

LIVING WITH PH1

A guide for caregivers



This guide was created to help both you and your child discuss primary hyperoxaluria type 1 (PH1).

 Alnylam[®]
PHARMACEUTICALS

TAKE
ON PH1 

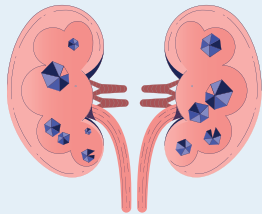
Covering the basics

Whether your child was diagnosed yesterday, or years ago, we could all use a refresher on the basics of PH1.

PH1 is a rare, inherited, recessive disease. **Inherited** means that it is passed down within families. **Recessive** means both the mother and the father have passed the mutated (changed) gene that causes PH1 down to a child. With PH1, the liver makes too much of a waste product called **oxalate**. Normally, oxalate is present only in small amounts, is not used by the body, and needs to be removed. However, with PH1, the overproduction of oxalate can cause **crystals** to form in the kidneys, which can cause progressive damage. **Progressive** means the damage is continuously happening and can be getting worse over time, even if sometimes symptoms aren't noticeable.

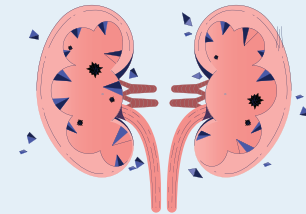
Crystals cause damage in different ways

1. Clumping together to create kidney stones.



2. Collecting in the kidneys and causing damage (nephrocalcinosis).

Over time, as kidneys are damaged, oxalate can spread and form crystals in other parts of the body (systemic oxalosis).



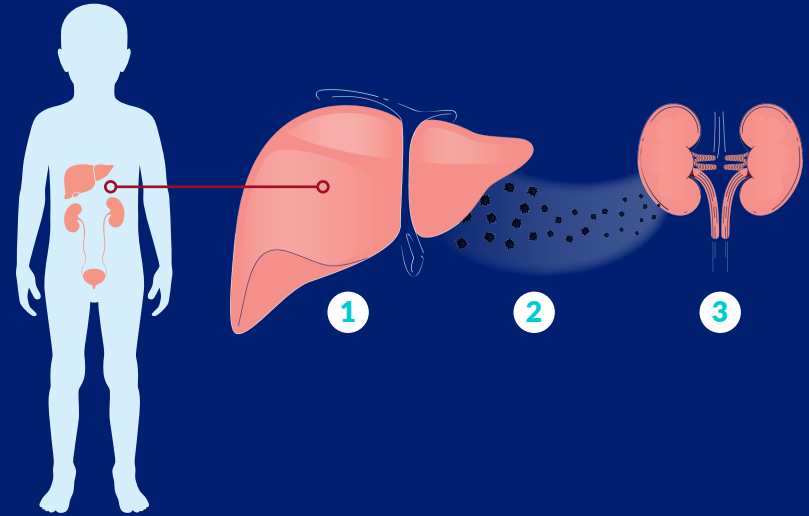
Could someone else in your family have PH1?

Because of the inherited nature of PH1, if your child has a genetically confirmed PH1 diagnosis, it is important that family members, especially siblings of a person with PH1, consider having a conversation with a healthcare provider about getting tested for the disease with a genetic test. If a healthcare provider decides genetic testing is right for an individual, one option is Alnylam Act[®], which you can **read more about on the back cover**.

Give your child a look inside PH1

PH1 starts in the liver but can hurt the kidneys

1. The liver makes **too much oxalate** the body can't use.
2. The body sends the oxalate to the **kidneys** to try to get rid of it.
3. But **oxalate can turn into crystals**, which can build up and damage the kidneys. Over time, the kidneys can't keep up with all they need to do.



Looking for more?

Watch *How PH1 Happens* at TakeOnPH1.com/aboutPH1 and brush up on the science behind your child's condition.



Have a curious child?

Look inside the back pocket to find the *My PH1 Activity Book*! It's filled with games, puzzles, and activities that can help your child learn about PH1.



Working with your healthcare team

While managing PH1 can feel overwhelming at times, **you are not alone**. Your child's healthcare team will help you to navigate the challenges ahead.

Based on how PH1 is progressing, your nephrologist will create a customized care plan.



MY CHILD'S PH1 DOCTOR:

NAME:

CONTACT INFO:

Monitoring your child's health

There are several different ways your healthcare team may track PH1 and check for kidney damage. The frequency of the tests and procedures used will depend on your child's care plan. Below are some terms you may hear your healthcare team use when discussing how they monitor your child's health.

