Case Study

French Early Access Program management for a US pharmaceutical company

PharmaBlue (a BlueReg company) as French "exploitant" company can provide operational, pharmacovigilance and regulatory support in the management of early access programs in France.

Indeed, PharmaBlue partners with a lot of US companies looking to outsource early access program in France and Exploitant responsibilities in the field of rare or serious disease & unmet medical need

Reminder :
French regulatory authorities require having an « Exploitant » authorized and established in France to manage an early access program.

Scope :

An American pharmaceutical company starting to build its European affiliates entrusted PharmaBlue with the responsibility of submitting, obtaining, and implementing a French Early Access Program for its orphan drug.

PharmaBlue support them for the whole process of submission (advice, writing activities and support for meeting with the Competent Authorities) as well as for the early access operational management through to Marketing Authorization.

Challenges :

An American pharmaceutical company wants to submit an early access dossier and implement this early access program in France for one of its orphan drugs. This project represents a major challenge for the client who need partnership strategy and for patients who had no other therapeutic alternative treatment in France for their disease.

Benefits of early access program in France:

- Allowing continuous access to the drug for patients
- Building the company image before commercial launch
- Generate turnover before being approved
- Setting the scene for future market access of commercial product

It is essential for the client that the treatment is known to the physicians in order to improve their understanding and facilitate the prescription of the treatment after it has been marketed in France.

A comprehensive understanding of the client clinical and commercial expectations is important to develop a solution specific to their needs, no matter how complex it is.

This essential first step allows clients to realize the full value of PharmaBlue's expertise and experience in French Early access Program. The client and PharmaBlue worked together to complete this project.

It was a long-term collaboration between the client and PharmaBlue since several months passed between the submission of the early access dossier and the approval by the HAS. Further to this approval. This collaboration has resulted in the success of this project.

BlueReg Support:

There are some situations where patients have a life threatening or orphan disease, and their physicians have exhausted all available treatment options or there is simply no treatment. Some country-specific mechanisms exist across the globe to enable access to pre-approved or unlicensed medicines. These mechanisms support and an earlier access to more and more innovative medicines for patients.

PharmaBlue is composed of experts in preparation and submission of early access dossiers in compliance with the current regulations and also through the new pilot schemes.

PharmaBlue has the skills and expertise to implement early access programs and patient follow-up in line with the current regulations and with pharmacovigilance (PV) requirements.

The PharmaBlue team worked in close collaboration internally and with the client.

Scientific writing

- Preparation and writing of early access dossier including the letter of intent, the application form, justification part and the Protocol for Therapeutic Use and information collection (PUT-RD)

Operational activities and project management

- PharmaBlue has set up an early access task force in order to ensure the validation of patient inclusion, drug orders, patient follow-up in the program.
- Patients' data collected by PharmaBlue were analysed for early access report writing.

Regulatory advice

- Advice for early access renewal dossier, MA dossier.
- Advice and warning on the specificities of the French regulations, particularly with regards to the different early access schemes and follow-up activities once early access ended.

Pharmacovigilence

- Management of PV cases (analysis of PV cases and submission to the French authorities).
- Management of medical information requests.

Quality Management

- Management and follow-up of treatment batches.
- Management of quality complaints.

Achievements:

- Submission of early access dossier in accordance with agreed timelines.
- Submission of PV cases to French authorities with compliant deadlines.
- Operational activities efficiently managed: successful collaboration with all client partners involved in this project (distributors, PV provider).
- Client's satisfaction PharmaBlue's expertise recognition: PharmaBlue entrusted to continue to act as the "exploitant" of the commercial product on the French market once it was granted a marketing authorisation.

Benefits for clients

Peace of mind operations on French Market + Immediate activation of French affiliate + Access to rare skills and experienced early access teams in France