

## Brineura

Abu Dhabi · access guide

# Brineura (cerliponase alfa) for an Abu Dhabi family with a child diagnosed with CLN2 Batten disease: what the pathway looks like in 2026 via SKMC, Cleveland Clinic Abu Dhabi, and SSMC

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

An Abu Dhabi family of a child newly diagnosed with CLN2 disease, the classic late-infantile form of Batten disease, walks into this decision in the UAE emirate that has the in-emirate paediatric infrastructure for ultra-rare paediatric ICV enzyme replacement therapy. Sheikh Khalifa Medical City, Cleveland Clinic Abu Dhabi, and Sheikh Shakhbout Medical City each combine paediatric neurology, paediatric neurosurgery, paediatric anaesthesia, and paediatric infusion services on one campus. Reserve Meds coordinates around whichever centre your treating consultant chooses.

This page is the first honest read you get on Brineura for an Abu Dhabi family. We will be specific about what CLN2 disease is, why the intracerebroventricular route matters, what the UAE EDE regulatory pathway and the DoH Abu Dhabi emirate-level layer look like in 2026, what it costs in AED and US dollars, and what life looks like for a family settling into this therapy.

## 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 in lysosomes inside brain cells, 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. Initiating before significant decline is the operative rule.

## The intracerebroventricular route and the Ommaya reservoir

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Brineura is not an IV drug. Intravenous cerliponase alfa would not cross the blood-brain barrier and would not reach the diseased neurons. Brineura is infused directly into the cerebrospinal fluid via a surgically-implanted intraventricular reservoir, often called an Ommaya reservoir or a Rickham reservoir, placed by a paediatric neurosurgeon under general anaesthesia in a separate hospital 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 capable of managing the reservoir. 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.

## Abu Dhabi paediatric infrastructure for CLN2

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- **Sheikh Khalifa Medical City (SKMC), Abu Dhabi.** Paediatric neurology and paediatric neurosurgery on the same campus. SKMC is the most documented UAE site for paediatric rare-disease infrastructure and has handled both gene therapy and other ICV-route protocols for paediatric metabolic and neurological diseases. - **Cleveland Clinic Abu Dhabi.** Paediatric neurology and neurosurgery with MD Anderson-style multidisciplinary depth; has placed Ommaya-style reservoirs for paediatric indications. - **Sheikh Shakhboub Medical City (SSMC), Abu Dhabi.** Paediatric neurology, neurosurgery, and rare-disease pharmacy. Coordinates with SKMC and Cleveland Clinic Abu Dhabi for complex paediatric cases. - **Tawam Hospital, Al Ain.** Paediatric metabolic and neurology service with long history of rare-disease care; complex neurosurgical procedures typically routed to SKMC or Cleveland Clinic Abu Dhabi. - **Burjeel Medical City, Abu Dhabi.** Paediatric subspecialty service. - **NMC Royal Khalifa City Hospital, Abu Dhabi.** Paediatric service.

For Abu Dhabi families, the realistic primary anchor is SKMC or Cleveland Clinic Abu Dhabi, with SSMC and Tawam as alternatives depending on family preference and consultant relationships.

## The workup that decides eligibility and shapes the plan

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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. The UAE-based reference laboratories and the international laboratories used by SKMC, SSMC, and Cleveland Clinic Abu Dhabi can run both prongs.

**Second, paediatric neurology baseline.** Motor-language summary score on the modified Hamburg CLN2 scale. Baseline seizure burden and current anti-seizure medication. Baseline vision and developmental status. The motor-language score becomes the primary efficacy surveillance marker.

**Third, paediatric neurosurgery consultation.** Brain MRI; anaesthesia review.

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

**Fifth, multidisciplinary team discussion.** Paediatric neurology, paediatric neurosurgery, paediatric anaesthesia, infusion-centre nursing, pharmacy, and the family.

A clinical rationale letter from your paediatric neurologist documents the diagnosis, the baseline motor-language score, the recommended treatment plan, the Ommaya placement plan, and the long-term monitoring schedule.

## **The UAE EDE regulatory pathway and DoH Abu Dhabi emirate layer**

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The Emirates Drug Establishment is the federal authority that the treating hospital's import pharmacy files through. Brineura's UAE EDE registration status is mixed and the realistic pathway is the named-patient mechanism filed via [ede.gov.ae](http://ede.gov.ae) by SKMC, Cleveland Clinic Abu Dhabi, or SSMC pharmacy on the treating paediatric neurologist's behalf. The Department of Health Abu Dhabi adds the emirate-level layer.

