

No room for error when delivering life-saving spinal muscular atrophy (SMA) treatments to young patients

When Amelia from Chile was diagnosed with Spinal Muscular Atrophy (SMA), she needed a one-time gene replacement therapy to save her life. World Courier worked around the clock to support in delivering the product on time.

At just six months old, Amelia¹ was diagnosed with SMA, a rare genetic disease that results in the loss of motor neurons and progressive muscle wasting. The condition is found in just 1 in 10,000 children born around the world and, if left untreated, more than 90% of patients may die or require permanent ventilation by the age of two².

“SMA affects a child’s ability to crawl, sit up and control their own head movements, even breathe or swallow. There is no guaranteed cure, but treatment and support from a young age can reduce symptoms, and improve quality of life.”

Amelia’s doctor

Faced with this news, Amelia’s family set out to obtain a one-time, potentially life-saving gene replacement therapy produced in the US.

The logistics challenge

Amelia, like many other vulnerable patients living in remote areas, struggle to access cutting-edge gene therapies. Many products require storage at a very specific temperature range, as low as -70°C and only have a shelf life of 48 hours. Failure to meet these strict requirements can lead to the degradation of the therapy, have devastating ramifications for patients’ health and wellbeing, and cost millions of dollars to rectify.

The leading biotechnology innovator behind this pioneering solution engaged World Courier for this challenge. Impressed by our extensive track record on quality, regulatory compliance and our carefully thought-out plan to deliver Amelia’s treatment, the company felt we were the ideal partner for the task.





The solution

The World Courier team harnessed our unique global network of local logistics and regulatory experts to make certain that the dose reached Amelia on time and in good condition.

The box used for transportation was a 30L GDI (Global Dry Ice) Thermal Container, chosen for its ability to keep its contents at a consistent temperature of -70°C , whatever the external conditions.

To minimize the impact of routing disruptions due to the COVID-19 pandemic and safeguard against delays, we fitted a GPS tracker in addition to our existing advanced tracking capabilities. This allowed us to monitor the location of the thermal container from departure through to where Amelia was being treated.

At every stage, our experts reviewed the documents, permits and custom requirements. With our unique experience and insight, we made sure that each piece of documentation was compliant with local regulations in the US and Chile to prevent any delays in the customs process.

“We wanted to make sure that nothing would go wrong for Amelia, so we also asked for a police escort from the airport to the hospital, to ensure that we would be there on time.”

Jaqueline Escotero

Regional Vice President, Latin America, World Courier

The outcome

Amelia received her gene replacement therapy safely and on time, thanks to our well-established global logistics infrastructure and team of dedicated people with their local regulatory, customs and transport expertise.

At World Courier we understand the importance of on-time and in-temperature delivery for every shipment. We are committed to supporting all of our clients with their life-saving medications so they can reach the people who need them most, wherever they are in the world.

“Our work goes beyond transporting boxes, we bring hope to the families who wait to receive the medicine to continue or begin their medical treatment.”

Maria Jose Tapia

Logistics Supervisor, World Courier Chile

For peace of mind in delivering your high-value, fragile therapies to patients, contact us [today](#).

¹ The name has been modified to protect the identity of the child portrayed in the story.

² Finkel RS, et al. Neurology. 2014;83(9):810-817.