

# Nityr<sup>TM</sup>

nitisinone  
Tablets

## Patient Initiation Brochure

for NITYR<sup>TM</sup> (nitisinone) tablets

This brochure is intended for patients or caregivers  
in the US who have been prescribed NITYR.

### In this leaflet:

- Information about hereditary tyrosinemia type 1 (HT-1) and what to expect after diagnosis
- Advice for helping your child manage HT-1 throughout the different stages of their life
- Information about the **NITYR Patient Support Program**
- Important safety information about NITYR



# Nityr<sup>™</sup> nitisinone Tablets

The nitisinone  
tablet for all  
life stages

## Contents

### Introduction

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Information About HT-1	5
What to Expect After an HT-1 Diagnosis	6
A New Era of Choice	7

### Managing HT-1

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#### Help and Advice for Parents and Caregivers of Children under 3

Helping Infants and Young Children with HT-1 Thrive	8
How to Give NITYR Tablets Using an Oral Syringe	10
How to Give NITYR Tablets with Applesauce	12

#### Help and Advice for Parents and Caregivers of 3 Year Olds to Teenagers

Important Considerations for Children 6 Years to 12 Years Old with HT-1	13
Helping Your Young Teen Transition to a Healthy Adult	14

#### Help and Advice for Adults

Making the Most of Living with HT-1 as a Young Adult	15
Living Life to the Fullest as an Adult with HT-1	16

### Further Information

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Where to Turn Anytime to Find Answers and Solutions	17
Important Safety Information Regarding NITYR (nitisinone) Tablets	18
About Cycle <sup>™</sup> Pharmaceuticals	20

If you or your child are living with HT-1, an ultra-rare metabolic disorder, you've probably received a lot of information and reached out to various sources for help. This Patient Initiation Brochure is designed to answer many questions patients and parents have regarding the disease management and treatment. It is our hope that this information will help you or your child lead a full life.

Note: This brochure is provided for information purposes only. It is not intended to be, and should not be used as, a substitute for medical advice.



#### Useful sources:

**About Tyrosinemia: New Parents' Guide**  
[depts.washington.edu/tyros/abouttyr.htm](https://depts.washington.edu/tyros/abouttyr.htm)

**Metabolic Support UK**  
[www.metabolicsupportuk.org](http://www.metabolicsupportuk.org)

**Baby's First Test**  
[www.babysfirsttest.org](http://www.babysfirsttest.org)

**Network of Tyrosinemia Advocates (NOTA)**  
[www.notacares.org](http://www.notacares.org)

**National Organization for Rare Disorders (NORD)**  
<https://rarediseases.org/>

## Information About HT-1

### What is HT-1?

Hereditary tyrosinemia type 1 (HT-1) is an ultra-rare disorder that leaves a person unable to fully break down the amino acid tyrosine. This causes a dangerous build-up of toxic metabolites in the body – which can lead to serious liver and kidney problems – those affected require a special diet and medication (nitisinone, sometimes known as NTBC) to control the disorder.

It is estimated that there are more than 170 patients in the US.

### What causes HT-1?

HT-1 is a lifelong disorder. It is an autosomal recessive genetic condition, which means the baby inherits a defective gene from each parent. Unaffected adults are often unaware they carry a defective gene until they have an affected child. People born with HT-1 cannot metabolize the amino acid tyrosine, meaning they are missing an essential enzyme that helps to break down protein in the food they eat.

### How is the condition diagnosed?

In the United States HT-1 is mostly diagnosed at birth. Newborn screening typically involves a heel stick blood test. However, a positive screening result may not be definitive. Your healthcare professional may order additional blood and urine tests to confirm the diagnosis. Early testing gives the opportunity to start treating your baby as early as possible, minimizing the risk of further complications.

### Who can I turn to for more information and support?

Although HT-1 is an ultra-rare amino acid disorder, you're not alone. Your healthcare professional can help you find a parent support group or organization focused on helping families adjust to a child with an amino acid disorder. Dieticians with special training and skills can help parents navigate the special dietary needs of a child or young adult with HT-1.



Cycle Pharmaceuticals, maker of NITYR also provides a patient support program. The **NITYR Patient Support Program** is available 24/7 and you can gain advice and support with access to a pharmacist when you need help or have any questions. For more information visit [www.nityr.us](http://www.nityr.us) or call **1-800-847-8714**.

## What to Expect After an HT-1 Diagnosis

Like many parents and caregivers before you, the first few days, weeks and months following a confirmed diagnosis can be confusing and challenging.



