RxAdvance

Clinical Policy Title:	pegvaliase-pqpz
Policy Number:	RxA.247
Drug(s) Applied:	Palynziq®
Original Policy Date:	02/07/2020
Last Review Date:	8/28/2024
Line of Business Policy Applies to:	All lines of business (except Medicare)

Criteria

I. Initial Approval Criteria

A. Phenylketonuria (must meet all):

1. Diagnosis of phenylketonuria;
2. Recent (within 90 days) phenylalanine blood level > 600 µmols/L;
3. Member is currently on a phenylalanine-restricted diet and will continue this diet during treatment;
4. Palynziq is not prescribed concurrently with Kuvan.

Approval Duration

All Lines of Business (except Medicare): 12 months

II. Continued Therapy Approval

A. Phenylketonuria (must meet all):

1. Member is currently receiving or has been treated with this medication within the past 120 days, excluding manufacturer samples;
2. Member is currently on a phenylalanine-restricted diet and will continue this diet during treatment;
3. Member meets one of the following (a, b, or c):
   a. Blood Phe level is ≤ 600 µmols/L;
   b. Request is for 40 mg per day and member has previously used 20 mg per day continuously for at least 6 months without achieving blood Phe control;
   c. Request is for 60 mg per day and member meets both of the following (i and ii):
      i. Member has previously used 40 mg per day continuously for at least 16 weeks without achieving blood Phe control;
      ii. Member has not used 60 mg per day continuously for more than 16 weeks without achieving blood Phe control.

Approval Duration

All Lines of Business (except Medicare): 12 months

References

1. Thomas J, Levy H, et al. Pegvaliase for the treatment of phenylketonuria: results of a long-term phase 3 clinical trial program (PRISM). Molecular Genetics and Metabolism. 2018; 124:27-38. Available at: https://pubmed.ncbi.nlm.nih.gov/29653686/. Accessed August 28, 2024.

Review/Revision History	Review/Revised Date	P&T Approval Date

Policy established.	01/2020	02/07/2020
Policy was reviewed. 1. Clinical policy table was updated. 2. Continued Therapy criteria II.A.1 was rephrased to "Currently receiving medication that has been authorized by RxAdvance..." 3. Reference reviewed and updated.	06/28/2020	09/14/2020
Policy was reviewed. 1. Initial Approval Criteria I.A.6 was updated from “20 mg per day” to “40 mg per day”. 2. Continued Therapy Approval Criteria II.A.1 was rephrased to "Member is currently receiving medication that has been authorized by RxAdvance...". 3. Continued Therapy Criteria II.A.2.c was updated from “Member has been using 20 mg per day for at least 6 months, but a dose titration to 40 mg per day is being…” to “The member has not responded to Palynziq® at a dose of 20 mg/day for 24 weeks followed by 40 mg/day for 16 weeks.”. 4. References were reviewed and updated.	07/13/2021	9/14/2021
Policy was reviewed: 1. Initial Approval Criteria I.A.6:Updated from Dose does not exceed 40 mg per day to Dose does not exceed 60 mg per day. 2. References were reviewed and updated.	02/04/2022	04/18/2022
Policy was reviewed: 1. Initial Approval Criteria, I.A.5: Updated to include new criteria pertaining to indication Phenylketonuria, Member is currently on a phenylalanine-restricted diet and will continue this diet during treatment with Palynziq®. 2. Continued Therapy Approval, II.A.2: Updated to include new criteria pertaining to indication Phenylketonuria, Member is currently on a phenylalanine-restricted diet and will continue this diet during treatment with Palynziq®. 3. Continued Therapy Approval, II.A.3.a: Updated to remove prior lab criteria "Blood Phe level has decreased by ≥ 20% from pre-treatment baseline." 4. Continued Therapy Approval, II.A.3.c: The member has not responded to Palynziq® at a	12/29/2022	04/13/2023

dose of 20 mg/day for 24 weeks followed by 40 mg/day for 16 weeks, but a dose titration to 60 mg per day is being requested after failure to meet therapeutic targets (a or b above) [only the 60 mg per day dose will be approved] was replaced with (b or c): b. Request is for 40 mg per day and member has previously used 20 mg per day continuously for at least 6 months without achieving blood Phe control; c. Request is for 60 mg per day and member meets both of the following (i and ii): i. Member has previously used 40 mg per day continuously for at least 16 weeks without achieving blood Phe control; ii. Member has not used 60 mg per day continuously for more than 16 weeks without achieving blood Phe control; 5. References were reviewed and updated.		
Policy was reviewed.	10/19/2023	10/19/2023
Policy was reviewed: 1. Removed age restrictions. 2. Removed prescriber restrictions. 3. Removed dose restrictions. 4. Updated Continued therapy approval with the new verbiage containing 120 days lookback period. 5. Updated approval duration verbiage. 6. References were reviewed and updated.	08/28/2024	09/13/2024