

Brineura

Bahrain · access guide

Brineura (cerliponase alfa) for a Bahraini family with a child diagnosed with CLN2 Batten disease: what the pathway looks like in 2026 via cross-border referral to Sidra Doha or KFSHRC Riyadh

By Reserve Meds clinical & regulatory team. Last reviewed 2026-05-20.

A Bahraini family of a child newly diagnosed with CLN2 disease, the classic late-infantile form of Batten disease, walks into this decision with one operational reality that needs to be on the table from the first conversation. Bahrain does not have an in-country paediatric neurosurgery centre capable of placing an intracerebroventricular reservoir for ultra-rare paediatric ERT, and Bahrain does not have a paediatric infusion centre delivering ICV-route ERT every 2 weeks. The realistic pathway for Brineura is cross-border referral, and the natural anchor is Sidra Medicine in Doha. KFSHRC Riyadh is the second cross-border option.

This page is the first honest read you get on Brineura for a Bahraini family. We will be specific about what CLN2 disease is, why the cross-border arrangement is the operative model, what the NHRA regulatory pathway looks like in 2026, what it costs in BHD and US dollars, and what life looks like for a family settling into this therapy with the procedural component at Sidra and continuity-of-care in Bahrain.

What CLN2 disease is, in plain terms

CLN2 disease, also called late-infantile neuronal ceroid lipofuscinosis type 2 or classic late-infantile Batten disease, is an autosomal recessive lysosomal storage disorder. The CLN2 / TPP1 gene normally produces an enzyme called tripeptidyl peptidase-1 that breaks down peptide fragments inside lysosomes in neurons. When the gene is faulty, the substrate accumulates, and the accumulation damages the brain.

The disease usually presents between ages 2 and 4 with seizures and language regression as the earliest signs. Untreated children lose ambulation, lose meaningful speech, lose vision, develop intractable epilepsy, and become bedbound, with median age at death of approximately 8 to 12 years.

The pivotal Schulz et al. trial published in the New England Journal of Medicine in 2018 demonstrated that intracerebroventricular cerliponase alfa slowed the decline in motor and language function compared to a matched natural-history cohort. The therapy preserves function. It does not restore function that has already been lost. The earlier therapy begins after diagnosis, the more function is preserved.

Why the cross-border arrangement is the operative model

For ICV-route paediatric ERT, the operational requirements are unusually specific. The centre needs paediatric neurology with experience in CLN2 disease management. It needs paediatric neurosurgery capable of placing an Ommaya-style reservoir under general anaesthesia in a child. It needs paediatric anaesthesia. It needs a paediatric infusion service delivering sterile intraventricular infusions every 2 weeks for the duration of the child's life. It needs paediatric pharmacy with cold-chain handling for biologics. And it needs the MDT alignment across all of those services on every single case.

Bahrain has good paediatric neurology services at Salmaniya Medical Complex, King Hamad University Hospital, and BDF Hospital, and the team at SMC paediatric neurology coordinates the diagnostic confirmation, the family-facing care, and the in-Bahrain workup. What Bahrain does not have is a paediatric neurosurgery centre that has placed Ommaya-style intraventricular reservoirs for ultra-rare paediatric ERT, and Bahrain does not have a centre that delivers ICV-route ERT every 2 weeks. The realistic pathway is cross-border.

The two cross-border anchors are:

1. **Sidra Medicine, Doha.** 90 minutes by air, paediatric-only mandate, paediatric neurology, paediatric neurosurgery, paediatric anaesthesia, and paediatric infusion infrastructure on one campus. The natural fit for Bahraini families. 2. **KFSHRC Riyadh.** Paediatric neurology under Dr Brahim Tabarki has the most CLN2 case-series experience in the region; KAMC Riyadh paediatric neurosurgery handles the Ommaya placement; paediatric infusion at KFSHRC. A second strong cross-border option.

For most Bahraini families, Sidra is the closer and operationally simpler option, particularly because the q2-weekly infusion calendar means travel back and forth for the duration of therapy.