### What does this mean for your baby?

A baby born with HT-1 will require medication (nitisinone, sometimes known as NTBC) and a special, tightly-controlled diet to restrict tyrosine and phenylalanine, to minimize risk of associated health problems. Get advice from healthcare professionals you trust, including a metabolic dietician that specializes in helping people with an amino acid disorder. Even as you navigate the early days of the diagnosis, keep in mind that babies born with HT-1 can live full and fulfilling lives.

### Navigating day-to-day management of living with HT-1

If you're like many parents of a child with HT-1, you quickly learn what your child can and cannot eat or drink. Many parents find they become experts on avoiding forbidden food and substituting formula and food that helps their baby thrive just like any other. As your baby starts socializing with other babies and goes on "play dates" – you'll learn to pack the best food and snacks ahead of time.



### Medical Team/Support

Your healthcare professional will also work with you, the dietician and other clinicians to prescribe special formula, nutritional supplements and/or medication to help your child thrive at every stage of development. Don't hesitate to ask questions or reach out to your pediatrician or other healthcare professionals when you have concerns.

HT-1 is an ultra-rare disorder, so reputable sources of information and guidance may feel limited at times. For this reason, Cycle Pharmaceuticals, maker of NITYR tablets, also provides a patient support program.

**The NITYR Patient Support Program** provides access to a pharmacist when you need help or have any questions. For more information visit [www.nityr.us](http://www.nityr.us) or call **1-800-847-8714**.

Sometimes questions come up during an overnight feed. Remember, you're not alone. The NITYR Patient Support Team is available 24/7.

## A New Era of Choice

We now have a new option for the first time in years to treat babies, adolescents and adults with HT-1. This new formulation of nitisinone (also known as NTBC), NITYR tablets, may give parents, caregivers, adolescents and adults living with HT-1 a more convenient choice in treatment.

Unlike other conditions that affect more Americans, where we continue to see new therapy options, the rarity of HT-1 has not prompted pharmaceutical companies to develop new medications to help your child. At Cycle Pharmaceuticals, we want to change that. We truly believe people and families affected by HT-1 deserve more.

We've been working behind the scenes for years to develop a more convenient

formulation of nitisinone, one that may offer convenient advantages to patients and caregivers alike.

Even if your child's condition is well controlled on your current nitisinone therapy, this new formulation aims to make patients' lives easier and improve quality of life at every stage.

NITYR tablets are bioequivalent to Orfadin<sup>\*</sup> (nitisinone) capsules. This means NITYR is clinically proven to have the same efficacy, safety and side effects, but may offer patients and their families greater convenience and a price advantage. Both medications are therapeutically equivalent.

NITYR is supplied in 2 mg, 5 mg and 10 mg tablets. The recommended initial dose is 0.5 mg/kg twice daily, administered orally, with or without food. The dose of nitisinone should be adjusted individually by your healthcare professional.

### NITYR tablets:

- Are bioequivalent<sup>1</sup> to Orfadin
- The tablet for all life stages
- Are very small<sup>2</sup> (baby aspirin sized) and tasteless
- Can be stored at room temperature, do not require refrigeration<sup>3</sup> and have a shelf life of 36 months (3 years)
- Can be taken with or without food
- Have a Food Program<sup>4</sup>
- Have a \$0 co-pay for eligible commercially insured patients<sup>5</sup>

*"It was reassuring to know that the chemical compounds were the same in this drug as they were in Orfadin. What I like about NITYR is it's a really small tablet. It comes in the size of baby aspirin, and it can be taken with or without food."*

### Carrie

Mother of Ethan (5 years old) NITYR patient.

See [www.nityr.us/mystory](http://www.nityr.us/mystory) to read the family's story.

<sup>1</sup>Data on File NIT/02/17. <sup>2</sup>Data on file NIT/03/18. <sup>3</sup>Data on file NIT/01/17. <sup>4</sup>Diet and Low Protein Food Program: Subject to terms and conditions and eligibility criteria. This program is for eligible commercially insured patients only. Not available for government-insured patients and subject to other federal and state law. <sup>5</sup>\$0 co-pay for eligible commercially insured patients (up to \$15,000 per year per patient). <sup>\*</sup>Orfadin is a registered trademark, licensed by Sobi, Inc. or its affiliates.

