This guide was created to help both you and your child integrate primary hyperoxaluria type 1 (PH1) management into your daily lives.
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 your mother and your father have passed the mutated (changed) gene that causes PH1 down to you. 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.

Could someone else in your family have PH1?

Because of the inherited nature of PH1, if your child has a genetically confirmed PH1 diagnosis, family members, especially siblings, should be tested as well. One way your doctor can request genetic testing and counseling is through Alnylam Act®, which is offered at no charge. Find more information on Alnylam Act 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.
**MANAGING PH1**

**Working with your care team**

While managing PH1 can feel overwhelming at times, you are not alone. Your child’s care team will help you navigate the challenges ahead. Based on how PH1 is progressing, your nephrologist will create a customized care plan.

**Monitoring your child’s health**

There are several different ways your care 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 care team use when discussing how they monitor your child’s health.

<table>
<thead>
<tr>
<th>TERM</th>
<th>DEFINITION</th>
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<tbody>
<tr>
<td>LITHOTRIPSY</td>
<td>Non-invasive procedure to break up kidney stones*</td>
</tr>
<tr>
<td>URETERO AND RENAL ENDOSCOPY</td>
<td>Minimally-invasive procedure to remove/break up stones*</td>
</tr>
<tr>
<td>URINARY OXALATE TEST</td>
<td>Test that keeps track of oxalate levels in urine</td>
</tr>
<tr>
<td>KIDNEY ULTRASOUND</td>
<td>Imaging that detects and monitors any kidney stones that may form</td>
</tr>
<tr>
<td>KIDNEY FUNCTION BLOOD TEST</td>
<td>Test that checks how well the kidneys are filtering waste from blood</td>
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* A urologist will select a procedure based on the size/amount of stone(s).

**MY CHILD’S PH1 DOCTOR:**

- NAME: 
- CONTACT INFO: 

**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), it’s important to check eGFR levels and kidney function regularly. The stages of CKD range from low risk of kidney damage (stage 1) to kidney failure (stage 5).

1 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. Try sharing these points to help them understand why managing PH1 is important:

**Hyperhydrating and taking medicine**
- Drinking lots of water helps dilute oxalate—that means making it weaker.
- Taking vitamin B6 may help your body make less oxalate.
- Taking alkali citrate may help lower the number of crystals that are made up of oxalate.
- 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 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.
Helping your child succeed

A solid routine can help your child stay on top of management, so they can stay involved with the things they love.

**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**
- Reward healthy habits and celebrate small wins
- 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 stone or kidney failure. If you think your child is experiencing these symptoms, you may consider contacting your nephrologist or urologist.

<table>
<thead>
<tr>
<th>SIGNS OF A KIDNEY STONE</th>
<th>SIGNS OF KIDNEY FAILURE</th>
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<tbody>
<tr>
<td>Pain in side or back</td>
<td>Decreased urine or no urine output</td>
</tr>
<tr>
<td>Blood in urine</td>
<td>Loss of appetite, nausea, vomiting</td>
</tr>
<tr>
<td>Frequent need to urinate</td>
<td>Pale skin color</td>
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<tr>
<td>Difficulty urinating</td>
<td>Swelling of hands and feet</td>
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<tr>
<td></td>
<td>Extreme fatigue and weakness</td>
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**Looking for more?**
To learn about other ways you can help your child manage PH1, go to 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 means 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 instance, telling your child’s coach about PH1 may be helpful so they can help ensure that your child is drinking enough water and they can appreciate the need to take 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.

By communicating your child’s unique needs to other people, 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

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.

Alnylam Act®: No-charge genetic testing and counseling
Your doctor can request genetic testing and counseling through the Alnylam Act program if you or your family members meet certain criteria. The test is simple—it’s done using a blood or saliva sample.

The Alnylam Act program was created to provide access to 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 Canada. 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 payers, including government payers, are billed for this program

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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!
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?

- LIVER
- KIDNEY
- BLADDER
- OXALATE
- CALCIUM
- CRYSTALS
- STONES
- WATER
- DIALYSIS
- URINE
- DOCTOR
- MUTATION

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You are more than PH1—you can do anything you put your mind to. 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 _________ home from my doctor appointment when we saw a bunch of ___________. They were ___________ _________ a soccer ball across a big ___________. It looked ___________ to me. My friend ___________ came over and asked if I could go to ___________ with them. Once we arrived, we had a contest to see who could ___________ the most ___________. And I won! All of my _________ were so ___________. I guess you could call me a champion ___________!
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