

# Niemann-Pick Disease Type C Agents Prior Authorization with Quantity Limit Program Summary

## POLICY REVIEW CYCLE

**Effective Date**  
02-01-2026

**Date of Origin**

## POLICY AGENT SUMMARY PRIOR AUTHORIZATION

Target Brand Agent(s)	Target Generic Agent(s)	Strength	Targeted MSC	Available MSC	Final Age Limit	Preferred Status
Miplyffa	arimoclolomol citrate cap	124 MG ; 47 MG ; 62 MG ; 93 MG	M ; N ; O ; Y	N		
Aqneursa	levacetylleucine for susp packet	1 GM	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
Aqneursa	levacetylleucine for susp packet	1 GM	120	Packets	30	DAYS			
Miplyffa	arimoclolomol citrate cap	47 MG	90	Capsules	30	DAYS			
Miplyffa	arimoclolomol citrate cap	62 MG	90	Capsules	30	DAYS			
Miplyffa	arimoclolomol citrate cap	93 MG	90	Capsules	30	DAYS			
Miplyffa	arimoclolomol citrate cap	124 MG	90	Capsules	30	DAYS			

## CLIENT SUMMARY – PRIOR AUTHORIZATION

Target Brand Agent Name(s)	Target Generic Agent Name(s)	Strength	Client Formulary
Aqneursa	levacetylleucine for susp packet	1 GM	Balanced ; Balanced Biosimilar ; Basic ; Basic Annual ; Enhanced ; Enhanced Annual ; HIM Quarterly 2024 ; HIM Quarterly 2025 ; HIM Quarterly 2026 ; IL HIM Annual 2025 ; IL HIM Annual 2026 ; IL HMO Performance Quarterly ; IL non-HMO Performance Full ; Jade ; MT Performance Full ; NM HIM Annual 2025

Target Brand Agent Name(s)	Target Generic Agent Name(s)	Strength	Client Formulary
			; 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 ; Whole Foods
Miplyffa	arimoclolom citrate cap	124 MG ; 47 MG ; 62 MG ; 93 MG	Balanced ; Balanced Biosimilar ; Basic ; Basic Annual ; Enhanced ; Enhanced Annual ; HIM Quarterly 2024 ; HIM Quarterly 2025 ; HIM Quarterly 2026 ; IL HIM Annual 2025 ; IL HIM Annual 2026 ; IL HMO Performance Quarterly ; IL non-HMO Performance Full ; Jade ; MT Performance Full ; 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 ; Whole Foods

### CLIENT SUMMARY – QUANTITY LIMITS

Target Brand Agent Name(s)	Target Generic Agent Name(s)	Strength	Client Formulary
Aqneursa	levacetylleucine for susp packet	1 GM	Balanced ; Balanced Biosimilar ; Basic ; Basic Annual ; Enhanced ; Enhanced Annual ; HIM Quarterly 2024 ; HIM Quarterly 2025 ; HIM Quarterly 2026 ; IL HIM Annual 2025 ; IL HIM Annual 2026 ; IL HMO Performance Quarterly ; IL non-HMO Performance Full ; Jade ; MT Performance Full ; 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 ; Whole Foods
Miplyffa	arimoclolom citrate cap	62 MG	Balanced ; Balanced Biosimilar ; Basic ; Basic Annual ; Enhanced ; Enhanced Annual ; HIM Quarterly 2024 ; HIM Quarterly 2025 ; HIM Quarterly 2026 ; IL HIM Annual 2025 ; IL HIM Annual 2026 ; IL HMO Performance Quarterly ; IL non-HMO Performance Full ; Jade ; MT Performance

Target Brand Agent Name(s)	Target Generic Agent Name(s)	Strength	Client Formulary
			Full ; 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 ; Whole Foods
Miplyffa	arimoclolomol citrate cap	47 MG	Balanced ; Balanced Biosimilar ; Basic ; Basic Annual ; Enhanced ; Enhanced Annual ; HIM Quarterly 2024 ; HIM Quarterly 2025 ; HIM Quarterly 2026 ; IL HIM Annual 2025 ; IL HIM Annual 2026 ; IL HMO Performance Quarterly ; IL non-HMO Performance Full ; Jade ; MT Performance Full ; 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 ; Whole Foods
Miplyffa	arimoclolomol citrate cap	93 MG	Balanced ; Balanced Biosimilar ; Basic ; Basic Annual ; Enhanced ; Enhanced Annual ; HIM Quarterly 2024 ; HIM Quarterly 2025 ; HIM Quarterly 2026 ; IL HIM Annual 2025 ; IL HIM Annual 2026 ; IL HMO Performance Quarterly ; IL non-HMO Performance Full ; Jade ; MT Performance Full ; 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 ; Whole Foods
Miplyffa	arimoclolomol citrate cap	124 MG	Balanced ; Balanced Biosimilar ; Basic ; Basic Annual ; Enhanced ; Enhanced Annual ; HIM Quarterly 2024 ; HIM Quarterly 2025 ; HIM Quarterly 2026 ; IL HIM Annual 2025 ; IL HIM Annual 2026 ; IL HMO Performance Quarterly ; IL non-HMO Performance Full ; Jade ; MT Performance Full ; NM HIM Annual 2025 ; NM HIM Annual 2026 ; NM Performance Full ; OK

