**Brineura**

Kuwait · access guide

# Brineura (cerliponase alfa) for a Kuwaiti family with a child diagnosed with CLN2 Batten disease: what the pathway looks like in 2026 via cross-border referral and MoH Foreign Medical Treatment funding

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

A Kuwaiti 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 and one financial mechanism that frame the rest of the conversation. Kuwait does not have an in-country paediatric neurosurgery + paediatric ICV-infusion centre delivering ultra-rare paediatric ERT every 2 weeks. The realistic pathway for Brineura is cross-border referral. The natural anchors are Sidra Medicine in Doha and KFSHRC Riyadh. The financial mechanism for Kuwaiti nationals is the Ministry of Health Foreign Medical Treatment (FMT) funding pathway, which has delivered ultra-rare paediatric specialty care abroad for many Kuwaiti families and is the realistic conversation for Brineura cost coverage.

This page is the first honest read you get on Brineura for a Kuwaiti family. We will be specific about what CLN2 disease is, why the cross-border arrangement is the operative model, what the Kuwait MoH FMT funding pathway looks like in practice, what it costs in KWD and US dollars, and what life looks like for a family settling into this therapy with the procedural component at Sidra or KFSHRC and continuity-of-care in Kuwait.

## What CLN2 disease is, in plain terms

CLN2 disease is an autosomal recessive lysosomal storage disorder caused by deficiency of the TPP1 enzyme. 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. Initiating before significant decline is the operative rule.

In Kuwait, the diagnostic infrastructure for CLN2 is centred at the Kuwait Medical Genetics Centre (KMGC), which has handled the enzyme-activity and gene-sequencing workup for several paediatric lysosomal storage disorders. KMGC routes the diagnostic confirmation and refers the family to Sabah Hospital paediatric neurology, Mubarak Al-Kabeer Hospital paediatric neurology, or Al Sabah Hospital paediatric neurology for the clinical pathway.

## 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.

Kuwait has good paediatric neurology services and Kuwait has paediatric neurosurgery. What Kuwait does not have, today, is an established programme delivering ICV-route paediatric ERT every 2 weeks for ultra-rare diseases. The case count is too low to support standing up a dedicated programme, and the realistic pathway is cross-border referral.

The two cross-border anchors are:

1. **Sidra Medicine, Doha.** Paediatric-only mandate, paediatric neurology, paediatric neurosurgery, paediatric anaesthesia, and paediatric infusion infrastructure on one campus. The natural fit for Kuwaiti families given the regional flight time and the paediatric-specific infrastructure. 2. **KFSHRC Riyadh.** Paediatric neurology under Dr Brahim Tabarki has the most CLN2 case-series experience in the region; paediatric neurosurgery at KAMC Riyadh handles the Ommaya placement.

For most Kuwaiti families, Sidra is the operationally simpler option. The MoH FMT mechanism has historically directed Kuwaiti ultra-rare paediatric cases to Sidra and to KFSHRC; Reserve Meds coordinates whichever anchor the MoH FMT committee approves.

## 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 at the cross-border centre.

After the device is in place, every infusion is delivered through it under sterile conditions in the paediatric infusion setting at the cross-border centre. 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 workup that decides eligibility and shapes the plan

The Kuwait-side workup is co-managed by KMGC and the family's paediatric neurology home (typically Sabah Hospital, Mubarak Al-Kabeer Hospital, Al Adan Hospital, or one of the private-sector paediatric services), with the cross-border centre's paediatric neurology and neurosurgery picking up the procedural component.

Five components.

**First, definitive diagnostic confirmation.** Deficient TPP1 enzyme activity and confirmation of two pathogenic variants in the CLN2 / TPP1 gene. KMGC routes the assay through reference laboratories.

**Second, paediatric neurology baseline.** Motor-language summary score, baseline seizure burden and anti-seizure medication, baseline vision and developmental status.

**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 discussion** between Kuwait-side and cross-border teams.

## The Kuwait MoH Foreign Medical Treatment funding pathway

The Foreign Medical Treatment pathway, administered through the Kuwait Ministry of Health, is the established mechanism for funding paediatric specialty care abroad when the case cannot be delivered in-country. For ultra-rare paediatric ERT, FMT has historically approved cross-border referrals to Sidra Medicine, to KFSHRC Riyadh, and to international centres.

