Javygtor™

(sapropterin dihydrochloride)

Tablets or Powder for Oral Solution

JAVYGTOR Patient Trial Support Guide

This guide will support you in your JAVYGTOR Free Trial.

This guide also contains useful resources and information about Cycle Vita™, our dedicated hub support platform, which delivers individualized support for patients.*

This brochure is intended for patients and caregivers in the US who have been prescribed JAVYGTOR. It is provided for information purposes only. It is not intended to be, and should not be used as, a substitute for medical advice.

Important Safety Information[1]

Javygtor™
(sapropterin dihydrochloride)
Tablets or Powder for Oral Solution

JAVYGTOR Tablets for Oral Use and JAVYGTOR Powder for Oral Solution

What Important Information Should I Know About JAVYGTOR Tablets for Oral Use and Powder for Oral Solution?

• Sapropterin Dihydrochloride can cause serious side effects including:

Severe allergic reactions, including anaphylaxis – JAVYGTOR is not recommended in patients with a history of anaphylaxis to Sapropterin Dihydrochloride. Stop taking JAVYGTOR and get medical help right away if you develop any of these symptoms of severe allergic reaction:

• Wheezing or trouble breathing

• Coughing

• Flushing

• Nausea

• Feeling lightheaded or you faint

• Rash

Inflammation of the lining of the stomach (gastritis) or esophagus (esophagitis) - Gastritis or esophagitis can happen with Sapropterin Dihydrochloride and may be severe. Patients should be monitored by their doctor for signs and symptoms of upper gastrointestinal mucosal inflammation. Call your doctor right away if you have any of these signs or symptoms:

• Severe upper stomach area (abdominal) discomfort or pain, nausea and vomiting

• Blood in your vomit or stool

• Black, tarry stool

• Difficulty swallowing

• Loss of appetite

• Pain in the throat

Phenylalanine (Phe) levels that are too low - Some PKU patients have experienced low blood Phe during treatment. Some children under the age of 7 years who take high doses of Sapropterin Dihydrochloride each day may experience low Phe levels

Too much or constant activity (hyperactivity) can happen with Sapropterin Dihydrochloride - Tell your doctor if you have any signs of hyperactivity, including:

• Fidgeting or moving around too much

• Talking too much

• It is not possible to know if Sapropterin Dihydrochloride will work for you until you start treatment. Your doctor will check your blood Phe levels when you start taking JAVYGTOR to see if the medicine is working

• All patients with PKU who are being treated with JAVYGTOR should also be treated with a Phe-restricted diet, including dietary protein and Phe restriction

• Your doctor should continue to monitor your blood Phe levels often during your treatment with JAVYGTOR to make sure that your blood Phe levels are not too high or too low. Frequent blood monitoring is recommended in the pediatric population

• Avoid any dietary changes which may affect your blood Phe level. Follow your doctor's instructions carefully and do not make any changes to your dietary Phe intake without first talking with your doctor. Even if you take JAVYGTOR and your Phe blood levels are not well controlled, you can develop severe neurologic problems

Important Safety Information[1], continued

• Tell your doctor as soon as possible if you are sick or have fever as this may increase your blood Phe level

• Take JAVYGTOR exactly as your doctor tells you to

• Do not take 2 doses in a day. If you take too much JAVYGTOR, call your doctor for advice or seek medical attention

Who Should Not Use JAVYGTOR Tablets for Oral Use and Powder for Oral Solution?

• JAVYGTOR Tablets for Oral Use and Powder for Oral Solution are not recommended in patients with a history of anaphylaxis to Sapropterin Dihydrochloride or any of the ingredients in Sapropterin Dihydrochloride

What Should I Tell My Doctor Before Taking JAVYGTOR Tablets for Oral Use and Powder for Oral Solution?

Before taking JAVYGTOR Tablets for Oral Use or Powder for Oral Solution, tell your doctor if you:

• Are allergic to Sapropterin Dihydrochloride or any of the ingredients in Sapropterin Dihydrochloride

• Have poor nutrition or have loss of appetite

• Are pregnant or plan to become pregnant

• Are breastfeeding or plan to breastfeed

Provide your doctor with a complete list of medications you take, including prescription and over-the-counter medications, vitamins, and dietary or herbal supplements. JAVYGTOR can interact with other medications that you are taking. Especially tell your doctor if you take:

• A medication that contains levodopa

• An antifolate medication, such as methotrexate, phenobarbital, trimethoprim, valproic acid

• Sildenafil (Revatio, Viagra), tadalafil (Adcirca, Cialis), vardenafil (Staxyn, Levitra)

Tell your doctor if you are unsure if your medication is one of the above listed. Always share your complete list of medications with your doctor and pharmacist.

What Are the Most Common Side Effects of JAVYGTOR Tablets for Oral Use and Powder for Oral Solution?

