propharma

case study

EMA approval under exceptional circumstances

Regulatory Sciences

How We Helped Our Client Obtain EMA Approval for the Treatment of an Ultra-Rare Condition Using Science as the Driver of Success

ProPharma's Regulatory Sciences team supported a US biotech company in obtaining approval for the first therapy licensed to target the underlying metabolic defect responsible for the ultra-rare condition known as lipodystrophy. Due to the extreme rarity of the condition, the client was seeking EMA approval under exceptional circumstances. Our team of consultants provided expert regulatory guidance throughout the entire process, resulting in EMA approval.

How can a product receive EMA approval when it's not possible or ethical to collect the comprehensive patient safety and efficacy data required for MAA submission? ProPharma's expert FDA and EMA regulatory scientists worked together with the client to successfully obtain EMA approval for the therapy.

challenge

The client had developed one of the few treatments for lipodystrophy – a condition in which patients do not make sufficient leptin and, consequently, are unable to properly store ingested fats. Lipodystrophy causes hypertriglyceridemia, leading to potentially extreme insulin resistance, diabetes, pancreatitis, and cardiovascular disease. The therapy developed by our client would be the first to target the underlying etiology of the condition.

After obtaining Biologics License Application (BLA) approval from the FDA, the company came to us seeking assistance with the development and submission of a Marketing Authorization Application (MAA) to EMA.

Due to the extreme rarity of the condition, it was not possible or ethical for the client to collect comprehensive safety and efficacy data. As a result, it was not possible for the company to provide EMA with a comprehensive clinical data package, as is typically required for MAA submissions.

solution

In the EU, there is the possibility to gain approval for new medicines despite the inability to provide a comprehensive clinical data package. This type of approval is known as an approval under exceptional circumstances and is most often used for extremely rare indications, such as lipodystrophy.

ProPharma's team of regulatory experts supported the client with fi filing the MAA in the EU, which required updating the previously FDA-approved BLA, writing an EU Module 1, and publishing and submitting the application.

Our team also assisted with the responses to questions from the EMA, which included complex discussions around immunogenicity assessments, given that the product in question was a biologic.

results

At the end of the project, our client obtained EMA approval for their product. The approval also involved a complex orphan strategy, since the indication encompassed four distinct forms of lipodystrophy, each requiring an orphan designation and the activities to maintain these designations at the time of marketing authorization.

Achieving success for our client was fueled by ProPharma's set of unparalleled global regulatory capabilities. We have regulatory scientists on both sides of the Atlantic, each with expert knowledge of the applicable local regulatory requirements. **Our client needed a team with a deep understanding of the science of the product, a detailed appreciation of the data available, and an innovative approach to working with the available BLA to make this fi fit for the purpose of an EU MAA.** Using the same approach we take to every project, we were able to work closely with the client to understand the organization's unique needs to help them achieve the goal of European marketing authorization.

Our team of regulatory scientists is able to work with situations such as this, which provide unique challenges, and apply the available data to bring an innovative product to regulatory approval in the EU.

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