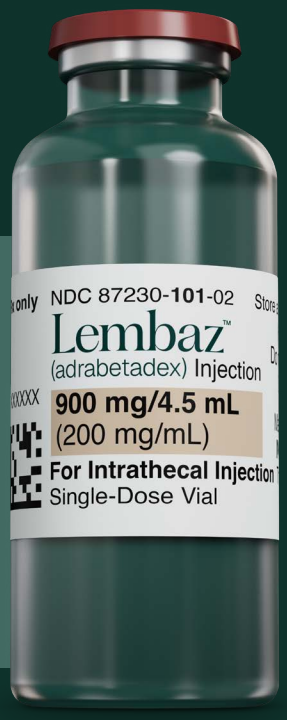
Lembaz™
(adrabetadex) injection
900 mg/4.5 mL, for intrathecal use

Product, Dosing and Administration Guide for Lembaz

A guide to preparing, dosing, and administering Lembaz by intrathecal injection



Each single-dose vial contains
900 mg/4.5 mL
(200 mg/mL) Lembaz.
NDC: 87230-101-01

INDICATION AND IMPORTANT SAFETY INFORMATION

INDICATION

LEMBAZ™ (adrabetadex) is indicated to reduce the risk of mortality in adult and pediatric patients, who weigh 7 kg or greater, with Niemann-Pick disease type C (NPC) with neurological manifestations that started at less than 6 years of age.

IMPORTANT SAFETY INFORMATION

WARNING: HEARING IMPAIRMENT INCLUDING PERMANENT HEARING LOSS

Treatment with LEMBAZ has resulted in permanent hearing impairment.

- **Prior to LEMBAZ use, perform a comprehensive audiology evaluation (e.g., pure tone audiometry, speech recognition, tympanometry) to establish baseline hearing levels.**
- **During LEMBAZ treatment, modify LEMBAZ dosage based on comprehensive audiology evaluations as follows:**
  - **To determine the subsequent dosage level, repeat comprehensive audiology evaluation 1 to 2 weeks after the first LEMBAZ dose at each dosage level but before the next dose, and then 1 to 2 weeks after the second LEMBAZ dose at each dosage level but before the next dose.**
  - **When a patient remains at the same LEMBAZ dosage level for 2 consecutive doses or has reached the maximum dosage, perform comprehensive audiology evaluations at one-month intervals until 3 consecutive, stable audiology results are obtained, and then repeat the comprehensive audiology evaluation every 12 months.**

Please see Important Safety Information on page 7 and the full Prescribing Information, including Boxed WARNING.

beren™

Establishing the Starting Dosage for Lembaz

Key baseline assessments before initiating treatment with Lembaz

Before initiating Lembaz, perform a comprehensive audiology evaluation (eg, pure tone audiometry, speech recognition, tympanometry) to establish baseline hearing levels.

Note: Lembaz has been associated with permanent hearing impairment, primarily affecting high-frequency but hearing impairment at mid and lower frequencies was also observed.

Dosing for patients with infantile-onset Niemann-Pick disease, type C (NPC)

Lembaz is administered once every 2 weeks intrathecally over 1 to 2 minutes, by or under the supervision of a healthcare provider experienced in performing lumbar punctures.

Lembaz dosing schedule: 1 intrathecal bolus injection every 2 weeks

Dosing at a glance

Starting dosage	IT Q2W
<6 months	100 mg
6 mo to <4 years	200 mg
4 years and older	400 mg

Maximum dosage	by weight
7 to <12 kg	600 mg
12 kg or more	900 mg

Titration step

+100–200 mg

Give at least 2 doses at each dosage level, then step up as tolerated to the weight-based maximum, each step guided by audiology. A dosage level consists of the same dose of Lembaz given two weeks apart.

