Brineura

United Arab Emirates · access guide

Brineura (cerliponase alfa) for a UAE family with a child diagnosed with CLN2 Batten disease: what the pathway looks like in 2026

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

A UAE family of a child newly diagnosed with CLN2 disease, the classic late-infantile form of Batten disease, walks into this decision with more than a treatment question. There is a clinical question about how far the disease has progressed and what Brineura can and cannot do at the stage your child is at. There is a regulatory question about how a paediatric intracerebroventricular enzyme replacement therapy moves into the country. There is a surgical question, because Brineura is not an IV drug; it requires an Ommaya-style reservoir to be placed into a cerebral ventricle by a paediatric neurosurgeon before therapy can start. There is a financial question that runs for the rest of your child's life. And there is a family question, often a heavy one, about what your home and your school week will look like with every-2-week ICV infusions for the foreseeable future.

This page is meant to be the first honest read you get on Brineura in the UAE, written by the team that would coordinate around your child's case if you decided you wanted operational support on the workup, the reservoir placement, the import, the infusion centre, the long-term surveillance, and the cost picture.

We will be specific about what CLN2 disease is, what the intracerebroventricular route means in practice, what the UAE regulatory pathway looks like in 2026, what it costs in AED and US dollars, where the surgery and the every-2-week infusions can be done in the UAE, 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 gene (also called TPP1) normally produces an enzyme called tripeptidyl peptidase-1, which lives inside the lysosomes of neurons and breaks down peptide fragments as part of the cell's normal recycling. When CLN2 is faulty, the substrate accumulates in lysosomes inside neurons. The accumulation is what damages the brain.

The disease usually presents between ages 2 and 4. The first signs are commonly seizures and a slowing or reversal of language development. Within months to a few years, untreated children lose ambulation, lose meaningful speech, lose vision, develop intractable epilepsy, and become bedbound. Median age at death in untreated CLN2 is approximately 8 to 12 years. This is one of the harder paediatric neurological diseases families face.

We mention this honestly at the start because it determines the window for Brineura. The pivotal data, the 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. Treated children lost on average about 0.27 motor-language scale points per 48 weeks against approximately 2.12 points per 48 weeks in untreated controls. The therapy preserves the function your child still has. It does not restore function that has already been lost. Initiating before significant decline is the operative rule.

If your child has been newly diagnosed and is still ambulant and still communicating, the conversation is about how quickly we can move. If your child has already lost ambulation, lost meaningful language, and become substantially neurologically affected, the conversation your paediatric neurologist will have with you is harder, and we will talk about it candidly.

The intracerebroventricular route: what an Ommaya reservoir is and why it matters

This is what makes Brineura operationally different from every other paediatric ERT in the Reserve Meds catalogue.

Brineura is not given as an IV infusion. Intravenous cerliponase alfa would not cross the blood-brain barrier and would not reach the diseased neurons. Instead, Brineura is infused directly into the cerebrospinal fluid via a surgically-implanted intraventricular reservoir, often called an Ommaya reservoir or a Rickham reservoir, with a catheter that runs from the reservoir under the scalp into a lateral cerebral ventricle. A paediatric neurosurgeon places the device under general anaesthesia in a separate hospital admission, typically before the first dose of Brineura. The wound is allowed to heal and the CSF stability is confirmed before the first infusion.

After the device is in place, every infusion of Brineura is delivered through it, in a paediatric infusion or day-surgery setting capable of managing the reservoir under sterile conditions. The infusion is 300 mg of cerliponase alfa diluted to 10 mL, given at 2.5 mL per hour over approximately 4.5 hours, then followed by intraventricular electrolytes flush. The frequency is every 2 weeks. There is very limited tolerance for schedule disruption. Missed infusions are not made up by doubling the next dose.

The device adds two clinical considerations. First, the small but real risk of device-related infection, particularly meningitis and ventriculitis. Centres routinely monitor the scalp condition, the temperature, and (where clinically indicated) the cerebrospinal fluid for early signs of infection. Second, the small but real risk of CSF leak or device malfunction over time, occasionally requiring revision surgery.

