WEP

With Every Patient.

CASE STUDY:

EXPERTLY MANAGING ACCESS REGULATORY COMPLEXITIES THROUGHOUT A DRUG'S DEVELOPMENT LIFECYCLE

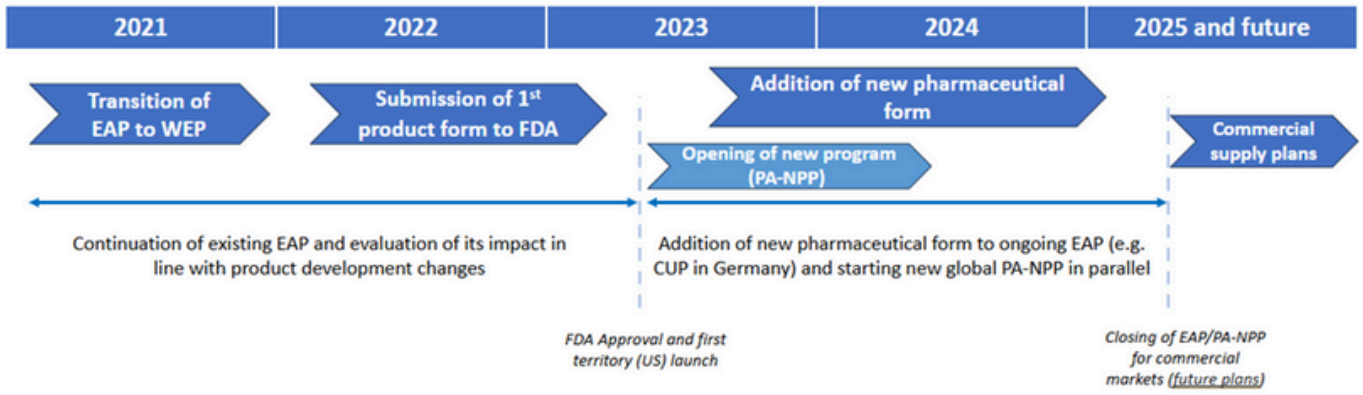
Background

WEP partnered with a global biopharmaceutical company (the Sponsor) to take over the management of an existing Expanded Access Program (EAP) for a rare disease investigational medicinal product (IMP) that the Sponsor acquired from another manufacturer. The purpose of the EAP was to allow greater access for patients around the world who were unable to enroll into active clinical trials.

This case study highlights how the WEP team expertly managed the regulatory complexities involved as the IMP progressed through its development lifecycle. This included multiple changes to marketing authorization stages and introducing additional pharmaceutical forms and new patient populations.

Successfully navigating these scenarios and proactively adapting our regulatory strategy in real-time allowed us to ensure no disruption to treatment for patients and continued program compliance.

The following schematic illustrates the main project evolution milestones:



Milestone 1: The EAP project ownership was transitioned over to WEP

WEP Objective:

The WEP team had to ensure seamless continuation of treatment for all patients enrolled under the existing EAP, set up by the initial IMP owner. The Sponsor also wanted WEP to find a way to extend access to patients in additional territories that were not covered by the initial EAP framework.

Key Take-aways:

At the outset, we worked closely with the Sponsor to fully understand its specific goals for maintaining the EAP and expanding access to patients in additional countries. Based on what we heard from the Sponsor, we then established a tailored and comprehensive project plan that covered both existing territories and new ones. This was created with strategic input from all key WEP stakeholder teams, including Regulatory Affairs, Business Development, Project Management, Supply Chain and Quality.

We then developed a tailored regulatory strategy for each territory, which:

- Differentiated individual patient access pathways vs. cohort applications
- Took into consideration any potential long-term Sponsor obligations
- Fulfilled any local specific labelling requirements
- Outlined any in-country vendor management and support
- Managed direct coordination with local authorities

Milestone 2: The Sponsor submitted the medicinal product marketing authorization application (MAA) in the US for the main pharmaceutical form (tablets) in adult population

WEP Objective 1:

The WEP team had to prepare to close-out the EAP in the US, transitioning patients from EAP supply over to commercial supply. We also had to evaluate the impact of MAA approval on the EAP strategy, for all ex-US territories, including the EU, UK, Canada, Australia and Japan.

