Packaging Containment Solutions for Gene Therapy Products

Case Study: Overcoming the Challenge of Maintaining Sterility at Low Temperature Storage

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Research in the area of cell and gene therapies started almost 40 years ago, however, recently there has been a surge of activity including landmark regulatory approvals for therapies treating chronic and rare diseases. In 2017 and 2018 the FDA approved the first two CART-T cell therapies for cancer treatment and the first two gene therapies for rare diseases. All four of these cell and gene therapies were approved under the FDA Accelerated Approval Program, aimed at expediting approvals for life saving drug therapies. These regulatory programs have the potential to shorten drug development as much as 1-3 years. Currently, there are more than 1,000 regenerative medicine clinical trials underway globally[1], and the market is expected to grow rapidly over the next 10+ years.

As more of these life changing therapies are granted accelerated approvals and the speed of development increases, it is critical to plan for your packaging decisions early in the development cycle to avoid potential delays. West has partnered with many companies who have launched drugs via expedited approval pathways, and we understand the need for a solution to support you quickly through development. As advancements in gene therapy products continue to grow, West is committed to providing packaging solutions for these complex treatments.

Learn more about a biotechnology company, with a recently approved gene therapy product, that partnered with West to select a packaging solution that would allow them to meet their needs for storage at low temperatures and still get to market quickly.

The Challenge

Bringing the first gene therapy product 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

When planning gene therapy product 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 product 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

The company selected West's Ready Pack™ system which is an ideal solution for gene therapy product companies that need a proven containment system at cold temperatures and a fast delivery to meet the short timelines associated with accelerated approvals. West's highest quality components are included in the Ready Pack system: Daikyo Crystal Zenith® vials available in multiple sizes; NovaPure® vision verified stoppers with FluroTec® barrier film; and West Flip-Off® Clean Certified Sterile seals. These high-quality components combine to create a proven system which ensures CCI over the product shelf life and low temperatures. In 2019 the FDA approved the first gene therapy product treatment for pediatric Spinal Muscular Atrophy using West's Ready Pack containment system.

Customers can purchase the small quantity Ready Pack system directly from West's online store. 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 product to market.

Visit our booth to learn more about how West can support your gene therapy product development with solutions for your containment needs. Read more about our containments solutions for gene therapy products or Contact Us to talk with an Account Manager or Technical Customer Support Representative.

Source: [(1)] Alliance for Regenerative Medicine January 2020