

EXPANDED ACCESS PROGRAM • IMMUNOLOGY & GENETIC DISORDERS

Expertly Managing Access Regulatory Complexities Throughout a Drug's Development Lifecycle

How WEP Clinical helped a rare disease Sponsor scale an Existing EAP and transition a new pediatric patient population onto treatment within one month – without a single missed dose.

PROGRAM SNAPSHOT

Therapeutic Area

Immunology &
Genetic Disorders

Program Type

Expanded Access
Program (EAP)

Pediatric

Yes

Country in Scope

26 globally

BACKGROUND

WEP Clinical partnered with a global biopharmaceutical company to take over management of an existing Expanded Access Program (EAP) for a rare disease investigational medicinal product (IMP) that the Sponsor had acquired from another manufacturer.

The purpose of the EAP was to allow greater access for patients globally who were unable to enroll in active clinical trials.

As the product moved through its development lifecycle, WEP had to adapt the regulatory strategy in real time to account for commercial planning, evolving marketing authorization stages, new pharmaceutical forms, lower dosages, and additional patient populations. The central objective was clear: maintain uninterrupted patient access while keeping the program compliant across changing regulatory, commercial, and clinical development milestones.

THE CHALLENGE

This EAP was not a static program. It evolved as the Sponsor's development and commercialization plans progressed. WEP needed to manage the existing EAP while also preparing for major changes, including:

- Transitioning EAP ownership from the initial IMP owner
- Expanding access into additional territories not covered by the original framework
- Preparing for US commercial approval and patient transition to commercial supply
- Evaluating the impact of approval planning on ex-US EAP territories
- Developing a potential Paid-for Post-Approval Named Patient Program (PA-NPP)
- Incorporating a new pharmaceutical form and lower dosages
- Supporting access for pediatric patients
- Managing country-specific regulatory requirements, including Germany's BfArM CUP process

The key challenge was clear: WEP needed to anticipate how each product lifecycle change would affect patients, sites, supply, eligibility criteria, local approvals, and long-term Sponsor obligations.

WEP CLINICAL'S SOLUTION

At the outset, WEP worked closely with the Sponsor to understand its goals for maintaining the EAP, expanding access to additional countries, and aligning the access strategy with broader development and commercial plans.

WEP then created a tailored project plan covering both existing and new territories, with input from key WEP stakeholder teams including Regulatory Affairs, Business Development, Project Management, Supply Chain, and Quality.

For each territory, WEP developed a regulatory strategy that considered:

- Individual patient access pathways versus cohort applications
- Long-term Sponsor obligations
- Local labeling requirements
- In-country vendor management
- Local authority engagement
- Supply continuity
- Patient transition planning



Milestone 1: EAP Ownership Transitioned to WEP

The first priority was to ensure seamless continuation of treatment for all patients already enrolled under the existing EAP. WEP also needed to identify ways to extend access to additional territories that were not covered under the original EAP framework. This required a country-by-country review of available access pathways, operational feasibility, and regulatory requirements.

WEP Impact: WEP helped the Sponsor maintain continuity for existing patients while creating a more structured framework for expanding access into new territories.



Milestone 2: Preparing for US Commercial Approval and Global EAP Impact

As the Sponsor moved toward US approval for the main pharmaceutical form in the adult population, WEP had to prepare for EAP close-out in the United States and transition patients from EAP supply to commercial supply.

At the same time, WEP evaluated how US approval would affect the EAP strategy in ex-US territories, including the EU, UK, Canada, Australia, and Japan.

WEP supported this transition by:

- Revising program eligibility criteria
- Developing patient termination and transition plans for the US and future launch countries
- Preparing a temporary extended access plan in case commercial access was delayed
- Creating clear messaging for healthcare providers about commercial availability and next steps
- Adjusting supply chain logistics based on new commercial distribution channels
- Ensuring adequate supply for ongoing EAPs in regions where the program would continue
- Working proactively with health authorities to capture regulatory requirements and maintain compliance

This helped WEP prepare for US EAP termination while maintaining EAP supply and management in the rest of the world.

WEP Impact: WEP helped the Sponsor manage the transition from EAP supply to commercial supply in the US while protecting access continuity in ex-US markets.