The intracerebroventricular route and the Ommaya reservoir

Brineura is not an IV drug. Intravenous cerliponase alfa would not cross the blood-brain barrier. The drug is infused directly into the cerebrospinal fluid via a surgically-implanted intraventricular reservoir placed by a paediatric neurosurgeon under general anaesthesia in a separate admission before therapy starts. A catheter runs from the reservoir under the scalp into a lateral cerebral ventricle.

After the device is in place, every infusion of Brineura is delivered through it under sterile conditions in a paediatric infusion setting. 300 mg of cerliponase alfa diluted to 10 mL, infused at 2.5 mL per hour over approximately 4.5 hours, followed by intraventricular electrolytes flush, every 2 weeks.

The Ommaya placement happens at the cross-border centre (Sidra or KFSHRC) as the first inpatient event of therapy. Subsequent infusions also happen at that centre. Between infusions, your child is at home in Bahrain under the continuity-of-care arrangement with SMC paediatric neurology.

The workup that decides eligibility and shapes the plan

The Bahrain-side workup is co-managed by SMC paediatric neurology (or King Hamad University Hospital or BDF Hospital, depending on your family's primary paediatric clinic) and the cross-border centre.

Five components.

First, definitive diagnostic confirmation of CLN2 disease. Deficient TPP1 enzyme activity in leukocytes, fibroblasts, or dried blood spot, AND confirmation of two pathogenic variants in the CLN2 / TPP1 gene by sequencing. Most Bahraini CLN2 diagnoses are confirmed through international reference laboratories with the sample collected on Bahrain soil. SMC paediatric neurology routes the assay.

Second, paediatric neurology baseline. Motor-language summary score on the modified Hamburg CLN2 scale. Baseline seizure burden and anti-seizure medication. Baseline vision and developmental status. Co-recorded between SMC and the cross-border centre.

Third, paediatric neurosurgery consultation at the cross-border centre. Brain MRI; anaesthesia review.

Fourth, baseline brain MRI, baseline ECG, baseline CSF studies at the time of reservoir placement.

Fifth, multidisciplinary team discussion between SMC paediatric neurology and the cross-border centre's paediatric neurosurgery and infusion team.

The Bahraini regulatory pathway and cross-border supply

The Bahrain National Health Regulatory Authority (NHRA) is the federal regulator. Brineura's NHRA registration status is mixed and the realistic pathway is one of two: (a) the named-patient mechanism filed in Bahrain with the drug supplied to the cross-border centre, or (b) the cross-border centre's own regulatory mechanism (Qatar MOPH or Saudi SFDA) handles the supply with Bahrain referring the patient.

In practice, the second arrangement is more common: the Bahraini child is referred to Sidra or KFSHRC, the cross-border centre's pharmacy handles the supply under its own regulatory framework, and Bahrain SMC continues the home-country continuity of care between infusions.

For Bahraini nationals, the Supreme Council of Health and the public health-funding pathway include mechanisms for cross-border paediatric specialty care. SMC paediatric neurology files the cross-border referral case alongside the clinical recommendation; the funding decision is made through the Bahrain MoH referral committee.

The access pathway for a Bahraini family: step by step

1. Diagnostic confirmation of CLN2 (enzyme assay + gene sequencing) through SMC paediatric neurology or via international reference laboratory. 2. Bahrain-side paediatric neurology baseline at SMC, King Hamad University Hospital, or BDF Hospital. 3. Cross-border referral package prepared by SMC paediatric neurology with Reserve Meds providing the documentation; sent to Sidra Medicine Doha (primary) or KFSHRC Riyadh (alternative). 4. Bahrain MoH cross-border funding case filed in parallel. 5. Cross-border centre paediatric neurology and neurosurgery MDT; Ommaya reservoir placement admission at the cross-border centre. 6. First Brineura infusion at the cross-border centre. 7. Stable every-2-week infusion calendar with cross-border travel; Bahrain-side continuity at SMC. 8. Ongoing surveillance.

The cost conversation, in the form a Bahraini family needs

The 2026 indicative annual drug cost is approximately USD 730,000 to USD 750,000.

Annual cost of care in stable years (Year 2 onwards) is approximately USD 850,000 to USD 1.05 million, or approximately BHD 320,000 to BHD 396,000, excluding the travel and accommodation costs of the q2-weekly cross-border infusion calendar. Year 1 adds the Ommaya reservoir placement admission at the cross-border centre, approximately BHD 41,000 to BHD 62,000.

