Challenge

An US-based company was developing a new drug to treat a rare disease, and they needed to obtain Orphan Drug Designation (ODD) in Europe to accelerate the drug's regulatory approval process. They have already obtained an ODD in the USA, however, they were unfamiliar with the complex regulations and requirements for obtaining ODD in Europe.

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Phortas Solution

The company reached out to Phortas, as an SME registered EU-based regulatory consultancy, for assistance. Our team of experts worked closely with the company to understand the clinical development program and the target population for the drug. We analyzed the EU regulations, standard of care and disease prevalence applicable to company's targeted therapeutic indication/drug and provided guidance on how to develop an effective strategy for obtaining ODD in Europe. Based on the previous USA ODD dossier, we prepared a comprehensive EU dossier that included all the necessary documentation and data required by the European Medicines Agency (EMA) for ODD. We also worked closely with the company to ensure that the dossier was of the highest quality and met all the necessary regulatory requirements.

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Outcome

Thanks to Phortas' SME status, regulatory and clinical expertise and guidance, the company successfully obtained ODD in Europe. The company appreciated our in-depth involvement into the disease landscape and our understanding of company's development plans.

Obtaining an ODD in Europe was a critical step in the company's development program, as it allowed them to access various incentives, such as market exclusivity and fee reductions. This enabled the company to focus their resources on developing the drug and bringing it to market as quickly as possible.

Overall, it was a successful partnership that demonstrated the value of Phortas' expertise and commitment to client satisfaction. Our consultancy's services saved the company time and resources, as they managed to access to the EMA SME incentives and did not have to spend time navigating the complex EU regulatory landscape on their own. We are proud to have played a role in bringing a much-needed drug to patients with a rare disease.

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