UREA CYCLE DISORDER PATIENT ENROLLMENT FORM INSTRUCTIONS

The Urea Cycle Disorder Patient Enrollment Form is required to initiate treatment with Horizon urea cycle disorder (UCD) medicines.

Instructions:

1. Complete all required patient information.
2. Complete all required insurance information for the patient and attach copies of the front and back of the patient's medical and prescription insurance cards.
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 By Your Side, a patient support program, at **1-877-695-8304** or email it to **UCDHBYS@horizontherapeutics.com**.
7. Check with your patient to ensure he or she has printed, signed, and dated the required Patient Authorization Form providing HIPAA authorization for Horizon By Your Side, in order to initiate patient support.
8. If you have any questions or comments, please contact Horizon By Your Side at **1-855-823-7878.**

Please see the Important Safety Information for RAVICTI® (glycerol phenylbutyrate) Oral Liquid on page 4, and click here for the RAVICTI Full Prescribing Information.

Please see the Important Safety Information for BUPHENYL® (sodium phenylbutyrate) Tablets on page 5, and click here for the BUPHENYL Full Prescribing Information.

HORIZON BY your side

UREA CYCLE DISORDER PATIENT ENROLLMENT FORM

Please fax the completed form to Horizon By Your Side at 1-877-695-8304 or email it to UCDHBYS@horizontherapeutics.com.

HORIZON

Phone: 1-855-823-7878
Fax: 1-877-695-8304
HorizonByYourSide.com

1. PATIENT INFORMATION

First Name: _____ MI: ____ Last Name: _____ DOB: ____/____/____ Gender: ☒ Male ☐ Female

Address: _____ City: _____ State: _____ ZIP: _____

Preferred Phone: (____) _____ Alternate Phone: (____) _____ Email: _____

Caregiver/Alternate Contact Name: _____ Relationship: _____ Phone: (____) _____

Preferred Contact: ☐ Patient ☐ Caregiver Preferred Type: ☐ Phone (Day) ☐ Phone (Evening) ☐ Email Preferred Language: _____

2. INSURANCE INFORMATION — Please attach copies of the front and back of patient's medical and prescription insurance cards. ☐ No Insurance

Primary Insurance Company: _____

Phone: (____) _____

Policy Type: ☐ Medicare ☐ Medicaid ☐ Commercial ☐ Other

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 ☐ No If Yes, Carrier: _____ Phone: (____) _____

Identification #: _____ Policy/Group #: _____

Policyholder Name: _____ Relationship: _____ DOB: ____/____/____

3. DIAGNOSIS

☐ Ornithine transcarbamylase deficiency/OTC (E72.4) ☐ Argininosuccinate lyase deficiency/ASL (E72.22) ☐ Argininemia/ARG (E72.21)

☐ Carbamoyl phosphate synthetase/CPS (E72.29) ☐ Citrullinemia/ASSD (E72.23)

☐ Hyperammonemia-hyperornithinemia-homocitrullinuria syndrome/HHH (E72.4) ☐ Disorder of urea cycle metabolism, unspecified (E72.20)

☐ Other diagnosis, ICD-10 _____ Please visit www.icd10data.com/Convert/270.6 for more information.

Current Nitrogen Scavenger: ☐ Sodium phenylbutyrate ☐ Sodium benzoate ☐ Sodium phenylbutyrate and sodium benzoate ☐ No nitrogen scavenger

4. PRESCRIPTION INFORMATION (ALL FIELDS REQUIRED TO BE CONSIDERED COMPLETE)

☐ RAVICTI® (glycerol phenylbutyrate) Oral Liquid, 1.1 g/mL ☐ BUPHENYL® (sodium phenylbutyrate) Tablets, 500 mg ☐ BUPHENYL® (sodium phenylbutyrate) Powder, 250 g/bottle

Patient Weight: _____ ☐ lb ☐ kg (check one) Patient Height: _____ ☐ in ☐ cm (check one)

Dose: _____ Doses per Day: _____ Total Daily Dose: _____ Days' Supply: _____

Total Quantity: _____ # Refills: _____ Instructions: _____

5. PRESCRIBER INFORMATION

Preferred Method of Contact ☐ Email ☐ Phone

First and Last Name: _____ Credentials: _____

NPI #: _____ State License #: _____ State Issued: _____ Tax ID: _____ Specialty: _____