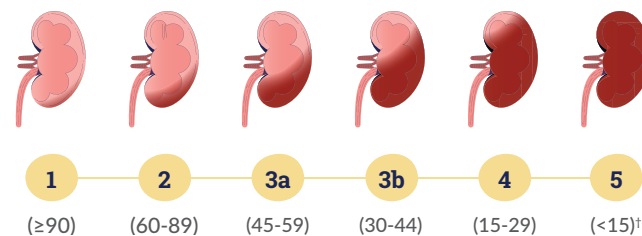
TERM	DEFINITION
LITHOTRIPSY	Non-invasive procedure to break up kidney stones*
URETERO AND RENAL ENDOSCOPY	Minimally-invasive procedure to remove/break up stones*
URINARY OXALATE TEST	Test that keeps track of oxalate levels in urine
KIDNEY ULTRASOUND	Imaging that detects and monitors any kidney stones that may form
KIDNEY FUNCTION BLOOD TEST	Test that checks how well the kidneys are filtering waste from blood

*A urologist will select a procedure based on the size/amount of stone(s).

Checking on kidney function

Doctors measure kidney function by checking the **estimated glomerular filtration rate (eGFR)**. Your child's eGFR number is based on a blood test for creatinine, a waste product in the blood.

Because PH1 can lead to **chronic kidney disease (CKD)**, your doctor may regularly check eGFR levels and kidney function. The stages of CKD range from low risk of kidney damage (stage 1) to kidney failure (stage 5).



[†]These values correspond to how many milliliters per minute the kidney is filtering, and is adjusted for body area.

Talking to your child about PH1

Teaching your child the value of their care plan can help them stay motivated. Consider sharing these points to help them understand why managing PH1 is important:



Hydrating and taking medicine

- The doctor may recommend drinking more water to help dilute oxalate—that means making it weaker.
- It's not always easy drinking so much water—especially if you're not feeling thirsty. What are some good ways I can help you?
- Taking medications or supplements may help your body make less oxalate or help lower the number of crystals that are made up of oxalate.



Taking care of body and mind

- You can help keep your body as strong as possible by eating healthy foods, playing outside with friends, and getting a good night's sleep.
- It's okay to feel tired, angry, or sad about having PH1 sometimes. Come talk to me, and I'll do whatever I can to help.



Going to dialysis

- When kidneys can't do their job anymore, they will need some extra help. There is a machine that can work like kidneys—it's called a dialysis machine.
- Dialysis will help get rid of the excess oxalate.
- While the dialysis machine does its job, you'll need to sit still and wait patiently. Are there any activities, games, or books you'd like to bring?



Going to the doctor

- Doctors play an important role in managing your PH1. Doing tests helps them know how to take care of you.
- Doctors can help if you get a stone or if your kidneys aren't strong enough. They can also answer any questions you may have about PH1. Is there anything you want to ask the doctor at our next visit?



Have a curious child?

PH1 of a Kind® is an animated video series made just for kids living with PH1. Watch them with your child at [TakeOnPH1.com/support](https://www.TakeOnPH1.com/support).

Helping your child succeed

Your doctor will work with you and your child to develop a management plan to try to reduce symptoms and try to prevent the disease from getting worse. A solid routine can help your child stay on top of management.

Tips for appointment prep

- Ask if there's anything your child needs to do beforehand
- Record any symptoms, even if they seem unrelated to PH1
- Track how much your child drinks and urinates over the course of a day
- Make a list of all medications, vitamins, or other supplements your child takes
- Write down questions to ask your doctor

Tips for sticking to the care plan

- Consider rewarding healthy habits and celebrate small wins
- If hydration is part of their management plan:
 - Set reminders on your child's phone/watch to remind them to drink water
 - Designate a special water bottle or bracelet as a reminder to drink enough water
 - Anticipate frequent bathroom breaks
- Plan ahead for events that could throw off the usual care plan (camp, vacations, school trips, etc.)

Tips for reading the signs

Because your child might have trouble describing how they feel, you may notice symptoms before they can articulate them. Keep in mind that **your child may not feel any of these symptoms**, and still have a kidney stone or kidney failure. If you think your child is experiencing these symptoms, you should immediately contact your child's doctor.

SOME SIGNS OF A KIDNEY STONE

- Pain in side or back
- Blood in urine
- Frequent need to urinate
- Difficulty urinating

SOME SIGNS OF KIDNEY FAILURE

- Decreased urine or no urine output
- Loss of appetite, nausea, vomiting
- Pale skin color
- Swelling of hands and feet
- Extreme fatigue and weakness



Looking for more?

