



Regulatory Affairs Leadership

Synteract Helps Client Obtain Orphan Drug and First PRiority MEdicines Designation

Highlights

- Synteract provided regulatory support to obtain orphan drug and PRIME designations.
- It encouraged the client to participate in a pre-submission meeting with the EMA and provided guidance.
- The drug was designated an orphan medicinal product. Access to PRIME was granted.

Introduction

A clinical-stage biopharmaceutical client obtained orphan designation in the U.S. for its lead product and wanted to also obtain the designation in Europe. The respective rare disease can cause pain, leading to physical disabilities, organ damage, and early death. Orphan designation in Europe can

provide development and commercial incentives, market exclusivity for a period of time, product consultation from the EMA, and lower, or reduced, regulatory fees. However, the client did not have a presence in the region.

Challenges

- To be accepted for orphan designation and the PRIME scheme in Europe, a therapy must demonstrate the potential to benefit patients with unmet medical needs through early clinical data. However, calculation of the prevalence in this case across Europe was not easy to determine, as it is less widespread in the region and significant differences exist between EU member states.
- Although the disease in question is a known rare disease and orphan designations have been granted in the past, Synteract recognized the client's application needed to be supported by solid data on the nature of the disease, available therapies, whether they are considered satisfactory or not, and justification on how the intended product meets an unmet need, or could be a significant benefit to existing treatment alternatives.

Our Solutions

- Synteract acted as sponsor for the orphan designation request and encouraged the client to participate in a pre-submission meeting with the EMA to ensure a smooth process and understanding of requirements. Synteract experts emphasized that while a more thorough procedure around

submission preparation could extend timelines, having all requirements properly addressed could shorten back and forth communication between the client and the Committee for Orphan Medicinal Products (COMP) overall and be more likely to ensure success.

- Synteract leveraged its regulatory experience to calculate detailed information on prevalence of the disease in the EU and potential benefits of the drug for patients with unmet medical needs.
- **Synteract provided strong medical and regulatory leadership throughout the process and performed a final regulatory review of the complete package before it was submitted to make sure it included all information required by authorities.**

Program Success

- Recommendations from the pre-submission meeting played a large role in creating a meaningful submission, and providing information upfront, eliminating the need for COMP members to raise questions afterwards.
- **The European Commission (EC), acting on a positive recommendation from the COMP, designated the drug an orphan medicinal product with no comments, signifying that the opinion was unanimous amongst the 36 members of the COMP and Eurordis reviewers.**
- Synteract is the official holder of the orphan drug designation in the EU for this product on behalf of the client.

- Based on the availability of early clinical data, the potential to significantly address an unmet medical need was appropriately justified. **Synteract acted successfully as applicant for the PRIME eligibility request on behalf of the client and access to the PRIME scheme was granted.**

Medicines eligible for PRIME can also expect to be eligible for accelerated assessment at the time of MAA. As of May 2018 only 40 medicines out of 145 applications have been granted access to the PRIME scheme.

About Synteract

With employees across 21 countries, Synteract is an innovative, full-service CRO supporting biopharma companies across all phases of drug development to help bring new medicines to market. Synteract has conducted 4,000 studies on six continents and in more than 60 countries, working with more than 26,000 investigative sites and 750,000 patients. It has contributed to more than 240 product approvals. Synteract offers a notable depth of therapeutic expertise in oncology, dermatology, and neuro degenerative indications, as well as rare and orphan, pediatric, and immunotherapy studies.

For more information on how we can support your clinical trial, please visit www.synteract.com/Therapeutic-Expertise/Rare-Orphan-Diseases or ContactUs@synteract.com.

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