

UREA CYCLE DISORDER PATIENT ENROLLMENT FORM INSTRUCTIONS

The Urea Cycle Disorder Patient Enrollment Form is required to initiate treatment with Horizon UCD Medications.

Instructions:

1. Complete all required patient information.
2. Complete all required insurance information for the patient and, if possible, attach a copy of the patient's insurance card.
3. Complete the diagnosis and prescription information in its entirety; all fields are required. The patient's healthcare provider should fill out this section.
4. Complete all required prescriber information, including the contact information for the practice or facility.
5. A signature is required from the patient's healthcare provider.
6. Fax the completed form to Horizon Patient Services at **1-877-695-8304**.
7. Check in with your patient to ensure he or she has completed the HIPAA Authorization Form. It must be completed and sent in to initiate support.
8. If you have any questions or comments, please contact Horizon Patient Services at **1-855-823-7878**.

UREA CYCLE DISORDER PATIENT ENROLLMENT FORM

Patients must also complete the Urea Cycle Disorder HIPAA Authorization Form for all new referrals.

Patient Information (* indicates required field)

Patient Name*: _____ DOB*: ____/____/____ Gender*: Male Female
 Address*: _____ City*: _____ State*: _____ Zip Code*: _____
 Preferred Phone*: (____) _____ Alternate Phone: (____) _____ Email*: _____
 Caregiver/Alternate Contact Name: _____ Relationship: _____ Phone: (____) _____
 Preferred Contact: Patient Caregiver Preferred Type: Phone (Day) Phone (Evening) Email Preferred Language: _____

Insurance Information (* indicates required field) Please attach copies of insurance card(s), if available.

Primary Insurance Company*: _____ Secondary Insurance Company: _____
 Phone*: (____) _____ Phone: (____) _____
 Policy Type: Medicare Medicaid Commercial Other Policy Type: Medicare Medicaid Commercial Other
 Policy #*: _____ Group #: _____ Policy #: _____ Group #: _____
 Policyholder Name*: _____ Policyholder Name: _____
 Relationship: _____ DOB: ____/____/____ Relationship: _____ DOB: ____/____/____
 Prescription Card: Yes No If Yes, Carrier: _____ Phone: (____) _____
 Identification #: _____ Policy/Group #: _____
 Policyholder Name: _____ Relationship: _____ DOB: ____/____/____

Diagnosis and Prescription Information (ALL fields required)

DIAGNOSIS:

Ornithine transcarbamylase deficiency/OTC (E72.4) Argininosuccinate lyase deficiency/ASL (E72.22) Argininemia/ARG (E72.21)
 Argininosuccinate synthetase/ASSD (E72.22) Carbamoyl phosphate synthetase/CPS (E72.29) Citrullinemia/CITRIN (E72.23)
 Hyperammonemia-hyperornithinemia-homocitrullinuria syndrome/HHH (E72.4) Disorder of urea cycle metabolism, unspecified (E72.20)
 Other diagnosis, ICD-10 _____ Please visit <http://www.icd10data.com/Convert/270.6> for more information.
 Patient Weight: _____ lb/kg (circle one) Patient Height: _____ in/cm (circle one) Date of Diagnosis (month/year): _____
 Current Nitrogen Scavenger: Sodium phenylbutyrate Sodium benzoate Sodium phenylbutyrate and sodium benzoate No nitrogen scavenger

PRESCRIPTION:

RAVICTI® (glycerol phenylbutyrate) Oral Liquid (mL) BUPHENYL® (sodium phenylbutyrate) Tablets BUPHENYL® (sodium phenylbutyrate) Powder (g)
 Dose: _____ Doses/Day: _____ Total Daily Dose: _____ Days Supply: _____
 Total Quantity: _____ # Refills: _____ Instructions: _____

Prescriber Information (* indicates required field)

First and Last Name*: _____ Credentials: _____
 NPI #: _____ State License #: _____ State Issued: _____ Tax ID: _____ Specialty*: _____
 Practice/Facility Name*: _____ Primary Contact Name: _____
 Address*: _____ City*: _____ State*: _____ Zip Code*: _____
 Phone*: (____) _____ Fax: (____) _____ Prescriber Email: _____

