Sucraid (sacrosidase) Prior Authorization with Quantity Limit Program Summary

POLICY REVIEW CYCLE

Effective Date 12/1/2022 **Date of Origin**

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

Overview	Congenital sucrase-isomaltase deficiency (CSID) is a rare, chronic, autosomal recessive disorder characterized by the absence or deficiency of endogenous sucrase and isomaltase activity. Patients with CSID have two defective copies of the sucrase-isomaltase (SI) gene. The SI enzyme complex is naturally produced in the brush border of the small intestine, primarily the distal duodenum and jejunum. Unhydrolyzed sucrose and starch are not absorbed from the intestine and their presence in the intestinal lumen may lead to osmotic retention of water. This may result in diarrhea. Unabsorbed sucrose in the colon is fermented by bacterial flora to produce increased amounts of hydrogen, methane, and water. As a consequence, excessive gas, bloating, abdominal cramps, nausea, and vomiting may occur. Chronic malabsorption of disaccharides may result in malnutrition. Undiagnosed/untreated CSID patients often fail to thrive and fall behind in their expected growth and development curves.(2-4) A number of diagnostic tests are available to support the diagnosis of CSID, with assay of duodenal biopsy specimens considered the gold standard.(2,4) Endoscopic biopsy of the small bowel should demonstrate 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.(4) Genetic sequencing of the SI gene can identify homozygous and compound heterozygous mutations responsible for CSID.(2,4) 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 which include false-positive or false-negative results, and lack of validation data.(4) Previously, the treatment of CSID has required the continual use of a strict sucrose-free diet and starch-restricted diets. Treatment has improved considerably with the availability of enzyme replacement therapy (sacrosidase), which is derived from baker's yeast.(2-4) Access to a physician or dietician who is knowledgeable about CSID is essential for guiding patients and their families.(4)

Efficacy(1, 5)	The efficacy of Sucraid was studied in a two-phase (dose response preceded by a breath hydrogen phase) double blind, multi-site, crossover trial conducted in 28 patients with confirmed CSID. Patients showed a marked decrease in breath hydrogen output when they received sacrosidase in comparison to placebo in the breath hydrogen phase. This first phase consisted of three sucrose breath hydrogen tests with three single-dose treatments (placebo, sacrosidase, and sacrosidase plus milk), each test undergone after 1 week of a sucrose-free, low starch diet and a 12-hour fasting interval. At the conclusion of the breath tests, each patient underwent another week of a sucrose-free, low-starch diet before beginning the second phase, dose response. During the dose response phase, the patients were challenged with an ordinary sucrose-containing diet while receiving each of four doses of sacrosidase: full strength (9000 IU/mL) and three dilutions (1:10, 1:100, and 1:1000) in random order for a period of 10 days. The primary efficacy endpoints were fewer total stools and lower total symptom scores. Symptom scores were based on severity of symptoms (gas, bloating, nausea, vomiting, and abdominal cramps) recorded daily by each patient. The symptoms were assigned values of 0 = none, 1 = mild, 2 = moderate, and 3 = severe. A dose response relationship was shown between the two higher and the two lower doses, with the two higher doses meeting the primary efficacy endpoints.
Safety(1)	Sucraid is contraindicated in patients known to be hypersensitive to yeast, yeast products, glycerin (glycerol), or papain.

REFERENCES

Number	Reference
1	Sucraid prescribing information. QOL Medical, LLC. May 2022.
2	Treem WR. Clinical Aspects and Treatment of Congenital Sucrase-Isomaltase Deficiency. J Pediatr Gastroenterol Nutr. 2012;55:S7-S13.
3	National Organization for Rare Disorders (NORD). Congenital Sucrase-Isomaltase Deficiency. Rare Disease Database; 2005. Available at: https://rarediseases.org/rare-diseases/disaccharide-intolerance-i/.
4	Chey WD, Cash B, Lembo A, et al. Congenital Sucrase-Isomaltase Deficiency: What, When, and How? Gastroenterol Hepatol. 2020;16(10):1-11.
5	Treem WR, McAdams L, Stanford L, et al. Sacrosidase Therapy for Congenital Sucrase-Isomaltase Deficiency, J Pediatr Gastroenterol Nutr. 1999;28(2):137-142

POLICY AGENT SUMMARY PRIOR AUTHORIZATION

Agent Names	Strength	Targeted MSC	Available MSC	Preferred Status	Effective Date
SUCRAID*sacrosidase soln	8500 UNIT/ML	M ; N ; O ; Y	N		

POLICY AGENT SUMMARY QUANTITY LIMIT

Target Agent GPI	Agent Names	Strength	QL Amount	Dose Form	Days Supply	Duration	Addtl QL Info	Allowed Exceptions	Targeted NDCs When Exclusions Exist	Effective Date
51200060002030	SUCRAID*Sacrosidase Soln 8500 Unit/ML	8500 UNIT/ML	300.0	MLS	30	Days				

CLIENT SUMMARY – PRIOR AUTHORIZATION

Agent Names	Strength	Client Formulary
SUCRAID*sacrosidase soln	8500 UNIT/ML	Commercial ; HIM

CLIENT SUMMARY – QUANTITY LIMITS

Agent Names	Strength	Client Formulary
SUCRAID*Sacrosidase Soln 8500 Unit/ML	8500 UNIT/ML	Commercial ; HIM

PRIOR AUTHORIZATION CLINICAL CRITERIA FOR APPROVAL

Module	Clinical Criteria for Approval
	Initial Evaluation **Target Agent** 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, endocrinologist) or the prescriber has consulted with a specialist in the area of the patient's diagnosis **AND** 3. The patient does NOT have any FDA labeled contraindications to the requested agent **Length of Approval:** 3 months NOTE: Quantity Limit applies, please refer to Quantity Limit Criteria section below.

Module	Clinical Criteria for Approval
	Renewal Evaluation **Target Agent** 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, 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 **AND** **Length of Approval:** 12 months NOTE: Quantity Limit applies, please refer to Quantity Limit Criteria section below.

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) is greater than 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) is greater than the program quantity limit **AND** B. The requested quantity (dose) is greater than 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