Process to enroll patients in Horizon By Your Side

for RAVICTI® (glycerol phenylbutyrate) Oral Liquid or
BUPHENYL® (sodium phenylbutyrate) Tablets and Powder

1. Fill out all required fields to ensure a thorough benefits investigation.

2. Complete the prescriber signature and date within the Patient Enrollment Form. Make sure your patient or their legally authorized representative has completed, signed, and dated the Patient Authorization Form for Horizon By Your Side, a patient support program.

3. Send both the front and back of the patient's insurance card and both completed forms to Horizon By Your Side.

Please see Important Safety Information for RAVICTI Oral Liquid at the end of this guide, and click here for Full Prescribing Information for RAVICTI.
Please see Important Safety Information for BUPHENYL Tablets and Powder at the end of this guide, and click here for Full Prescribing Information for BUPHENYL.

HORIZON®

1 PATIENT INFORMATION

Fill out all patient information.

- Required fields are needed to conduct a benefits investigation, to contact the patient for any follow-up, and to provide support from Horizon By Your Side.
- Alternate contact information is optional.
  - — It may be helpful to include a caregiver's contact information here.

2 INSURANCE INFORMATION

Provide the patient's primary insurance information.

- Select the "No Insurance" box if the patient does not have any insurance.
- Include secondary insurance plan information, if applicable.

Please include copies of both sides of your patient's insurance card(s), if available, along with the completed Patient Enrollment Form.

- If not available, or if the patient is uninsured, you may attach the electronic medical record demographics page as an alternative to the image of the cards.

3 DIAGNOSIS

Provide the diagnosis code.

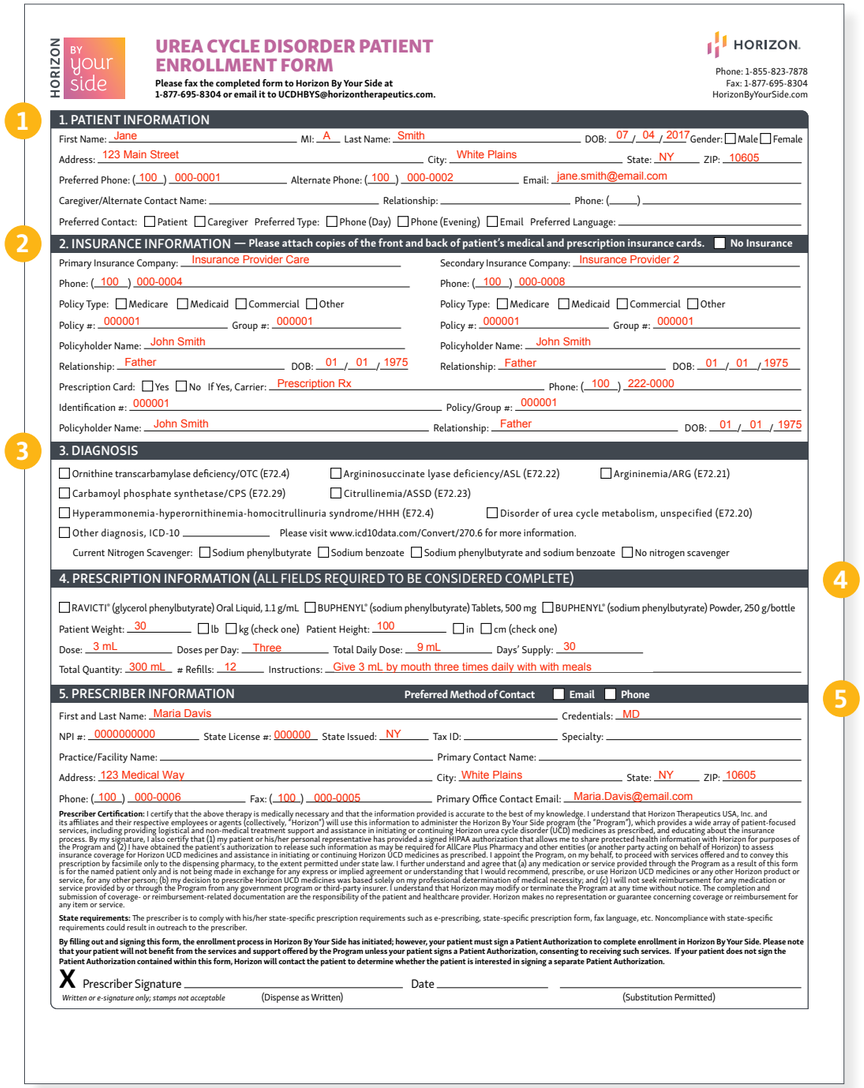
- If there is no box, select "Other ICD-10 code" and note the primary diagnosis code.
- Select the patient's current nitrogen scavenger or that the patient is not taking one.

