WEP

With Every Patient.

CASE STUDY:

PROVIDING A PAID-FOR POST-APPROVAL NAMED PATIENT PROGRAM

Background:

A global biopharmaceutical company (the Sponsor) with a Market Cap of $28bn, developing medicines for patients with rare and life-threatening diseases, approached WEP to request our support in providing global access to one of its rare disease products

Challenge:

The product had recently been approved by the US FDA to treat a rare autoimmune disorder. At the time of FDA approval, there were no disease modifying treatment options available for patients suffering from this condition. Because of this, the Sponsor was receiving requests to access the new medicine from physicians around the world, who were aware of the FDA approval and wanted access to the medicine to treat their patients.

The Sponsor wanted to find a way to manage these requests and help fulfil the unmet clinical need. However, like most biopharmaceutical companies, it was not in a position to open a free of charge Expanded Access Program on a global scale. This was particularly significant because global regulatory submissions had not started, and a free of charge access program on this scale would be extremely costly.

Solution:

WEP engaged with the Sponsor's International Executive team at a strategic level to discuss and consider all options available internationally, from a medicines access perspective.

WEP's recommendation, based on the development stage of the new medicine outside of the US and the objectives of the Sponsor, was to set up a paid-for Post-Approval Named Patient Program (PA-NPP).

WEP advised the Sponsor to open this program on a global basis, excluding the US, where the product was commercially available, so that it could fulfill requests from any country. We also advised that the Sponsor use the US commercial price for the PA-NPP, thereby preserving any future Market Access strategy in additional countries worldwide.

WEP's Approach:

WEP has deep internal knowledge of the Named Patient regulatory pathways in 120 countries around the world. Named Patient access exists in all of these countries as an option for physicians to secure local approval to import a medicine, not yet approved in their own country, under specific criteria, including:

- High unmet clinical need for the patient
- Lack of approved treatments available to the patient
- The patient is ineligible for any clinical trials

It is possible for physicians and their relevant pharmacist to purchase US-approved medicines through International Pharmacy. However, it is not available in all countries, it is often very expensive, and the Sponsor has no PV or Quality oversight of where its product is being used to treat patients.

As an alternative, WEP can establish a global PA-NPP which ensures that the Sponsor has full oversight of the patients being treated and the requesting hospitals and physicians, as well as compliance with PV, Quality, GMP and GDP in all countries.

WEP's cross-functional team, including Medical Affairs, Regulatory, Quality Assurance, PV, Supply Chain and Logistic experts, ensures that product is stored, handled and distributed to treating physicians compliantly, on time, and cost-effectively.

WEP has a Market Access team that understands the different funding pathways available in certain countries, which can help predict where most of the unlicensed demand for the medicine will come from.

We develop a robust communication plan, so that physicians worldwide are aware that the program exists, dependent on eligibility criteria. It also allows WEP and the Sponsor to share accurate and timely information with treating physicians making unsolicited requests for the medicine.

End-to-end Project Management

WEP's cross-functional approach managed the PA-NPP end-to-end, so that the Sponsor could focus on internal efforts. Our team's activities include:

- Responding to requests for the medicine from physicians worldwide through a dedicated mailbox, answering questions about the program, and supporting with local regulatory knowledge
- Reviewing and approving physician requests according to defined Patient Eligibility criteria
- Storage and international distribution of the medicine worldwide, according to a Quality Technical Agreement and strict GxP requirements.
- Ensuring PV compliance and any AE reporting requirements
- Invoicing the hospital or Healthcare Institute and managing the full Order to Cash process
- Site support and follow up for patient re-supply and any site questions

We have a dedicated Project Management team overseeing all aspects of the program and managing all communications with the Sponsor and physicians.

Benefits for Sponsors:

This program allowed the Sponsor to provide a much-needed treatment option for patients around the world, which aligned with its corporate objectives as well as its commitment to addressing critical needs for patients impacted by rare diseases.

The unlicensed medicine use also helped establish real world early clinical experience and allowed the Sponsor to build a network of KOLs and advocates to support future clinical trials and launch efforts across Europe and ROW.

The Sponsor had a long-term strategy to develop its own infrastructure in Europe and the PA-NPP supported this. Not least, the revenue from the PA-NPP allowed the Sponsor to reinvest in the medicine's further development and paid for the EU infrastructure build.

If you have any questions or want to learn more about WEP capabilities, please click the button below to send us an email.

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