Sucraid (sacrosidase) Prior Authorization with Quantity Limit Program Summary

This program applies to MN Medicaid.

POLICY REVIEW CYCLE

Effective Date	Date of Origin
1/1/2024	1/1/2021

FDA APPROVED INDICATIONS AND DOSAGE

Agent(s)	FDA Indication(s)	Notes	Ref#
Sucraid® (sacrosidase) Oral solution	Oral replacement therapy for treatment of genetically determined sucrase deficiency, which is part of congenital sucrase-isomaltase deficiency (CSID)		1

See package insert for FDA prescribing information: https://dailymed.nlm.nih.gov/dailymed/index.cfm

CLINICAL RATIONALE

CSID	Congenital sucrase-isomaltase deficiency (CSID) is a rare, chronic, autosomal recessive disorder characterized by the absence or deficiency of the enzymes sucrase and isomaltase. Patients with CSID have two defective copies of the sucrase-isomaltase (SI) gene. The SI enzyme complex is naturally produced in the brush border lining of the small intestine and assists in the breakdown of certain sucrose and products of starch digestion (dextrins). When sucrase-isomaltase is absent or deficient, non-absorbed carbohydrates enter the distal small intestine and colon where they are fermented, leading to the excessive production of short-chain fatty acids and gases such as hydrogen, methane, and hydrogen sulfide. This in turn can lead to abdominal distension, cramping, pain, excessive flatulence, nausea/vomiting, and osmotic diarrhea. If left untreated, significant sucrase-isomaltase deficiency (SID) can result in inadequate growth and failure to thrive in children as well as weight loss in adults. The gold standard for the diagnosis of CSID remains small intestinal biopsy specimens assayed for lactase, sucrase, isomaltase, and maltase activity. Criteria to make the diagnosis of CSID include normal small bowel morphology in the presence of markedly reduced or absent sucrase activity, isomaltase activity varying from zero to full activity, and reduced maltase activity. Lactase activity can be normal or reduced in children with a sucrase:lactase ratio of less than 1.0. Genetic sequencing of the SI gene can identify homozygous and compound heterozygous mutations responsible for CSID. A number of noninvasive diagnostic tests can also help establish the diagnosis, including the sucrose challenge test, lactose breath test, and hydrogen-methane breath test. However, many of these tests have limitations including false-positive results, false-negative results, and lack of validation data. Previously, treatment of CSID has required lifelong adherence to a sucrose-free diet.(2-4) Data suggest that even after diagnosis and dietary treatment, major gastrointestinal symptoms persists, and there is a high frequency of decreased weight for height and age in these patients.(5) Treatment has improved considerably with the availability of enzyme replacement therapy (sacrosidase) which has allowed consumption of a more normal diet and decreased the high incidence of chronic

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	gastrointestinal problems.(2-5) Access to a physician or dietician who is knowledgeable about CSID is essential for guiding patients and their families.(4)
Efficacy	The efficacy of Sucraid was studied in a two-phase randomized, double blind, multisite, crossover trial conducted in 28 patients with confirmed CSID. Phase 1 was the breath hydrogen phase which consisted of three single-dose treatments (placebo, sacrosidase, and milk with sacrosidase). Phase 2 was the dose-response phase which consisted of four multidose treatment groups (full-strength sacrosidase, 1:10, 1:100, and 1:1000 dilution). The first phase consisted of each patient undergoing three hydrogen breath tests after 1 week of a sucrose-free, low starch diet, and a 12-hour fasting interval. A baseline breath hydrogen level of less than 10 parts per million was required before each patient ingested a 20% sucrose solution of 2g/kg followed by placebo, sacrosidase, or milk with sacrosidase. Breath hydrogen levels were obtained every 30 minutes during each 3-hour breath test. Stool frequency and consistency (watery, soft, formed, hard), and the frequency and severity (0 = none, 1 = mild, 2 = moderate, 3 = severe) of symptoms (diarrhea, gas, bloating, cramps, and vomiting) were recorded during the test and for 24 hours thereafter. At the conclusion of the breath hydrogen tests, each patient underwent another week of a sucrose-free, low-starch diet before beginning the dose-response phase. During the second dose-response phase, patients consumed a normal sucrose-containing diet while receiving each of four doses of sacrosidase in random order for a period of 10 days. Stool frequency and consistency measures, symptoms, and dietary data were recorded on a daily basis and compared with a baseline period during which patients had consumed a sucrose-free diet and had not taken any enzyme. The primary efficacy endpoints included total stools and total symptoms score collected during the dose-response phase. All other measurements assessed during the dose-response phase and the breath hydrogen phase were secondary. A dose response relationship was shown between the two higher and the two lower concentrations, with the two higher concentrations meeting the primary efficacy endpoints. Higher doses of sacrosidase were associated with a significantly greater number of hard and formed stools as well as with fewer watery and soft stools, the secondary efficacy endpoints. Additionally, patients showed a marked decrease in breath hydrogen output when they received sacrosidase in comparison to placebo in the first phase.(1,5)
Safety	Sucraid is contraindicated in patients known to be hypersensitive to yeast, yeast products, glycerin (glycerol), or papain.(1)