To learn about other ways you can help your child manage PH1, talk to your doctor. You can also visit [TakeOnPH1.com/management](https://www.TakeOnPH1.com/management).

Finding strength & support

As a caregiver, it's important to remember your own well-being.

Practice self-care

You are the strongest part of your child's support system.

It takes time, energy, and perseverance, but don't forget to take care of yourself. That may mean setting aside time to eat healthy, stay active, unwind, and pay attention to your own psychological well-being.

Expand your circle of support

It's important that those who your child interacts with regularly understand how PH1 can affect your child's day-to-day—like babysitters, teachers, and coaches. For example, if hydration is part of your child's management plan, telling their coach about PH1 may be helpful so they can help ensure that your child is drinking enough water and they may need frequent bathroom breaks.

There's a form you can download, fill out, and share with others to inform them about your child's PH1. You can find it at TakeOnPH1.com/support.

Consider having a conversation with your child about sharing their unique needs with others. By doing this, you can expand the network of knowledgeable adults looking out for your child's well-being.



Looking for more?

To hear stories from others living with PH1, go to TakeOnPH1.com/support.

You've got this.

Learn more about PH1, hear from members of the community, and find support at [TakeOnPH1.com](https://www.TakeOnPH1.com)



The Oxalosis & Hyperoxaluria Foundation (OHF)

The OHF is an advocacy group dedicated to finding treatments and a cure for all forms of hyperoxaluria, and supports thousands of healthcare professionals, patients, and their families.



American Kidney Fund (AKF)

AKF works on behalf of the 37 million Americans living with kidney disease and millions more at risk, providing resources that support people in their fight against kidney disease, including rare diseases such as PH1.



Alnylam Act®: One option for genetic testing and counseling

You or your healthcare provider can request genetic testing and counseling through the Alnylam Act® program if your child meets certain criteria. The test is done using a blood, saliva, or buccal sample. Eligible siblings and family members may also be tested through Alnylam Act® if they meet eligibility criteria.

The Alnylam Act® program was created to provide access to no-charge genetic testing and counseling to patients as a way to help people make more informed decisions about their health.

- While Alnylam provides financial support for this program, tests and services are performed by independent third parties
- Healthcare professionals must confirm that patients meet certain criteria to use the program
- Alnylam receives de-identified patient data from this program, but at no time does Alnylam receive patient-identifiable information. Alnylam uses healthcare professional contact information for research and commercial purposes
- Genetic testing is available in the US and certain other countries. Genetic counseling is only available in the US
- Healthcare professionals or patients who use this program have no obligation to recommend, purchase, order, prescribe, promote, administer, use, or support any Alnylam product
- No patients, healthcare professionals, or payers, including government payers, are billed for this program

For more information and program rules, download the Alnylam Act® Genetic Testing and Counseling Brochure for PH1. [AlnylamActPH1.com](https://www.AlnylamActPH1.com)



Isabelle

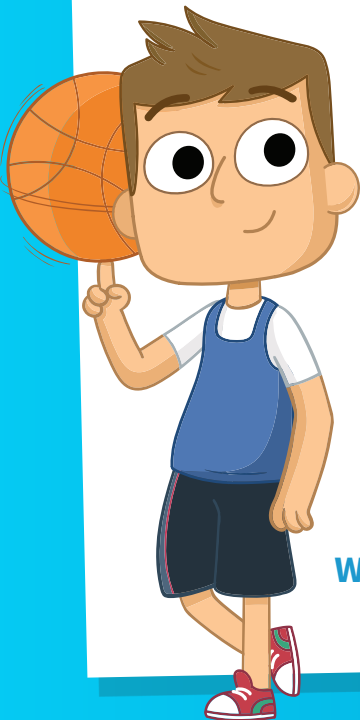


Luuk

my PH1 ACTIVITY BOOK

Look inside for games and activities to help you learn about living with PH1.