Prescriber Acknowledgement: I certify that the above therapy is medically necessary and that the information provided is accurate to the best of my knowledge. I understand that Horizon Pharma and its employees or agents (collectively, "Horizon") will use this information to administer the Horizon Patient Services™ program (the "Program"), which provides assistance to patients in obtaining coverage for Horizon UCD Medications and assistance in initiating or continuing Horizon UCD Medications. By my signature, I also acknowledge that my patient or his or her personal representative has provided a signed HIPAA authorization that allows me to share protected health information with Horizon for purposes of the Program. I appoint the Program, on my behalf, to convey this prescription to the dispensing pharmacy, to the extent permitted under state law. I further understand and agree that (a) any medication or service provided through the Program as a result of this form is for the named patient only and is not being made in exchange for any express or implied agreement or understanding that I would recommend, prescribe, or use Horizon UCD Medications, or any other Horizon product or service, for any other person, (b) my decision to prescribe Horizon UCD Medications was based solely on my professional determination of medical necessity, and (c) I will not seek reimbursement for any medication or service provided by or through the Program from any government program or third-party insurer. I understand that Horizon may modify or terminate the Program at any time without notice.

State requirements: The prescriber is to comply with his/her state specific prescription requirements such as e-prescribing, state specific prescription form, fax language, etc. Noncompliance with state specific requirements could result in outreach to the prescriber.

Prescriber Name*: _____ Date: _____
 Prescriber Signature*: _____ (Dispense as Written) _____ (Substitution Allowed)

HIPAA Authorization

I hereby authorize my healthcare providers, my health insurance carriers, and my pharmacies to use and disclose my individually identifiable health information, including my medical records, insurance coverage information, and my name, address and telephone number to Horizon Pharma USA, Inc. and its affiliates and their respective agents and representatives (collectively, "Horizon"), including third parties authorized by Horizon to administer drug support and to dispense drugs (collectively, "Horizon Patient Services") for the following purposes: (1) to establish eligibility for benefits; (2) to communicate with healthcare providers and me about my medical care; (3) to facilitate the provision of products, supplies, or services by a third party including, but not limited to, specialty pharmacies; (4) to register me in any applicable product registration program required for my treatment; (5) to enroll me in eligible patient support programs offered by Horizon Patient Services and/or Horizon, including nursing or patient access support services (government-reimbursed programs may not be eligible for all support services offered; please contact Horizon Patient Services for determination); and (6) to send me marketing information related to my treatment or condition (or related products or services in which I might be interested) and to contact me occasionally to obtain my feedback (for market research purposes only) about my treatment, my condition, or my experience with Horizon and/or Horizon Patient Services otherwise as required or permitted by law. I understand the pharmacies may receive a fee from Horizon in exchange for (1) providing me with certain materials and information described above, and (2) using or disclosing certain health information pursuant to this Authorization.

I understand that Horizon, as well as my healthcare providers, cannot require me, as a condition of having access to medications, prescription drugs, treatment, or other care, to sign this Authorization. I understand that I am entitled to a copy of this Authorization. I understand that information disclosed pursuant to this Authorization in some cases may be redisclosed by the recipient and no longer protected by HIPAA or other privacy laws. But Horizon has agreed to use and disclose my information only for purposes of operating the program.

I understand that I may cancel this Authorization at any time by mailing a signed letter requesting such cancellation to Horizon Patient Services, 150 South Saunders Rd, Lake Forest, IL 60045, but that this cancellation will not apply to any information used or disclosed by my healthcare providers and/or health insurance carriers based on this Authorization before they are notified that I have cancelled it. Unless required by state law, this Authorization is valid for whichever is greater: (a) the duration of remaining on this treatment or (b) 10 years from the date signed below.

A photocopy of this Authorization will be treated in the same manner as the original.