Target Brand Agent Name(s)	Target Generic Agent Name(s)	Strength	Client Formulary
			Performance Full ; Performance ; Performance Annual ; Performance Biosimilar ; Performance Select ; Performance Select Biosimilar ; TX HIM Annual 2025 ; TX HIM Annual 2026 ; Topaz ; Whole Foods

## PRIOR AUTHORIZATION CLINICAL CRITERIA FOR APPROVAL

Module	Clinical Criteria for Approval
Aqneurs a	<p><b>Initial Evaluation</b></p> <p><b>Target Agent(s)</b> will be approved when ALL of the following are met:</p> <ol style="list-style-type: none"> <li>1. The patient has a diagnosis of Niemann-Pick disease Type C <b>AND</b></li> <li>2. Genetic analysis confirms mutation in the <i>NPC1</i> or <i>NPC2</i> genes <b>AND</b></li> <li>3. The patient has disease-related neurological symptoms (e.g., loss of motor function, difficulty swallowing, speech impairment, cognitive impairment) <b>AND</b></li> <li>4. The patient weighs greater than or equal to 15 kg <b>AND</b></li> <li>5. The prescriber is a specialist in the area of the patient's diagnosis (e.g., geneticist), or the prescriber has consulted with a specialist in the area of the patient's diagnosis <b>AND</b></li> <li>6. The patient will NOT be using the requested agent in combination with Miplyffa <b>AND</b></li> <li>7. The patient does NOT have any FDA labeled contraindications to the requested agent</li> </ol> <p><b>Length of Approval:</b></p> <p>BCBSOK: 36 months BCBSIL and BCBSMT: 12 months All other plans: 6 months</p> <p>NOTE: If Quantity Limit applies, please refer to Quantity Limit Criteria.</p> <p><b>The requested agent will also be approved when ONE of the following is met:</b></p> <ol style="list-style-type: none"> <li>1. The request is for a BCBS NM Fully Insured or NM HIM member and ALL of the following: <ol style="list-style-type: none"> <li>A. The patient does NOT have any FDA labeled contraindications to the requested agent <b>AND</b></li> <li>B. The requested indication is a rare disease <b>AND</b></li> <li>C. ONE of the following: <ol style="list-style-type: none"> <li>1. The patient has another FDA labeled indication for the requested agent and route of administration <b>OR</b></li> <li>2. The patient has another indication that is supported in compendia for the requested agent and route of administration <b>OR</b></li> </ol> </li> </ol> </li> <li>2. ALL of the following: <ol style="list-style-type: none"> <li>A. The member resides in Ohio <b>AND</b></li> <li>B. The plan is Fully Insured or HIM Shop (SG) <b>AND</b></li> <li>C. The patient does NOT have any FDA labeled contraindications to the requested agent <b>AND</b></li> <li>D. ONE of the following: <ol style="list-style-type: none"> <li>1. The patient has another FDA labeled indication for the requested agent and route of administration <b>OR</b></li> <li>2. The patient has another indication that is supported in compendia for the requested agent and route of administration <b>OR</b></li> <li>3. The prescriber has submitted TWO articles from major peer-reviewed professional medical journals [e.g., Journal of American Medical</li> </ol> </li> </ol> </li> </ol>

Module	Clinical Criteria for Approval
	<p>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> <p><b>Non-oncology compendia allowed:</b> DrugDex level 1, 2A or 2B, AHFS-DI (narrative text must be supportive)</p> <p><b>Oncology compendia allowed:</b> 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><b>Length of Approval:</b></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> <p><b>Renewal Evaluation</b></p> <p><b>Target Agent(s)</b> will be approved when ALL of the following are met:</p> <ol style="list-style-type: none"> <li>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) <b>AND</b></li> <li>2. The patient has had clinical benefit with the requested agent <b>AND</b></li> <li>3. The prescriber is a specialist in the area of the patient's diagnosis (e.g., geneticist), or the prescriber has consulted with a specialist in the area of the patient's diagnosis <b>AND</b></li> <li>4. The patient will NOT be using the requested agent in combination with Miplyffa <b>AND</b></li> <li>5. The patient does NOT have any FDA labeled contraindications to the requested agent</li> </ol> <p><b>Length of Approval:</b></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>
Miplyffa	<p><b>Initial Evaluation</b></p> <p><b>Target Agent(s)</b> will be approved when ALL of the following are met:</p> <ol style="list-style-type: none"> <li>1. ONE of the following: <ol style="list-style-type: none"> <li>A. The requested agent is eligible for continuation of therapy AND the following:</li> </ol> <p style="text-align: center;"><b>Agents Eligible for Continuation of Therapy</b></p> <p style="text-align: center;">Miplyffa</p> <ol style="list-style-type: none"> <li>1. The prescriber states the patient has been treated with the requested agent (starting on samples is not approvable) within the past 90 days AND is at risk if therapy is changed <b>OR</b></li> </ol> </li> </ol>