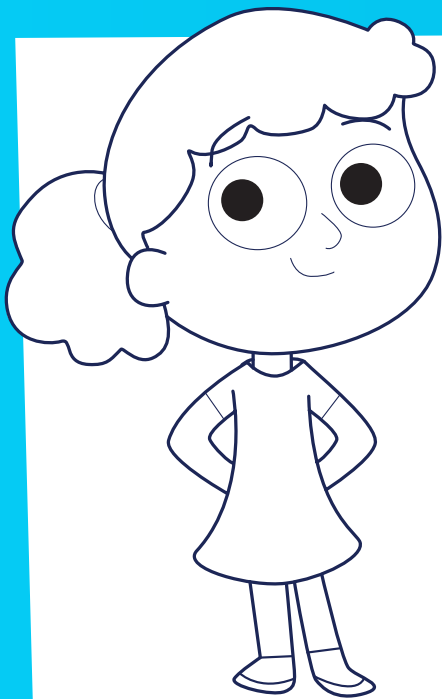
Here to guide you are Isabelle, Luuk, Asha, and Will—kids from all over the world who have PH1, too!



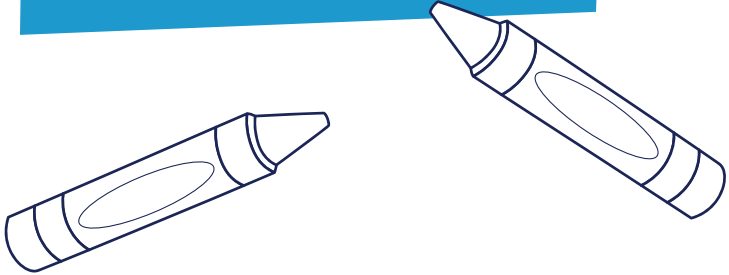
Will



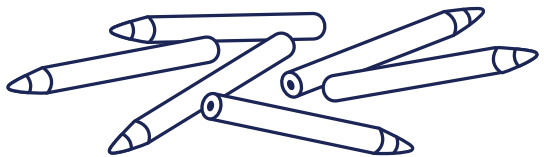
Asha



When Isabelle learned she had PH1, she heard some new words she'd never heard before. Can you help her find all the PH1 words listed below?



