

Brineura

Dubai · access guide

Brineura (cerliponase alfa) for a Dubai family with a child diagnosed with CLN2 Batten disease: what the pathway looks like in 2026 via Dubai-side workup and cross-emirate referral to SKMC or Cleveland Clinic Abu Dhabi

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

A Dubai-resident family of a child newly diagnosed with CLN2 disease faces the same clinical and operational picture as any UAE family, with one practical specific. Dubai has strong paediatric neurology services and excellent family-facing paediatric care, but the paediatric neurosurgery + paediatric ICV-infusion infrastructure for ultra-rare paediatric ERT is concentrated in Abu Dhabi. The realistic pathway for Brineura is Dubai-side workup with cross-emirate referral to SKMC, Cleveland Clinic Abu Dhabi, or SSMC for the Ommaya reservoir placement and the every-2-week infusion calendar.

This page is the first honest read you get on Brineura for a Dubai family. We will be specific about what CLN2 disease is, why the cross-emirate arrangement is the operative model, what the UAE EDE regulatory pathway looks like in 2026, what it costs in AED and US dollars, and what life looks like for a family settling into this therapy with the procedural component in Abu Dhabi and continuity-of-care in Dubai.

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 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 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, the more function is preserved.

Why the cross-emirate 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.

In the UAE in 2026, the centres that combine all of these on one campus are concentrated in Abu Dhabi: - **Sheikh Khalifa Medical City (SKMC), Abu Dhabi.** Paediatric neurology and paediatric neurosurgery on the same campus; the most documented UAE site for paediatric rare-disease infrastructure. - **Cleveland Clinic Abu Dhabi.** Paediatric neurology and neurosurgery with MD Anderson-style multidisciplinary depth. - **Sheikh Shakhbout Medical City (SSMC), Abu Dhabi.** Paediatric neurology, neurosurgery, and rare-disease pharmacy.

Dubai-side paediatric services are strong on the family-facing and continuity-of-care side: - **Al Jalila Children's Specialty Hospital.** Paediatric neurology depth; the natural Dubai-side anchor for paediatric rare-disease workup. - **Mediclinic City Hospital.** Paediatric neurology and subspecialty depth. - **American Hospital Dubai.** Paediatric service. - **NMC Specialty Hospital.** Paediatric service.

The realistic Dubai-family arrangement is: Dubai-side diagnostic confirmation and workup at Al Jalila or Mediclinic City; cross-emirate referral to SKMC, Cleveland Clinic Abu Dhabi, or SSMC for the Ommaya placement and the q2-weekly infusions; Dubai-side continuity for the in-between weeks. Reserve Meds coordinates the cross-emirate handoff.

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 Abu Dhabi cross-emirate centre.

After the device is in place, every infusion is delivered through it under sterile conditions in the paediatric infusion setting at the Abu Dhabi 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

Five components, co-managed Dubai-side and cross-emirate-side.

First, definitive diagnostic confirmation. Deficient TPP1 enzyme activity and confirmation of two pathogenic variants in the CLN2 / TPP1 gene. Al Jalila molecular genetics or Mediclinic City reference laboratory can route both prongs.

Second, paediatric neurology baseline. Motor-language summary score, baseline seizure burden and anti-seizure medication, baseline vision and developmental status. Recorded at Al Jalila or Mediclinic City; shared with the Abu Dhabi cross-emirate centre.

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

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

Fifth, multidisciplinary discussion between Dubai-side paediatric neurology and the Abu Dhabi cross-emirate team.

The UAE EDE regulatory pathway and DHA emirate-level layer

The Emirates Drug Establishment is the federal authority that the cross-emirate centre's import pharmacy files through. Brineura's EDE registration status is mixed and the realistic pathway is the named-patient mechanism filed through SKMC, Cleveland Clinic Abu Dhabi, or SSMC pharmacy. The Department of Health Abu Dhabi adds the emirate-level layer for the procedural and infusion components.

For Dubai residents, the Dubai Health Authority (DHA) maintains the emirate-level layer for the Dubai-side workup and continuity-of-care, but the EDE filing is handled by the cross-emirate centre because that is where the drug is dispensed and administered. DHA Pharmaceutical Affairs and DoH Abu Dhabi coordinate the cross-emirate referral.