Module	Clinical Criteria for Approval
	<p>B. ALL of the following:</p> <ol style="list-style-type: none"> <li>1. The patient has a diagnosis of Niemann-Pick disease Type C <b>AND</b></li> <li>2. Genetic analysis confirms mutation in the NPC1 or NPC2 genes <b>AND</b></li> <li>3. The patient has disease-related neurological symptoms (e.g., loss of motor function, difficulty swallowing, speech impairment, cognitive impairment) <b>AND</b></li> <li>4. The requested agent will be used in combination with miglustat <b>AND</b></li> <li>5. If the patient has an FDA labeled indication, then ONE of the following: <ol style="list-style-type: none"> <li>A. The patient's age is within FDA labeling for the requested indication for the requested agent <b>OR</b></li> <li>B. There is support for using the requested agent for the patient's age for the requested indication <b>AND</b></li> </ol> </li> </ol> <ol style="list-style-type: none"> <li>2. The prescriber is a specialist in the area of the patient's diagnosis (e.g., geneticist), or the prescriber has consulted with a specialist in the area of the patient's diagnosis <b>AND</b></li> <li>3. The patient will NOT be using the requested agent in combination with Aqneursa <b>AND</b></li> <li>4. The patient does NOT have any FDA labeled contraindications to the requested agent</li> </ol> <p><b>Length of Approval:</b></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> <p><b>The requested agent will also be approved when the following are met:</b></p> <ol style="list-style-type: none"> <li>1. The member resides in Ohio <b>AND</b></li> <li>2. The plan is Fully Insured or HIM Shop (SG) <b>AND</b> BOTH of the following <ol style="list-style-type: none"> <li>A. The patient does NOT have any FDA labeled contraindications to the requested agent <b>AND</b></li> <li>B. ONE of the following: <ol style="list-style-type: none"> <li>1. The patient has another FDA labeled indication for the requested agent and route of administration <b>OR</b></li> <li>2. The patient has another indication that is supported in compendia for the requested agent and route of administration <b>OR</b></li> <li>3. 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]</li> </ol> </li> </ol> </li> </ol> <p><b>Non-oncology compendia allowed:</b> DrugDex level 1, 2A or 2B, AHFS-DI (narrative text must be supportive)</p> <p><b>Oncology compendia allowed:</b> 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><b>Length of Approval:</b></p> <p>BCBSOK: 36 months</p> <p>ALL other plans: 12 months</p>

Module	Clinical Criteria for Approval
	<p>NOTE: If Quantity Limit applies, please refer to Quantity Limit Criteria</p> <p><b>Renewal Evaluation</b></p> <p><b>Target Agent(s)</b> will be approved when ALL of the following are met:</p> <ol style="list-style-type: none"> <li>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) <b>AND</b></li> <li>2. The patient has had clinical benefit with the requested agent <b>AND</b></li> <li>3. The prescriber is a specialist in the area of the patient's diagnosis (e.g., geneticist), or the prescriber has consulted with a specialist in the area of the patient's diagnosis <b>AND</b></li> <li>4. The patient will NOT be using the requested agent in combination with Aqneursa <b>AND</b></li> <li>5. The patient does NOT have any FDA labeled contraindications to the requested agent</li> </ol> <p><b>Length of Approval:</b></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><b>Quantity Limit for the Target Agent(s)</b> will be approved when ONE of the following is met:</p> <ol style="list-style-type: none"> <li>1. The requested quantity (dose) does NOT exceed the program quantity limit <b>OR</b></li> <li>2. The requested quantity (dose) exceeds the program quantity limit AND ONE of the following: <ol style="list-style-type: none"> <li>A. BOTH of the following: <ol style="list-style-type: none"> <li>1. The requested agent does NOT have a maximum FDA labeled dose for the requested indication <b>AND</b></li> <li>2. There is support for therapy with a higher dose for the requested indication <b>OR</b></li> </ol> </li> <li>B. BOTH of the following: <ol style="list-style-type: none"> <li>1. The requested quantity (dose) does NOT exceed the maximum FDA labeled dose for the requested indication <b>AND</b></li> <li>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 <b>OR</b></li> </ol> </li> <li>C. BOTH of the following: <ol style="list-style-type: none"> <li>1. The requested quantity (dose) exceeds the maximum FDA labeled dose for the requested indication <b>AND</b></li> <li>2. There is support for therapy with a higher dose for the requested indication</li> </ol> </li> </ol> </li> </ol> <p><b>Length of Approval:</b></p> <p>BCBSIL 12 months</p> <p>ALL other plans--</p>

Module	Clinical Criteria for Approval
	INITIAL: Aqneursa 6 months, Miplyffa 12 months  RENEWAL: 12 months