IMPORTANT SAFETY INFORMATION (cont'd)

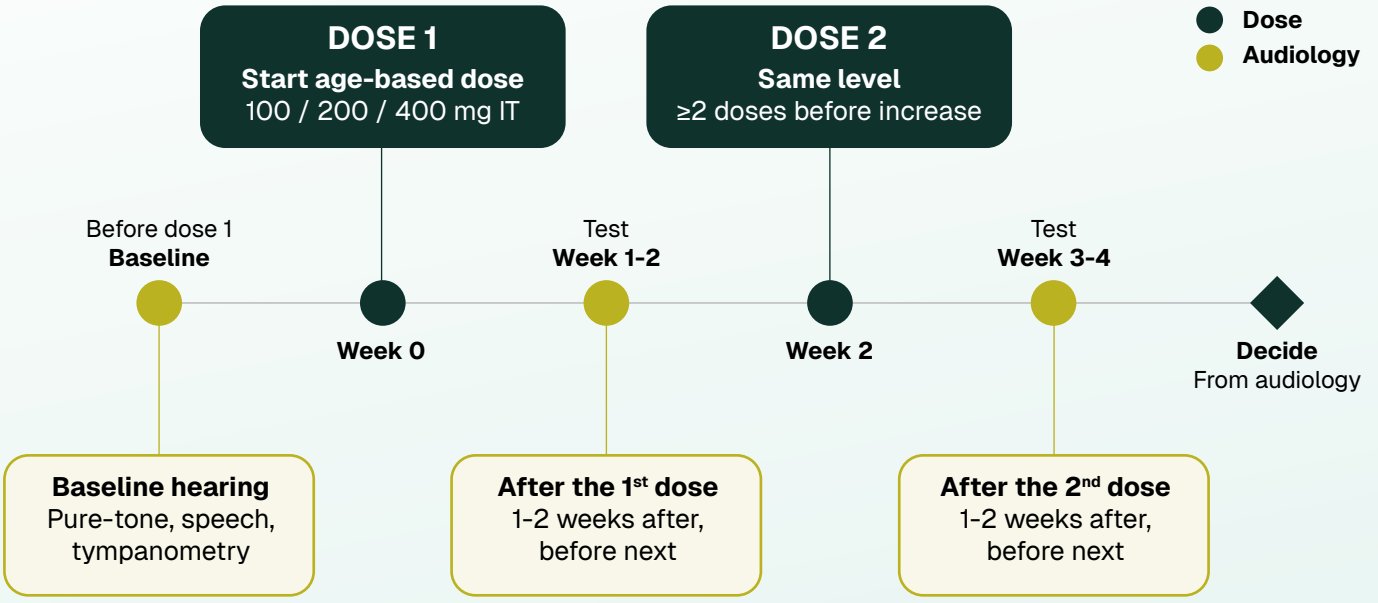
WARNINGS AND PRECAUTIONS

Hearing Impairment Including Permanent Hearing Loss: LEMBAZ-treated patients experienced permanent hearing impairment that ranged from mild to profound hearing loss, including in some patients with normal hearing at baseline. Do not increase the dosage if audiology results show hearing impairment below 4,000 Hz that exceeds the thresholds defined in the full Prescribing Information. In patients with LEMBAZ-associated hearing impairment, data are insufficient to determine whether continuing or lowering the dose affects the risk of further hearing impairment.

Please see Important Safety Information on page 7 and the full Prescribing Information, including Boxed WARNING.

Dose Management

Audiology-guided titration at each dosage level



Week labels are illustrative. The label specifies dosing every 2 weeks, with audiology 1 to 2 weeks after the first and second dose at each dosage level.

Dosage modifications for hearing loss

When a patient remains at the same Lembaz dosage level for 2 consecutive doses or has reached the maximum dosage, perform comprehensive audiology evaluations at 1-month intervals until 3 consecutive, stable audiology results are obtained and then repeat the comprehensive audiology evaluation every 12 months.

Dosage and administration modifications due to ataxia or fatigue

In patients with severe ataxia or fatigue associated with Lembaz treatment, consider reducing the Lembaz dosage. If the Lembaz dosage is decreased due to severe ataxia or fatigue and the ataxia or fatigue becomes mild or resolves, consider increasing the Lembaz dosage. Subsequently, if the ataxia or fatigue worsens, consider decreasing the dosage.

IMPORTANT SAFETY INFORMATION (cont'd)

WARNINGS AND PRECAUTIONS (cont'd)

Ataxia: LEMBAZ can cause ataxia, including fall, abnormal gait and/or coordination, dizziness, and hypotonia. This may occur shortly after a dose, typically in the first few days. Severe ataxia requiring hospitalization has occurred. Ataxia may be more severe at higher doses. Monitor patients for ataxia severity and consider reducing the dosage to reduce ataxia severity.