The FMT case is filed by the Kuwait-side paediatric neurologist with the clinical recommendation, the diagnostic confirmation, and the cross-border centre's acceptance letter. The FMT committee reviews the case and authorises funding for the cross-border centre's services. Reserve Meds coordinates the documentation packet for the FMT filing in cooperation with the Kuwait-side paediatric neurologist.

FMT approval timelines vary; complete, well-documented ultra-rare paediatric files typically clear in six to twelve weeks. We do not promise a specific timeline but we can give you a realistic forecast based on similar cases we have coordinated.

## The Kuwait DFC registration pathway for drug import (if applicable)

If the FMT mechanism does not apply (for example, for expatriate families who are not Kuwaiti nationals), the alternative is a Kuwait MoH Drug and Food Control (DFC) named-patient filing for Brineura import to Kuwait, paired with a paediatric centre arrangement. In practice this is rarely the chosen route for Brineura because the in-country ICV-infusion infrastructure is not in place. Most expatriate Kuwaiti residents pursue Brineura through one of two routes: (a) commercial insurance plus cross-border arrangement to Sidra or KFSHRC at family expense, or (b) return to home country if applicable.

## The access pathway for a Kuwaiti family: step by step

1. Diagnostic confirmation of CLN2 through KMGC and reference laboratory. 2. Kuwait-side paediatric neurology baseline at Sabah, Mubarak Al-Kabeer, Al Adan, or private-sector paediatric clinic. 3. Cross-border referral package prepared with Reserve Meds providing the documentation; sent to Sidra Medicine Doha (primary) or KFSHRC Riyadh (alternative). 4. Kuwait MoH FMT funding case filed in parallel. 5. Cross-border centre 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; Kuwait-side continuity at the home paediatric neurology clinic. 8. Ongoing surveillance.

## The cost conversation, in the form a Kuwaiti 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 KWD 261,000 to KWD 323,000, excluding travel and accommodation. Year 1 adds the Ommaya reservoir placement admission at the cross-border centre, approximately KWD 34,000 to KWD 50,000.

Travel and accommodation for the q2-weekly cross-border calendar adds approximately KWD 18,000 to KWD 35,000 per year depending on the cross-border centre, the family's accommodation choices, and the duration of each visit.

For Kuwaiti nationals approved through the MoH FMT pathway, the cross-border centre's services (drug, infusion delivery, neurosurgical admission, paediatric neurology surveillance) are typically covered through the FMT mechanism. The funding case includes Reserve Meds coordination fees disclosed in writing. For expatriate residents, the cost picture is family-pay or commercial-insurance based.

When we issue a quote at intake, we separate every line. We do not put a markup on the manufacturer's drug price.

## Safety: what to watch for

- **Device-related infection.** Meningitis or ventriculitis is rare but the most clinically serious complication. Cross-border centre and Kuwait-side 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. - **Seizures.** CLN2 children typically have a baseline seizure disorder. Kuwait-side 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 Kuwaiti family pursuing Brineura via cross-border referral to Sidra or KFSHRC, our scope is the diagnostic-confirmation pathway routing through KMGC, the Kuwait-side paediatric neurology MDT documentation packet, the cross-border referral package preparation, the Kuwait MoH FMT funding case documentation, coordination with the cross-border centre's pharmacy and intake, the sourcing logistics through DSCSA-compliant chain of custody where applicable, 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.

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 or the MoH FMT decision-maker.

We work cash-pay where applicable. Our coordination fee is disclosed in writing and included in the MoH FMT funding case where applicable.

## Frequently asked parent questions

**Q: Why does Kuwait not have an in-country centre?** The case count is too low to justify a standing paediatric neurosurgery + paediatric ICV-infusion programme for ultra-rare diseases. The MoH FMT pathway is the established mechanism for these cases.

**Q: How long does FMT approval take?** For complete, well-documented ultra-rare paediatric files, typically six to twelve weeks. We can give you a realistic forecast based on similar cases.

**Q: Can the q2-weekly travel be sustained?** Yes. Most Kuwaiti families settle into a cross-border cycle within the first 2 to 3 months. The school year, work schedule, family events, and Ramadan are planned around the calendar.

**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 part of the genetics counselling that KMGC offers.

### *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.

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