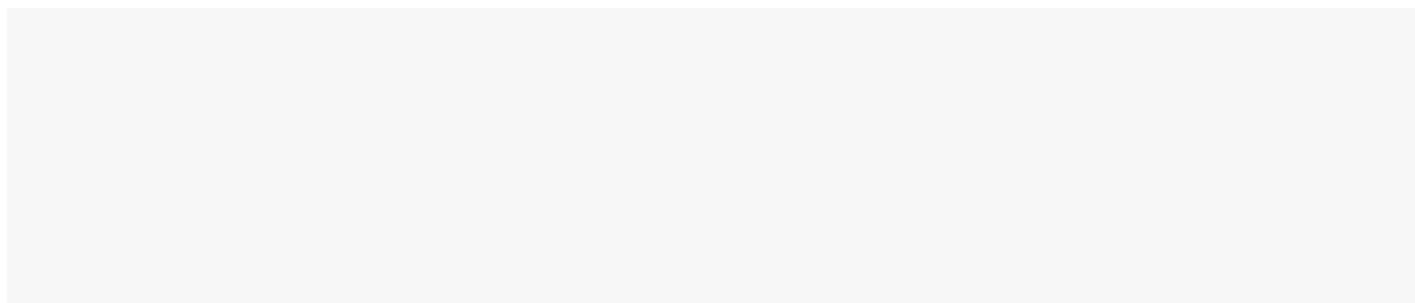
- I enjoy the following activities/hobbies, but I can't do as much as I want to

These steps and examples of discussion questions can help you prepare for your next appointment. Symptoms can affect people's lives differently, so speaking from your own experiences will be best to help your doctor understand how PK deficiency affects you.

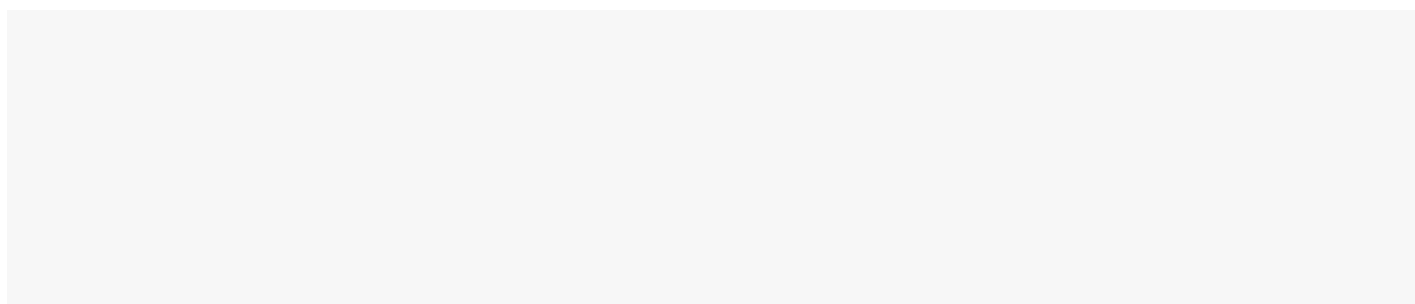
- The symptoms that most interfere with my daily life are



- I notice my symptoms are worse during this part of my day



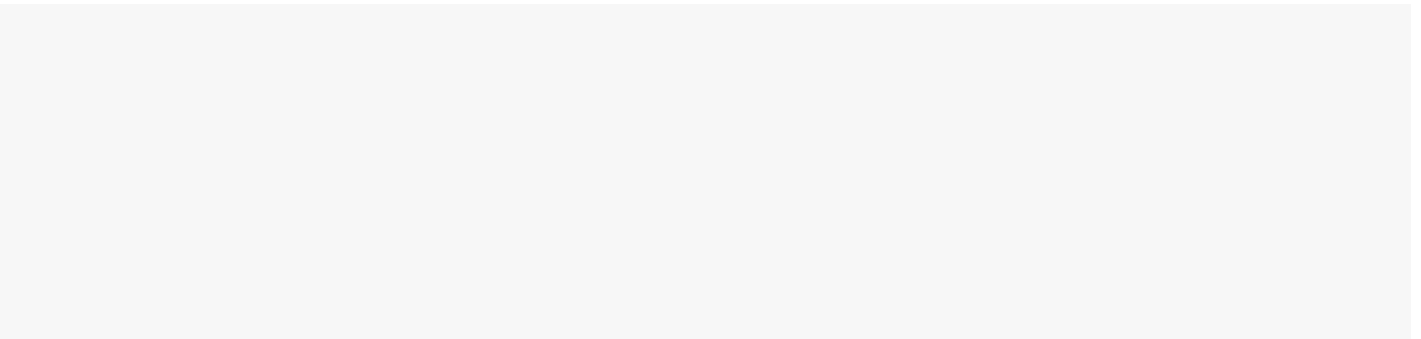
- The symptom that bothers me the most and why is