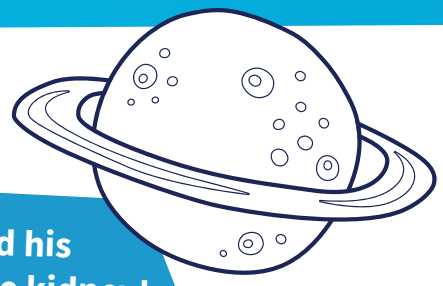
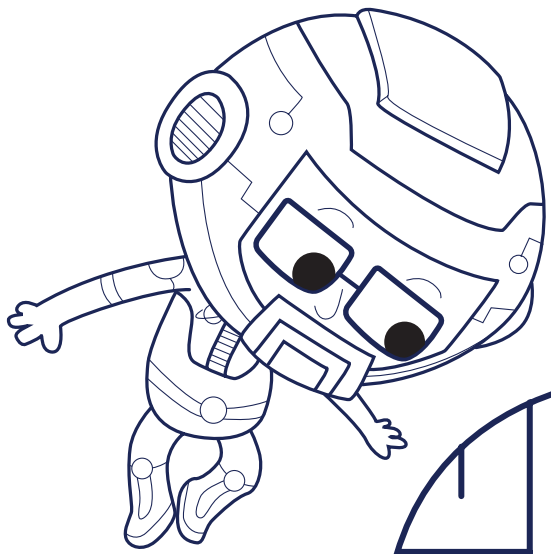
N	K	N	X	U	G	M	I	D	D	C	S	C	P	O	LIVER
D	I	Z	Q	R	L	L	Q	I	F	I	E	P	B	H	KIDNEY
F	D	O	C	I	I	I	X	A	O	H	Q	E	X	J	BLADDER
C	N	Y	V	N	V	V	C	L	Q	J	D	Q	Y	S	OXALATE
J	E	P	A	E	J	E	L	Y	N	G	O	Y	L	I	CALCIUM
L	Y	Y	T	W	D	R	O	S	F	Y	C	A	B	S	CRYSTALS
G	D	N	U	A	E	I	B	I	K	I	T	N	S	F	STONES
F	B	V	X	D	F	G	R	S	V	S	O	Q	A	L	WATER
G	E	J	D	C	T	E	V	M	Y	T	R	A	V	O	DIALYSIS
Y	X	A	T	F	I	H	U	R	A	O	U	S	T	X	URINE
X	L	O	K	D	E	I	C	X	J	N	F	W	N	A	DOCTOR
B	M	F	Q	V	C	L	K	L	B	E	H	B	S	L	MUTATION
D	M	U	T	A	T	I	O	N	I	S	W	U	Q	A	
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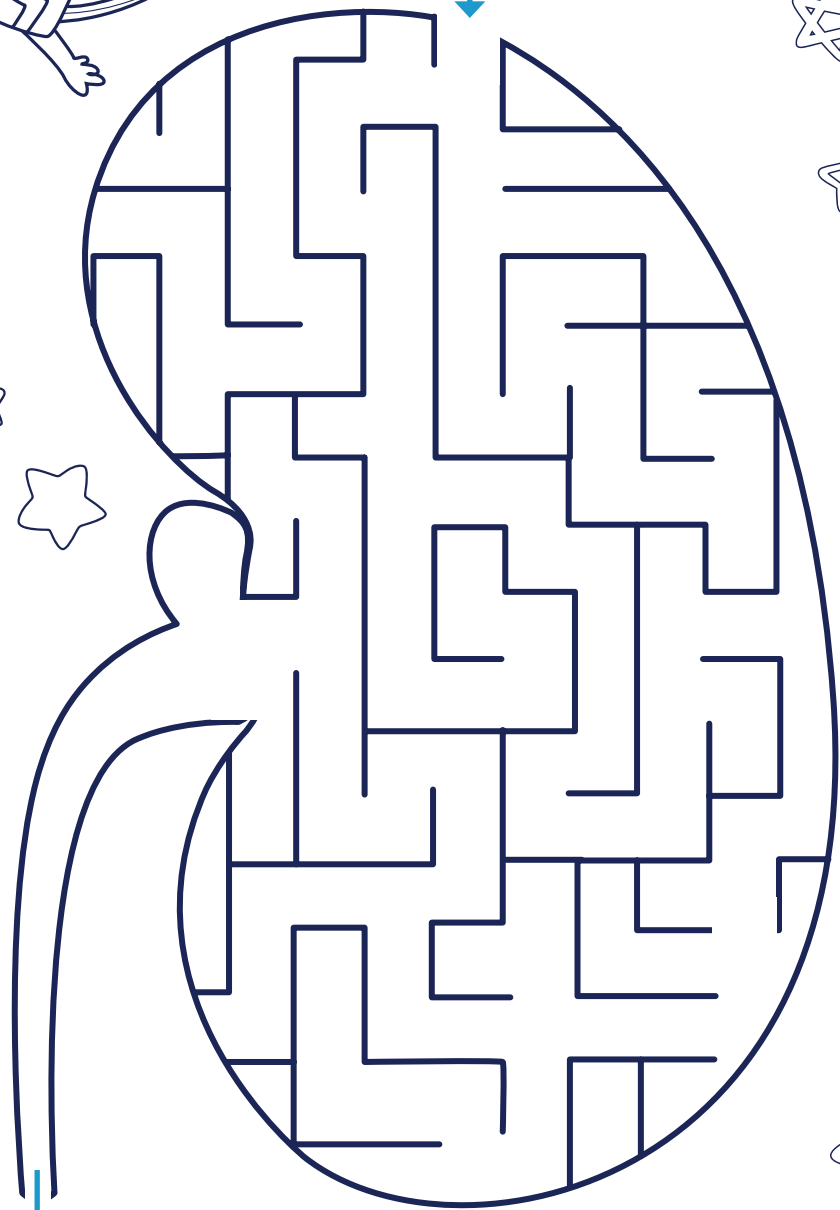
You are more than PH1 so you need a story just for you! Ask an adult to help you with this section: they'll ask you for certain types of words, and then read you the funny story you just created.

WHAT A DAY!

The other day, my _____ and I were coming
(noun)
home from my doctor appointment when we saw a
bunch of _____. They were _____
(plural noun) (ing-verb)
a soccer ball across a big _____. It looked
(noun)
_____ to me. My friend _____
(adjective) (friend's name)
came over and asked if I could go to _____
(a place)
with them. Once we arrived, we had a contest
to see who could _____ the most
(verb)
_____. And I won! All of my _____
(plural noun) (plural noun)
were so _____. I guess you could call me a
(adjective)
champion _____!
(noun)



Help Luuk find his way out of the kidney!



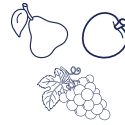
Asha likes to spend lots of time playing outside. Connect the dots to see what she's up to!



These things are all part of Will's life with PH1—but he can't find them! Can you help him find all of his missing things?



Get plenty of sleep



Eat healthy food



Visit your doctor



Spend time with friends



Drink water to stay hydrated



Get exercise