• Headache

• Runny nose and nasal congestion

• Sore throat

• Diarrhea

• Vomiting

• Cough

These are not all of the possible side effects of JAVYGTOR. Call your doctor for medical advice about side effects.

Contents

Important Safety Information	2-3
Welcome	5
What is Phenylketonuria (PKU)?	6
Why do I need a sapropterin trial?	7
How does the trial work?	8
Preparing for success	9
My Calendar	10
My Trial Log	10
Support from Cycle Vita	11

Javygtor™
(sapropterin dihydrochloride)
Tablets or Powder for Oral Solution

NOT ACTUAL PATIENTS

CYCLE VITA™
Support for life

Welcome from Cycle Vita!

Hello and congratulations on taking the first step to undertake the free JAVYGTOR (sapropterin dihydrochloride) trial.

Through discussion with your healthcare provider, you've decided to see if you, or your loved one, respond to sapropterin. This is an important decision and we're here to support you.

You'll find lots of useful information and tools to help you during your trial in this brochure. We recommend you read it through as it really will help prepare you for success.

And remember, if you ever have any questions or need any support then our team is just a call away.

Best Wishes,

Cycle Vita

You can contact the friendly, familiar Cycle Vita team on: 888-360-8482 hello@cyclevita.life

Important Safety Information[1]
It is not possible to know if Sapropterin Dihydrochloride will work for you until you start treatment. Your doctor will check your blood Phe levels when you start taking JAVYGTOR to see if the medicine is working.

What is Phenylketonuria (PKU)?

PKU is an inherited disorder caused by a deficiency of an enzyme (phenylalanine hydroxylase, sometimes known as PAH). The PAH enzyme is needed to process phenylalanine (Phe), which is found in all foods containing protein. High levels of Phe can cause brain damage.[2] The incidence of PKU is highest amongst caucasians, occurring in approximately 1 in 10,000 births.[3]

How is PKU treated?

The first line of therapy to treat PKU is a Phe-restricted diet. This involves foods which have been modified to be low in protein, and medical formula (a special nutritional supplement), which provides the nutrients a person without PKU would typically get from protein.[4]

In addition to the Phe-restricted diet, some patients with PKU also take a medication called sapropterin dihydrochloride which is the active ingredient in JAVYGTOR. This medication helps the PAH enzyme work more effectively to break down Phe in the body.[1]

JAVYGTOR is not a cure for PKU, but when combined with a Phe-restricted diet it can help keep Phe levels under control,[1] and may help you better manage your condition.

For more information on PKU or JAVYGTOR, visit www.javygtor.com

Important Safety Information[1]
All patients with PKU who are being treated with JAVYGTOR should also be treated with a Phe-restricted diet, including dietary protein and Phe restriction.

Why do I need a sapropterin trial?

Javygtor™
(sapropterin dihydrochloride)
Tablets or Powder for Oral Solution

Not all patients with PKU respond to sapropterin dihydrochloride and so in some cases a trial is required before JAVYGTOR treatment can initiate.

Your doctor and PKU team have probably selected you for a trial for one of the following reasons:

You have been newly diagnosed with PKU and are starting out on your treatment journey; testing your response to sapropterin is an essential step.

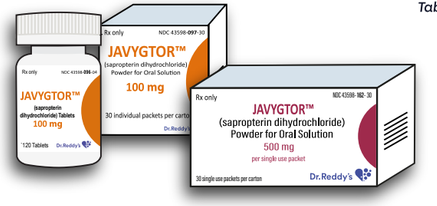
You have not needed medication to help manage your PKU to date, but you may now require additional treatment options. A sapropterin trial is part of understanding what the best treatment is for you.

Important Safety Information[1]
Your doctor should continue to monitor your blood Phe levels often during your treatment with JAVYGTOR to make sure that your blood Phe levels are not too high or too low. Frequent blood monitoring is recommended in the pediatric population.

How does the trial work?

Javygtor™
(sapropterin dihydrochloride)
Tablets or Powder for Oral Solution

The trial will take place over 30 days and will assess if you respond to sapropterin dihydrochloride by closely monitoring your blood Phe levels.



During your medication trial you can expect:

- Free JAVYGTOR Tablets or Powder for Oral Solution for the duration of your trial. Your doctor will talk to you about which form of JAVYGTOR best suits your needs and how much you will need to take each day.
- A Phe-restricted diet, including medical formula (a special nutritional supplement), will be prescribed by your clinic and must be carefully followed at all times throughout the trial.
- Clinical support from the Cycle Vita team, who are here for you if you have any questions about taking JAVYGTOR, or need advice in following the diet your clinic has set for you.

You can contact the friendly, familiar Cycle Vita team on:

888-360-8482 hello@cyclevita.life

CYCLE VITA™ Support for life

Important Safety Information[1]
Take JAVYGTOR exactly as your doctor tells you to. Do not take 2 doses in a day. If you take too much JAVYGTOR, call your doctor for advice or seek medical attention.