Date: _____

Patient's Printed Name: _____

Patient's/Legally Authorized Representative's Signature: _____

Legally Authorized Representative's Printed Name (if required): _____

Patient's/Legally Authorized Representative's Home Address:

Street Address: _____

City: _____ State: _____ Zip Code: _____

Patient's/Legally Authorized Representative's Telephone: _____ Home Mobile

Patient's/Legally Authorized Representative's Email Address: _____

Legally Authorized Representative's Relationship to Patient: Spouse Parent/Legal Guardian Representative per Power of Attorney

Is there someone else with whom we may discuss your protected health information? No Yes

Name: _____

Relationship to you: _____

Name: _____

Relationship to you: _____

APPROVED USES AND IMPORTANT SAFETY INFORMATION

What is RAVICTI?

RAVICTI (glycerol phenylbutyrate) Oral Liquid is a prescription medicine used for long-term management of high blood levels of ammonia (hyperammonemia) caused by a condition called a urea cycle disorder (UCD). RAVICTI should be used if the UCD cannot be managed with a low-protein diet and dietary supplements alone. RAVICTI must be used along with a low-protein diet and in some cases, dietary supplements.

RAVICTI is not used to treat extremely high levels of ammonia in the blood (hyperammonemic crisis) in people with UCDS.

It is not known if RAVICTI is safe and effective for the treatment of *N*-acetylglutamate synthase (NAGS) deficiency.

IMPORTANT SAFETY INFORMATION

What is the most important safety information I should know about RAVICTI?

RAVICTI may cause serious side effects, including:

Nervous system side effects (Neurotoxicity). The breakdown of RAVICTI produces the byproduct phenylacetate (PAA), which may cause nervous system side effects.

Call your doctor or get medical help right away if you have any of these symptoms while taking RAVICTI:

- sleepiness
- lightheadedness
- change in taste
- problems with hearing
- confusion
- problems with memory
- worsening of numbness, tingling, or burning in your hands or feet
- headache
- feeling very tired (fatigue)
- nausea
- vomiting

Who should not take RAVICTI?

Do not take RAVICTI if you are allergic to phenylbutyrate. Call your doctor or go to the nearest hospital emergency room if you experience any of the following symptoms of an allergic reaction while taking RAVICTI:

- wheezing
- shortness of breath
- cough
- low blood pressure
- flushing
- nausea
- skin rash

What should I tell my doctor before taking RAVICTI?

Tell your doctor about any medical conditions and if you:

- Have liver or kidney problems.
- Have pancreas or bowel (intestine) problems.
- Are pregnant or plan to become pregnant. It is not known if RAVICTI will harm your unborn baby.
 - **Pregnancy Registry:** There is a Pregnancy Registry for women who take RAVICTI just before becoming pregnant or who become pregnant during treatment with RAVICTI. The purpose of this registry is to collect information about the health of you and your baby. Talk to your doctor about how you can join the Pregnancy Registry. For more information about this registry, call 1-855-823-2595 or visit www.ucdregistry.com.
- Are breastfeeding or plan to breastfeed. It is not known if RAVICTI passes into your breast milk. Breastfeeding is not recommended during treatment with RAVICTI. Talk to your doctor about the best way to feed your baby if you take RAVICTI.

What are possible side effects of RAVICTI?

RAVICTI may cause serious side effects, including:

- See “What is the most important information I should know about RAVICTI?”

The most common side effects of RAVICTI in adults include:

- diarrhea
- gas
- headache
- abdomen (stomach) pain
- vomiting
- tiredness
- decreased appetite
- indigestion or heartburn

The most common side effects of RAVICTI in children 2 years to 17 years of age include:

- upper abdomen (stomach) pain
- rash
- nausea
- vomiting
- diarrhea
- decreased appetite
- headache

The most common side effects of RAVICTI in children 2 months to less than 2 years of age include:

- low white blood cell count (neutropenia)
- vomiting
- diarrhea
- fever
- reduced food intake
- cough
- stuffy nose
- runny nose
- skin rash
- small round bumps on the skin

Please fax the completed form to 1-877-695-8304.

Phone: 1-855-823-7878 • Please visit www.UCDsupport.com.

