RECORDATI RARE DISEASES Inc.
100 CORPORATE DRIVE LEBANON,
NJ 08833 TEL. +1 (908) 236-0888
FAX +1 (908) 236-0028

Dear Healthcare Provider,

Recordati Rare Diseases Inc. has developed a:

CARBAGLU® (carglumic acid) tablets for oral suspension 200mg: Letter of Appeal EXEMPLAR

In an effort to support your patients and make the Appeal process as smooth as possible, we have developed a Letter of Appeal exemplar for CARBAGLU.

The appeals process with most insurance plans often requires the submission of a Letter of Appeal. The purpose of this exemplar letter is to assist your office in developing a customized Letter of Appeal, which addresses the reasons why coverage for CARBAGLU was denied, as well as outline the medical justification for CARBAGLU therapy.

Please note that this letter exemplar should only be used as a guide; however, it is suggested that your Letter of Appeal include:

1. The reason(s) CARBAGLU therapy was denied,
2. Response or rebuttal to each reason CARBAGLU was denied, and
3. Supporting documentation (such as lab results) justifying the medical need for CARBAGLU.

As you know, each patient will have their own unique and specific reasons requiring CARBAGLU therapy. In addition, each insurance plan may have their own rules and guidelines for approving CARBAGLU coverage.

For full Prescribing Information and Instructions for Use, please go to www.CarbagluPrescribingInfo.com.

Sincerely,
The Carbaglu Team
Phone: (888) 454-8860
Fax: (888) 454-8488

PP-CBGL-US-0327

ON OFFICE LETTERHEAD INLCUDING PROVIDER NAME AND ADDRESS

Name

Address

Phone

Fax

CARBAGLU® (carglumic acid) tablets for oral suspension 200mg

Letter of Appeal

EXEMPLAR

(Date)

(Payer Name)

(Payer Address)

Patient Name: (Patient Name)

Patient Date of Birth: (Patient DOB)

Policy Number: (Policy Number)

Group Number: (Group Number)

Case Number: (Case Number)

Subject: Letter of Appeal regarding CARBAGLU (carglumic acid) tablets for oral suspension 200mg

To Whom It May Concern:

I am writing to request an APPEAL of the decision to deny CARBAGLU coverage for my patient (**Patient Name**). (**Patient name**) has been diagnosed with N-acetylglutamate synthase (NAGS) deficiency and requires treatment for chronic hyperammonemia associated with this condition. My patient has taken CARBAGLU for (**X years/months**) and it is important this patient continue to receive CARBAGLU as I prescribed.

CARBAGLU (carglumic acid) tablets for oral suspension 200mg is a carbamoyl phosphate synthetase (CPS1) activator indicated in pediatric and adult patients as maintenance therapy for the treatment of chronic hyperammonemia due to NAGS deficiency.

Our office received a denial for CARBAGLU on (date). In that denial, CARBAGLU was denied due to the following reasons:

1.
2.
3.

I disagree with this decision. In my clinical judgement, treatment with CARBAGLU is medically necessary due to the following reasons (answer each reason why CARBAGLU was denied):

1.
2.
3.

NAGS deficiency:
N-acetylglutamate synthase (NAGS) deficiency is a rare, autosomal recessive urea cycle disorder. NAGS is an essential mitochondrial liver enzyme that produces an essential cofactor, NAG, which triggers activation of the urea cycle. When NAGS is deficient, ammonia fails to convert to urea and accumulates in the blood, resulting in hyperammonemia. Rapid accumulation of ammonia and other precursor metabolites may result in cerebral edema with severe neurological complications.

Treatment Plan:
CARBAGLU acts as a CPS1 activator, improves or restores the function of the urea cycle, and facilitates ammonia detoxification and urea production, enabling the urea cycle to function as it should to maintain normal blood ammonia levels. My intended use of CARBAGLU is (**ADD DOSE HERE**).
Please note that according to the CARBAGLU Prescribing Information, the efficacy of CARBAGLU in the treatment of acute and chronic hyperammonemia due to NAGS deficiency was evaluated in a retrospective case series. A subset of 13 patients was selected based on documented plasma ammonia levels prior to and after long-term treatment. All 13 patients had increased plasma ammonia levels at baseline (overall mean baseline plasma ammonia level was 271 micromol/L). Study results showed normal plasma ammonia levels were maintained with long-term treatment (median duration of 7.9 years). Most common adverse reactions (≥13%) are vomiting, abdominal pain, pyrexia, tonsillitis, anemia, diarrhea, ear infection, infections, nasopharyngitis, hemoglobin decreased, and headache.

I would appreciate your reconsideration of this denial and ask that you reverse your decision and approve CARBAGLU for (**patient name**).

If you have any questions or wish to conduct a Peer-to-Peer discussion, feel free to contact me at (**enter phone number**).

Thank you for your time and consideration!
(**First and Last name, MD**)