Ensure that you submit **pages 1 and 2 of the Patient Enrollment Form**, along with copies of **both sides of the patient's insurance card(s).** Retain a copy of this form in the patient's records.

Please contact the team at Horizon By Your Side with any questions about completing this form.

HORIZON BY your side

1-855-823-7878
Monday to Friday, 9 AM to 8 PM (EST)



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: Jane MI: A Last Name: Smith DOB: 07 / 04 / 2017 Gender: ☐ Male ☐ Female
Address: 123 Main Street City: White Plains State: NY ZIP: 10605
Preferred Phone: (100) 000-0001 Alternate Phone: (100) 000-0002 Email: jane.smith@email.com
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: Insurance Provider Care
Phone: (100) 000-0004
Policy Type: ☐ Medicare ☐ Medicaid ☐ Commercial ☐ Other
Policy #: 000001 Group #: 000001
Policyholder Name: John Smith
Relationship: Father DOB: 01 / 01 / 1975
Prescription Card: ☐ Yes ☐ No If Yes, Carrier: Prescription Rx Phone: (100) 222-0000
Identification #: 000001 Policy/Group #: 000001
Policyholder Name: John Smith Relationship: Father DOB: 01 / 01 / 1975

Secondary Insurance Company: Insurance Provider 2
Phone: (100) 000-0008
Policy Type: ☐ Medicare ☐ Medicaid ☐ Commercial ☐ Other
Policy #: 000001 Group #: 000001
Policyholder Name: John Smith
Relationship: Father DOB: 01 / 01 / 1975

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: 30 ☐ lb ☐ kg (check one) Patient Height: 100 ☐ in ☐ cm (check one)
Dose: 3 mL Doses per Day: Three Total Daily Dose: 9 mL Days' Supply: 30
Total Quantity: 300 mL # Refills: 12 Instructions: Give 3 mL by mouth three times daily with with meals

5. PRESCRIBER INFORMATION Preferred Method of Contact ☐ Email ☐ Phone

First and Last Name: Maria Davis Credentials: MD
NPI #: 0000000000 State License #: 000000 State Issued: NY Tax ID: Specialty:
Practice/Facility Name: Primary Contact Name:
Address: 123 Medical Way City: White Plains State: NY ZIP: 10605
Phone: (100) 000-0006 Fax: (100) 000-0005 Primary Office Contact Email: Maria.Davis@email.com

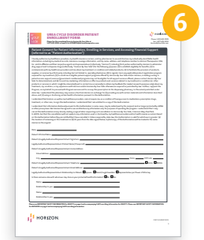
Prescriber Certification: I certify that the above therapy is medically necessary and that the information provided is accurate to the best of my knowledge. I understand that Horizon Therapeutics USA, Inc. and its affiliates and their respective employees or agents (collectively, "Horizon") will use 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 urea cycle disorder (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 UCD medicines and assistance in initiating or continuing Horizon 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 UCD medicines or any other Horizon product or service, for any other person; (b) my decision to prescribe Horizon 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)

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



4 PRESCRIPTION INFORMATION

All prescription fields must be fully completed. Incomplete information may result in delays at specialty pharmacies and additional outreach for prescription clarification.

- Reference the select RAVICTI or BUPHENYL dosing instructions included in the Patient Enrollment Form or the Full Prescribing Information for RAVICTI at RAVICTIhcp.com and BUPHENYL at HorizonByYourSide.com for complete dosing information.

5 PRESCRIBER INFORMATION

Fill out all prescriber information, including prescriber name, contact information, and NPI number.

- Include the office contact name, phone number, and email address.
- Review, sign, and date the prescriber certification. In signing, you are indicating that RAVICTI or BUPHENYL should be dispensed as written. If a substitution is allowed, it should be noted.
  - — Must be a written or e-signature, stamps are not accepted.

6 PATIENT CONSENT

The Patient Authorization Form is located on the second page of the Patient Enrollment Form.

- A patient or patient's legally authorized representative signature is required for the team at Horizon By Your Side to provide non-medical logistical support to the patient.
- If the patient/legally authorized representative is not available to sign the form at your office, the Horizon By Your Side team can follow up to obtain HIPAA consent.

Submit the Patient Enrollment Form using one of the methods below:

EMAIL
UCDHBYS@horizontherapeutics.com

FAX
1-877-695-8304

INDICATION and IMPORTANT SAFETY INFORMATION FOR RAVICTI (GLYCEROL PHENYLBUTYRATE) ORAL LIQUID

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.

HORIZON®

INDICATION and IMPORTANT SAFETY INFORMATION FOR BUPHENYL (SODIUM PHENYLBUTYRATE) TABLETS AND POWDER

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
  - H*ematologic:* anemia, leukopenia, leukocytosis, and thrombocytopenia

USE IN SPECIAL 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.

HORIZON®