## STEP 2 continued

**It may be helpful to describe how the symptoms affect your life directly; for example:**

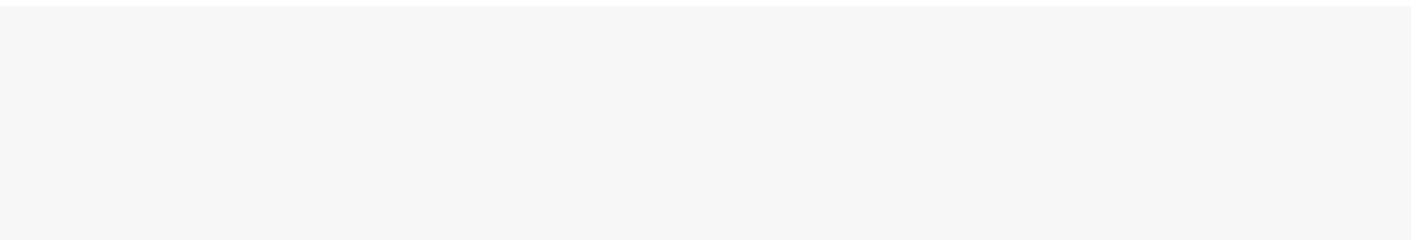
- I always get a headache in the late afternoon, so instead of spending time in my garden, I have to lie down
- I want to be able to attend my child's school events, but can't find the energy to leave the house after 5 PM
- Sometimes I can't finish my homework because my brain gets foggy and reading my textbook becomes a struggle



## STEP 3: Make connections

**The chart on page 15 lists assessments and their recommended frequency. Using what you've learned, talk to your hematologist about your monitoring frequency. Think about these conversation starters and write any other topics you want to discuss:**

- I've read that tests to monitor for PK deficiency complications can vary in frequency depending on transfusions, the need for other therapies, and discoveries from previous assessments. Based on my history, can we discuss how often we should be testing?



- Some assessments become more relevant at a certain age, such as a DXA scan to monitor osteoporosis and an echo to monitor pulmonary hypertension. When should we start thinking about these tests for me?



**Iron overload can occur in people with PK deficiency regardless of age, transfusion history, or degree of anemia. It may be a topic worth discussing on its own. Some prompts to start a conversation include:**

- I came across research that suggests people with PK deficiency should be monitored regularly for iron overload and its complications. Recent research shows that iron overload can be a concern when ferritin levels are high (greater than 500 nanograms per milliliter). How are my ferritin levels?
- I also learned that people who undergo regular transfusions, or who are on chelation therapy, may need to have their ferritin levels tested more often. What do you think about testing my ferritin levels more frequently, like every 3 or 6 months instead of every year?

## STEP 4: Advocate for yourself

**When discussing support options, it can be helpful to mention the methods you've learned about in your own research. You may even find resources that are new to your physician. Consider sharing a resource and any questions you have about its content.**

Talking about your specific situation along with the hematologist's recommendations can help you both create an effective plan.

See some examples of discussion points below and add in your own.

- I want to be proactive about my PK deficiency. Can we talk about managing my condition and build a plan together?

- Based on the symptoms I've checked off in this brochure, what else can we do to manage my anemia?

- Some of my symptoms are affecting my daily life. What changes in my management plan can we consider based on what I'm still feeling?

- I've read that transfusions or a splenectomy could help my anemia and fatigue. It would be helpful to talk through the added risks and benefits of these options together, as well as what they might mean for me.

