

# Substrate Reduction Therapy Prior Authorization with Quantity Limit Program Summary

## POLICY REVIEW CYCLE

**Effective Date**  
11-01-2025

**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
Cerdelga	Eliglustat Tartrate Cap 84 MG (Base Equivalent)	84 MG	M ; N ; O ; Y	N		
Opfolda	miglustat (gaa deficiency) cap	65 MG	M ; N ; O ; Y	N		
Zavesca	miglustat cap	100 MG	M ; N ; O ; Y	O ; Y		

## 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
Cerdelga	Eliglustat Tartrate Cap 84 MG (Base Equivalent)	84 MG	60	Capsules	30	DAYS			
Opfolda	miglustat (gaa deficiency) cap	65 MG	8	Capsules	28	DAYS			
Zavesca	Miglustat Cap 100 MG	100 MG	90	Capsules	30	DAYS			

## CLIENT SUMMARY – PRIOR AUTHORIZATION

Target Brand Agent Name(s)	Target Generic Agent Name(s)	Strength	Client Formulary
Cerdelga	Eliglustat Tartrate Cap 84 MG (Base Equivalent)	84 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 ;

Target Brand Agent Name(s)	Target Generic Agent Name(s)	Strength	Client Formulary
			Performance Annual ; Performance Biosimilar ; Performance Select ; Performance Select Biosimilar ; TX HIM Annual 2025 ; TX HIM Annual 2026 ; Topaz ; Whole Foods
Opfolda	miglustat (gaa deficiency) cap	65 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
Zavesca	miglustat cap	100 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
Cerdelga	Eliglustat Tartrate Cap 84 MG (Base Equivalent)	84 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 ;

Target Brand Agent Name(s)	Target Generic Agent Name(s)	Strength	Client Formulary
			Performance Annual ; Performance Biosimilar ; Performance Select ; Performance Select Biosimilar ; TX HIM Annual 2025 ; TX HIM Annual 2026 ; Topaz ; Whole Foods
Opfolda	miglustat (gaa deficiency) cap	65 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 ; Whole Foods
Zavesca	Miglustat Cap 100 MG	100 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 ; Whole Foods

**PRIOR AUTHORIZATION CLINICAL CRITERIA FOR APPROVAL**

Module	Clinical Criteria for Approval
Cerdelga , miglustat	<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>ONE of the following:</li> </ol>

Module	Clinical Criteria for Approval
	<p>A. The requested agent is eligible for continuation of therapy AND the following:</p> <p style="text-align: center;"><b>Agents Eligible for Continuation of Therapy</b></p> <p style="text-align: center;">All target agents are eligible for continuation of therapy</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> <p>B. ALL of the following:</p> <ol style="list-style-type: none"> <li>1. The patient has a diagnosis of Gaucher disease type 1 (GD1) <b>AND</b></li> <li>2. ONE of the following: <ol style="list-style-type: none"> <li>A. The patient has a baseline (prior to therapy for the requested indication) glucocerebrosidase enzyme activity of less than or equal to 15% of mean normal in fibroblasts, leukocytes, or other nucleated cells <b>OR</b></li> <li>B. Genetic analysis confirmed two (2) pathogenic alleles in the glucocerebrosidase (GBA) gene <b>AND</b></li> </ol> </li> <li>3. 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> <li>4. The patient does NOT have any neuronopathic symptoms indicative of Gaucher disease type 2 or type 3 [e.g., bulbar signs (e.g., stridor, strabismus, swallowing difficulty), pyramidal signs (e.g., opisthotonos, head retroflexion, spasticity, trismus), oculomotor apraxia, tonic-clonic seizures, myoclonic epilepsy, dementia, ataxia] <b>AND</b></li> <li>5. The patient has at least ONE of the following clinical presentations at baseline (prior to therapy for the requested indication): <ol style="list-style-type: none"> <li>A. Anemia defined as mean hemoglobin (Hb) level below the testing laboratory's lower limit of the normal range based on age and gender <b>OR</b></li> <li>B. Thrombocytopenia (platelet count &lt; 100,000/microliter on at least 2 measurements) <b>OR</b></li> <li>C. Hepatomegaly <b>OR</b></li> <li>D. Splenomegaly <b>OR</b></li> <li>E. Growth failure (i.e., growth velocity is below the standard mean for age) <b>OR</b></li> <li>F. Evidence of bone disease with other causes ruled out <b>AND</b></li> </ol> </li> <li>6. The patient is a CYP2D6 extensive metabolizer (EM), intermediate metabolizer (IM), or poor metabolizer (PM), as detected by an FDA-cleared test for determining CYP2D6 genotype <b>AND</b></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., endocrinologist, 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 another substrate reduction therapy agent (e.g., Opfolda, miglustat, Zavesca) for the requested indication <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>

