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A Truly Global Expanded Access Program:

Navigating Diverse Regulatory Requirements to Deliver a Gene Therapy

THE CASE

A pharmaceutical client contracted Durbin to create and implement an expanded access program (EAP) for a first-of-its-kind gene therapy. The therapy was designed to treat a genetic disorder with few satisfactory treatment options available. As the disorder is a leading genetic cause of infant mortality and causes affected infants and children to swiftly decline, a rapid and successful implementation of the EAP was crucial.

THE CHALLENGE

The EAP presented several unique challenges, beginning with the uncertainty of whether running an EAP for a gene therapy was possible, necessitating Durbin's consultancy services. As the treatment was the first of its kind, even countries with established regulations had never seen a therapy like this before and did not have appropriate approval processes in place. The therapy was also very high in value, leading to the possibility of significant shipping taxes and duty charges for the client and patients. The EAP incorporated a few payment models, beginning with a paid program and later expanding to include a free-of-charge model along with other innovative payment methods. In addition, the therapy had to be stored at -80° C, could not be X-rayed in customs, and had to be used within 14 days of shipment.

Perhaps the most significant challenge consisted of the varied and complex regulatory requirements for each country participating in the EAP — an obstacle that was further compounded because many markets had not yet considered how a gene therapy would be imported into the country, resulting in unknown regulatory requirements. The program's objective was to provide worldwide access to this therapy for all qualifying patients. Each country with qualifying patients had different regulations for the provision of unlicensed or locally unavailable medicines. Some of the markets offered thorough guidance, some had informal programs, and others, including unique locales like Kazakhstan, Russia, India, and Malaysia, had no established system or legislation at all. This dramatic variance in both regulatory vigor and clarity created a maze of processes and documentation requirements to be navigated.

DURBIN'S EXPANDED ACCESS PROGRAM AND REGULATORY SOLUTIONS

We used our 50 years of experience with the distribution of unlicensed medicines and the related regulatory frameworks, as well as our passionate team of high-caliber logistics specialists, to meet the EAP's unique needs and ensure timely delivery of this potentially lifesaving therapy in all the countries in the program.

Because of the EAP's global coverage, our team collaborated closely with the client to craft a plan detailing how to distribute the therapy into key markets, with specifics on shipping mechanisms and customs documentation. For high-risk markets where the local teams wanted to be involved, we conducted thorough research and met with the client, resulting in an even deeper understanding of each market's specific requirements and fostering a sense of collaboration that spanned the entire program. We also worked with local authorities, insurance providers, and legal teams to minimize the taxes and duties involved with the high-value product, even creating templates to help guide caregivers applying for tax exemptions.

Once the therapy was ready to ship, we supported the HCP teams in obtaining import and export licenses and customs clearance. Because of the product's strict temperature requirements and its need to be used within 14 days of shipment, we closely followed each shipment and provided support to ensure proper, timely delivery. As part of our white-glove service, the team remained in contact with the client on shipment progress for specific locations.

> As an example of our ongoing commitment to EAP success, we went above and beyond to facilitate a sustainable program.

The Durbin team navigated additional challenges in implementing the EAP because of the COVID-19 pandemic. For example, when a treatment was postponed due to a patient testing positive for the virus, it resulted in an urgent need for in-country storage solutions to be arranged in order to maintain its integrity. Durbin was committed to overcoming any challenge to ensure as many patients as possible accessed the potentially lifesaving therapy.

Whatever problem or need arose, the team not only came back with an effective solution, but they also often predicted and prepared for potential difficulties before they could impede the progress of the EAP. For example, because of the significantly limited treatment options for the disorder, we knew the client would be flooded with requests for access. Our team effectively set up a call center to handle these inquiries and provide concierge assistance.

PROGRAM RESULTS

This was the first global EAP for a high-value gene therapy product in a critical disease area, carving a hopeful path for many more therapies to come. The program exceeded the client's expectations, inspiring comments including:

> "Engagement with Durbin has been first rate."
>
> "[Durbin was a] true partner that has been able to help deal with very complex situations under tremendous time pressure."
>
> "[We've] never seen such a high level of commitment to the cause in a partner before."

The most important success, however, was that the EAP provided a significant number of patients across more than 40 markets access to essential, timely treatment. The impact of the EAP on the lives of these children and their families can't be quantified.

YOUR 360º EAP PARTNER

If you want high-quality, end-to-end support, optimal value, and peace of mind, **make Durbin your EAP partner.**

ABOUT DURBIN EAP

Durbin is a specialist medical supplier distributing unlicensed pharmaceuticals to 160 different countries. Durbin works in partnership with global pharmaceutical and biotech companies to provide Expanded Access Programs, including Named Patient Supply and Cohort Programs.

The company has 25 years' experience designing and implementing EAPs from concept and specializes in developing robust and compliant voluntary data collection initiatives, which run seamlessly alongside the programs they manage.

Those initiatives help partners capture real-world insights from outside the clinical trial environment that can then have a variety of potential uses, ranging from regulatory and payer negotiations to informing future study design.

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