DoH Abu Dhabi has an established rare-disease desk and the Thiqa funding pathway covers ultra-rare paediatric biologics for Emirati nationals. The Daman funding pathway for employer-covered cases and the SEHA-network funding for SKMC and Tawam are the operational mechanisms for non-Thiqa-covered children.

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

## **The access pathway in Abu Dhabi: step by step**

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1. Diagnostic confirmation (enzyme assay + gene sequencing) on UAE soil or through reference laboratory referral. 2. Paediatric neurology MDT at SKMC, Cleveland Clinic Abu Dhabi, or SSMC. 3. Paediatric neurosurgery consultation; brain MRI; anaesthesia review. 4. EDE named-patient filing through the hospital's import pharmacy with Reserve Meds providing the documentation packet. 5. Ommaya reservoir placement admission, typically 1 to 3 inpatient days; wound healing window of 1 to 2 weeks before first infusion. 6. First Brineura infusion at the paediatric infusion centre under paediatric neurology supervision. 7. Stable every-2-week infusion routine established over the next 2 to 3 months. 8. Ongoing surveillance: motor-language score reassessment, scalp and reservoir surveillance, periodic brain MRI, seizure-management adjustment, family support.

## **The cost conversation, in the form an Abu Dhabi family needs**

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The 2026 indicative annual drug cost is approximately USD 730,000 to USD 750,000, calculated as approximately USD 28,100 per 300 mg vial times 26 infusions per year.

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 includes the Ommaya reservoir placement admission, which adds approximately AED 110,000 to AED 160,000 depending on the centre.

When we issue a quote at intake, we separate every line: drug per infusion, infusion-suite charges, neurosurgical admission charges in Year 1, monitoring labs, brain MRI surveillance, paediatric neurology visits, our coordination fee. Nothing is bundled. We do not put a markup on the manufacturer's drug price.

For Emirati nationals, the Thiqa rare-disease funding pathway through DoH Abu Dhabi is the realistic conversation. SKMC, SSMC, and Tawam coordinate the funding case alongside the EDE filing for Emirati national patients. For non-Emirati residents, the Daman commercial cover or other commercial insurance applies depending on the policy, and the cost picture is typically a mix of insurance, employer support where applicable, and family-pay. We supply your insurer with the documentation packet at no charge.

## Cross-emirate and cross-border options

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If the Abu Dhabi-side capacity is delayed or if your family prefers a regional alternative, Sidra Medicine in Doha is the closest regional paediatric centre with all the required services on one campus. KFSHRC Riyadh is the second cross-border option. Reserve Meds coordinates either alternative pattern. For most Abu Dhabi families, the in-emirate option at SKMC, Cleveland Clinic Abu Dhabi, or SSMC is operationally simpler.

## Safety: what to watch for

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- **Device-related infection.** Meningitis or ventriculitis is rare but the most clinically serious complication. Centres monitor scalp condition, temperature, behaviour change, and CSF on suspicion. - **Infusion reactions.** Pyrexia, vomiting, and hypersensitivity. Anaphylaxis-management capability is on site for every infusion. - **Seizures.** CLN2 children typically have a baseline seizure disorder. Paediatric neurology adjusts the anti-seizure medication regimen. - **CSF leak or reservoir malfunction.** Uncommon but possible; may require revision surgery. - **ECG changes.** Typically minor.

## What Reserve Meds does, and what we do not do

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Reserve Meds is a US-based concierge coordinator for cross-border and complex paediatric specialty medicine. For an Abu Dhabi family pursuing Brineura, our scope is the diagnostic-confirmation pathway routing, the paediatric neurology MDT documentation packet, the EDE filing in collaboration with SKMC, Cleveland Clinic Abu Dhabi, or SSMC pharmacy, the sourcing logistics from BioMarin's authorised distribution through DSCSA-compliant chain of custody, cold-chain shipment to the qualified centre, the family-side logistics for the Ommaya placement admission, 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 infusion 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

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**Q: Will Brineura cure my child?** No. It slows the decline. The earlier therapy starts, the more function is preserved.

**Q: Which Abu Dhabi centre is best?** SKMC and Cleveland Clinic Abu Dhabi are the two centres with the most documented paediatric neurosurgery and paediatric ICV-protocol experience. SSMC and Tawam are alternatives. Your treating paediatric neurologist will recommend based on the MDT.

**Q: Can the infusions ever be done in Dubai or elsewhere?** For ICV-route paediatric ERT, the q2-weekly infusion is typically continued at the centre that placed the reservoir. Reservoir-related management is best handled by the team that knows the device. Continuity of place matters here more than for IV ERT.

**Q: What if we miss an infusion?** Missed infusions are not made up by doubling subsequent doses. The q2-weekly schedule is part of the therapy.

**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.

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### **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