Milestone 3: Developing a PA-NPP Strategy Alongside Launch Planning

WEP also helped the Sponsor assess the potential for a PPA-NPP. The purpose of the PA-NPP was to fulfill individual physician requests for patients who could benefit from treatment with the US commercial product after approval and launch, in territories where the product was not yet licensed and where EAP supply was not available.

WEP developed the PA-NPP opportunity alongside the Sponsor's market launch plans, enabling the program to begin in parallel with the US launch.

This meant the Sponsor could continue supporting access needs in unlicensed markets while aligning the program with broader pre-launch and commercialization strategy.

WEP Impact: WEP helped the Sponsor move beyond reactive access management and build a strategic access pathway that supported future market preparation.



Milestone 4: Adding a New Pharmaceutical Form, Lower Dosages, and Pediatric Access

The Sponsor later added a different pharmaceutical form and lower dosages to support treatment for an additional patient population: pediatric patients. WEP had to incorporate this development change into the existing EAP scope and evaluate whether the new dosage form and patient population could be supported in each intended territory.

Because pediatric access can face increased regulatory scrutiny, WEP worked with the Sponsor to review new development data from active clinical trials and ensure the treatment option was medically justified for early access use.

WEP also implemented program change management so treatment could continue with the existing medicinal product while allowing access to the additional dosage form. In some territories, this required a combined strategy covering both patient populations and dosage forms. In others, access was managed on an individualized request basis.

WEP Impact: WEP helped the Sponsor expand the EAP in a controlled way while maintaining continuity for existing patients and enabling access for a new pediatric population.



Country-Specific Example: Germany BfArM CUP Application

Germany became a key example of WEP's regulatory problem-solving.

BfArM required WEP to maintain the active German Compassionate Use Program for the original IMP form, strength, and adult patient population through annual renewals. At the same time, WEP had to introduce substantial modifications to add the new pharmaceutical form, strengths, and pediatric population.

WEP conducted preliminary consultations with BfArM to obtain pre-submission scientific advice on the product-specific scenario. This supported a more efficient submission strategy and smoother assessment of the application.

WEP also worked closely with the Sponsor's medical and safety teams to present clinical data supporting the CUP modification. In parallel, WEP engaged with the local KOL network to justify the unmet need and emphasize the lack of satisfactory alternative treatment options. WEP then executed the parallel application for CUP re-notification and substantial CUP modifications. This resulted in approval of updated CUP criteria, supporting timely treatment for both adult and pediatric patients in Germany.

WEP Impact: WEP helped maintain the existing German CUP while expanding the program to support a new formulation, lower dosages, and pediatric patients.

THE OUTCOME

WEP helped the Sponsor manage a complex EAP through multiple regulatory, commercial, and clinical development changes while maintaining treatment continuity for patients. By adapting the regulatory strategy in real time, the Sponsor maintained program compliance, supported treatment continuity, managed US commercial transition planning, developed a PA-NPP strategy, and expanded access to a new pediatric patient population.

To date, the program has supported:

150+ Patients

90 sites

26 countries/markets in scope

Countries Included:

Europe & Eurasia: Denmark, UK, France, Italy, Spain, Netherlands including Aruba, Germany, Switzerland, Sweden, Finland, Czechia, Serbia, Slovakia, Russia, and Turkey

Asia-Pacific: Australia, South Korea, China, Hong Kong, and Singapore

North America: United States and Canada

Middle East: Israel and Saudi Arabia

Latin America: Argentina and Mexico

WHY THIS MATTERS

EAPs often evolve as a product moves through development. Clinical data matures, new formulations may be introduced, additional patient populations may become eligible, and commercial launch planning can change the access pathway in some countries while the EAP continues in others. Without a proactive regulatory strategy, these changes can create treatment disruption, compliance risk, supply challenges, and confusion for sites and physicians. This case demonstrates how WEP Clinical can help Sponsors manage access programs across the full product lifecycle, from investigational access through commercial planning and post-approval named patient strategy.

NEED TO MANAGE REGULATORY COMPLEXITY IN AN EXPANDED ACCESS PROGRAM?

Let the WEP team help navigate regulatory change without disrupting patient access.

WEP Clinical supports Sponsors with regulatory strategy, project management, supply planning, country pathway assessment, patient transition planning, PA-NPP strategy, and lifecycle access program management.



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