Practice/Facility Name: _____ Primary Contact Name: _____

Address: _____ City: _____ State: _____ ZIP: _____

Phone: (____) _____ Fax: (____) _____ Primary Office Contact Email: _____

Prescriber Certification: 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 Therapeutics USA, Inc. and its affiliates and their respective employees or agents (collectively, "Horizon") will use this information to administer the Horizon By Your Side program (the "Program"), which provides a wide array of patient-focused services, including providing logistical and non-medical treatment support and assistance in initiating or continuing Horizon UCD medicines as prescribed, and educating about the insurance process. By my signature, I also certify that (1) my patient or his/her personal representative has provided a signed HIPAA authorization that allows me to share protected health information with Horizon for purposes of the Program and (2) I have obtained the patient's authorization to release such information as may be required for AllCare Plus Pharmacy and other entities (or another party acting on behalf of Horizon) to assess insurance coverage for Horizon urea cycle disorder (UCD) medicines and assistance in initiating or continuing Horizon urea cycle disorder (UCD) medicines as prescribed. I appoint the Program, on my behalf, to proceed with services offered and to convey this prescription by facsimile only 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 urea cycle disorder (UCD) medicines or any other Horizon product or service, for any other person; (b) my decision to prescribe Horizon urea cycle disorder (UCD) medicines 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. The completion and submission of coverage- or reimbursement-related documentation are the responsibility of the patient and healthcare provider. Horizon makes no representation or guarantee concerning coverage or reimbursement for any item or service.

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.

By filling out and signing this form, the enrollment process in Horizon By Your Side has initiated; however, your patient must sign a Patient Authorization to complete enrollment in Horizon By Your Side. Please note that your patient will not benefit from the services and support offered by the Program unless your patient signs a Patient Authorization, consenting to receiving such services. If your patient does not sign the Patient Authorization contained within this form, Horizon will contact the patient to determine whether the patient is interested in signing a separate Patient Authorization.

X Prescriber Signature _____ Date _____

Written or e-signature only; stamps not acceptable.

(Dispense as Written) _____ (Substitution Permitted)

Please see the Important Safety Information for RAVICTI on page 4 and the Full Prescribing Information and Medication Guide available at RAVICTI.com. Please see the Important Safety Information for BUPHENYL on page 5, and visit horizontherapeutics.com/medicines/rare-diseases to download a copy of the BUPHENYL Full Prescribing Information and Patient Package Insert.

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Fax: 1-877-695-8304
HorizonByYourSide.com

Patient Consent for Patient Information, Enrolling in Services, and Accessing Financial Support (referred to as "Patient 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 Therapeutics 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 By Your Side") for the following purposes: (1) to establish eligibility for benefits; (2) to communicate with my healthcare providers and me about my treatment or condition and related products; (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 By Your Side 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 By Your Side for determination); and (6) to send me marketing information or offer me products and services related to my treatment or condition (or other 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 By Your Side otherwise as required or permitted by law. Further, I appoint the Program, on my behalf, to proceed with Program services and to convey this prescription to the dispensing pharmacy, to the extent permitted under state 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 By Your Side, 1 Horizon Way, Deerfield, IL 60015, 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: _____

INDICATION and IMPORTANT SAFETY INFORMATION

INDICATION

RAVICTI (glycerol phenylbutyrate) Oral Liquid is indicated for use as a nitrogen-binding agent for chronic management of patients with urea cycle disorders (UCDs) who cannot be managed by dietary protein restriction and/or supplementation alone. RAVICTI must be used with dietary protein restriction and, in some cases, dietary supplements (e.g. essential amino acids, arginine, citrulline, protein-free calorie supplements).

LIMITATIONS OF USE

- RAVICTI is not indicated for the treatment of acute hyperammonemia in patients with UCDs because more rapidly acting interventions are essential to reduce plasma ammonia levels.
- The safety and efficacy of RAVICTI for the treatment of *N*-acetylglutamate synthase (NAGS) deficiency has not been established.

IMPORTANT SAFETY INFORMATION

CONTRAINDICATIONS

- *Patients with known hypersensitivity to phenylbutyrate:* Reactions include wheezing, dyspnea, coughing, hypotension, flushing, nausea, and rash.

WARNINGS AND PRECAUTIONS

- *Neurotoxicity:* Phenylacetate (PAA), the major metabolite of RAVICTI, may be toxic at levels of 500 micrograms/mL or greater. If symptoms of vomiting, nausea, headache, somnolence, or confusion, are present in the absence of high ammonia or other intercurrent illness which explains these symptoms, consider the potential for PAA neurotoxicity which may need reduction in the RAVICTI dosage.
- *Pancreatic Insufficiency or Intestinal Malabsorption:* Low or absent pancreatic enzymes or intestinal disease resulting in fat malabsorption may result in reduced or absent digestion of RAVICTI and/or absorption of phenylbutyrate and reduced control of plasma ammonia. Monitor ammonia levels closely.