For UAE families, the practical implication is that you need two centres to align on the case: the paediatric neurosurgery centre that will place the reservoir, and the paediatric infusion centre that will deliver the every-2-week infusions. In most UAE arrangements these are the same hospital. Reserve Meds coordinates the MDT alignment.

The UAE regulatory pathway: how it actually works in 2026

The Emirates Drug Establishment, which absorbed 44 of the Ministry of Health and Prevention's regulatory functions by early 2026, is the federal authority that the treating hospital files through. Brineura's UAE EDE registration status is mixed: the prevalence is too low to support standalone domestic registration in most years, and the realistic pathway is the named-patient mechanism filed via ede.gov.ae by the hospital's import pharmacy on the treating paediatric neurologist's behalf. The Department of Health Abu Dhabi or the Dubai Health Authority adds the emirate-level layer depending on where the surgery and the infusions are given.

In our experience coordinating ultra-rare paediatric ERT cases in the UAE, EDE coordination on a complete, well-documented file runs four to eight weeks from filing to first dose. The longer end of that range accounts for the time needed to align paediatric neurology, paediatric neurosurgery, paediatric anaesthesia, and the infusion-suite team, and to confirm cold-chain shipping and customs release for a temperature-sensitive biologic.

The realistic UAE infrastructure for CLN2: - **Sheikh Khalifa Medical City, 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 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 Shakhbout Medical City, 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; neurosurgical referral typically routed to SKMC. - **Al Jalila Children's Specialty Hospital, Dubai.** Paediatric neurology depth; complex neurosurgical procedures usually routed cross-emirate to SKMC or Cleveland Clinic Abu Dhabi. - **Mediclinic City Hospital, Dubai.** Paediatric subspecialty service for Dubai-side workup. - **American Hospital Dubai.** Paediatric service for the Dubai-emirate component of the workup.

If your family is in Dubai or in the Northern Emirates, the surgical and infusion legs typically route through Abu Dhabi. We coordinate the cross-emirate logistics and the family-side accommodation.

The workup that decides eligibility and shapes the plan

Five components.

First, definitive diagnostic confirmation of CLN2 disease. Two prongs are needed: 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. Where a family arrives at Reserve Meds with the genetic confirmation but without the enzyme assay, or vice versa, we route the missing test.

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 is the primary efficacy surveillance marker after therapy starts.

Third, paediatric neurosurgery consultation. The neurosurgeon assesses your child's anatomy via brain MRI (ventricular size, scalp condition, prior CNS surgery or infection history), discusses anaesthesia risk with paediatric anaesthesia, and plans the implantation admission.

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. The MDT decides on the readiness for therapy and on the operational plan for the next 12 months.

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 access pathway in the UAE: step by step

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, with the documentation packet from Reserve Meds. 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; sterile technique; paediatric anaesthesia available if needed. 7. Stable every-2-week infusion routine established over the next 2 to 3 months. 8. Ongoing surveillance: motor-language score reassessment at intervals; scalp and reservoir surveillance at each visit; periodic brain MRI; seizure-management adjustment as needed; family support.

The cost conversation, in the form a UAE family needs

The 2026 indicative annual list price of Brineura is approximately USD 730,000 to USD 750,000 for the drug alone, calculated as approximately USD 28,100 per 300 mg vial times 26 infusions per year.

Annual cost of care, including drug, infusion-centre delivery, paediatric neurology surveillance, and monitoring, is approximately USD 850,000 to USD 1.05 million per year, or approximately AED 3.12 million to AED 3.86 million per year, in stable years (Year 2 onwards). Year 1 includes the Ommaya reservoir placement admission, which adds approximately AED 100,000 to AED 150,000 depending on the centre. Over a 10 to 15 year therapy window, cumulative cost can reach USD 10 to 16 million, before counting the supportive-care infrastructure your child will also need.

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. We charge a transparent coordination fee for the case-management work, disclosed in writing before any funds move.

