Clinical Policy Title:	sapropterin dihydrochloride
Policy Number:	RxA.787
Drug(s) Applied:	sapropterin dihydrochloride
Original Policy Date:	04/13/2023
Last Review Date:	12/11/2025
Line of Business Policy Applies to:	All lines of business (except Medicare)

Clinical Policy

I. Initial Approval Criteria

A. Phenylketonuria (PKU) (must meet all):

1. Diagnosis of hyperphenylalaninemia due to PKU;
2. Recent (within 90 days) phenylalanine blood level > 600 µmols/L;
3. Member is currently on a phenylalanine-restricted diet and will continue diet during treatment;
4. Not prescribed concurrently with Palynziq®.

Approval Duration

All Lines of Business (except Medicare): 3 months

II. Continued Therapy Approval

A. Phenylketonuria (must meet all):

1. Member is currently receiving medication in the past 120 days that has been authorized by RxAdvance or the member has met initial approval criteria.
2. Member is responding positively to therapy as demonstrated by a reduction in Phe blood levels since initiation of therapy;
3. Member is currently on a phenylalanine-restricted diet and will continue this diet during treatment.

Approval Duration

All Lines of Business (except Medicare): 12 months

References

1. Sapropterin dihydrochloride. Prescribing Information. Malvern, PA: Endo USA; July 2024. Available at: https://dailymed.nlm.nih.gov/dailymed/fda/fdaDrugXsl.cfm?setid=eafec3a2-4f29-4afa-8728-a62b34cefd18&type=display. Accessed March 24, 2025.
2. Vockly J, Andersson HC, Antshel KM, et al. ACMG practice guidelines: phenylalanine hydroxylase deficiency: diagnosis and management guideline. Genet Med. 2014;16(2):188- 200. Available at: https://pubmed.ncbi.nlm.nih.gov/24385074/. Accessed March 24, 2025.
3. Camp KM, Parisi MA, Acosta PB, et al. Phenylketonuria scientific review conference: state of the science and future research needs. Mol Genet Metab. June 2014;112(2):87-122. Available at: https://pubmed.ncbi.nlm.nih.gov/24667081/. Accessed March 24, 2025.

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

This clinical policy has been developed to authorize, modify, or determine coverage for individuals with similar conditions. Specific care and treatment may vary depending on individual need and benefits covered by the plan. This policy is not intended to dictate to providers how to practice medicine, nor does it constitute a contract or guarantee regarding payment or results. This document may contain prescription brand name drugs that are trademarks of pharmaceutical manufacturers that are not affiliated with RxAdvance.

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Policy established.	01/27/2023	04/13/2023
Policy was reviewed: 1. Removed brand names from drug applied section. 2. Removed background, dosing information, dosage forms, Appendices. 3. Removed prescriber criteria. 4. Removed dosing criteria. 5. Updated approval duration verbiage. 6. Updated continuation of therapy language. 7. References were reviewed and updated.	03/25/2025	04/10/2025
Policy reviewed	12/11/2025	12/11/2025

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