How Efficient Logistics Can Change the Lives of Rare Disease Patients

Dr. Danial Arkwell, Head of Global Key Accounts, Pharma at Envirotainer highlights the vital role of efficient logistics in mitigating risk and finding the right solutions to get orphan drugs to patients in desperate need.

There are approximately eight thousand rare diseases in the world and many of these are chronic, life-threatening conditions. To fight these elusive illnesses, the medical community focuses on developing specialised medications known as orphan drugs. Originating from the United States Orphan Drug Act (ODA) in 1983, the term "Orphan Drug" reflects the lack of attention and investment necessary for their development.

The diseases targeted by orphan drugs often have low prevalence rates, making them economically unattractive to develop treatments for, due to the limited potential market. The result is that many of these diseases are being historically neglected as a result of the perceived low profitability.

The Orphan Drug Act and similar legislation aim to progress the development of drugs for rare diseases by offering various incentives to drug manufacturers. This includes tax credits for clinical research costs, seven years of market exclusivity upon approval, and assistance with clinical trials, design and funding.

Despite government support, orphan drugs have been priced significantly higher than non-orphan drugs, owing to the inherent complexities and high costs of production. The significant upfront investment required to bring these much-needed treatments to market not only slows down their development but also contributes to incredibly high prices for patients, potentially putting them out of reach for many.

Yet, there are other barriers to patients receiving life-saving treatments. One that's frequently overlooked is the complexity of transporting these highly sensitive drugs from the lab to the end patient. If manufacturers cannot ensure safe, cost-effective delivery, then access to these rare treatments will remain out of reach for many patients. Efficient logistics must take centre stage.

Logistical Barriers Stand Tall

Companies developing orphan drugs and other rare treatments often do so at a net financial loss. This cost is not solely placed on the research and production, the safe transport and distribution of these specialised treatments is also a consideration. Unfortunately, there is a lot that can impact delivery.

One of the most significant challenges in shipping orphan drugs is their temperature sensitivity. Many of these products are biologics, gene therapies, or other advanced therapies that require strict temperature control to protect their efficacy. Some require storage at extremely low temperatures, often below freezing, while others may need to be stored at controlled room temperatures or other conditions.

Even a small temperature deviation during shipment can compromise the product and render the drug ineffective or even harmful to patients. Furthermore, when developed as personalised medicines, the transportation complexities and logistics costs increase further, often resulting in higher total landed cost (TLC) for shipping.

Despite the small patient pool, orphan drugs often need to be delivered to different, sometimes hard-to-reach, locations around the world, adding another layer of complexity

to the process. Import and export regulations and differing international standards can create bottlenecks and delays, increasing the risk of temperature deviations if a shipment is stuck in customs.

Last-mile delivery is another critical challenge when ensuring patients receive their treatments safely and efficiently. Unlike many medications, orphan drugs often require direct delivery to patients' homes or specialised medical facilities, introducing additional requirements to consider. Challenges such as geographical remoteness, limitations in infrastructure (*e.g.* storage space, electricity, and cooling capabilities), and the need for personalised handling, add further to the complexities of last-mile delivery.

All these costs and logistical challenges only push the cost of treatment up higher, creating significant barriers to affordable treatments and potentially pricing out-patients who need the treatment most. For manufacturers, however, the safe shipping of these drugs is non-negotiable, no matter how much it costs.

Finding the Right Balance

For the pharmaceutical industry, efficient logistics isn't just about cost savings; it's about ensuring life-saving medications reach the right patients, at the right time, in the right condition.

Finding the optimal shipping solution involves balancing performance, environmental impact, service level and cost.

For strict temperature-controlled drugs, it's crucial to choose packaging that's designed to maintain a consistent temperature range throughout the shipping process. It's vital that the optimal packaging for the shipment mitigates the risk of temperature deviations and prevents potential product loss and any consequential increase in transport costs and drug usability at the destination.

In addition, as many as 20% of these products are spoiled and unable to be delivered due to failures in the cold chain, resulting in severe implications for patients. However, there are strategies to mitigate risk, such as 'smart' secondary packing solutions with real-time temperature and location monitoring, which send alerts in real-time if an unexpected event occurs during transit, enabling swift corrective action - preventing costly product spoilage and re-shipping and saving money in the long run.

Both active and passive containers have their role to play, but the best choice depends on varying factors including product temperature requirements, tradeline complexity, TLC and ultimately the level of risk one is willing to take with one's medicine.

Because orphan drugs are manufactured in smaller quantities, these drugs have often been seen sharing cargo space with other pharmaceutical products, potentially increasing the risk of exposure to unsuitable conditions. To mitigate this risk, manufacturers can prioritise collaboration and coordination with logistics partners to optimise trans-portation. By leveraging shared logistics networks and implementing Just-In-Time inventory strategies, distribution processes can be streamlined while also minimising costs and maximising efficiency.

The Bottom Line of Efficient Logistics

The journey of an orphan drug, from research lab to patient delivery, is a complex process and often an expensive one. However, the cost of rare disease drugs isn't just in development, it's in delivery. Inefficient logistics can eat up resources and can even lead to patients not getting the treatment they so desperately need.

Yet, with the right packaging choice, ongoing collaboration between pharma manufacturers and specialists in temperature-controlled logistics, and a patient-centric approach, the barriers to shipping rare disease treatments can be removed. This means that more patients get access to the drugs they need, regardless of how rare their condition is. After all, it's not just about saving money, it's about saving lives.

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Dr. Danial Arkwell has a Ph.D in Molecular Biology, and with over 20 years commercial experience in healthcare, clinical diagnostics and pharmaceuticals, he is an industry expert in temperature controlled logistics. Working with Envirotainer, he believes that close collaboration, open communication and a passion for patient safety are key to navigating the challenges facing this industry.