Applying for Orphan Designation

Orphan designation in a nutshell

If you are developing a small molecule, biologic, cell or gene therapy for a rare disease, an orphan designation in the EU is critical to commercial success. Orphan drug designation also comes with several incentives, including:

- Providing an additional layer of exclusivity.
- Creating a more favourable pricing and reimbursement environment.
- Regulatory fee reductions.

An orphan application can be submitted any time prior to filing your marketing authorization application (MAA), and it is ideal if you have limited clinical data available to include in your submission.

Orphan designation provides 10 years of market exclusivity. In practice, this means the European Medicines Agency (EMA) cannot accept an MAA for a similar product during this period, such that the exclusivity in effect runs during the MAA of the competitor – allowing for nearly 11 years of exclusivity. Exclusivity can also be extended by 2 years for completion of a paediatric investigation plan.

New medicines for orphan conditions also have to demonstrate significant benefit over existing treatments, in order to maintain orphan designation and ensure commercial viability.

It is also worth noting that some products which are off patent can become commercially viable through orphan exclusivity.

Finally, it is not possible to combine orphan and non-orphan products in the same MAA – this entails planning the initial versus lifecycle indications for the product carefully to maximize commercial success.

We have submitted numerous orphan applications across different therapeutic areas and modalities.

How we can help you

1. **We can write and submit your orphan designation application.**

We can draft the orphan application and submit it to the EMA. In particular, we can address the medical plausibility and significant benefit sections of the orphan application – these are critical components that can dictate the success or failure of the submission.

2. **We can plan your orphan strategy.**

More importantly, we can also plan the broader orphan strategy – obtaining the designation is only the first step. The sponsor is expected to collect quantitative data (usually through pivotal clinical trials) to demonstrate that the criteria under which the designation was awarded are still valid at the time of the MAA (including the need to address significant benefit). The strategy for maintenance can be complex and should be evaluated through scientific advice with the Committee for Orphan Medicinal Product.

Your roadmap to working with Somerville Development Partner

Initial conversation

It is always a good idea to start with an open-ended discussion about your needs and challenges, and we can give you some preliminary advice on how to approach your regulatory interactions in Europe and the US.

Project scoping

To provide a specific proposal for how we would approach your project, we require access to some of your project details. Therefore, we always recommend signing a confidentiality agreement before discussing the scope of your project.

Next, we will provide a scope of work for you to review. Once this is agreed, we will start work on the project.

Kick-off

We will assign an experienced consultant to act as your Regulatory Lead from the very beginning—your expert will take overall responsibility and accountability for your project from start to finish.

Your Regulatory Lead will always be someone who has:

- 10–15 years of experience
- tenure in established pharmaceutical and biotechnology companies, and
- been the regulatory lead on a global project team.

Your Regulatory Lead will be your primary point of contact and will also draw on support and expertise from additional consultants, as needed.

We ask that you securely share all product-related documents so that we can conduct a detailed evaluation of the data. This will enable us to provide informed recommendations for how we will deliver the project.

The most common documents we request include:

- study protocols,
- the investigator brochure,
- clinical study reports,
- briefing books from prior regulatory interactions, and
- meeting minutes from prior regulatory interactions.

This also means that we can repurpose as much content as possible to reduce costs.

We will agree the timelines, overall project delivery and desired outcomes with you. We will also identify the relevant stakeholders who will need to review and provide input into the project.

Delivery

During the project, we look out for your best interests and work to help you achieve your corporate objectives. We work as a partner and proactively identify issues, solutions and opportunities, rather than acting as a passive or transactional vendor.

Our consulting team will produce the agreed deliverables to the required timetable.

We will meet with you as often as needed to discuss the progress of the project and request any additional information. We will also meet—as necessary—to discuss review cycles, final signoff and submission.

Every project we work on undergoes peer review, as well as quality control of the final submission. That way we effectively draw on all additional experience from among our team of life sciences experts.

Feedback

Feedback is very important to us, so most of all we want to hear your thoughts on how the project went.

Getting in touch

We always welcome the opportunity to discuss a potential project with you!

Tom Oakley
Founder & Principal Consultant
tom@somerville-partners.com

SOMERVILLE
DEVELOPMENT PARTNERS