Brineura

Saudi Arabia · access guide

Brineura (cerliponase alfa) for a Saudi 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 Saudi family of a child newly diagnosed with CLN2 disease, the classic late-infantile form of Batten disease, faces a decision that has several moving parts at once. There is the clinical question about stage and timing. There is the surgical question, because Brineura requires a reservoir to be placed into a cerebral ventricle by a paediatric neurosurgeon before therapy can start. There is the regulatory question about how an ultra-rare paediatric ICV enzyme replacement therapy reaches Saudi soil. There is the financial question, which runs for the rest of your child's life. And there is the family question about how every-2-week infusions reshape your week, your school year, and your travel patterns.

This page is the first honest read you get on Brineura in Saudi Arabia, written by the team that would coordinate around your child's case at KFSHRC Riyadh, KFSH-RC Jeddah, KAMC Riyadh, or whichever paediatric centre your treating consultant chooses.

We will be specific about what CLN2 disease is, why the intracerebroventricular route matters, what the SFDA regulatory pathway looks like in 2026, what it costs in SAR and US dollars, where the surgery and the q2-weekly infusions can be done in the Kingdom, 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 breaks down peptide fragments inside lysosomes in neurons. When CLN2 is faulty, the substrate accumulates in lysosomes inside brain cells. That accumulation is what 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 treatment preserves function. It does not restore function that has already been lost. The earlier therapy begins after diagnosis, the more function is preserved.

In Saudi Arabia, the paediatric neurology service at KFSHRC Riyadh under Dr Brahim Tabarki published the only documented Saudi CLN2 case series, and the KFSHRC team has the most CLN2-specific experience in the region. The Kingdom has more documented CLN2 paediatric infrastructure than most MENA countries because the consanguinity-linked carrier frequency makes autosomal-recessive ultra-rare diseases more clinically visible in Saudi practice.

The intracerebroventricular route and the Ommaya reservoir

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. The infusion is 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.

For Saudi families, this means two clinical services must align on the case: paediatric neurology and paediatric neurosurgery. KFSHRC Riyadh, KAMC Riyadh, and KFSH-RC Jeddah have both services on the same campus. Reserve Meds coordinates the MDT alignment.

The workup that decides eligibility and shapes the plan

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 KFSHRC molecular genetics laboratory and the major Saudi reference laboratories (including the genetics services at KAMC and at KFMC) can run both prongs. Where a family arrives with one prong only, 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 becomes the primary efficacy surveillance marker.

Third, paediatric neurosurgery consultation. Brain MRI to assess ventricular anatomy, scalp condition, and any prior CNS surgery or infection. Anaesthesia review.

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

Fifth, multidisciplinary 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 plan, the Ommaya placement plan, and the surveillance schedule.

The Saudi regulatory pathway: how it actually works in 2026

The Saudi Food and Drug Authority (SFDA) is the federal regulator. Brineura's SFDA registration status is mixed, and the realistic pathway for most cases is the named-patient mechanism filed by the treating hospital's pharmacy on the paediatric neurologist's behalf. SFDA has a workable framework for ultra-rare paediatric biologics, and SFDA coordination on a complete file typically runs four to eight weeks.

The Ministry of Health, the Council of Cooperative Health Insurance (CCHI), and the rare-disease desks at the major Saudi paediatric centres are part of the operational flow once the SFDA approval is in hand.

The realistic Saudi infrastructure for CLN2: - **King Faisal Specialist Hospital and Research Centre (KFSHRC), Riyadh.** Paediatric neurology under Dr Brahim Tabarki and team; the most documented Saudi CLN2 case series; paediatric neurosurgery on campus; paediatric infusion infrastructure; the natural anchor for the Kingdom. - **KFSHRC Jeddah.** Paediatric neurology and neurosurgery; the Western Region anchor. - **King Abdulaziz Medical City (KAMC), Riyadh.** Paediatric neurology, paediatric neurosurgery (one of the strongest paediatric neurosurgery programmes in the Kingdom), paediatric infusion; an alternative anchor for the Central Region. - **King Fahd Medical City (KFMC), Riyadh.** Paediatric neurology and metabolic service. - **King Fahd Specialist Hospital, Dammam.** Eastern Region paediatric neurology; complex neurosurgical procedures may be routed to KFSHRC or KAMC. - **Prince Sultan Military Medical City (PSMMC), Riyadh.** Paediatric neurology service.

For families in regions without a paediatric neurosurgery centre capable of Ommaya placement, the case routes to KFSHRC Riyadh or KAMC Riyadh, with the family-side travel coordinated.

The access pathway in Saudi Arabia: step by step

1. Diagnostic confirmation (enzyme assay + gene sequencing) at a Saudi reference laboratory. 2. Paediatric neurology MDT at KFSHRC, KAMC, KFSH-RC Jeddah, or KFMC. 3. Paediatric neurosurgery consultation; brain MRI; anaesthesia review. 4. SFDA named-patient filing through the hospital's 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 a Saudi family needs

The 2026 indicative annual drug cost is approximately USD 730,000 to USD 750,000, calculated as 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 SAR 3.19 million to SAR 3.94 million. Year 1 adds the Ommaya reservoir placement admission, approximately SAR 120,000 to SAR 180,000 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. We charge a transparent coordination fee, disclosed in writing before any funds move.

For Saudi nationals, the Ministry of Health rare-disease pathway and the funding mechanisms attached to KFSHRC, KAMC, and KFMC are the realistic conversation. KFSHRC has historically been able to deliver ultra-rare paediatric biologics for Saudi nationals through the MoH-funded mechanism, with the rare-disease desk filing the funding case alongside the SFDA filing. Your treating consultant will confirm whether and how the funding stream applies to your child's case.

For expatriate residents, CCHI commercial cover 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. We do not process the claim or guarantee coverage.

Cross-border options

For Saudi families in the Eastern Region, Sidra Medicine in Doha, Qatar, is an alternative paediatric centre with paediatric neurology, paediatric neurosurgery, and paediatric infusion infrastructure on one campus. Cross-border to Sidra is workable if KFSHRC or KAMC capacity is delayed. Reserve Meds coordinates the cross-border logistics.

For Saudi families weighing access to clinical trials or to investigational therapies for related CLN subtypes (CLN1 in particular), the cross-border conversation extends to international centres. The Saudi paediatric neurology service can refer to international centres where appropriate, and Reserve Meds coordinates the travel.

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, and CSF on suspicion. - **Infusion reactions.** Pyrexia, vomiting, and hypersensitivity can occur. 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; may require revision surgery. - **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 Saudi family pursuing Brineura, our scope is the diagnostic-confirmation pathway routing, the paediatric neurology MDT documentation packet, the SFDA filing in collaboration with the hospital's pharmacy, the sourcing logistics from BioMarin's authorised distribution through DSCSA-compliant chain of custody, cold-chain shipment to the qualified Saudi centre, 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 neurology team; we are the operational layer.

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. The earlier therapy starts, the more function is preserved.

Q: Is the Ommaya reservoir permanent? The reservoir is implanted for the duration of therapy. Revision surgery is occasionally needed but is not the standard expectation.

Q: Can infusions be done at home? No. Every infusion is given in a paediatric centre under sterile conditions with paediatric neurology supervision.

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. Saudi families typically consult their religious advisors before committing.

Q: What about siblings and the extended family? CLN2 is autosomal recessive. Given the consanguinity rates in many Saudi families, carrier testing for siblings, for cousins, and for the extended family is part of the standard genetics-counselling referral that your paediatric neurologist will offer.

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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