Dose Management (cont'd)

May increase the dose:

NO BASELINE IMPAIRMENT

- No hearing impairment observed at frequencies below 4,000 Hz, OR
- Hearing impairment observed but with a hearing threshold less than or equal to 40 dB HL at frequencies below 4,000 Hz

BASELINE MILD TO MODERATE

- No hearing threshold shift at 2 consecutive frequencies below 4,000 Hz, OR
- Hearing threshold shift that is not greater than 15 dB at 2 consecutive frequencies below 4,000 Hz

Do not increase the dose:

NO BASELINE IMPAIRMENT

Hearing impairment with a threshold greater than 40 dB HL at frequencies below 4,000 Hz (hearing impairment in the speech hearing range).

BASELINE MILD TO MODERATE

Hearing threshold shift of greater than 15 dB at 2 consecutive frequencies below 4,000 Hz.

In patients with Lembaz-associated hearing impairment, there are insufficient data to determine if continuing Lembaz treatment at the current dosage level increases the risk of further hearing impairment or if lowering the Lembaz dosage reduces the risk of further hearing impairment

Maintenance monitoring

Begins once stable across 2 dosage levels or at the maximum

Audiology	Audiology	Audiology	Audiology
Month 1 Monthly check	**Month 2** Monthly check	**Month 3** Monthly check	**Then every 12 months** Once 3 stable results

Dosing continues every 2 weeks at the established dose

MISSED DOSE REMINDER

If one or more doses are missed, **resume treatment as soon as possible while maintaining the 2-week dosing interval**. Do not administer an extra dose to make up for a missed dose.

IMPORTANT SAFETY INFORMATION (cont'd)

WARNINGS AND PRECAUTIONS (cont'd)

Fatigue: LEMBAZ can cause fatigue, including asthenia, fatigue, malaise, and somnolence. This may occur shortly after a dose, typically in the first few days. Severe fatigue requiring hospitalization has occurred. Fatigue may be more severe at higher doses. Monitor patients for fatigue severity and consider reducing the dosage to reduce fatigue severity.

Preparing for Administration

1. Visually inspect the solution in the vials for particulate matter and discoloration. The solution should be clear and colorless. Discard Lembaz if the solution is discolored or if visible particulate matter is present.
2. Withdraw the required volume of Lembaz from the vial and discard unused contents of the vial.
3. Dilute Lembaz with 0.9% Sodium Chloride Injection (preservative-free) to a total administration volume of 3 mL, 5 mL, or 10 mL based on patient's weight. For patients who weigh 7 kg to less than 12 kg treated with Lembaz 600-mg dose, do not dilute Lembaz (a volume of 3 mL is administered undiluted in these patients).
4. Attach a syringe cap to the syringe if not administered immediately.

Administration Volumes

Weight (kg)	Dose (mg)	Volume of Lembaz (mL)	Volume of 0.9% saline (mL)	Total volume for administration (mL)
7 to <12	100	0.5	2.5	3
7 to <12	200	1	2	3
7 to <12	300	1.5	1.5	3
7 to <12	400	2	1	3
7 to <12	500	2.5	0.5	3
7 to <12	600*†	3	0	3
12 to 25	200	1	4	5
12 to 25	300	1.5	3.5	5
12 to 25	400	2	3	5
12 to 25	500	2.5	2.5	5
12 to 25	600	3	2	5
12 to 25	750	3.75	1.25	5
12 to 25	900*	4.5	0.5	5
>25	200	1	9	10
>25	300	1.5	8.5	10
>25	400	2	8	10
>25	500	2.5	7.5	10
>25	600	3	7	10
>25	750	3.75	6.25	10
>25	900*	4.5	5.5	10

*Maximum dose for weight.

†For patients who weigh 7 kg to less than 12 kg on the Lembaz 600-mg dose, the Lembaz dose volume of 3 mL is administered undiluted.

IMPORTANT SAFETY INFORMATION (cont'd)

ADVERSE REACTIONS

The most common adverse reactions (reported in ≥25% of patients) were hearing impairment, ataxia, fatigue, vomiting, back pain, headache, pyrexia, diarrhea, nausea, and cough.

Administration Instructions



Apply local anesthetic (topical and subcutaneous lidocaine) to the skin.



Consider sedation as indicated by the clinical condition of the patient.



If there is difficulty accessing the intrathecal space, consider imaging to guide intrathecal administration.



Prior to administration, remove up to 5 mL of cerebrospinal fluid.



Administer Lembaz as an intrathecal bolus injection over 1 to 2 minutes using a spinal needle. Do not administer in areas of the skin where there are signs of injection or inflammation.



