Case Study

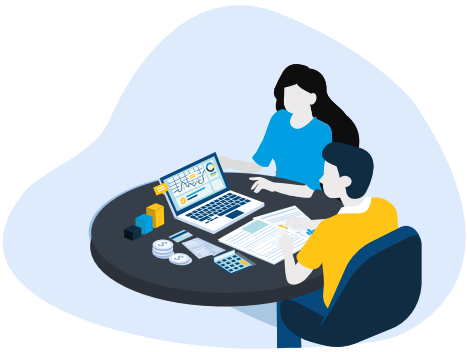
Coordination and Regulatory support across Europe for an innovative product/orphan drug.

Context :

The Vice-President Global Regulatory Affairs of a US based company developing an important innovative product (orphan drug) contacted BlueReg to seek regulatory support to discuss the different stages of development in Europe.

Our Client Objectives:

- Understand the pathway towards approval in the proposed market and target therapeutic indication.
- Anticipate potential discussion highlights with regulators and take advantage of pathways according to product type/category (ATMPS, ODD).
- Prepare a robust regulatory dossier that details application in order to optimize chance of approval and success.
- Alignment of EU with US strategy (streamline & capitalize, minimize change & cost).



Our Client Obstacles:

- Unfamiliar with EU health authorities.
- Inexperience with EU accelerated pathways or existing incentives (e.g. SME Status).
- Very limited resources or expertise in Europe (no affiliates or partners).

The project:

1. Supporting access to SME (small and medium sized enterprises) incentives granted by the European Medicines Agency (EMA)

The client's SME status application was prepared and submitted on behalf of the US company; this was subsequently granted within the short timelines agreed.

The client's SME status then enabled the US client to benefit from administration and financial incentives as defined in the Commission Regulation (EC) 2049/2005, through BlueReg acting as their EU legal representative.

2. Facilitation of Competent Authority meetings during drug development, in the course of submitting a new drug application or marketing authorization application:

- Support in developing Client scientific advice strategy to ensure that meaningful and useful advice is the achieved outcome.
- Strategic advice on selecting the optimal competent regulatory authorities in order to gain individual member state perspectives on program development .
- Author the required documents with Client input: briefing package, protocol synopsis, responses to list of questions and positions, meeting presentation and subsequent meeting minutes.
- Preparation for the competent authority advice meetings, by way of rehearsal and/or represent or assist the Client at face-to-face discussion meetings, as required.
- Coordination of the complete procedures (letter of intent, pre-submission phase, evaluation phase) – in order to manage overall timelines to ensure a smooth and timely procedure.

3. EMA / HTA Parallel Consultation

Our consultants advised our client on all steps of the procedure and authoring the required documents with the support of the affiliate partners:

- Provide recommendation on HTA advice strategy.
- Check the eligibility for the consolidated parallel consultation pathway.
- Review the proposed consultation strategy on regulatory, market access and writing aspects.
- Author the required documents with Client input: briefing package, protocol synopsis, responses to list of issues, slide deck, meeting minutes.
- Coordinate on Client behalf the complete procedures at local level with the support of local partners.

4. Paediatric Investigation Plans (PIPs)

Our consultants advise in the overall preparation and design of Client initial PIP/PSP:

- Assess the right strategy concerning the PIP/PSP scope and timelines to meet Client objectives.
- Agree on the right condition and indications to be studied through a systemic approach and rigorous methodology.
- For all age subsets, assess whether a waiver / deferral can be considered based on available data and propose risk mitigation.
- Interact with agencies in case of Paediatric SA, pre-submission meeting and complete PIP procedure.
- Write and prepare the corresponding documents: Briefing pack, presentations, paediatric plans and corresponding synopses or justifications for waivers/deferral.
- Coordinate the full procedures (scientific advice/ agency meeting, presubmission meeting, PIP/ PSP) and represent Client at agency level in the event of face-to-face meeting.
- Support with PIP amendments in Client paediatric developments, the compliance check procedure or annual deferral report preparation and submission.

The team:

Project Manager (PM)

- Central contact at BlueReg for any questions or requests linked to the contract, budget, timelines & deliverables.
- Ensures coordination of the project from innovation to conclusion, sets up a dedicated share point between BlueReg and the Client for secure document exchange, and will ensure that the appropriate resources are assigned to the project.
- Coordinate, supervise and follow up on the team's activities and BlueReg partners (if any) with the objective of delivering quality product on-time within budget.
- Manages the scope of the project; organizing and preparing the kick-off meeting, provide the RACI, develop the project plan/schedule, ensure appropriate communication channels, and organize meetings as planned and follow-up on the defined metrics/KPIs.

Program Regulatory Lead (PRL) and Program Scientific Writing Lead (PSWL)

- The role of a PRL is to oversee/manage the project from a regulatory/technical standpoint and the PSWL from a scientific writing point. The PRL/PSWL are senior regulatory or scientific writer consultants.
- The PRL will oversee the development of a regulatory strategy, the technical aspects during the project, including the coordination of regulatory consultants.
- The PRL is the primary contact point for all general regulatory questions/issues, who will work in close collaboration with the PM, the PSWL and the consultants team.
- The PSWL will oversee the preparation, writing and update of all scientific documentations in close collaboration with the PM and PRL and the consultants team.

Team of consultants

- The team of consultants is a multidisciplinary team of regulatory affairs consultants able to provide expert advice as well as hands-on operational support.
- The allocated resources have strong experience in the different fields of the project, and possess solid collaborative and communication skills, as a cooperative approach among all the stakeholders is key for the success of those projects.

BlueReg Solutions benefits:

- Anticipate regulatory milestones to optimize development.
- Immediate access to rare skills & qualified resources in the target market.
- Take advantage of European Knowledge & Expertise to optimize EU expansion.
- Gain time and control budget on Development process.