Module	Clinical Criteria for Approval
	<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> <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., endocrinologist, geneticist), or the prescriber has consulted with a specialist in the area of patient's diagnosis <b>AND</b></li> <li>4. The patient will NOT be using the requested agent in combination with another substrate reduction therapy agent (e.g., Opfolda, miglustat, Zavesca) for the requested indication <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>

Module	Clinical Criteria for Approval
	<p>ALL other plans: 12 months</p> <p>NOTE: If Quantity Limit applies, please refer to Quantity Limit Criteria.</p>
Opfolda	<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: <p style="text-align: center;"><b>Agents Eligible for Continuation of Therapy</b></p> <p>All target agents are eligible for continuation of therapy</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> <li>B. ALL of the following: <ol style="list-style-type: none"> <li>1. The patient has a diagnosis of late-onset Pompe disease (acid maltase deficiency [AMD]; glycogen storage disease type II [GSDII]) confirmed by ONE of the following: <ol style="list-style-type: none"> <li>A. Genetic analysis confirms biallelic mutation (two pathogenic variants) in the <i>GAA</i> gene <b>OR</b></li> <li>B. The patient has deficient acid alpha-glucosidase glycogen enzyme activity in dried blood spots, leukocytes, skin fibroblasts, and/or skeletal muscle tissue <b>AND</b></li> </ol> </li> <li>2. The patient is not improving on their current enzyme replacement therapy (ERT) <b>AND</b></li> <li>3. The patient weighs greater than or equal to 40 kg <b>AND</b></li> <li>4. The requested agent will be taken in combination with Pombiliti <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>OR</b></li> </ol> </li> </ol> </li> <li>C. The requested agent will be used in combination with Miplyffa (arimoclomol) and ALL of the following: <ol style="list-style-type: none"> <li>1. The patient has a diagnosis of Niemann-Pick type C disease (NPC) <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 <b>AND</b></li> <li>4. The patient's age is within Miplyffa FDA labeling for the requested indication <b>AND</b></li> </ol> </li> </ol> </li> <li>2. The prescriber is a specialist in the area of the patient's diagnosis (e.g., endocrinologist, 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 another substrate reduction therapy agent (e.g., Cerdelga, eliglustat, Zavesca) for the requested indication <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>

Module	Clinical Criteria for Approval
	<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> <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., endocrinologist, 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 another substrate reduction therapy agent (e.g., Cerdelga, eliglustat, Zavesca) for the requested indication <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>

Module	Clinical Criteria for Approval
	<p>ALL other plans: 12 months</p> <p>NOTE: If Quantity Limit applies, please refer to Quantity Limit criteria.</p>
Zavesca, miglustat	<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: <div data-bbox="467 516 1182 594" style="border: 1px solid black; padding: 5px; margin: 10px 0;"> <p><b>Agents Eligible for Continuation of Therapy</b></p> <p>All target agents are eligible for continuation of therapy</p> </div> </li> </ol> </li> <li>B. ONE of the following: <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> <li>1. The patient has a diagnosis of Gaucher disease type 1 (GD1) ALL of the following: <ol style="list-style-type: none"> <li>A. If the patient has an FDA labeled indication, then ONE of the following: <ol style="list-style-type: none"> <li>1. The patient’s age is within FDA labeling for the requested indication for the requested agent <b>OR</b></li> <li>2. There is support for using the requested agent for the patient’s age for the requested indication <b>AND</b></li> </ol> </li> <li>B. ONE of the following: <ol style="list-style-type: none"> <li>1. The patient has baseline (prior to therapy for the requested indication) glucocerebrosidase enzyme activity of less than or equal to 15% of mean normal in fibroblasts, leukocytes, or other nucleated cells <b>OR</b></li> <li>2. Genetic analysis confirmed two (2) pathogenic alleles in the glucocerebrosidase (<i>GBA</i>) gene <b>AND</b></li> </ol> </li> <li>C. The patient does NOT have any neuronopathic symptoms indicative of Gaucher disease type 2 or type 3 [e.g., bulbar signs (e.g., stridor, strabismus, swallowing difficulty), pyramidal signs (e.g., opisthotonos, head retroflexion, spasticity, trismus), oculomotor apraxia, tonic-clonic seizures, myoclonic epilepsy, dementia, ataxia] <b>AND</b></li> <li>D. The patient has at least ONE of the following clinical presentations at baseline (prior to therapy for the requested indication): <ol style="list-style-type: none"> <li>1. Anemia defined as mean hemoglobin (Hb) level below the testing laboratory’s lower limit of the normal range based on age and gender <b>OR</b></li> <li>2. Thrombocytopenia (platelet count less than 100,000/microliter on at least 2 measurements) <b>OR</b></li> <li>3. Hepatomegaly <b>OR</b></li> <li>4. Splenomegaly <b>OR</b></li> <li>5. Growth failure (i.e., growth velocity is below the standard mean for age) <b>OR</b></li> <li>6. Evidence of bone disease with other causes ruled out <b>AND</b></li> </ol> </li> <li>E. Enzyme replacement therapy (ERT) is NOT a therapeutic option (e.g., due to allergy, hypersensitivity, poor venous access, previous ERT failure) <b>OR</b></li> </ol> </li> <li>2. The requested agent will be used in combination with Miplyffa (arimoclomol) and ALL of the following: <ol style="list-style-type: none"> <li>A. The patient has a diagnosis of Niemann-Pick type C disease (NPC) <b>AND</b></li> <li>B. Genetic analysis confirms mutation in the NPC1 or NPC2 genes <b>AND</b></li> </ol> </li> </ol> </li> </ol>