Insurance coverage of Brineura in the UAE is uneven and the ultra-rare disease pathway is the realistic conversation. Daman has approved cases through prior authorisation for certain employer plans and for Thiqa-covered Emirati nationals where the rare-disease pathway applies. Private insurers vary widely. We supply your insurer with the documentation packet at no charge. We do not process the claim or guarantee coverage.

For Emirati nationals being treated at SKMC, SSMC, or Tawam under the public system, much of the cost may be underwritten through the government health funding pathways, and the rare-disease desk at DoH Abu Dhabi has an established mechanism for ultra-rare paediatric cases. 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.

Cross-border options if the UAE cannot deliver

Sidra Medicine in Doha, Qatar, is the natural regional paediatric centre for ICV-route ERT. Sidra is paediatric-only and has the paediatric neurology, paediatric neurosurgery, and paediatric infusion infrastructure all on one campus. For families in the Northern Emirates or where the UAE-side MDT process is delayed, cross-border to Sidra is workable; the flight is 90 minutes and the family-side logistics are well-trodden.

KFSHRC Riyadh is the second cross-border option. The paediatric neurology team at KFSHRC, including the case series published by Dr Brahim Tabarki, has handled CLN2 cases in the region.

Reserve Meds coordinates either pattern. The home-emirate continuity-of-care plan stays the same: the UAE-side paediatric neurologist remains your child's primary clinician, with surveillance visits scheduled around the cross-border infusion calendar.

Safety: what to watch for

- **Device-related infection.** Meningitis or ventriculitis is rare but is the most clinically serious complication. Centres monitor scalp condition, temperature, behaviour change, headache (in verbal children), and CSF on suspicion. - **Infusion reactions.** Pyrexia, vomiting, and hypersensitivity can occur, particularly in the first months. Anaphylaxis-management capability must be on site for every infusion. - **Seizures.** CLN2 children typically have a baseline seizure disorder. Seizure frequency during infusion is monitored; anti-seizure medication regimen is optimised by paediatric neurology. - **CSF leak or reservoir malfunction.** Uncommon but possible over time; may require revision surgery. - **ECG changes.** Typically minor; baseline ECG is the comparator.

The peri-infusion monitoring routine becomes familiar over the first months. Families adjust to the every-2-week rhythm. Paediatric neurology adjusts the seizure-management regimen as needed.

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 UAE family pursuing Brineura, our scope is the diagnostic-confirmation pathway routing, the paediatric neurology MDT documentation packet, the EDE filing in collaboration with the hospital's import pharmacy, the sourcing logistics from BioMarin's authorised distribution through DSCSA-compliant chain of custody, cold-chain shipment (2-8 degrees Celsius, do not freeze) to the qualified UAE 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. Clinical decisions stay with your paediatric neurologist and the neurosurgery and infusion team; we are the operational layer that turns those decisions into a coordinated case.

We work cash-pay where applicable. Our coordination fee is disclosed in writing. We will not start work without a signed engagement.

Frequently asked parent questions

Q: Will Brineura cure my child? No. It slows the decline. Treated children in the pivotal trial lost on average about 0.27 motor-language scale points per 48 weeks versus approximately 2.12 points in untreated historical controls. The earlier therapy starts, the more function is preserved.

Q: Is the Ommaya reservoir permanent? The reservoir is implanted for the duration of therapy, which is the duration of your child's life on Brineura. Revision surgery is occasionally needed if a device malfunctions, but the standard expectation is that one well-placed reservoir lasts.

Q: Can the infusions be done at home? No. Every infusion is given in a paediatric centre under sterile conditions with paediatric neurology supervision and anaphylaxis-management capability. Home infusion is not part of the standard Brineura protocol.

Q: What if we miss an infusion? Missed infusions are not made up by doubling subsequent doses. Schedule discipline is part of the therapy. Travel, school holidays, Ramadan, and family events are planned around the q2-weekly 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. Families typically consult with their religious advisors before committing.

Q: What about siblings? CLN2 is autosomal recessive. Carrier testing for siblings and for the extended family is a separate but important thread, and your paediatric neurologist will offer the genetic counselling referral.

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.
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reservemeds.com · hello@reservemeds.com