Brineura

Qatar · access guide

Brineura (cerliponase alfa) for a Qatari family with a child diagnosed with CLN2 Batten disease: what the pathway looks like in 2026 with Sidra Medicine as the natural centre

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

A Qatari family of a child newly diagnosed with CLN2 disease, the classic late-infantile form of Batten disease, walks into this decision with one important advantage. Qatar has Sidra Medicine, and for paediatric intracerebroventricular enzyme replacement therapy, Sidra is the natural regional centre. Paediatric neurology, paediatric neurosurgery, paediatric anaesthesia, and paediatric infusion infrastructure are all on the same campus. This page is the first honest read you get on Brineura in Qatar, written by the team that would coordinate around your child's case with the Sidra paediatric team or, where applicable, with Hamad Medical Corporation paediatric neurology.

We will be specific about what CLN2 disease is, why the intracerebroventricular route matters, what the MOPH regulatory pathway looks like in 2026, what it costs in QAR and US dollars, where the surgery and the q2-weekly infusions can be done in Qatar, 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 normally 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 the accumulation damages the brain.

The disease usually presents between ages 2 and 4 with seizures and language regression. 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.

Why Sidra Medicine is the natural Qatar centre, and the regional anchor

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 intraventricular reservoir under general anaesthesia in a child. It needs paediatric anaesthesia. It needs a paediatric infusion service capable of 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.

Sidra Medicine has all of those services on one Doha campus, with a paediatric-only mandate that makes the operational alignment cleaner than at any mixed adult-paediatric hospital in the region. Sidra is the natural centre for Qatari CLN2 families, and it is the cross-border anchor for Bahraini, Kuwaiti, eastern Saudi, and several UAE-based families.

For families whose Qatari national paediatric care has historically been at HMC Hamad General Hospital or at the National Center for Cancer Care and Research, the conversation with HMC paediatric neurology and the cross-referral to Sidra for the ICV procedural component is the standard arrangement.

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. The drug is infused directly into the cerebrospinal fluid via a surgically-implanted intraventricular reservoir placed by a paediatric neurosurgeon under general anaesthesia in a separate 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.

The device requires monitoring: scalp condition, temperature, behaviour change in younger children, and CSF on suspicion of infection. Sidra paediatric neurosurgery handles the reservoir surveillance and any revision surgery that may be needed over the years.

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. Sidra's molecular genetics laboratory and HMC's reference laboratory can run both prongs in-country.

Second, paediatric neurology baseline. Motor-language summary score on the modified Hamburg CLN2 scale. Baseline seizure burden and anti-seizure medication. Baseline vision and developmental status.

Third, paediatric neurosurgery consultation. Brain MRI to assess ventricular anatomy. 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 Qatari regulatory pathway: how it actually works in 2026

The Ministry of Public Health (MOPH) is the federal regulator. Brineura's MOPH registration status is mixed and the realistic pathway is the named-patient mechanism filed by Sidra's pharmacy (or HMC's pharmacy where applicable) on the paediatric neurologist's behalf. MOPH coordination on a complete file typically runs four to six weeks for ultra-rare paediatric biologics.

The HMC funding pathway for Qatari nationals through the rare-disease desk is the established mechanism for cost coverage. Sidra coordinates the funding case in parallel with the MOPH filing.

For non-Qatari residents and for cross-border families coming to Sidra from Bahrain, Kuwait, or Saudi Arabia, the supply chain is the same but the funding mechanism follows the home-country funder.

The access pathway in Qatar: step by step

1. Diagnostic confirmation (enzyme assay + gene sequencing) at Sidra Medicine or HMC reference laboratory. 2. Paediatric neurology MDT at Sidra (or HMC paediatric neurology with referral to Sidra for the ICV procedural component). 3. Paediatric neurosurgery consultation at Sidra; brain MRI; anaesthesia review. 4. MOPH named-patient filing through Sidra pharmacy with Reserve Meds providing the documentation packet. 5. Ommaya reservoir placement admission at Sidra, typically 1 to 3 inpatient days; wound healing window of 1 to 2 weeks before first infusion. 6. First Brineura infusion at Sidra 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 Qatari 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 QAR 3.10 million to QAR 3.83 million. Year 1 adds the Ommaya reservoir placement admission, approximately QAR 110,000 to QAR 165,000 at Sidra.

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 Qatari nationals, the HMC and Sidra rare-disease funding pathway is the realistic conversation. The MOPH-coordinated funding for ultra-rare paediatric biologics has been delivered in-country for other paediatric ERTs and the precedent is workable for Brineura. Your Sidra consultant and the rare-disease desk will confirm the funding mechanism for your child's case.

For non-Qatari residents, commercial cover applies depending on the policy. We supply your insurer with the documentation packet at no charge.

Cross-border families coming to Sidra

If you are a Bahraini, Kuwaiti, eastern Saudi, or UAE-based family and your home-country centre has referred your child to Sidra for the ICV procedural component, the operational flow is well-trodden. Sidra paediatric neurology and neurosurgery accept cross-border referrals, the home-country MoH funding pathway (Kuwait FMT, Bahrain SMC referral, Saudi MoH cross-border) processes the financial side in parallel, and Reserve Meds coordinates the documentation, the travel, the family-side accommodation, and the continuity with your home-country paediatric neurologist who remains your child's primary clinician between Sidra visits.

For families coming to Sidra cross-border, plan for the Ommaya placement admission as the first Sidra-based event (1 to 3 inpatient days), then plan for the every-2-week infusion calendar with Sidra as the venue.

Safety: what to watch for

- **Device-related infection.** Meningitis or ventriculitis is rare but the most clinically serious complication. Sidra monitors scalp condition, temperature, behaviour change, and CSF on suspicion. - **Infusion reactions.** Pyrexia, vomiting, and hypersensitivity can occur. Anaphylaxis-management capability is on site at Sidra for every infusion. - **Seizures.** CLN2 children typically have a baseline seizure disorder. Seizure frequency during infusion is monitored; the anti-seizure medication regimen is optimised by Sidra 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 Qatari family pursuing Brineura at Sidra Medicine, our scope is the diagnostic-confirmation pathway routing, the Sidra MDT documentation packet, the MOPH filing in collaboration with Sidra pharmacy, the sourcing logistics from BioMarin's authorised distribution through DSCSA-compliant chain of custody, cold-chain shipment to Sidra, 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.

For cross-border families coming to Sidra, our scope additionally includes the home-country funder coordination and the home-country paediatric neurology continuity.

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

Q: Why Sidra rather than HMC? Sidra is paediatric-only with paediatric neurosurgery capable of Ommaya placement and paediatric infusion infrastructure on the same campus. For ICV-route paediatric ERT, Sidra is the natural regional anchor. HMC paediatric neurology remains involved for many Qatari families through the cross-referral arrangement.

Q: Can infusions be done at home? No. Every infusion is given at 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. Families typically consult their religious advisors before committing.

Q: What about siblings? CLN2 is autosomal recessive. Carrier testing for siblings and for the extended family is part of the genetics counselling that Sidra paediatric neurology 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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