## Helping Infants and Young Children with HT-1 Thrive

We understand the many challenges of living with HT-1 but administering and transporting the medication should not be one of them. For this reason, NITYR comes in convenient 2 mg, 5 mg, and 10 mg very small<sup>1</sup> (baby aspirin sized), tasteless tablets. They can be stored at room temperature for up to 36 months (3 years). These tablets can be converted to a suspension for administration to a younger child via oral syringe, or they can be crushed and added to applesauce.



### Administration using an oral syringe

To take NITYR tablets using a syringe, you will require an oral dosing with a cap. It only takes a minute to create the suspension, which can be stored for up to 24 hours at room temperature before use. This means you can prepare it the night before to administer in the morning, or in the morning to administer that evening. It's very simple. Visit the NITYR website for more information:

[www.nityr.us/patient/infant](http://www.nityr.us/patient/infant)

Our **NITYR Patient Support Program** will provide the reusable syringes for you along with your monthly refills. See pages 10–11 for full instructions on how to administer NITYR using an oral syringe.



### Administration using applesauce

Tablets may be crushed between two spoons and mixed with applesauce for administration. Tablet crushers are available from the **NITYR Patient Support Program** free of charge. See page 12 for full instructions on how to administer NITYR in applesauce.



### Designed for convenience

With your help, your child can lead a full life. With NITYR there is one less thing for you to worry about. If your child is newly diagnosed with HT-1, talk with your healthcare professional about how to manage the condition, including medication and diet.

If you already administer nitisinone to your child, moving to this new formulation is easy. NITYR (nitisinone) tablets are bioequivalent<sup>2</sup> to Orfadin\* (nitisinone) capsules. This means NITYR is clinically proven to have the same efficacy, safety and side effects, but is specially formulated aiming to be more convenient. Both medications are therapeutically equivalent. Creating a suspension using NITYR is a simple and clean process, which in turn can make your life easier.

NITYR is also stable at room temperature between 68° to 77°F (20° to 25°C), allowing for excursions between 59° and 86°F (15° and 30°C), and freeing you from having to refrigerate the medication<sup>3</sup> or to travel with ice packs in a container. To help families facing vigilant mealtimes every day, the new formulation can be taken with or without food.



### Access to support when you need it

Remember if you're navigating a new diagnosis of HT-1, many parents report feeling overwhelmed. But you're not alone and can always reach out to us at the **NITYR Patient Support Program** for support and to answer any questions.

This program will give you access to a pharmacist to give you the best available advice whenever you need help, 24/7, as well as proactive refills that put convenience first.



### The importance of ongoing monitoring and testing

HT-1 is not a one size fits all condition. Your healthcare professional and dieticians will make specific recommendations based on the weight of your baby and other factors. Physicians also regularly monitor and adjust therapy based on ongoing testing (blood, urine and other tests) – just one reason to keep your appointments over time.

Multiple routine tests help children with HT-1 thrive. These include measurement of urine succinylacetone (SA), 5- aminolevulinate (5-ALA), and erythrocyte porphobilinogen (PBG)-synthase as needed. In addition, your doctor will confirm that plasma tyrosine levels remain below 500 µmol/L, and that platelet and white blood cells counts, as well liver function testing levels, stay within a normal range. If you need help to understand these tests you can call today our **NITYR Patient Support Program: 1-800-847-8714**.

*“My 5-year-old son was really excited to be able to swallow the medication. He shows everyone that comes over how big he is, when he gets to take his medication with water. He asks for a crowd! He's really proud of himself. He feels like a big boy, really independent.”*

**Carrie**

Mother of Ethan (5 years old) NITYR patient.

See [www.nityr.us/mystory](http://www.nityr.us/mystory) to read the family's story.

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# How to Give NITYR Tablets Using an Oral Syringe

## To give one tablet

1. Use the reusable syringe provided by the **NITYR Patient Support Program**. Remove the plunger, add one NITYR tablet and replace the plunger.
2. Draw up 2.6 mL of room temperature water.
3. Put the cap back on and let the syringe sit for at least 60 minutes.
4. Once you are ready to administer (you can administer up to 24 hours after adding water to the tablet), shake the syringe well for at least 30 seconds and check that no large lumps of tablet remain. If there are large lumps, let the syringe sit for a further 10 minutes, and then reinspect the syringe for any large lumps.
5. Take the cap off, place the syringe in the child's mouth, and push down on the plunger until a small amount of air remains in the syringe. Do not press the plunger all the way to the end.
6. Rinse the syringe by drawing up another 2 mL of water. Replace the cap and shake the syringe well.
7. Take the cap off, place the syringe in the child's mouth and push down on the plunger until it is completely empty.

