

# Gene Therapy and Daikyo Crystal Zenith® Vials Case Study

## Overcoming the challenge of maintaining sterility at low temperature storage

### The Challenge

Bringing the first gene therapy for pediatric Spinal Muscular Atrophy to market in a leak and break resistant container which maintains sterility over product shelf life, through freeze and defrost cycles. Container Closure Integrity (CCI) must be proven and consistent for storage at -80°C, and the solution must be ready to scale-up quickly due to orphan designation and Fast Track status of the program.

### The Considerations

Gene therapy is no longer an area of pharmaceutical research merely restricted to academia; gene therapy now accounts for 10% of the Biologics pipeline. When planning gene therapy drug development, it is critical to ensure known challenges are mitigated; therefore a chosen packaging system needs to be proven, reliable and able to be scaled-up, at short notice.

The supply chain for gene therapies requires the drug to be frozen, then stored at -80°C before being thawed for injection into the patient. It is critical that packaging components used in this complex supply chain are compatible with both the drug and specialized conditions required for storage and distribution.

Having secured Fast Track designation from the FDA, the customer was able to bring its gene therapy to market quickly, allowing patients with the highest need to receive treatment in record time.

The packaging system selected helped to facilitate the speed with which the drug moved from development to commercialization. The chosen system provided a range of container sizes with quality components, in ready-to-use format, proven to work as a system and available in small quantities.

### The Solution

West's Ready Pack™ system was selected, which includes a Daikyo Crystal Zenith® vial, NovaPure® stopper and Flip-Off® CCS seal. These high-quality components combine to create a proven system which ensures CCI over the product shelf life and low temperatures.

Customers can purchase the small quantity Ready Pack™ system directly from [West's website](#). Buying this offering online allows for quick delivery and enables customers to meet the short timelines often associated with orphan drug designation and Fast Track approval.

Daikyo Crystal Zenith® vials and associated West components were the right choice for this customer, as vial sizes from 2-5mL supported expedited scale-up activities from lab to the patient. The Ready Pack™ system offers all these benefits without the unwanted cost of excess stock and is the perfect choice for bringing a gene therapy to market.



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*"The seals, the Crystal Zenith vials and the rubber stoppers, it's all an integrated component for us and it's been a terrific partnership and collaboration with West. Every vial that leaves our filler with the great components that have been supplied by West - those will go to a patient with this life changing medicine."*

### West Customer

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