ADVERSE REACTIONS

The most common adverse reactions reported in clinical trials (at least 10% of patients) were:

- *Adult patients:* diarrhea, flatulence, and headache occurred during 4-week treatment (n=45) with RAVICTI; nausea, vomiting, diarrhea, decreased appetite, dizziness, headache, and fatigue occurred during 12-month treatment (n=51) with RAVICTI.
- *Pediatric patients ages 2 to 17 years:* upper abdominal pain, rash, nausea, vomiting, diarrhea, decreased appetite, and headache occurred during 12-month treatment (n=26) with RAVICTI.
- *Pediatric patients ages 2 months to less than 2 years:* neutropenia, vomiting, constipation, diarrhea, pyrexia, hypophagia, cough, nasal congestion, rhinorrhea, rash, and papule occurred during 12-month treatment (n=17) with RAVICTI.
- *Pediatric patients less than 2 months of age:* vomiting, rash, gastroesophageal reflux, increased hepatic enzymes, feeding disorder (decreased appetite, hypophagia), anemia, cough, dehydration, metabolic acidosis, thrombocytosis, thrombocytopenia, neutropenia, lymphocytosis, diarrhea, flatulence, constipation, pyrexia, lethargy, and irritability/agitation occurred during 24-month treatment (n=16) with RAVICTI.

DRUG INTERACTIONS

- Corticosteroids, valproic acid, or haloperidol may increase plasma ammonia level. Monitor ammonia levels closely.
- Probenecid may affect renal excretion of metabolites of RAVICTI, including phenylacetylglutamine (PAGN) and PAA.
- CYP3A4 substrates with narrow therapeutic index (eg, alfentanil, quinidine, cyclosporine): RAVICTI may decrease exposure to the concomitant drug.
- Midazolam: Use of RAVICTI decreased exposure of midazolam with concomitant use.

USE IN SPECIFIC POPULATIONS

- *Pregnancy:* RAVICTI should be used with caution in patients who are pregnant or planning to become pregnant. Based on animal data, RAVICTI may cause fetal harm. Report pregnancies to Horizon at 1-866-479-6742.
- *Lactation:* breastfeeding is not recommended during treatment with RAVICTI. There are no data on the presence of RAVICTI in human milk, the effects on the breastfed infant, nor the effects on milk production.

Please see Full Prescribing Information.

INDICATION and IMPORTANT SAFETY INFORMATION

INDICATION

BUPHENYL® (sodium phenylbutyrate) Tablets for oral administration and BUPHENYL® (sodium phenylbutyrate) Powder for oral, nasogastric, or gastrostomy tube administration are indicated as adjunctive therapy in the chronic management of patients with urea cycle disorders (UCDs) involving deficiencies of carbamoyl phosphate synthetase (CPS), ornithine transcarbamylase (OTC), or argininosuccinic acid synthetase (AS).

BUPHENYL is indicated in all patients with neonatal-onset deficiency (complete enzymatic deficiency, presenting within the first 28 days of life). It is also indicated in patients with late-onset disease (partial enzymatic deficiency, presenting after the first month of life) who have a history of hyperammonemic encephalopathy.

BUPHENYL must be used with dietary protein restriction and, in some cases, essential amino acid supplementation.

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

IMPORTANT SAFETY INFORMATION

CONTRAINDICATIONS

- *Acute hyperammonemia:* BUPHENYL should not be used to manage acute hyperammonemia, which is a medical emergency.

WARNINGS AND PRECAUTIONS

BUPHENYL should not be administered to patients with known hypersensitivity to sodium phenylbutyrate or any component of this preparation.

- Use caution with administering BUPHENYL to patients with:
    - Congestive heart failure or severe renal insufficiency, and in clinical states in which there is sodium retention with edema.
    - Hepatic or renal insufficiency or inborn errors of beta oxidation.
- Probenecid may affect renal excretion of the conjugated product of BUPHENYL as well as its metabolite.
- Use of corticosteroids may cause the breakdown of body protein and increase plasma ammonia levels.
- There have been published reports of hyperammonemia being induced by haloperidol and by valproic acid.

ADVERSE REACTIONS

- The most common adverse reactions (≥3%) reported in BUPHENYL clinical trials were decreased appetite, body odor, bad taste or taste aversion.
- In female patients, amenorrhea/menstrual dysfunction (irregular menstrual cycles) occurred in 23% of the menstruating patients.
- Neurotoxicity was reported in cancer patients receiving intravenous phenylacetate. Manifestations were predominately somnolence, fatigue, and lightheadedness; with less frequent headache, dysgeusia, hypoacusis, disorientation, impaired memory, and exacerbation of a pre-existing neuropathy.
- Laboratory adverse events occurring in >2% of UCD patients by body system were:
    - *Metabolic:* acidosis, alkalosis, hyperchloremia, and hypophosphatemia
    - *Nutritional:* hypoalbuminemia and decreased total protein
    - *Hepatic:* increased alkaline phosphatase and increased liver transaminases
    - *Hematologic:* anemia, leukopenia, leukocytosis, and thrombocytopenia

USE IN SPECIFIC POPULATIONS

- *Pregnancy:* BUPHENYL should be used with caution in patients who are pregnant or planning to become pregnant. Animal reproduction studies have not been conducted with BUPHENYL. It is not known whether BUPHENYL can cause fetal harm when administered to a pregnant woman or can affect reproduction capacity.
- *Lactation:* breastfeeding is not recommended during treatment with BUPHENYL. There are no data on the presence of BUPHENYL in human milk.

Please see Full Prescribing Information.