Take the NITYR suspension within 24 hours of adding water to the tablets.

## To give two tablets

Follow the same instructions adjacent, except:

In step 1, remove the plunger from the syringe and insert two tablets, then replace the plunger and draw up 5 mL of water at room temperature.

Note: More than two tablets should **NOT** be prepared in the same syringe. If your healthcare professional advises you to give three tablets, follow the instructions to prepare one syringe with one tablet and another syringe with two tablets. The same instructions apply to more than three tablets.

Full Instructions For Use (IFU) is available at [www.nityr.us/ifu](http://www.nityr.us/ifu)

Watch the instructive video:  
[www.nityr.us/patient/infant](http://www.nityr.us/patient/infant)

If you didn't receive the oral syringes, please call the **NITYR Patient Support Team** on:  
**1-800-847-8714**

Step 1



Step 2



Step 3



Step 4



Step 5



Step 6



Step 7



For two tablets



## How to Give NITYR Tablets with Applesauce

- Crush one NITYR tablet at a time by placing it between two teaspoons and pushing down lightly on the top spoon. Press, rotate, and look at the tablet. If large particles are still there, repeat until finely crushed.
- Add all the crushed tablet powder to one full teaspoon of applesauce in a clean glass or bowl.
- If giving more than one tablet, repeat the process above, adding the powder to the same applesauce.
- Mix well.
- Feed the mixture to your child using a teaspoon.
- To make sure you give your child all the medicine, add another full teaspoon of applesauce to the same glass or bowl and stir. Again, feed the mixture to your child.
- Use the NITYR-applesauce mixture within 2 hours of preparation.

Full Instructions For Use (IFU) are available at [www.nityr.us/ifu](http://www.nityr.us/ifu)

If you would like to receive a tablet crusher, please call the **NITYR Patient Support Team** on: **1-800-847-8714**.

Step 1



Step 2



Step 3



Step 4



Step 5



Step 6



## Important Considerations for Children 6 Years to 12 Years Old with HT-1

NITYR is bioequivalent<sup>1</sup> to Orfadin\*, meaning it contains the nitisinone medication (NTBC) your child has always taken, but is formed with novel techniques that may make treating HT-1 more convenient.

Now that your child is in elementary school, they probably realize their diet and routine is very different from other children's. NITYR is a small<sup>2</sup>, tasteless and discreet tablet, which may mean that your child can swallow it easily, after consultation with a healthcare professional. Tablets should be swallowed whole with a drink of water, and can be taken with or without food. With the right support, your child can start swallowing tablets directly very easily, which may give you peace of mind knowing that they have taken the whole dose.

The tablets are also easy to carry – and strong enough to be stored in your active child's pocket or backpack without breaking apart. Nitisinone tablets are taken twice a day, but check with your doctor for the best dosage schedule that can accommodate your daily routine.

NITYR is stable at room temperature between 68° to 77°F (20° to 25°C), allowing for excursions between 59° and 86°F (15° and 30°C), and freeing you from having to refrigerate the medication<sup>3</sup>. NITYR can also be taken with or without food, which can make life more convenient for you and your child.

Moving your child from a suspension to a tablet may help get them ready for taking their medication more independently, gaining a sense of control over their HT-1.



### Support when you need it

If you're like many parents of a child this age with HT-1, you're probably already somewhat of an expert on dietary restrictions and exceptional circumstances. But even "expert" parents have questions or need support as a child with HT-1 grows and develops. Remember you're not alone and can always reach out to us for support and to answer any pressing questions. Cycle Pharmaceuticals is proud to act as an ongoing resource for parents and caregivers, so we are here for you every step of the way.

The leading **NITYR Patient Support Program** is available 24/7 and puts convenience first. The patient support team provides advice and support with access to a pharmacist when you need help or have any questions. We can even help you with advice on your child eating at school and going on sleepovers or school trips.

<sup>1</sup>Data on file NIT/02/17. <sup>2</sup>Data on file NIT/03/18. <sup>3</sup>Data on file NIT/01/17. \*Orfadin is a registered trademark, licensed by Sobi, Inc. or its affiliates.

