

Clinical Policy Title:	mitapivat
Policy Number:	RxA.755
Drug(s) Applied:	Pyrukynd®
Original Policy Date:	04/18/2022
Last Review Date:	12/11/2025
Line of Business Policy Applies to:	All lines of business (except Medicare)

Criteria

I. Initial Approval Criteria

A. Hemolytic anemia with pyruvate kinase deficiency (PKD) (must meet all):

1. Diagnosis of hemolytic anemia with pyruvate kinase deficiency;
2. Member has at least two mutant alleles in the PKLR gene, of which at least one is a missense variant;
3. Member is not homozygous for the R479H mutation or had two non-missense variants, without the presence of another missense variant, in the PKLR gene;
4. Documentation required for RBC transfusions for hemolytic anemia due to PKD within the previous year;
5. Recent hemoglobin level ≤ 10 g/dL;
6. Prescribed concurrently with oral folic acid.

Approval Duration

All Lines of Business (except Medicare): 3 months

II. Continued Therapy Approval

A. Hemolytic anemia in adults with pyruvate kinase (PK) deficiency (must meet all):

1. Auto-approval based on lookback functionality within the past 120 days as a proxy for member responding positively to therapy.

Approval Duration

All Lines of Business (except Medicare): 12 months

References

1. Al-Samkari, H., & van Beers, E. J. (2021). Mitapivat, a novel pyruvate kinase activator, for the treatment of hereditary hemolytic anemias. Therapeutic advances in hematology. Available at: <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC8721383/#:~:text=Mitapivat%20is%20a%20promising%2C%20first,thalassemia%2C%20and%20sickle%20cell%20disease>. Accessed September 4, 2024.

Review/Revision History	Review/Revision Date	P&T Approval Date
Policy established.	03/14/2022	04/18/2022
1. Initial Approval Criteria I.A.9: Updated to add prescribed concurrently with oral folic acid. 2. References were reviewed and updated.	3/30/2023	04/13/2023

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.

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 auto-approval based on lookback functionality within the past 120 days. 5. Removed reauthorization requirement for positive response to therapy. 6. Updated approval duration verbiage.	08/28/2024	09/13/2024
Policy was reviewed.	12/05/2024	N/A
Policy reviewed.	12/11/2025	12/11/2025