



Harliku Prior Authorization with Quantity Limit Program Summary

POLICY REVIEW CYCLE

Effective Date
01-01-2026

Date of Origin
08-21-2025

POLICY AGENT SUMMARY PRIOR AUTHORIZATION

Target Brand Agent(s)	Target Generic Agent(s)	Strength	Targeted MSC	Available MSC	Final Age Limit	Preferred Status
Harliku	nitisinone (aku) tab	2 MG	M ; N ; O ; Y	N		

POLICY AGENT SUMMARY QUANTITY LIMIT

Target Brand Agent Name(s)	Target Generic Agent Name(s)	Strength	QL Amount	Dose Form	Day Supply	Duration	Addtl QL Info	Allowed Exceptions	Targeted NDCs When Exclusions Exist
Harliku	nitisinone (aku) tab	2 MG	30	Tablets	30	DAYS			

CLIENT SUMMARY – PRIOR AUTHORIZATION

Target Brand Agent Name(s)	Target Generic Agent Name(s)	Strength	Client Formulary
Harliku	nitisinone (aku) tab	2 MG	IL HMO Performance Annual ; Balanced ; Balanced Biosimilar ; Basic ; Basic Annual ; Enhanced ; Enhanced Annual ; HIM Annual 2024 ; HIM Quarterly 2024 ; HIM Quarterly 2025 ; HIM Quarterly 2026 ; IL HIM Annual 2025 ; IL HIM Annual 2026 ; IL non-HMO Performance Full ; Jade ; MT Performance Full ; OK Performance Full ; Performance ; Performance Annual ; Performance Biosimilar ; Performance Select ; Performance Select Biosimilar ; TX HIM Annual 2025 ; TX HIM Annual 2026 ; Topaz ; Whole Foods

CLIENT SUMMARY – QUANTITY LIMITS

Target Brand Agent Name(s)	Target Generic Agent Name(s)	Strength	Client Formulary
Harliku	nitisinone (aku) tab	2 MG	IL HMO Performance Annual ; Balanced ; Balanced Biosimilar ; Basic ; Basic Annual ; Enhanced ; Enhanced Annual ; HIM Annual 2024 ; HIM Quarterly 2024 ; HIM Quarterly 2025 ; HIM Quarterly 2026 ; IL HIM Annual 2025 ; IL HIM Annual 2026 ; IL non-HMO Performance Full ; Jade ; MT Performance Full ; NM HIM Annual 2024 ; NM HIM Annual 2025 ; NM HIM Annual 2026 ; NM Performance Full ; OK Performance Full ; Performance ; Performance Annual ; Performance Biosimilar ; Performance Select ; Performance Select Biosimilar ; TX HIM Annual 2025 ; TX HIM Annual 2026 ; Topaz

PRIOR AUTHORIZATION CLINICAL CRITERIA FOR APPROVAL

Module	Clinical Criteria for Approval
PA	<p>Initial Evaluation</p> <p>Target Agent(s) will be approved when ALL of the following are met:</p> <ol style="list-style-type: none"> 1. The patient has a diagnosis of alkaptonuria AND 2. The patient has greater than or equal to 1 gram of homogentisic acid (HGA) in a 24-hour urine sample (medical records required) AND 3. If the patient has an FDA labeled indication, then ONE of the following: <ol style="list-style-type: none"> A. The patient’s age is within FDA labeling for the requested indication for the requested agent OR B. There is support for using the requested agent for the patient’s age for the requested indication AND 4. ONE of the following: <ol style="list-style-type: none"> A. The request is for a BCBS IL Fully Insured, ASO Cost/BBF, HIM, or Non-ERISA ASO/Self-insured Municipalities/Counties member OR B. The patient is currently being treated with the requested agent and the patient is currently stable on the requested agent [chart notes required] OR C. The patient has tried and had an inadequate response to TWO prerequisite agents (i.e., nitisinone capsule, Nityr tablet) that is NOT expected to occur with the requested agent [chart notes required] OR D. TWO prerequisite agents (i.e., nitisinone capsule, Nityr tablet) were discontinued due to lack of efficacy or effectiveness, diminished effect, or an adverse event [chart notes required] OR E. The patient has an intolerance or hypersensitivity to TWO prerequisite agents (i.e., nitisinone capsule, Nityr tablet) that is NOT expected to occur with the requested agent [chart notes required] OR F. The patient has an FDA labeled contraindication to ALL prerequisite agents that is NOT expected to occur with the requested agent [chart notes required] OR G. TWO prerequisite agents (i.e., nitisinone capsule, Nityr tablet) are expected to be ineffective based on the known clinical characteristics of the patient and the known characteristics of the prescription drug; OR cause a significant barrier to the patient’s adherence of care; OR worsen a comorbid condition; OR decrease the patient’s ability to achieve or maintain reasonable functional ability in