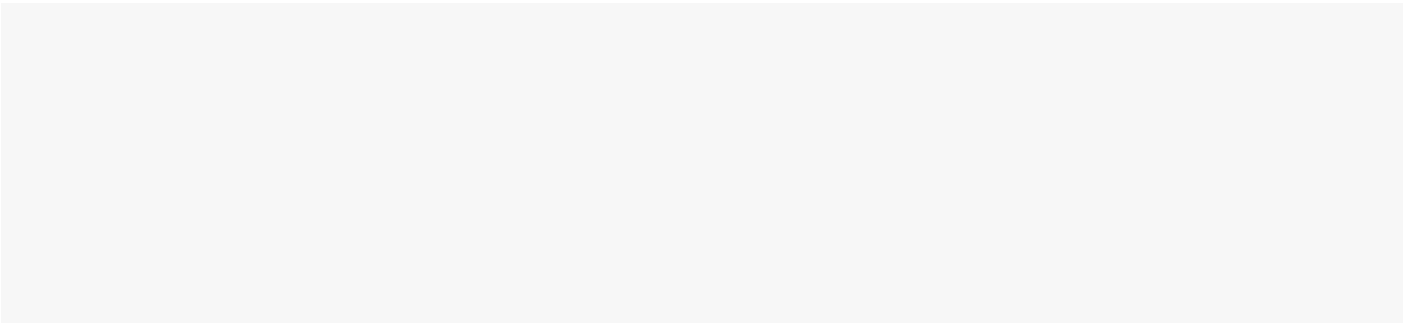
- I find my condition to be overwhelming sometimes, and think I may benefit from more support. Are there any groups recommended for people living with a rare condition?

- My yellow skin has started to take a toll on my self-esteem. What else can we do to reduce my bilirubin levels?

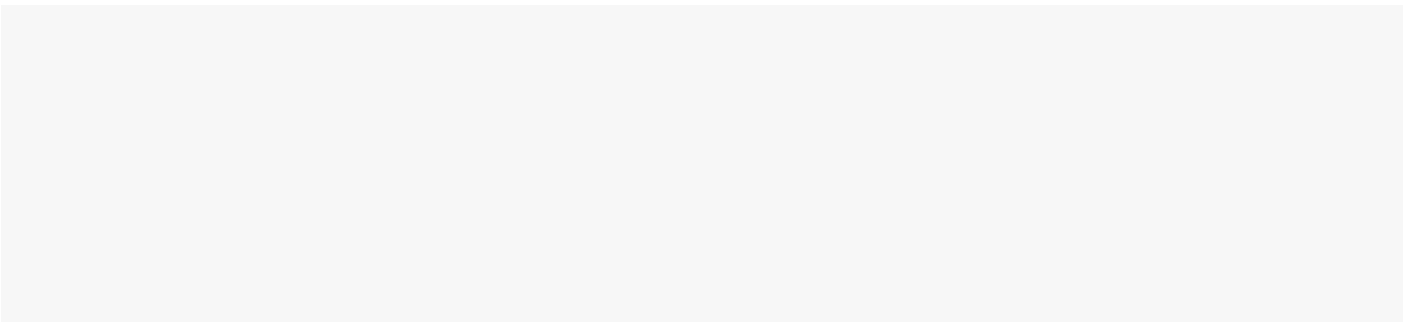
## STEP 5: Set goals

**Be open about the plans you have for the future. Think about what you want to do next, and ask your healthcare team if they can help you get there. Plan ahead by considering these prompts:**

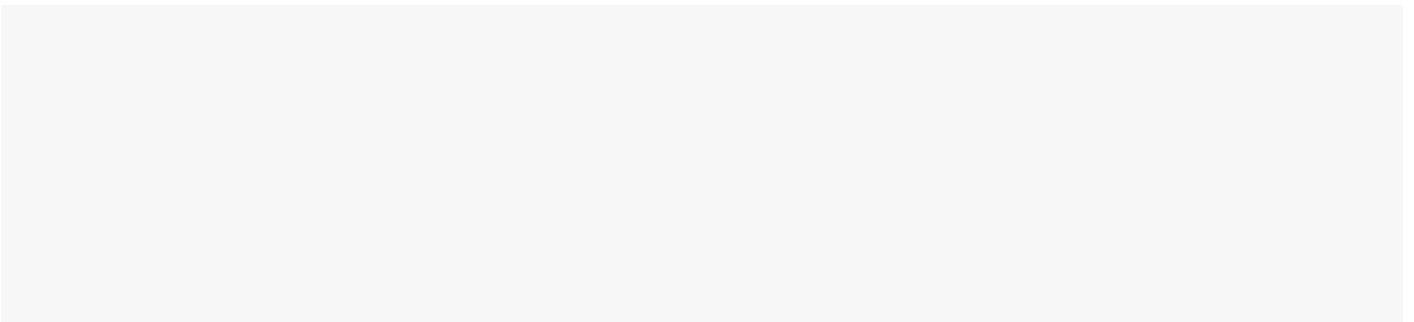
- My school and career plans include



- I have events with family/friends that I want to attend



- I want to be able to participate in the following activities or hobbies







The Resources section on KnowPKDeficiency.com includes additional information, including *Pyruvate Kinase Deficiency Through the Decade*. This resource includes summaries of journal articles that may help support conversations with your doctor. Consider sharing the title and author of the article, or the journal article itself.

