

Policy: MBP 124.0

Section: Medical Benefit Pharmaceutical Policy

Subject: Ruconest (C1 esterase inhibitor, recombinant)

I. Policy:

Ruconest (C1 esterase inhibitor, recombinant)

II. Purpose/Objective:

To provide a policy of coverage regarding Ruconest (C1 esterase inhibitor, recombinant)

III. Responsibility:

- A. Medical Directors
- B. Medical Management
- C. Pharmacy Department

IV. Required Definitions

1. Attachment – a supporting document that is developed and maintained by the policy writer or department requiring/authoring the policy.
2. Exhibit – a supporting document developed and maintained in a department other than
3. the department requiring/authoring the policy.
4. Devised – the date the policy was implemented.
5. Revised – the date of every revision to the policy, including typographical and grammatical changes.
6. Reviewed – the date documenting the annual review if the policy has no revisions necessary.

V. Additional Definitions

Medical Necessity or Medically Necessary means Covered Services rendered by a Health Care Provider that the Plan determines are:

- a. appropriate for the symptoms and diagnosis or treatment of the Member's condition, illness, disease or injury;
- b. provided for the diagnosis and the direct care and treatment of the Member's condition, illness disease or injury;
- c. in accordance with current standards good medical treatment practiced by the general medical community;
- d. not primarily for the convenience of the Member, or the Member's Health Care Provider; and
- e. the most appropriate source or level of service that can safely be provided to the Member. When applied to hospitalization, this further means that the Member requires acute care as an inpatient due to the nature of the services rendered or the Member's condition, and the Member cannot receive safe or adequate care as an outpatient

Medicaid Business Segment

Medically Necessary — A service, item, procedure, or level of care compensable under the Medical Assistance program that is necessary for the proper treatment or management of an illness, injury, or disability is one that:

- i. Will, or is reasonably expected to, prevent the onset of an illness, condition, injury or disability.
- ii. Will, or is reasonably expected to, reduce or ameliorate the physical, mental or developmental effects of an illness, condition, injury or disability.
- iii. Will assist the Member to achieve or maintain maximum functional capacity in performing daily activities, taking into account both the functional capacity of the Member and those functional capacities that are appropriate for Members of the same age.

DESCRIPTION:

Ruconest (C1 esterase inhibitor, recombinant) is indicated for the treatment of acute hereditary angioedema (HAE) attacks in adults and adolescent patients. Ruconest is a recombinant C1 esterase inhibitor (rhC1INH) which irreversibly binds target proteases (activated C1s, kallikrein, factor XIIa, and factor XIa) of the contact and complement systems.

CRITERIA FOR USE: Requires Prior Authorization by Medical Director or Designee

GRANDFATHER PROVISION – Members already established on therapy are eligible for approval as long as there is medical record documentation that the safety and effectiveness of use for the prescribed indication is supported by Food and Drug Administration (FDA) approval or adequate medical and scientific evidence in the medical literature

Ruconest (C1 esterase inhibitor, recombinant) will be considered medically necessary for the commercial, exchange and CHIP lines of business when all of the following criteria are met:

- Patient must be 13 years of age or older **AND**
 - Must be prescribed by an allergist, immunologist, hematologist, or dermatologist **AND**
 - Medication is being used for the treatment of an acute attack of hereditary angioedema **AND**
 - Must not be a laryngeal edema attack or have a history of laryngeal attacks **AND**
 - Not used in combination with other approved treatments for acute HAE attacks **AND**
 - Medical record documentation of diagnosis of hereditary angioedema supported by physician documentation of:
 - Recurrent, self-limiting non-inflammatory subcutaneous angioedema without urticarial, lasting more than 12 hours **OR**
 - Recurrent, self-remitting abdominal pain lasting more than 6 hours, without clear organic etiology **AND**
 - The presence of specific abnormalities in complement proteins, in the setting of a suggestive clinical history or episodic angioedema without urticaria; supported by
 - Medical record documentation of 2 or more sets of complement studies, separated by one month or more, showing consistent results of
 - Low C4 levels **AND**
 - Less than 50% of the lower limit of normal C1-INH antigenic protein levels **OR**
 - Less than 50% of the lower limit of normal C1-INH function levels
- AND**
- Medical record documentation of no allergy (as confirmed by IgE antibodies) to rabbits or rabbit derived products **AND**
 - Physician provided documentation of concurrent or failure on, intolerance to, or contraindication to prophylactic therapy (danazol).*

*Only applies to patients with more than one severe episode of angioedema per month, or those with a history of laryngeal attacks

AUTHORIZATION DURATION: Initial approval will be for 6 months or less if the reviewing provider feels it is medically appropriate. Subsequent approvals will be for an additional 6 months or less if the reviewing provider feels it is medically appropriate and will require medical record documentation of continued disease improvement. The medication will no longer be covered if the patient experiences toxicity or worsening of disease.

