Delivering living cells to patients

Cell and gene therapies pose new logistic challenges

The spotlight on rare diseases over the past three decades has helped spur the development of innovative cell and gene therapies that test the boundaries of science and offer the potential to improve health outcomes to patients, who in many cases have no other hope.

Moreover, the rapid advance of gene therapy into the area of oncology has opened up potentially huge markets for these medicines. This is illustrated by US approvals in 2017 for Kymriah and Yescarta for haematologic malignancies, and recent positive opinions for both drugs by the European Medicines Agency. Clinical activity for cell and gene therapy is also accelerating for a variety of indications. According to the Alliance for Regenerative Medicine, there were 959 clinical trials underway at the end of the first quarter, 90 of which were in Phase 3[1]. Treatments of this kind, which were only a dream a few years ago, are rapidly becoming a viable option for many patients.

With more therapies in the development stage, it is important that manufacturers take steps to ensure that they are ready to go to market once approved. Unlike traditional drugs, cell and gene therapies are composed of living cells and therefore need to operate under time critical and temperature sensitive parameters. Therefore, efficient logistics are a key consideration. Cell and gene therapies must be delivered on time and in pristine condition both to and from the manufacturer and to the treatment site for patient administration. Hyper-coordination and expertly managed logistics are critical in the administration of these products and their long term commercial viability.

The launch of a new product requires strategic focus on a number of different aspects of commercialisation, including managing late-stage clinical trials, creating reimbursement strategies and decreasing the cost of goods. The development of a viable, cost-effective logistics platform is equally important to optimise the supply chain and ensure that patients receive the intended benefit of the therapy.

The way in which manufacturers handle logistics has changed; the traditional cold chain model is not equipped for the intricacies involved in successfully transporting cell and gene therapies. Now developers must embed, early in their development process, a logistics platform that can reach the appropriate scale. The clinical trial stage can prove a useful testing ground for manufacturers and their logistics partners to design, build, test and optimise their logistics platforms. With this advanced planning, manufacturers will be better positioned to describe an established delivery system to regulators and investors, an important step as a product scales up and moves to market.

The patient as part of the supply chain

In the case of autologous therapies, the patient becomes part of the supply chain, which presents new challenges. The cells, which will be drawn from a patient and cryopreserved, need to be transported to a manufacturing hub where they will be engineered to meet the specific requirements of the patient. Companies need to have a firm grip on transportation details, including airline routes and schedules, and customs' requirements. Moreover, the cells must meet regulatory requirements for quality control throughout the journey to manufacture and back to the patient. Advanced contingency planning is essential to standardise operating procedures so that any possible unexpected situation can be resolved and clearly delineated lines of responsibility established. If incorporated, the lines of communication between each point on the supply chain will be stronger.

As manufacturers look to scale cell and gene therapies, coordinated support is essential for a smooth commercialisation transition. In this phase, the increase in patient and healthcare sites, geographic locations and shipment volumes necessitate significant planning and testing. Sourcing materials from patients, maintaining proper temperature controls and product distribution that ensures patient access are all elements that should be thought about ahead of time and implemented with the help of an experienced partner.

A clear vision of the logistics platform's critical attributes, post-marketing authorisation application, enables technical experts within logistics organisations to collaborate with manufacturers to design, test and streamline. Starting logistics planning while still in the clinical trial phases allows robust data analytics to be built into the supply chain. Logistics partners can manage the exchange of information, including shipping temperatures and geographic locations, and determine the most secure routes. Data collection also gives participants flexibility to create a truly customised solution to meet a therapy's unique requirements.

While manufacturers may wish to take advantage of the expertise a partner can offer, it is important that they evaluate and align with one that meets their specific needs. Manufacturers should look for partners that can offer both the experience and resources to work through unique challenges that a cell and gene therapy innovator is faced with and find alternate solutions that, to the greatest extent possible, guarantee the shipment's integrity and delivery deadlines on a global scale.

If the current pipeline is any indication, the cell and gene therapy market will continue to grow. From increased production volumes to geographical reach and the creation of patient programmes, manufacturers should feel confident in the communications that are now taking place among all stakeholders in the supply chain.

Reference:

1. Q1 2018 Data Report. Alliance for Regenerative Medicine, https://alliancerm.org/publication/q1-2018/

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