Module	Clinical Criteria for Approval				
	<p data-bbox="565 180 1349 210">C. The patient has disease-related neurological symptoms <b>AND</b></p> <p data-bbox="565 212 1409 266">D. The patient's age is within Miplyffa FDA labeling for the requested indication <b>AND</b></p> <p data-bbox="279 268 1417 325">2. If the request is for one of the following brand agents with an available generic equivalent (listed below), then ONE of the following:</p> <table border="1" data-bbox="526 363 1122 443"> <thead> <tr> <th data-bbox="526 363 824 401">Brand</th> <th data-bbox="824 363 1122 401">Generic Equivalent</th> </tr> </thead> <tbody> <tr> <td data-bbox="526 401 824 443">Zavesca</td> <td data-bbox="824 401 1122 443">miglustat</td> </tr> </tbody> </table> <p data-bbox="354 483 1369 543">A. The request is for a BCBS IL Fully Insured, ASO Cost/BBF, HIM, or Non-ERISA ASO/Self-insured Municipalities/Counties member <b>OR</b></p> <p data-bbox="354 546 1406 604">B. The patient is currently being treated with the requested agent and the patient is currently stable on the requested agent [chart notes required] <b>OR</b></p> <p data-bbox="354 606 1406 663">C. The patient has tried and had an inadequate response to the generic equivalent [chart notes required] <b>OR</b></p> <p data-bbox="354 665 1390 722">D. The generic equivalent was discontinued due to lack of efficacy or effectiveness, diminished effect, or an adverse event [chart notes required] <b>OR</b></p> <p data-bbox="354 724 1417 781">E. The patient has an intolerance or hypersensitivity to the generic equivalent that is not expected to occur with the brand agent <b>OR</b></p> <p data-bbox="354 783 1395 840">F. The patient has an FDA labeled contraindication to the generic equivalent that is not expected to occur with the brand agent <b>OR</b></p> <p data-bbox="354 842 1401 1010">G. The generic equivalent is expected to be ineffective based on the known clinical characteristics of the patient and the known characteristics of the prescription drug; <b>OR</b> cause a significant barrier to the patient's adherence of care; <b>OR</b> worsen a comorbid condition; <b>OR</b> decrease the patient's ability to achieve or maintain reasonable functional ability in performing daily activities; <b>OR</b> cause an adverse reaction or cause physical or mental harm [chart notes required] <b>OR</b></p> <p data-bbox="354 1012 1317 1068">H. The generic equivalent is not in the best interest of the patient based on medical necessity [chart notes required] <b>OR</b></p> <p data-bbox="354 1071 1411 1186">I. The patient has tried another prescription drug in the same pharmacologic class or with the same mechanism of action as the generic equivalent and that prescription drug was discontinued due to lack of efficacy or effectiveness, diminished effect, or an adverse event [chart notes required] <b>OR</b></p> <p data-bbox="354 1188 1328 1245">J. There is support for the use of the requested brand agent over the generic equivalent <b>AND</b></p> <p data-bbox="279 1247 1395 1329">3. The prescriber is a specialist in the area of the patient's diagnosis (e.g., endocrinologist, geneticist), or the prescriber has consulted with a specialist in the area of the patient's diagnosis <b>AND</b></p> <p data-bbox="279 1331 1403 1413">4. The patient will NOT be using the requested agent in combination with another substrate reduction therapy agent (e.g., Cerdelga, eliglustat, Opfolda) for the requested indication <b>AND</b></p> <p data-bbox="279 1415 1357 1444">5. The patient does NOT have any FDA labeled contraindications to the requested agent</p> <p data-bbox="228 1482 500 1512"><b>Length of Approval:</b></p> <p data-bbox="228 1549 483 1579">BCBSOK: 36 months</p> <p data-bbox="228 1617 570 1646">ALL other plans: 12 months</p> <p data-bbox="228 1684 1084 1713">NOTE: If Quantity Limit applies, please refer to Quantity Limit Criteria.</p> <p data-bbox="228 1808 1192 1837"><b>The requested agent will also be approved when the following are met:</b></p> <p data-bbox="279 1877 737 1906">1. The member resides in Ohio <b>AND</b></p> <p data-bbox="279 1908 1185 1938">2. The plan is Fully Insured or HIM Shop (SG) <b>AND</b> BOTH of the following</p> <p data-bbox="354 1940 1377 1997">A. The patient does NOT have any FDA labeled contraindications to the requested agent <b>AND</b></p>	Brand	Generic Equivalent	Zavesca	miglustat
Brand	Generic Equivalent				
Zavesca	miglustat				