Please see the complete Important Safety Information on pages 2-3 and accompanying full Prescribing Information available at www.JAVYGTOR.com/PI

Preparing for success

To support you during the trial you will be given a Trial Welcome Kit, which contains everything you will need to provide your clinic with blood samples, as well as useful items such as measuring cups and spoons.

During the trial it is very important that you follow the instructions provided by your clinic as closely as possible so the trial result is accurate. If you have any questions about JAVYGTOR during the trial, the clinical team at Cycle Vita are just a call away.

You can contact the friendly, familiar Cycle Vita team on:

888-360-8482 hello@cyclevita.life

CYCLE VITA™ Support for life

Important Safety Information[1]
Avoid any dietary changes which may affect your blood Phe level. Follow your doctor's instructions carefully and do not make any changes to your dietary Phe intake without first talking with your doctor. Even if you take JAVYGTOR and your Phe blood levels are not well controlled, you can develop severe neurologic problems.

Please see the complete Important Safety Information on pages 2-3 and accompanying full Prescribing Information available at www.JAVYGTOR.com/PI

My JAVYGTOR Calendar and Trial Log

Javygtor™
(sapropterin dihydrochloride)
Tablets or Powder for Oral Solution

When it comes to managing PKU, routine is everything!

Scan the QR code below, or visit www.javygtor.com/my-calendar to find a calendar, which you can either print off or fill in digitally, to track each day's treatment.

During your trial it will be important to keep track of key metrics such as your diet over the course of the trial as well as blood Phe levels. This information will then be fed back to your medical team to help them determine if the trial is working.

Scan the QR code or visit www.javygtor.com/trial-log for your copy of the log that you can either print off or fill in digitally.

Once your free trial has finished, your Phe levels will be measured by healthcare provider administered labs. During this period, eligible* patients will have access to the Bridge Program, which offers a free supply of JAVYGTOR while Cycle Vita assists with Prior Authorization, if required. You can continue to use the JAVYGTOR Calendar and Trial Log during this period to help with your routine.

Important Safety Information[1]
Tell your doctor as soon as possible if you are sick or have fever as this may increase your blood Phe level.

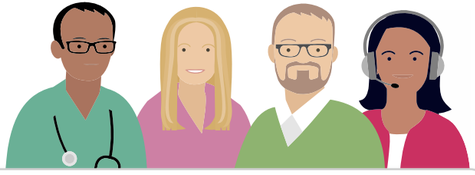
Cycle Vita - We're here for you, *for life*

At Cycle Pharmaceuticals we know that life-changing treatments need life-improving product support to match.

When you put the two together, great things can happen. That's why we created Cycle Vita, delivering you individualized product support which is as unique as you are. Cycle Vita is here to give you product, financial,* clinical and nutritional support at every step of your journey.

For as long as you need us, we're here to support you.

Cycle Vita



Get in touch with one of the team:

888-360-8482 | hello@cyclevita.life | www.cyclevita.life

CYCLE VITA
Support for life

Important Safety Information[1]
JAVYGTOR Tablets for Oral Use and Powder for Oral Solution are not recommended in patients with a history of anaphylaxis to Sapropterin Dihydrochloride or any of the ingredients in Sapropterin Dihydrochloride.

Javygtor™

(sapropterin dihydrochloride)

Tablets or Powder for Oral Solution

NOT ACTUAL PATIENTS

For more information on PKU or JAVYGTOR, please visit:

www.JAVYGTOR.com

Please see the complete Important Safety Information on pages 2-3 and accompanying full Prescribing Information available at www.JAVYGTOR.com/PI

*Some areas of support may not be accessible to all patients. Eligibility criteria may apply to ensure compliance with all applicable federal and state requirements, and benefits may be limited to commercially insured patients only. For more detailed information about eligibility, terms and conditions, please contact the Cycle Vita team at 888-360- 8482.

References:

1. JAVYGTOR (sapropterin dihydrochloride). Prescribing Information. Dr Reddy's Laboratories Ltd.
2. National Institutes of Health. Phenylketonuria (PKU). Available at: https://www.nichd.nih.gov/health/topics/pku. (Accessed March 22nd, 2024).
3. Williams, R. A. et al. 2008. Phenylketonuria: An Inborn Error of Phenylalanine Metabolism. The Clinical Biochemist Reviews. 29(1) 31-41.
4. Vockley J., et al; for the American College of Medical Genetics and Genomics Therapeutic Committee. Phenylalanine hydroxylase deficiency: diagnosis and management guideline. Genet Med. 2014;16(2):188-200. doi:10.1038/gim.2013.157.

©2024 Cycle Pharmaceuticals Limited. All rights reserved. JAVYGTOR is a trademark of Cycle Pharmaceuticals Limited in the United States. Cycle Vita is a trademark of Cycle Pharmaceuticals Ltd in the United States. US-SAP-2400023 | RDY-0324-627 | April 2024