## Helping your Young Teen Transition to a Healthy Adult

You've done an excellent job helping your child thrive. Now your son or daughter is entering their early teen years, a time that can be challenging for parents and teenagers, even for those without special dietary and medication needs, but don't despair. We are here to help ease this transition time through our specialized **NITYR Patient Support Program**. If ever you have a question or concern use our patient support program to access a pharmacist for the best possible advice or guidance.

Your teenager is going to want to spend more time at after school activities or with friends, so taking nitisinone (NTBC) that does not require refrigeration or being taken with a meal can make sense. NITYR tablets may bring greater peace of mind for you and may offer your child a discreet way to take their medication around their friends. All they'll need is a drink of water. The tablets can be stored at room temperature for up to 36 months (3 years).



After carefully controlling your child's diet, medication and medical appointments, it can be difficult to transfer some responsibility to your child to start controlling their condition on their own. But don't worry, we are here for you. Just contact the **NITYR Patient Support Program** on **1-800-847-8714** for any questions you need answered, 24/7.

## Making the Most of Living with HT-1 as a Young Adult

You've now got more freedom and are making more choices on your own. Chances are this includes taking control of what you eat and how you manage HT-1.

NITYR is bioequivalent<sup>1</sup> to Orfadin\*. This means NITYR is clinically proven to have the same efficacy, safety and side effects, but may offer you and your family greater convenience. Both medications are therapeutically equivalent.

NITYR is stable at room temperature between 68° to 77°F (20° to 25°C), allowing for excursions between 59° and 86°F (15° and 30°C), and so frees you from having to refrigerate the medication<sup>2</sup>. NITYR can also be taken with or without food, you only need a drink of water to take the tablet. The tablets can be stored at room temperature for up to 36 months (3 years).

You've probably got enough challenges just being a young adult. Consider the greater convenience of NITYR that may make your life easier. You'll appreciate the convenience that NITYR may bring when you're eating with friends at school or eating out with your family. NITYR will be with you when you get older and start college or when traveling abroad.



### NITYR tablets:

- Are bioequivalent to Orfadin
- The tablet for all life stages
- Are very small<sup>3</sup> (baby aspirin sized) and tasteless
- Can be stored at room temperature, do not require refrigeration and have a shelf life of 36 months (3 years)
- Can be taken with or without food
- Have a Food Program<sup>4</sup>
- Have a \$0 co-pay for eligible commercially insured patients<sup>5</sup>

<sup>1</sup>Data on File NIT/02/17. <sup>2</sup>Data on file NIT/01/17. <sup>3</sup>Data on file NIT/03/18. <sup>4</sup>Diet and Low Protein Food Program: Subject to terms and conditions and eligibility criteria. This program is for eligible commercially insured patients only. Not available for government-insured patients and subject to other federal and state law. <sup>5</sup>\$0 co-pay for eligible commercially insured patients (up to \$15,000 per year per patient). \*Orfadin is a registered trademark, licensed by Sobi, Inc. or its affiliates.

## Living Life to the Fullest as an Adult with HT-1

You made it through all those years of everyone else telling you what to eat, when to take your medication and how to live your life around your HT-1.

NITYR is bioequivalent<sup>1</sup> to Orfadin\*. This means NITYR is clinically proven to have the same efficacy, safety and side effects, but may offer you and your family greater convenience. Both medications are therapeutically equivalent.

As a typical adult, you're probably on the go between home, work, college classes and all your new responsibilities. NITYR is stable at room temperature between 68° to 77°F (20° to 25°C), allowing for excursions between 59° and 86°F (15° and 30°C), freeing you from having to refrigerate the medication<sup>2</sup>. NITYR can be taken with or without food and stored at room temperature for up to 36 months (3 years).

It's a new formulation that may give you more flexibility.



### It's a new era in nitisinone therapy

NITYR is supplied in 2 mg, 5 mg and 10 mg tablets. The recommended initial dose is 0.5 mg/kg twice daily, administered orally, with or without food. The dose of nitisinone should be adjusted individually by your healthcare professional.

Cycle Pharmaceuticals, maker of NITYR, also provides the **NITYR Patient Support Program**, available 24/7, giving advice and support when you need help or have any questions.

For more information visit [www.nityr.us](http://www.nityr.us) or call **1-800-847-8714**.

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## Where to Turn Anytime to Find Answers and Solutions

HT-1 is an ultra-rare disorder, so reputable sources of information and guidance may feel limited at times. For this reason, we provide additional information and FAQs on our website, [www.nityr.us](http://www.nityr.us), as well as the **NITYR Patient Support Program**.