Key Takeaways:

We ensured clear and timely adaptations of the EAP regulatory strategy in line with MAA planning – this included:

- Revising program eligibility criteria
- Developing a patient termination and transition plan for the US, as well as future launch countries, to manage the process of switching patients over to commercial supply
- Ensuring a temporary extended access plan was in place, just in case commercial access was delayed
- Ensuring clear and readily available messaging about commercial availability and next steps for healthcare providers in the US
- Adjusting supply chain logistics based on new commercial distribution channels
- Ensuring adequate drug supply for ongoing EAPs in regions where the program was to continue.
- Proactively working with health authorities to ensure all regulatory requirements were captured and our processes were in compliance.

This ensured we were successfully prepared for the termination of the EAP in the US, with no disruption to EAP supply or management in the rest of the world. During this process, we provided regular communication with the Sponsor team with real-time information and updates, which was key to compliantly maintaining the EAP.

WEP Objective 2:

At this time, the WEP team also had to consider and present to the Sponsor the potential for a new Paid-for Post-Approval-Named Patient Program (PA-NPP) – to fulfill requests from individual physicians for patients that might benefit from treatment with US commercial product, once approved and launched, in the territories where the product had not been licensed and where EAP supply was not made available.

Key Takeaways:

We developed strategic additional program opportunities alongside the Sponsor's key market launch plans, which enabled the PA-NPP to commence in parallel with the US launch.

We ensured that the PA-NPP was designed to align with the Sponsor's broader pre-launch strategy, providing real-world experience in new markets while generating revenue to support the program and future commercialization efforts.

Milestone 3: The Sponsor added a different product pharmaceutical form and lower dosages to treat an additional patient population (pediatric patients)

WEP Objective:

The WEP team had to incorporate this change in product development into the EAP scope, as requested by Sponsor. This meant evaluating access possibilities for the new dosage form/new patient population within the current EAP for intended territories.

Key Takeaways:

Regulatory authorities require proper justification of the medicinal product's proven safety and efficacy for usage in early access treatment, with increased scrutiny when pediatric patients are involved. As such, we worked with the Sponsor to evaluate the new development data gathered from active clinical trials, to ensure that this treatment option was medically justified.

We implemented robust program change management to ensure the continuity of treatment with the existing medicinal product, while simultaneously allowing treatment with an additional dosage form. In some territories, this meant utilizing a combined strategy addressing both patient populations and dosage forms, while in others, access was individualized based on specific requests.

Country-Specific Example – German (BfArM) CUP Application:

In Germany, the regulatory agency (BfArM) required the WEP team to maintain the active German compassionate use program (CUP) for the initial IMP form, strength, and patient population (adult patients), with annual renewals. At the same time, we had to incorporate substantial modifications to introduce the additional scope, i.e. new pharmaceutical form, strengths, and patient population (pediatric patients). We conducted preliminary consultations with BfArM to receive the pre-submission scientific advice on the product-specific scenario.

This facilitated an efficient submission strategy and resulted in smoother assessment of the application.

We also worked closely with the Sponsor's medical and safety teams to present the clinical data supporting the CUP modification, and we engaged proactively with the local KOL network to justify unmet need, emphasizing the lack of alternative satisfactory treatment options.

We then ensured timely execution of the parallel application for CUP re-notification, along with the substantial CUP modifications. This resulted in a positive outcome and the approval of updated CUP criteria, ensuring timely treatment for both adult and pediatric patients, which is still ongoing in Germany.

Conclusion

As this case study demonstrates, the key to efficient EAP management by WEP involves open communication with the Sponsor and a clear understanding of the Sponsor's IMP development strategy and commercial plans, while effectively accounting for all local and international regulatory requirements. In all of our programs, any potential risks that can influence program execution are recognized and mitigated in a timely manner, allowing for the smooth implementation of an EAP as well as a seamless transition to commercial supply. Furthermore, WEP has the capability to support Sponsors in commercial launch distribution of their approved product, as was the case with this client.