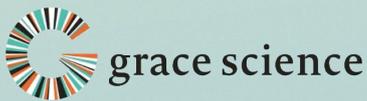




Case study

Harnessing hope: how Grace Science used Almac Adapt™ to deliver for children living with NGLY1 Deficiency

Supply With Care™





When a family learns their child has NGLY1 Deficiency, an incredibly rare autosomal recessive disease that devastates the central nervous system, hope must be exceptionally well supported. Father to Grace, who was diagnosed with this ultra rare disease in 2013, Matt Wilsey - co-founder and CEO of Grace Science, LLC - set out to change the world. Determined to discover a cure for Grace, and all children with NGLY1 Deficiency, Grace Science embarked on a clinical trial to assess the safety and efficacy of GS-100 – a gene therapy developed to treat the disease with a single intracerebroventricular infusion of an AAV9 vector delivering the full length human NGLY1 gene to children aged 2-18.

To overcome several challenges, assure timely supply to patients, and take a leap forward on the journey towards curing NGLY1 Deficiency, Grace Science partnered with Almac Clinical Services who understood the assignment. Tailoring a Just in Time Manufacturing (JTM) approach,

supported by deeply aligned teamwork and white glove service, Almac made it their mission to ensure this promising gene therapy arrived at the right time, in the right condition, and ready to potentially transform young lives.

Zero margin for error

The path towards successfully dosing patients wasn't straightforward. Several factors combined to create zero margin for error:

- Paediatric patients, their families, and medical teams were pinning their hopes on receiving this potentially transformative therapy – raising the stakes for providing timely drug access.
- GS-100 is an incredibly high-value therapy for one of the rarest diseases known to science. As such, availability was limited. Getting it right first time was essential.
- Due to the study's small scale, orders were manual. With no IRT system, without appropriate oversight and expertise, miscommunication and timing errors could negatively impact patients and delay study milestones.
- A two-step screening process created timeline uncertainty. With patient eligibility hanging in the balance until both screening and hospital board approval was complete, triggering orders at the optimum time required around the clock communication.
- GS-100 needs to be administered during brain surgery. Because of this, the drug needed to be delivered within a precise timeframe, aligned with the surgical procedure. Missing a surgery window was not an option, given the expense of re-scheduling a theatre and surgical team, the emotional investment of children and their families awaiting treatment, and the inability to repurpose extremely expensive drug product.
- GS-100 is temperature sensitive – demanding –80°C handling. Any deviation had the potential to render the drug product unfit for use.



Helping to harness hope

Almac approached the programme as more than a logistics challenge — it was a shared mission to bring hope to families who had waited years for a treatment. Working as an extension of the Grace Science team, every person at Almac understood what was at stake – creating 'white glove' operational ownership powered by a shared mission, multifunctional alignment, and personal accountability across all teams – from project services, operations and warehouse to distribution, quality, labels and temperature services.

Crucially, Almac Adapt™, Almac's JTM solution, was harnessed to ensure each dose was packaged, labelled and released only when a child was ready to receive it, with orders triggered precisely on confirmed board approval to avoid premature activities. This removed the risk of labelled product expiring and prevented bulk drug waste that would take months to re-manufacture/re-supply. This approach was critical, given that GS-100 is produced in extremely limited quantities and at substantial cost, has a short shelf life, and complex storage requirements.

With every dose requiring strict temperature control at –80°C and destined for administration during surgery, timing and temperature control had to be flawless. Teams in Almac's Durham facility remained on constant standby, ready to act at the appropriate time. Project plans were created for each patient and dynamically managed with close collaboration to ensure real-time situational awareness - from screening and hospital board approval through to order trigger, packaging and release, courier instruction, and theatre planning.

That dedication extended beyond process. Internal communication channels, daily cross-team updates, and a full 'dummy run' ahead of first dosing, which was crucial in activating the site prior to the surgery, reflected the collective commitment to getting it right first time, every time.



Hope in action

The collaboration between Grace Science and Almac demonstrated what's possible when purpose and precision align. On February 28, 2024 (Rare Disease Day) and the exact date little Grace received her NGLY1 Deficiency diagnosis in 2013, the first patient was dosed with GS-100 in Houston, Texas. This marked a major moment of hope not only for one family, but for the entire NGLY1 Deficiency community. Using Almac Adapt™ ensured Grace Science could deliver an unprecedented 'half the time' fast-tracked dosing schedule.

Since then, Almac has continued to support the delivery of individual patient orders across US clinical sites, each requiring the same level of coordination, care, and control that defined the inaugural dose. Every shipment has been executed to plan, with zero product loss or temperature deviation reported, and each patient receiving their treatment exactly when clinical teams were ready.

The success of this programme has created a close partnership between Grace Science and Almac Clinical Services, uniting both organisations around a shared mission to deliver life-changing therapies safely and reliably. For Almac's people, the reward went beyond operational success — it was knowing that behind every order was a child, a family, and the possibility of a different future.

Writing to thank Almac following the success of the first patient dosed with GS-100, Matt Wilsey said:

'We could not have gotten here without your help. Thank you. Let's go cure some more people.'

Because with everything we do, we Supply With Care™



*The images shown are of actual study participants, shared with the kind permission of their families.

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