Our Patient Support Program gives people, parents and providers access to knowledgeable pharmacists. You can contact the **NITYR Patient Support Program** anytime you have questions or concerns, 24/7, just call 1-800-847-8714.

HT-1 advocacy and support groups can be a great resource for information and interacting with others affected by this condition. Just make sure you're seeking information and help from a reputable source online.

Cycle Pharmaceuticals can put you in touch with HT-1 patient advocacy groups such as the Network of Tyrosinemia Advocates (NOTA) [www.notacares.org](http://www.notacares.org), and the National Organization for Rare Diseases (NORD) [www.rarediseases.org](http://www.rarediseases.org)

You can also visit the NITYR website, [www.nityr.us](http://www.nityr.us), to access the latest information, NITYR news in the US, new offers and updates on HT-1.

Many patients and families find support when they connect with others affected by HT-1. Many have faced the same challenges you have, and they can offer guidance, recommend clinicians and coping strategies that work well for their family. When families communicate, they feel less isolated and more empowered to take control of living with HT-1. NOTA also maintains a Facebook group to connect and support people affected by tyrosinemia.

*"We are such a small community that we don't always get the same attention as some other conditions."*

**Mike**

Father of Ethan (5 years old) NITYR patient.

See [www.nityr.us/mystory](http://www.nityr.us/mystory) to read the family's story.

## Important Safety Information Regarding NITYR (nitisinone) Tablets

NITYR tablets are bioequivalent<sup>1</sup> to Orfadin\* capsules. This means NITYR is clinically proven to have the same efficacy, safety and side effects, but may offer you and your families greater convenience. Both products are therapeutically equivalent.

Do not take NITYR if you are allergic to nitisinone or any other ingredients. Tell your healthcare professional about any health problems, and if you are pregnant or planning to become pregnant, before starting treatment.

The warnings and precautions that come with NITYR include:

### **Increased levels of plasma tyrosine, eye symptoms, developmental delay and skin changes**

- Inadequate restriction of tyrosine and phenylalanine intake can result in elevations of plasma tyrosine
- At plasma tyrosine levels above 500 micromol/L, symptoms can occur including intellectual disability and developmental delay or painful hyperkeratotic plaques (thickening of the skin) on the soles and palms
- Do not adjust the dosage of NITYR in order to lower the levels of tyrosine in the blood

### **Changes in blood profile**

- Patients can develop leukopenia (reduction in the number of white blood cells, which form part of the immune system) and severe thrombocytopenia (abnormally low levels of platelets, which help the blood clot)
- Your healthcare professional will monitor platelet and white blood cell counts, and will adjust your medication accordingly

If you have a question about your dosage, or if you experience any of the symptoms explained above, please speak to your healthcare professional.

Keep NITYR and all medicines out of reach of children.

For more detailed information, please refer to the full prescribing information, which can be found at [www.nityr.us/pi](http://www.nityr.us/pi)

If you get any side effects, talk to your healthcare practitioner. This includes possible side effects not listed in this brochure. You can also report side effects directly by calling Cycle Pharmaceuticals at 1-855-831-5413 or FDA at 1-800-FDA-1088 or [www.fda.gov/medwatch](http://www.fda.gov/medwatch)

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# About Cycle Pharmaceuticals – What Motivates Us

Cycle Pharmaceuticals is a pioneering pharmaceutical company, reimagining how drugs can benefit patients, to make their lives easier and improve their quality of life at every stage.

Cycle has three core areas of pharmaceutical development:

- Improving drugs – optimizing an existing drug
- Repurposing drugs – creating a new indication for an already existing drug
- Generics – reinstating generic drugs, previously available in the market

These three areas of focus are underpinned by formulation technology – creating new drug delivery technologies to improve the efficacy and effectiveness of drugs, allowing Cycle to give patients greater freedom and choice.

With Headquarters in Cambridge (UK) and offices in Boston, Mass. (US), Cycle has developed unique partnerships with renowned universities around the world. Cycle is not limited to any one disease area. The company is actively working in a broad range of disease areas, from cardiovascular and inflammatory conditions, to rare diseases such as Cystic Fibrosis and Tyrosinemia type 1 (HT-1).

NITYR (nitisinone) is the first product from Cycle and demonstrates the company's commitment to improving patients' lives. NITYR contains the same nitisinone medication that patients with HT-1 have always used, but has a new formulation, aiming to provide patients with greater convenience.



[www.cyclepharma.com](http://www.cyclepharma.com)



@CyclePharma

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