The most common side effects of RAVICTI in children less than 2 months of age include:

- vomiting
- rash
- gastroesophageal reflux
- increased levels of liver enzymes in the blood
- decreased appetite and reduced food intake
- low red blood cell count (anemia)
- cough
- loss of too much body fluid (dehydration)
- too much acid in the blood (acidosis)
- high blood platelet count (thrombocytosis)
- low blood platelet count (thrombocytopenia)
- low blood neutrophil count (type of white blood cell) (neutropenia)
- high white blood cell count (lymphocytosis)
- diarrhea
- gas
- constipation
- fever
- drowsiness (lethargy)
- irritability
- agitation

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

For additional important safety information, please see the Full Prescribing Information and Medication Guide available at RAVICTI.com and discuss with your doctor.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit <http://www.fda.gov/medwatch> or call 1-800-FDA-1088.

IMPORTANT SAFETY INFORMATION

Approved Uses for BUPHENYL®

BUPHENYL (sodium phenylbutyrate) Tablets is a prescription medicine that can be taken by mouth and BUPHENYL (sodium phenylbutyrate) Powder is a prescription medicine that can be taken by mouth or feeding tube for the long-term management of high blood levels of ammonia (hyperammonemia) caused by a condition called a urea cycle disorder (UCD). BUPHENYL should be used if the UCD cannot be managed with a low-protein diet and dietary supplements alone.

BUPHENYL only treats high blood levels of ammonia in patients with the following enzyme deficiencies:

- Carbamylphosphate synthetase (CPS)
- Ornithine transcarbamylase (OTC)
- Argininosuccinic acid synthetase (AS)

BUPHENYL can be used in infants up to 28 days old who have a complete enzyme deficiency (an enzyme in the urea cycle that does not work at all). It can also be used in people 1 month of age and up who have a partial enzyme deficiency (an enzyme in the urea cycle that only works partially) and have a history of brain damage from high blood levels of ammonia (hyperammonemia). It is important to have a healthcare provider diagnose this condition and prescribe a medication as early as possible to improve chance of survival.

BUPHENYL must be used along with a low-protein diet and, in some cases, dietary supplements.

Any episode related to acute hyperammonemia should be treated as a life-threatening emergency.

Important Safety Information (ISI)

Do not take BUPHENYL if you are allergic to phenylbutyrate, or for the treatment of acute hyperammonemia in people with UCDS.

Use of BUPHENYL may cause serious side effects to the nervous system due to phenylacetate, a breakdown product of BUPHENYL. Call your doctor or get medical help right away if you experience any of the following symptoms while taking BUPHENYL: sleepiness, weakness, lightheadedness, problems with memory, worsening neuropathy (numbness, tingling, or burning in your hands or feet), change in taste, problems with hearing, confusion, and headache.

Talk to your doctor before taking BUPHENYL if you have heart failure or decreased kidney function, which may lead to retention of the sodium content of BUPHENYL with potentially serious consequences such as worsening heart failure, high blood pressure, and swelling. You and your doctor should decide if you will take BUPHENYL if you have these medical conditions. Do not take BUPHENYL if you have liver or kidney problems, have any other medical conditions, if your child is 20kg or less, or if you are planning to become pregnant or breastfeed, as it is unknown if BUPHENYL will harm your unborn baby or will pass into your breastmilk.

The most common side effects of BUPHENYL include absent or irregular periods in women, decreased appetite, body odor, and bad taste.

The most common side effects of BUPHENYL® seen in a laboratory setting include changes to blood pH and electrolyte levels (such as chloride and phosphate), low protein levels in the blood, high levels of certain bone and liver enzymes (such as alkaline phosphatase and transaminases), and decreased red and white blood cell and platelet count.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch or call 1-800-FDA-1088.

This information is not intended to replace discussions with your doctor. For additional information about BUPHENYL®, please consult the Full Prescribing Information and the Information for the Patient/Caregiver and talk to your doctor. BUPHENYL® is available by prescription only.

Please visit HorizonPharma.com/buphenyl to download a copy of the BUPHENYL Full Prescribing Information.