Module	Clinical Criteria for Approval				
	<p>B. ONE of the following:</p> <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> <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. If the request is for one of the following brand agents with an available generic equivalent (listed below), then ONE of the following:</li> </ol> <table border="1" data-bbox="526 1459 1122 1535" style="margin-left: auto; margin-right: auto;"> <thead> <tr> <th>Brand</th> <th>Generic Equivalent</th> </tr> </thead> <tbody> <tr> <td>Zavesca</td> <td>miglustat</td> </tr> </tbody> </table> <ol style="list-style-type: none"> <li>A. The request is for a BCBS IL Fully Insured, ASO Cost/BBF, HIM, or Non-ERISA ASO/Self-insured Municipalities/Counties member <b>OR</b></li> <li>B. The patient is currently being treated with the requested agent and the patient is currently stable on the requested agent [chart notes required] <b>OR</b></li> <li>C. The patient has tried and had an inadequate response to the generic equivalent [chart notes required] <b>OR</b></li> <li>D. The generic equivalent was discontinued due to lack of efficacy or effectiveness, diminished effect, or an adverse event [chart notes required] <b>OR</b></li> <li>E. The patient has an intolerance or hypersensitivity to the generic equivalent that is not expected to occur with the brand agent <b>OR</b></li> <li>F. The patient has an FDA labeled contraindication to the generic equivalent that is not expected to occur with the brand agent <b>OR</b></li> <li>G. The generic equivalent is expected to be ineffective based on the known clinical characteristics of the patient and the known characteristics of the prescription</li> </ol>	Brand	Generic Equivalent	Zavesca	miglustat
Brand	Generic Equivalent				
Zavesca	miglustat				

Module	Clinical Criteria for Approval
	<p>drug; <b>OR</b> cause a significant barrier to the patient’s adherence of care; <b>OR</b> worsen a comorbid condition; <b>OR</b> decrease the patient’s ability to achieve or maintain reasonable functional ability in performing daily activities; <b>OR</b> cause an adverse reaction or cause physical or mental harm [chart notes required] <b>OR</b></p> <p>H. The generic equivalent is not in the best interest of the patient based on medical necessity [chart notes required] <b>OR</b></p> <p>I. The patient has tried another prescription drug in the same pharmacologic class or with the same mechanism of action as the generic equivalent and that prescription drug was discontinued due to lack of efficacy or effectiveness, diminished effect, or an adverse event [chart notes required] <b>OR</b></p> <p>J. There is support for the use of the requested brand agent over the generic equivalent <b>AND</b></p> <p>4. The prescriber is a specialist in the area of the patient’s diagnosis (e.g., endocrinologist, geneticist), or the prescriber has consulted with a specialist in the area of patient’s diagnosis <b>AND</b></p> <p>5. The patient will NOT be using the requested agent in combination with another substrate reduction therapy agent (e.g., Cerdelga, eliglustat, Opfolda) for the requested indication <b>AND</b></p> <p>6. The patient does NOT have any FDA labeled contraindications to the requested agent</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>

## 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. If the requested agent is Zavesca or Opfolda (or the generic equivalent miglustat), the patient has a diagnosis of Niemann-Pick type C disease <b>AND</b> the requested quantity (dose) does NOT exceed 600 mg per day <b>OR</b></li> <li>3. The requested quantity (dose) exceeds the program quantity limit <b>AND</b> 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 of therapy with a higher dose for the requested indication</li> </ol> </li> </ol> </li> </ol> <p><b>Length of Approval:</b> 12 months</p>