Module	Clinical Criteria for Approval
	<p>performing daily activities; OR cause an adverse reaction or cause physical or mental harm [chart notes required] OR</p> <p>H. TWO prerequisite agents (i.e., nitisinone capsule, Nityr tablet) are not in the best interest of the patient based on medical necessity [chart notes required] OR</p> <p>I. The patient has tried another prescription drug in the same pharmacologic class or with the same mechanism of action as TWO prerequisite agents (i.e., nitisinone capsule, Nityr tablet) and that prescription drug was discontinued due to lack of efficacy or effectiveness, diminished effect, or an adverse event [chart notes required] AND</p> <p>5. The prescriber is a specialist in the area of the patient’s diagnosis (e.g., geneticist, rheumatologist), or the prescriber has consulted with a specialist in the area of the patient’s diagnosis AND</p> <p>6. The patient does NOT have any FDA labeled contraindications to the requested agent</p> <p>Length of Approval:</p> <p>BCBSOK: 36 months</p> <p>BCBSIL and BCBSMT: 12 months</p> <p>ALL other plans: 6 months</p> <p>NOTE: If Quantity Limit applies, please refer to Quantity Limit Criteria.</p> <p>The requested agent will also be approved when ALL of the following are met:</p> <ol style="list-style-type: none"> 1. The member resides in Ohio AND 2. The plan is Fully Insured or HIM Shop (SG) AND 3. The patient does NOT have any FDA labeled contraindications to the requested agent AND 4. ONE of the following: <ol style="list-style-type: none"> A. The patient has another FDA labeled indication for the requested agent and route of administration OR B. The patient has another indication that is supported in compendia for the requested agent and route of administration OR C. The prescriber has submitted TWO articles from major peer-reviewed professional medical journals [e.g., Journal of American Medical Association (JAMA), New England Journal of Medicine (NEJM), Lancet] supporting the proposed use(s) as generally safe and effective. Accepted study designs may include, but are not limited to, randomized, double blind, placebo controlled clinical trials. NOTE: Case studies are not acceptable [journal articles required] <p>Non-oncology compendia allowed: DrugDex level 1, 2A or 2B, AHFS-DI (narrative text must be supportive)</p> <p>Oncology compendia allowed: NCCN 1 or 2A, AHFS-DI (narrative text must be supportive), DrugDex level 1, 2A, or 2B, or Clinical Pharmacology (narrative text must be supportive), Lexi-Drugs evidence level A, peer-reviewed medical literature</p> <p>Length of Approval:</p> <p>BCBSOK: 36 months</p> <p>ALL other plans: 12 months</p>

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	<p>NOTE: If Quantity Limit applies, please refer to Quantity Limit Criteria.</p> <p>Renewal Evaluation</p> <p>Target Agent(s) will be approved when ALL of the following are met:</p> <ol style="list-style-type: none"> 1. The patient has been previously approved for the requested agent through the plan's Prior Authorization process (Note: patients not previously approved for the requested agent will require initial evaluation review) AND 2. The patient has had clinical benefit with the requested agent as indicated by a reduction in urinary homogentisic acid (medical records required) AND 3. The prescriber is a specialist in the area of the patient's diagnosis (e.g., geneticist, rheumatologist), or the prescriber has consulted with a specialist in the area of the patient's diagnosis AND 4. The patient does NOT have any FDA labeled contraindications to the requested agent <p>Length of Approval:</p> <p>BCBSOK: 36 months</p> <p>ALL other plans: 12 months</p> <p>NOTE: If Quantity Limit applies, please refer to Quantity Limit Criteria.</p>

QUANTITY LIMIT CLINICAL CRITERIA FOR APPROVAL

Module	Clinical Criteria for Approval
	<p>Quantity Limit for the Target Agent(s) will be approved when ONE of the following is met:</p> <ol style="list-style-type: none"> 1. The requested quantity (dose) does NOT exceed the program quantity limit OR 2. The requested quantity (dose) exceeds the program quantity limit AND ONE of the following: <ol style="list-style-type: none"> A. BOTH of the following: <ol style="list-style-type: none"> 1. The requested agent does NOT have a maximum FDA labeled dose for the requested indication AND 2. There is support for therapy with a higher dose for the requested indication OR B. BOTH of the following: <ol style="list-style-type: none"> 1. The requested quantity (dose) does NOT exceed the maximum FDA labeled dose for the requested indication AND 2. There is support for why the requested quantity (dose) cannot be achieved with a lower quantity of a higher strength that does NOT exceed the program quantity limit <p>Length of Approval:</p> <p>BCBSIL: 12 months</p> <p>ALL other plans: Initial 6 months; Renewal 12 months</p>