

EXPANDED ACCESS PROGRAM • PEDIATRIC RARE DISEASE

Using Expanded Access as a Strategic Alternative for Post-Trial Access Versus a Traditional Open Label Extension Study

How WEP Clinical helped a rare disease Sponsor transition more than 500 patients from an OLE to a global Expanded Access Program across 14 countries - without disrupting treatment.

PROGRAM SNAPSHOT

Therapeutic Area
Neurology

Program Type
Expanded Access Program (EAP)

Advanced Therapy
Yes

Country in Scope
14 globally

SUMMARY

Following completion of a pivotal Phase 3 clinical trial, a global pharmaceutical company needed a pathway to continue treatment access for patients with a debilitating neurological disorder.

The Sponsor had originally planned to provide continued treatment through an Open Label Extension (OLE) study managed by its incumbent CRO.

However, over time, the OLE became increasingly burdensome and costly to maintain, particularly because the Sponsor was no longer required by regulatory agencies to collect additional long-term follow-up data after the pivotal study.

At the time, more than 500 patients were receiving continued treatment through the OLE across 14 countries and 4 continents. The Sponsor remained committed to treatment continuity but needed a more practical and sustainable model for what had become primarily an access objective.

WEP Clinical worked with the Sponsor to evaluate an alternative Post-Trial Access (PTA) model and identified Expanded Access as the right pathway. Within three months, WEP supported the transition of patients from the OLE into an Expanded Access Program (EAP) with no disruption to treatment.

THE CHALLENGE

OLE studies can be valuable when additional long-term safety, efficacy, or tolerability data are needed. In this case, however, the Sponsor's primary objective was no longer evidence generation. It was treatment continuity.

The OLE had become difficult to sustain because it required the Sponsor to maintain clinical trial infrastructure across a large, geographically dispersed patient population.

More than 500 patients were receiving treatment across 14 countries, creating significant coordination requirements across sites, treating physicians, supply chains, regulatory pathways, and internal Sponsor teams.

The Sponsor also needed to preserve internal resources for ongoing clinical trials and development activities.

Continuing the OLE in its existing form would have required substantial oversight and site management, despite the fact that additional follow-up data collection was no longer required.

The Sponsor needed an alternative model that could:

- Maintain continued treatment access for patients already receiving therapy
- Reduce the operational and site burden associated with the OLE
- Provide a compliant structure outside the traditional clinical trial setting
- Support a coordinated transition across multiple countries
- Preserve Sponsor resources for ongoing development activities

The key challenge was clear: transition a large post-trial patient population from an OLE into an EAP while maintaining uninterrupted treatment access.

WHY EXPANDED ACCESS WAS THE RIGHT FIT

Expanded Access provides a pathway for eligible patients to access investigational medicines outside of a clinical trial when no satisfactory alternative treatment options are available.

For this Sponsor, Expanded Access offered a more practical alternative because the purpose of the program was access, not continued clinical data collection.

Unlike an OLE, which is structured as a clinical trial and may require more extensive protocol-driven procedures, an EAP can often be designed around treatment access and safety oversight, with additional data collection included only when appropriate for the program, country, and Sponsor objectives.

This distinction was critical. The Sponsor did not want to withdraw treatment from patients who had completed the Phase 3 trial, but it also needed to move away from a model that placed significant strain on internal resources and costs.

An EAP provided a way to maintain patient access while reducing the operational burden of the previous OLE model.

WEP'S APPROACH

1

Program Assessment and Transition Planning

WEP Clinical first supported the Sponsor in assessing whether an EAP was appropriate for the patient population, treatment objective, and countries involved. This included reviewing the differences between the OLE and EAP model, identifying key operational considerations, and defining a transition plan that could be implemented within the Sponsor's required timeline.

WEP Impact: WEP helped the Sponsor move from a resource-intensive clinical trial structure to a more appropriate access-focused model.

2

Country-by-Country Access Strategy

Because patients were located across 14 countries, the transition required careful coordination of country-specific access pathways.

WEP helped structure the program around local requirements, recognizing that Expanded Access regulations, documentation expectations, approval processes, and timelines vary by country.

WEP Impact: WEP created a global EAP framework that could be adapted locally, rather than relying on a one-size-fits-all transition model.

3

Physician and Site Coordination

A successful transition depended on clear communication with treating physicians and sites. WEP supported the operational steps required to move patients from the OLE into the EAP, helping reduce administrative burden while maintaining appropriate oversight.

WEP Impact: WEP helped sites and physicians move patients into the new access model while keeping the transition clear, coordinated, and practical.

4

Drug Supply and Logistics Continuity

Treatment continuity was the Sponsor's primary requirement. WEP took over supply chain management for the EAP and coordinated logistics to support ongoing drug supply across multiple countries.

This precise supply chain planning helped ensure patients continued to receive treatment without disruption during the transition.

WEP Impact: WEP helped protect the most important outcome of the program: uninterrupted treatment access for patients already receiving therapy.

5

Ongoing Program Management

After the transition, WEP continued to manage the EAP, providing operational oversight, warehousing and distribution, program documentation management, and ongoing site support.

This allowed the Sponsor to maintain its commitment to patients while reducing the internal burden and costs associated with the previous OLE.

WEP Impact: WEP provided the long-term operational structure needed to keep the access program running after the initial transition was complete.

THE OUTCOME

WEP helped the Sponsor transition a large post-trial patient population from an Open Label Extension study into an Expanded Access Program within three months, with no disruption to treatment.

To date, the program has supported:



For the Sponsor, the EAP provided a way to maintain continuity of care while redirecting internal resources toward ongoing clinical development activities. For patients and physicians, it enabled continued treatment access through a more appropriate PTA model.

Most importantly, the program's success was not measured only by operational efficiency. It was measured by WEP's ability to maintain uninterrupted access for patients who were already dependent on treatment.

KEY TAKEAWAYS

1. When the objective is access, an OLE may not always be the most practical model

OLE studies can be valuable when additional structured clinical data are required. However, when the primary goal is continued treatment access, an EAP may provide a more appropriate and sustainable alternative.

2. Early transition planning is essential

Moving patients from an OLE to an EAP requires careful planning, particularly when patients are spread across multiple countries. Regulatory requirements, physician engagement, supply continuity, and program documentation all need to be coordinated early.

3. Global access programs require local flexibility

Expanded Access requirements differ by country. A successful program needs a global strategy that can be adapted to local regulations, timelines, documentation expectations, and treatment pathways.

4. Continuity should remain the central measure of success

For patients already receiving treatment after a clinical trial, any transition must be designed around avoiding interruption. In this case, the program's success was defined by the ability to maintain uninterrupted treatment access for more than 500 patients.

NEED TO TRANSITION PATIENTS FROM AN OLE TO A MORE PRACTICAL PTA MODEL?

Let WEP help you transition from OLE to Expanded Access without disrupting patient treatment.

WEP Clinical supports Sponsors with PTA strategy, EAP design, country pathway assessment, physician and site coordination, supply continuity planning, and operational delivery for complex global transition programs.



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