After administration, keep patients in the Trendelenburg position (head down at 15 to 30 degrees) for approximately 40 minutes. If unable to tolerate the Trendelenburg position, keep the patient flat as tolerated for about 40 minutes.

If you have medical questions, call the Medical Information Call Center:

844-237-3600, prompt 1

medinfo@berentx.com

If you have ordering questions, contact CuraScript SD by Evernorth:

877-900-9223

866-628-8942

Wholesalefax@curascript.com

curascriptsd.com

IMPORTANT SAFETY INFORMATION (cont'd)

To report SUSPECTED ADVERSE REACTIONS, contact Mandos, LLC at 1-844-237-3600 or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

Administer LEMBAZ intrathecally by, or under the supervision of, a healthcare provider experienced in performing lumbar punctures.

Please see Important Safety Information on page 7 and the full Prescribing Information, including Boxed WARNING.

IMPORTANT SAFETY INFORMATION

WARNING: HEARING IMPAIRMENT INCLUDING PERMANENT HEARING LOSS

Treatment with LEMBAZ has resulted in permanent hearing impairment.

- **Prior to LEMBAZ use, perform a comprehensive audiology evaluation (e.g., pure tone audiometry, speech recognition, tympanometry) to establish baseline hearing levels.**
- **During LEMBAZ treatment, modify LEMBAZ dosage based on comprehensive audiology evaluations as follows:**
  - **To determine the subsequent dosage level, repeat comprehensive audiology evaluation 1 to 2 weeks after the first LEMBAZ dose at each dosage level but before the next dose, and then 1 to 2 weeks after the second LEMBAZ dose at each dosage level but before the next dose.**
  - **When a patient remains at the same LEMBAZ dosage level for 2 consecutive doses or has reached the maximum dosage, perform comprehensive audiology evaluations at one-month intervals until 3 consecutive, stable audiology results are obtained, and then repeat the comprehensive audiology evaluation every 12 months.**

WARNINGS AND PRECAUTIONS

Hearing Impairment Including Permanent Hearing Loss: LEMBAZ-treated patients experienced permanent hearing impairment that ranged from mild to profound hearing loss, including in some patients with normal hearing at baseline. Do not increase the dosage if audiology results show hearing impairment below 4,000 Hz that exceeds the thresholds defined in the full Prescribing Information. In patients with LEMBAZ-associated hearing impairment, data are insufficient to determine whether continuing or lowering the dose affects the risk of further hearing impairment.

Ataxia: LEMBAZ can cause ataxia, including fall, abnormal gait and/or coordination, dizziness, and hypotonia. This may occur shortly after a dose, typically in the first few days. Severe ataxia requiring hospitalization has occurred. Ataxia may be more severe at higher doses. Monitor patients for ataxia severity and consider reducing the dosage to reduce ataxia severity.

Fatigue: LEMBAZ can cause fatigue, including asthenia, fatigue, malaise, and somnolence. This may occur shortly after a dose, typically in the first few days. Severe fatigue requiring hospitalization has occurred. Fatigue may be more severe at higher doses. Monitor patients for fatigue severity and consider reducing the dosage to reduce fatigue severity.

ADVERSE REACTIONS

The most common adverse reactions (reported in ≥25% of patients) were hearing impairment, ataxia, fatigue, vomiting, back pain, headache, pyrexia, diarrhea, nausea, and cough.

To report SUSPECTED ADVERSE REACTIONS, contact Mandos, LLC at 1-844-237-3600 or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

Administer LEMBAZ intrathecally by, or under the supervision of, a healthcare provider experienced in performing lumbar punctures.

Please see full Prescribing Information, including Boxed WARNING.

Lembaz™ Family Support

If you need support, reach out to your Access & Reimbursement Manager (ARM) or Lembaz™ Family Support:

844-237-3600

855-595-2575

LembazFamilySupport@berentx.com

Monday-Friday, 8am-8pm ET

Reference: Lembaz Prescribing Information. Beren Therapeutics P.B.C.

This material is intended for distribution to healthcare professionals and sites of care only.

©2026 Beren Therapeutics P.B.C. All rights reserved. Beren, Mandos, and Lembaz and their respective logos are trademarks of Beren Therapeutics P.B.C.

PRO-1444-US-EN-v2, November 2026

beren™

Lembaz™ (adrabetadex) injection