CASE STUDY

EARLY ACCESS PROGRAMS (ATU)

Implementation of a French Early Access Program for orphan drug patients in nominative & cohort program

A European pharmaceutical company entrusted PharmaBlue (a BlueReg Company) as French «exploitant» with the responsibility of submitting, obtaining and implementing a French Early Access Program called "ATU" (Autorisation Temporaire d'Utilisation) for its orphan drug for patients in nominative and cohort program.

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Challenges

A European pharmaceutical company wants to submit an ATU dossier and implement this ATU 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. 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 marteked in France.

A comprehensive understanding of their clinical and commercial expectations is important for the client to develop a solution specific to their needs, no matter how complex it is. This essential first step allows clients to realise the full value of PharmaBlue's expertise and experience in French Early access Program. The client and Pharma Blue 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 ATU dossier and the approval by the ANSM. Further to this approval, the nATU (Autorisation Temporaire d'Utilisation nominative) and cATU (Autorisation Temporaire d'Utilisation de cohorte) lasted one year with more than 190 patients included.

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 ATU dossiers in compliance with the current regulations and also through the new pilot schemes.

PharmaBlue has the skills and expertise to implement nominative and cohort ATUs 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 ATU dossier (protocol for therapeutic use (“PUT”), intention letter to authorities, SmPC and Patient leaflet, ATU forms, etc.).

- 6-month periodic ATU reports (clinical and safety).

Operational activities and project management:

- PharmaBlue has set up an ATU unit in order to ensure the validation of patient inclusion, drug orders, patient follow-up in the cohort ATU.

- Patients data collected by PharmaBlue were analysed for ATU report writing.

Regulatory advice

- Advice for ATU renewal dossier, MA dossier.

- Advice and warning on the specificities of the French regulations, particularly with regards to the different ATU schemes and follow-up activities once ATU ended.

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Pharmacovigilance

- 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 ATU 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.

How BlueReg can support you ?

Early Access Programs (and ATU)

For some rare, life threatening conditions or those of major public interest, the early access to investigational drugs prior to regulatory approval is vital.

BlueReg can help navigate the complex environment of Early Access Programs (EAP) in the EU, we will work cross-functionally with your functional teams to ensure the correct timing of the EAP and strategically plan to avoid any possible hurdles. We have considerable experience in the arena of EAPs and can design and implement the programs on your behalf in the EU.

You can rely on the experience and expertise of our consultants for providing advice and efficient implementation of ATU projects (nominative and cohort):

- Provision of advice or responses to questions including strategy and/or positioning of ATU projects
- Preparation or review of ATU applications including the protocol for therapeutic use (PUT)
- Submission of ATU dossiers to the French National Agency for the Safety of Medicines and Health Products (ANSM) and follow up until ATU is granted

Via our in-house organization, PharmaBlue, we manage ATU programs in the following ways:

- Pre-launch activities for the management of ATU programs: setting up a dedicated ATU team (cell ATU), assigning toll-free phone / fax numbers and creation of a database for the management of the ATU
- Day-to-day management of ATU programs: Patient inclusion and follow up, validation of the pharmacist orders, processing and capturing all necessary data
- Generation and submission of ATU periodic reports
- Tailored approach to completely manage the end to end process or to complement existing supply chain

For more information please contact us

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