QUANTITY LIMIT: 4 doses per 30 days. Quantities requested above limits will be reviewed for necessity. Exceptions will be allowed for patients maximizing prophylactic therapies and medical record documentation of acute attacks requiring more than 4 doses monthly

Ruconest (C1 esterase inhibitor, recombinant) will be considered medically necessary for the Medicare line of business when all of the following criteria are met:

- Patient must be 13 years of age or older **AND**
- Must be prescribed by an allergist, immunologist, hematologist, or dermatologist **AND**
- Medication is being used for the treatment of an acute attack of hereditary angioedema **AND**
- Must not be a laryngeal edema attack or have a history of laryngeal attacks **AND**
- Not used in combination with other approved treatments for acute HAE attacks **AND**
- Medical record documentation of diagnosis of hereditary angioedema supported by physician documentation of:
 - Recurrent, self-limiting non-inflammatory subcutaneous angioedema without urticarial, lasting more than 12 hours **OR**
 - Recurrent, self-remitting abdominal pain lasting more than 6 hours, without clear organic etiology **AND**

- The presence of specific abnormalities in complement proteins, in the setting of a suggestive clinical history or episodic angioedema without urticaria; supported by
 - Medical record documentation of 2 or more sets of complement studies, separated by one month or more, showing consistent results of
 - Low C4 levels **AND**
 - Less than 50% of the lower limit of normal C1-INH antigenic protein levels **OR**
 - Less than 50% of the lower limit of normal C1-INH function levels

AND

- Medical record documentation of no allergy (as confirmed by IgE antibodies) to rabbits or rabbit derived products

AUTHORIZATION DURATION: Initial approval will be for 6 months or less if the reviewing provider feels it is medically appropriate. Subsequent approvals will be for an additional 6 months or less if the reviewing provider feels it is medically appropriate and will require medical record documentation of continued disease improvement. The medication will no longer be covered if the patient experiences toxicity or worsening of disease.

QUANTITY LIMIT: 4 doses per 30 days. Quantities requested above limits will be reviewed for necessity. Exceptions will be allowed for patients maximizing prophylactic therapies and medical record documentation of acute attacks requiring more than 4 doses monthly

Angiodema disorder	C4*	C1 inhibitor level	C1 inhibitor function	C1q	C3	Other tests (not routinely needed for diagnosis)
HAE I	Low	Low	Low (usually <50 percent of normal)	Normal	Normal	Genetic testing
HAE II	Low	Normal or elevated	Low (usually <50 percent of normal)	Normal	Normal	Genetic testing
HAE III	Normal	Normal	Normal	Normal	Normal	Mutations in gene for factor XII detected in some patients
AAE	Low	Normal or low	Low (usually <50 percent of normal)	Normal or low*	Normal or low	Anti-C1 inhibitor antibodies
Idiopathic angioedema	Normal	Normal	Normal	Normal	Normal	

LIMITATIONS (For all lines of business):

Effectiveness was not established in patients having HAE with laryngeal attacks.

LINE OF BUSINESS:

Eligibility and contract specific benefit limitations and/or exclusions will apply. Coverage statements found in the line of business specific benefit document will supersede this policy.

REFERENCES:

1. Ruconest [prescribing information]. Warren, NJ: Pharming Healthcare Inc; April 2020.
2. Busse, PJ, Christiansen SC, Riedl MA, et al. US HAEA Medical Advisory Board 2020 Guidelines for the Management of Hereditary Angioedema. US Hereditary Angioedema Association. Journal of Allergy clinical immunology Practice; 2020; 9(1):132-150 [cited 2023 Dec 27]. Available from: https://www.haea.org/pages/p/treatment_guidelines

This policy will be revised as necessary and reviewed no less than annually.

Devised: 1/20/15

Revised: 3/24/15 (criteria), 3/29/19 (grandfather), 1/17/23 (LOB carve out), 12/30/23 (references added), 1/9/24 (Medicaid business segment)

Reviewed: 3/31/16, 3/30/17, 3/29/18, 2/28/19, 2/1/20, 1/19/21, 1/18/22

MA UM Committee approval: 12/31/23