Travel and accommodation for the q2-weekly cross-border infusion calendar adds approximately BHD 25,000 to BHD 50,000 per year depending on the cross-border centre, the family's accommodation choices, and the duration of each visit. For Sidra (90-minute flight), most families do a same-day or one-night trip; for KFSHRC Riyadh (longer travel), most families do a 1 to 2 night arrangement.

When we issue a quote at intake, we separate every line. We do not put a markup on the manufacturer's drug price. We charge a transparent coordination fee for the case-management work.

For Bahraini nationals, the Bahrain MoH cross-border funding pathway is the realistic conversation. SMC paediatric neurology and the rare-disease desk handle the funding case. For expatriate residents, the cost picture is typically a mix of insurance, employer support where applicable, and family-pay.

Safety: what to watch for

- **Device-related infection.** Meningitis or ventriculitis is rare but the most clinically serious complication. Cross-border centre and SMC continuity-of-care team monitor scalp condition, temperature, behaviour change, and CSF on suspicion. - **Infusion reactions.** Pyrexia, vomiting, and hypersensitivity. Anaphylaxis-management capability is on site at the cross-border centre for every infusion. - **Seizures.** CLN2 children typically have a baseline seizure disorder. SMC paediatric neurology optimises the anti-seizure medication regimen in coordination with the cross-border centre. - **CSF leak or reservoir malfunction.** Uncommon but possible; revision surgery is handled at the cross-border centre. - **ECG changes.** Typically minor.

What Reserve Meds does, and what we do not do

Reserve Meds is a US-based concierge coordinator for cross-border and complex paediatric specialty medicine. For a Bahraini family pursuing Brineura via cross-border referral to Sidra or KFSHRC, our scope is the diagnostic-confirmation pathway routing, the Bahrain-side MDT documentation packet, the cross-border referral package preparation, coordination with the cross-border centre's pharmacy and intake, the sourcing logistics through DSCSA-compliant chain of custody, the family-side logistics for the Ommaya placement admission and the q2-weekly infusion calendar, and named case-lead coordination from intake through the establishment of a stable every-2-week infusion routine. We coordinate the Bahrain MoH cross-border funding case.

Reserve Meds is not your child's prescriber. We do not practise medicine. We do not perform the neurosurgical placement. We do not own or operate Sidra or KFSHRC. We do not manufacture Brineura. We are not your insurer.

We work cash-pay where applicable. Our coordination fee is disclosed in writing.

Frequently asked parent questions

Q: Why does Bahrain not have an in-country centre? The case count is too low to justify standing up a paediatric neurosurgery + paediatric ICV-infusion infrastructure for one or two ultra-rare diseases. Cross-border referral to Sidra or KFSHRC is the operational model and has been workable for other ultra-rare paediatric cases.

Q: Can the q2-weekly travel be sustained? Yes. Most Bahraini families settle into a Sidra-based same-day or one-night cycle within the first 2 to 3 months. The school year, work schedule, family events, and Ramadan are planned around the calendar. It is not easy but it is workable.

Q: Can the infusions ever shift to Bahrain? Not under current Bahrain infrastructure. If Bahrain establishes paediatric neurosurgery and paediatric ICV-infusion capability in the future, the conversation about onshoring the infusion calendar would open. For now, the cross-border arrangement is the realistic model.

Q: What about religious considerations? Brineura is recombinant CHO-produced enzyme, not derived from animal tissue or human plasma. The Islamic-bioethics consensus on life- and function-preserving paediatric therapies is broadly permissive.

Q: What about siblings? CLN2 is autosomal recessive. Carrier testing for siblings and for the extended family is offered by SMC paediatric neurology genetics counselling.

Reserve Meds's role

US-based concierge coordinator for cross-border specialty medicine. We are not the prescriber, not the dispensing pharmacy, and not the manufacturer. All clinical decisions remain with your treating physician.

Reserve Meds

reserved for you.

Composite case examples. This document is for general information only and does not constitute medical advice. Please consult your treating physician.

Reserve Meds is in pre-launch. Published timelines and cost ranges are indicative, not guarantees.

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