EDE coordination on a complete, well-documented file runs four to eight weeks from filing to first dose.

The access pathway for a Dubai family: step by step

1. Diagnostic confirmation of CLN2 (enzyme assay + gene sequencing) at Al Jalila Children's, Mediclinic City, or international reference laboratory. 2. Dubai-side paediatric neurology baseline at Al Jalila or Mediclinic City. 3. Cross-emirate referral package prepared with Reserve Meds providing the documentation; sent to SKMC, Cleveland Clinic Abu Dhabi, or SSMC. 4. EDE named-patient filing through the cross-emirate centre's pharmacy. 5. Cross-emirate centre MDT; Ommaya reservoir placement admission at SKMC, Cleveland Clinic Abu Dhabi, or SSMC. 6. First Brineura infusion at the cross-emirate centre. 7. Stable every-2-week infusion calendar with Dubai-to-Abu-Dhabi travel; Dubai-side continuity at Al Jalila or Mediclinic City. 8. Ongoing surveillance.

The Dubai-to-Abu-Dhabi drive is approximately 90 minutes by car, which makes the q2-weekly arrangement materially easier than a cross-border arrangement. Most Dubai families do a same-day round trip for each infusion.

The cost conversation, in the form a Dubai 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 AED 3.12 million to AED 3.86 million. Year 1 adds the Ommaya reservoir placement admission at the cross-emirate centre, approximately AED 110,000 to AED 160,000.

Cross-emirate travel costs are modest given the same-day round-trip pattern but include either family driving costs or chauffeured transport, plus the occasional overnight stay around the Ommaya placement admission. We typically estimate AED 10,000 to AED 25,000 per year for the cross-emirate logistics.

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 disclosed in writing before any funds move.

For Emirati nationals treated at SKMC, SSMC, or under the cross-emirate DoH Abu Dhabi rare-disease pathway, much of the cost may be underwritten through the government health funding pathways. Your treating consultant will confirm whether and how. For expatriate residents, the cost picture is typically a mix of insurance coverage, employer support where applicable, and family-pay. We supply your insurer with the documentation packet at no charge.

DHA Pharmaceutical Affairs in Dubai does not typically dispense Brineura because the q2-weekly ICV infusion is delivered at the cross-emirate centre. The Dubai-side insurance conversation is about reimbursement to the family or to the cross-emirate centre for the dispensing event.

Safety: what to watch for

- **Device-related infection.** Meningitis or ventriculitis is rare but the most clinically serious complication. The cross-emirate centre and Dubai-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-emirate centre. - **Seizures.** CLN2 children typically have a baseline seizure disorder. Dubai-side paediatric neurology optimises the anti-seizure medication regimen in coordination with the cross-emirate centre. - **CSF leak or reservoir malfunction.** Uncommon but possible; revision surgery is handled at the cross-emirate 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 Dubai family pursuing Brineura via cross-emirate referral to Abu Dhabi, our scope is the diagnostic-confirmation pathway routing, the Dubai-side MDT documentation packet, the cross-emirate referral package preparation, the EDE filing in collaboration with the cross-emirate centre's pharmacy, the sourcing logistics from BioMarin's authorised distribution through DSCSA-compliant chain of custody, cold-chain shipment to the cross-emirate centre, 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 the cross-emirate centre. 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 Dubai not have an in-emirate paediatric ICV-infusion centre? The case count is too low to justify standing up a dedicated paediatric neurosurgery + paediatric ICV-infusion programme for ultra-rare diseases in Dubai when the Abu Dhabi centres can deliver. The cross-emirate model is operationally simpler than the cross-border alternative.

Q: Can the q2-weekly travel be sustained? Yes. The 90-minute drive is materially easier than the cross-border arrangements that families in Bahrain or Kuwait face. Most Dubai families settle into a same-day round-trip cycle within the first 2 to 3 months.

Q: Can the Dubai-side continuity-of-care team handle a problem between infusions? Yes. Al Jalila Children's or Mediclinic City paediatric neurology handles in-between-infusion clinical issues including seizure-management adjustment, scalp-condition monitoring, and family support, in coordination with the Abu Dhabi cross-emirate centre.

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.

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.

reservemeds.com · hello@reservemeds.com