Discussion topics might include:

- I found an article focusing on the burden of PK deficiency, and it mentioned some common symptoms that I've been noticing in myself, like memory loss and difficulty concentrating. I don't want this to further affect my career/schoolwork/hobbies/family life. What else can we consider to help?
- I read a study that talked about bone fragility, and a key finding was that long-term complications should be monitored regularly, since there are few early predictors. What can we do to proactively support my bone health?



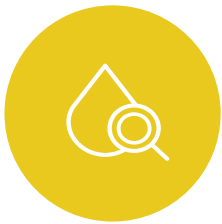


## Introducing a patient support program for people living with PK deficiency and their caregivers.



### Tailored support

Everyone's experience with PK deficiency is different. We will listen to understand your specific needs and interests, and work with you over time to deliver customized education and support.



### Educational resources

Whether you're newly diagnosed or you've been living with PK deficiency for a while, we will share resources that can help you or your family better understand the disease. This will help you communicate with your healthcare team.



### Community connections

Living with a rare disease can mean never having met someone else with the same condition. We can provide opportunities to connect with other patients and caregivers to allow you to share your experiences with PK deficiency.



**Enroll today to be connected to your dedicated Patient Support Manager.**

**Call 1-800-951-3889, Monday through Friday, 8 AM to 6 PM ET.**

**Visit [myagios.com/patient/pkd-enroll](https://myagios.com/patient/pkd-enroll) to learn more.**

**For patients in the United States only.** This program cannot offer medical or treatment-related advice. For these types of questions, contact your healthcare professional.



## Online PK deficiency support

These resources can offer more information and support.

### Agios KnowPKDeficiency.com

Download the *Fast Facts* brochure for additional in-depth information about PK deficiency.

Read *Pyruvate Kinase Deficiency Through the Decade* to understand more about PK deficiency and the latest breakthroughs in knowledge about the condition.

[KnowPKDeficiency.com](https://www.knowpkdeficiency.com)

### Agios Know PK Deficiency Facebook page

Join the community and connect with people who have PK deficiency and their caregivers.

[facebook.com/PKdeficiency](https://facebook.com/PKdeficiency)

### PK Deficiency Foundation

A national nonprofit organization whose mission is to enhance quality of life for patients and their families by providing awareness, expanding education, and promoting advocacy.

[pkdf.org](https://pkdf.org)

### Thrive with Pyruvate Kinase Deficiency Organization

This organization's mission is to connect the pyruvate kinase deficiency community through treatment support, increased social awareness, and advocacy.

[thrivewithpkd.org](https://thrivewithpkd.org)

### Thalassemia International Federation

This advocacy group offers information about hemoglobin disorders, including PK deficiency.

[thalassaemia.org.cy/pk-deficiency](https://thalassaemia.org.cy/pk-deficiency)



**Anemia ID** is an Agios-sponsored program that offers free genetic testing. The program can help determine the cause of hereditary anemias, confirm your diagnosis of PK deficiency, and identify family members who might be affected. Visit **AnemiaID.com** to learn more, then talk to your doctor about next steps.

This program is only available to residents of the United States. All testing provided to patients through **Anemia ID** is paid for by Agios Pharmaceuticals. While Agios provides financial support for this program, all tests and services are performed by PerkinElmer Genomics. Agios receives contact information for healthcare professionals who submit tests under this program and limited de-identified aggregate data. **Anemia ID** is sponsored by Agios in partnership with PerkinElmer Genomics. Other laboratories may also offer genetic testing.

To stay up-to-date and find additional support organizations, check the Resources page on **KnowPKDeficiency.com**.

“My life,  
my future,  
depends  
on this.”

Molly, 32  
Diagnosed with PK deficiency  
at 9 months old



## Goal-setting for life with PK deficiency

PK deficiency is a rare form of hemolytic anemia in which RBCs don't have enough energy to work properly, so they die more quickly. Symptoms of PK deficiency are different for everyone and can change over time, so it's important to keep track of how you feel.

Setting up a management plan with your hematologist can help you stay ahead of your PK deficiency symptoms and ensure you're informed about treatment options.

You have the power. Take action toward achieving the goals you've set for yourself.

### Interested in more information about PK deficiency?

Agios is dedicated to understanding rare disorders such as PK deficiency, and providing educational resources to the community.



To stay informed about the latest news, resources, and research related to PK deficiency, register for updates at **KnowPKDeficiency.com**.



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