

## 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 TranscendRare at **1-877-695-8304**.
7. Check in with your patient to ensure he or she has completed the Patient Authorization Form. It must be completed and sent in to initiate services.
8. If you have any questions or comments, please contact TranscendRare at **1-855-823-7878**.

## UREA CYCLE DISORDER PATIENT ENROLLMENT FORM

Patients must also complete the Urea Cycle Disorder Patient Enrollment Form for all new referrals.  
TranscendRare is available to all UCD-diagnosed patients regardless of prescription and/or treatment plan.

### 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  
 Program Enrollment Only (Enrollment-only patients will not receive medication through the program.) Preferred Language: \_\_\_\_\_

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

Primary Insurance Company\*: \_\_\_\_\_ Phone\*: (\_\_\_\_) \_\_\_\_\_  
 Policy Type:  Medicare  Medicaid  Commercial  Other Diagnosis Policy #: \_\_\_\_\_ Group #: \_\_\_\_\_  
 Policyholder Name\*: \_\_\_\_\_ Relationship: \_\_\_\_\_ DOB: \_\_\_\_/\_\_\_\_/\_\_\_\_  
 Secondary Insurance Company: \_\_\_\_\_ Phone: (\_\_\_\_) \_\_\_\_\_  
 Policy Type:  Medicare  Medicaid  Commercial  Other Policy #: \_\_\_\_\_ Group #: \_\_\_\_\_  
 Policyholder Name: \_\_\_\_\_ Relationship: \_\_\_\_\_ DOB: \_\_\_\_/\_\_\_\_/\_\_\_\_  
 Prescription Card?:  Yes If Yes, Carrier: \_\_\_\_\_ Phone: (\_\_\_\_) \_\_\_\_\_  
 Identification #: \_\_\_\_\_ Policy/Group #: \_\_\_\_\_  
 Policyholder Name: \_\_\_\_\_ Relationship: \_\_\_\_\_ DOB: \_\_\_\_/\_\_\_\_/\_\_\_\_

### Diagnosis Information (ALL fields required)

**DIAGNOSIS:**  Disorder of urea cycle metabolism, unspecified (E72.20)  Arginosuccinic aciduria (E72.22)  Citrullinemia (E72.23)  
 Specific Disorder:  CPS  OTC  ASS  ASL  ARG  HHH  CITRIN Date of Diagnosis (month/year): \_\_\_\_\_  
 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) Current Therapy: \_\_\_\_\_

### Prescription Information (Complete all fields if you are prescribing medication for your patient.)

**PRESCRIPTION:**  RAVICTI® (glycerol phenylbutyrate) Oral Liquid (mL) \_\_\_\_\_ Dose \_\_\_\_\_ Doses/Day \_\_\_\_\_ Total Daily Dose \_\_\_\_\_  
 BUPHENYL® (sodium phenylbutyrate) Tablets Days Supply: \_\_\_\_\_ Total Quantity: \_\_\_\_\_ # Refills: \_\_\_\_\_  
 BUPHENYL® (sodium phenylbutyrate) Powder (g) 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 TranscendRare 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. Non-compliance with state specific requirements could result in outreach to the prescriber.

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

Please see the Important Safety Information for RAVICTI on the following page and the Full Prescribing Information and Medication Guide available at [RAVICTI.com](http://RAVICTI.com). Please see the Important Safety Information for BUPHENYL on page 2, and visit [HorizonPharma.com/buphenyl](http://HorizonPharma.com/buphenyl) to download a copy of the BUPHENYL Full Prescribing Information.

## IMPORTANT SAFETY INFORMATION

### Approved Uses for RAVICTI® (glycerol phenylbutyrate) Oral Liquid

RAVICTI is a prescription medicine used in adults and children 2 months of age and older for long-term management of high levels of ammonia in the blood (hyperammonemia) caused by a condition called a urea cycle disorder (UCD). RAVICTI must be used along with a low-protein diet and, in some cases, dietary supplements. RAVICTI should only be used if the UCD cannot be managed with a low-protein diet and dietary supplements alone.

RAVICTI is not used to treat extremely high levels of ammonia in the blood (hyperammonemic crises) in people with UCDs as they may require rapidly acting medication.

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

It is not known if RAVICTI is safe and effective for children younger than 2 months.

### Detailed Important Safety Information

#### Who should not take RAVICTI:

Children younger than 2 months should not take RAVICTI because they may not be able to digest it.

Do not take RAVICTI if you are allergic to phenylbutyrate. Call your doctor or go to the nearest hospital emergency room if you have symptoms of an allergic reaction, such as wheezing, shortness of breath, cough, low blood pressure, flushing, nausea, or rash while taking RAVICTI.

#### RAVICTI may cause serious side effects:

The breakdown of RAVICTI produces the byproduct phenylacetate, 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, tiredness; nausea; or vomiting.

#### What are the possible side effects of RAVICTI?

The most common side effects of RAVICTI in adults include diarrhea, gas, headache, nausea, vomiting, tiredness, decreased appetite, and dizziness.

The most common side effects of RAVICTI in children ages 2 to 17 years include stomach pain, nausea, vomiting, diarrhea, decreased appetite, and headache.

The most common side effects of RAVICTI in children ages 2 months to younger than 2 years include decreased level of a type of white blood cell, vomiting, diarrhea, fever, decreased appetite, cough, nasal congestion, runny nose, and rash.

Tell your doctor if you have any side effect that bothers you or that does not go away. These are not all of the possible side effects of RAVICTI. Call your doctor for medical advice about side effects.

#### Before you take RAVICTI:

Tell your doctor if you have liver or kidney problems, pancreas or bowel (intestine) problems, or any other medical conditions. Tell your doctor if you are pregnant or plan to become pregnant. It is not known if RAVICTI will harm your unborn baby. Tell your doctor if you 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.

Talk to your doctor about participating in a UCD registry. The purpose of this registry is to collect information about people with UCDs to improve care. For more information about the registry program, call 1-855-823-2595 or visit [www.ucdregistry.com](http://www.ucdregistry.com).

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

This information is not comprehensive. To learn more, talk to your healthcare provider or pharmacist. The FDA-approved product labeling, including the Medication Guide, can be found at [Ravicti.com](http://Ravicti.com).

Please visit [RAVICTI.com](http://RAVICTI.com) to download a copy of the RAVICTI Full Prescribing Information and Medication Guide.

## 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](http://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](http://HorizonPharma.com/buphenyl) to download a copy of the BUPHENYL Full Prescribing Information.