REFERENCES

Number	Reference
1	Sucraid prescribing information. QOL Medical, LLC. May 2022.
2	Treem, W. R. (2012). Clinical aspects and treatment of congenital sucrase-isomaltase deficiency. Journal of Pediatric Gastroenterology and Nutrition, 55(2), S7–S13. https://doi.org/10.1097/01.mpg.0000421401.57633.90
3	Congenital sucrase-isomaltase deficiency - symptoms, causes, treatment: Nord. National Organization for Rare Disorders (NORD). (2005). https://rarediseases.org/rare-diseases/disaccharide-intolerance-i/
4	Chey, W. D., Cash, B., Lembo, A., Patel, D. B., & Scarlata, K. (2020, October). Congenital Sucrase-Isomaltase Deficiency: What, When, and How?. Gastroenterology and Hepatology. https://www.gastroenterologyandhepatology.net/supplements/congenital-sucrase-isomaltase-deficiency-what-when-and-how/

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Number	Reference
5	Treem, W. R., McAdams, L., Stanford, L., Kastoff, G., Justinich, C., & Hyams, J. (1999). Sacrosidase therapy for Congenital Sucrase-Isomaltase Deficiency. Journal of Pediatric Gastroenterology and Nutrition, 28(2), 137–142. https://doi.org/10.1097/00005176-199902000-00008

POLICY AGENT SUMMARY PRIOR AUTHORIZATION

Target Brand Agent(s)	Target Generic Agent(s)	Strength	Targeted MSC	Available MSC	Final Age Limit	Preferred Status
Sucraid	Sacrosidase Soln 8500 Unit/ML	8500 UNIT/ML	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
Sucraid	Sacrosidase Soln 8500 Unit/ML	8500 UNIT/ML	300	mL	30	DAYS			

CLIENT SUMMARY – PRIOR AUTHORIZATION

Target Brand Agent Name(s)	Target Generic Agent Name(s)	Strength	Client Formulary
Sucraid	Sacrosidase Soln 8500 Unit/ML	8500 UNIT/ML	Medicaid

CLIENT SUMMARY – QUANTITY LIMITS

Target Brand Agent Name(s)	Target Generic Agent Name(s)	Strength	Client Formulary
Sucraid	Sacrosidase Soln 8500 Unit/ML	8500 UNIT/ML	Medicaid

PRIOR AUTHORIZATION CLINICAL CRITERIA FOR APPROVAL

Module	Clinical Criteria for Approval
PA	**Initial Evaluation** **Target Agent(s)** will be approved when ALL of the following are met: 1. The patient has a diagnosis of congenital sucrase-isomaltase deficiency (CSID) confirmed by ONE of the following: A. Genetic testing of the sucrase-isomaltase (SI) gene indicates a pathogenic mutation **OR** B. Endoscopic biopsy of the small bowel indicates normal small bowel morphology in the presence of decreased (or absent) sucrase activity, isomaltase activity varying from decreased to normal activity, and decreased maltase activity **AND** 2. The prescriber is a specialist in the area of the patient's diagnosis (e.g., gastroenterologist, geneticist, endocrinologist), or the prescriber has consulted with a specialist in the area of the patient's diagnosis **AND**

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Module	Clinical Criteria for Approval
	3. The patient does NOT have any FDA labeled contraindications to the requested agent **Length of Approval:** 3 months NOTE: If Quantity Limit applies, please refer to Quantity Limit Criteria. **Renewal Evaluation** **Target Agent(s)** will be approved when ALL of the following are met: 1. The patient has been previously approved for the requested agent through the plan's Prior Authorization process **AND** 2. The patient has had clinical benefit with the requested agent **AND** 3. The prescriber is a specialist in the area of the patient's diagnosis (e.g., gastroenterologist, geneticist, endocrinologist), 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 **Length of Approval:** 12 months NOTE: If Quantity Limit applies, please refer to Quantity Limit Criteria.

QUANTITY LIMIT CLINICAL CRITERIA FOR APPROVAL

Module	Clinical Criteria for Approval
QL with PA	**Quantity Limit for the Target Agent(s)** will be approved when ONE of the following is met: 1. The requested quantity (dose) does NOT exceed the program quantity limit **OR** 2. ALL of the following: A. The requested quantity (dose) exceeds the program quantity limit **AND** B. The requested quantity (dose) does NOT exceed the maximum FDA labeled dose for the requested indication **AND** C. The requested quantity (dose) cannot be achieved with a lower quantity of a higher strength that does not exceed the program quantity limit **OR** 3. ALL of the following: A. The requested quantity (dose) exceeds the program quantity limit **AND** B. The requested quantity (dose) exceeds the maximum FDA labeled dose for the requested indication **AND** C. The prescriber has provided information in support of therapy with a higher dose for the requested indication **Length of Approval**: Initial - 3 months; Renewal - 12 months

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