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CASE STUDY

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Early licensure of a breakthrough cancer drug gives hope to patients

How named patient and expanded access programs helped speed the licensure of a breakthrough cancer drug

No clinical supply request could be a higher priority than that of a physician urgently seeking an investigational cancer drug for a patient who has exhausted all treatment options.

Fortunately, governments worldwide have made provisions for patients who don't qualify for clinical trials and have no remaining treatment options to gain access to drugs prior to licensure. In the European Union (EU), for example, named patient programs (NPPs) provide pre-approval access to drugs in response to requests of doctors on behalf of specific or "named" patients. The equivalent to NPPs in the United States are known as expanded access or compassionate use programs.

Late-stage development drug in high demand

A multinational pharmaceutical company began receiving dozens of requests from doctors worldwide for a cancer drug in late-stage development. It turned to Thermo Fisher Scientific for help. The drug was a PD-L1 inhibitor, in a category of drugs known as checkpoint inhibitors, being evaluated for the treatment of an aggressive form of skin cancer called Merkel cell carcinoma.

"With named patient programs, speed is of the essence," said Jennifer Worsfold, Thermo Fisher Scientific, Senior Director of Supply Chain Solutions & Strategic Projects at the Basel, Switzerland facility. "The NPPs require us to be expeditious in order to remove unnecessary bureaucracy and to ensure that everyone involved works for the same purpose."

Cross functional team at the ready

Acutely aware that time was a critical factor, the Thermo Fisher Scientific cross team – Clinical Supply Optimization Services (CSOS), Distribution, Clinical Label Services, Quality Assurance (QA), Qualified Persons (QP), Packaging and its in-house specialty courier – sprang into action.

Working quickly to deliver to patients

Supplies of the drug were delivered from a manufacturing facility in the EU to our clinical supply distribution center in Weil am Rhein, Germany. The Basel-based Thermo Fisher Scientific Label Services team prepared label and leaflet text for approval and obtained required information about the requesting physicians, who were considered to be investigators in single-patient trials. The team also:

- prepared and submitted mandatory import and other documentation
- obtained necessary product releases
- printed labels
- packaged clinical supplies for direct shipment to requesting physicians in the EU, U.S., Canada, Brazil, Argentina, Lebanon, Qatar and other countries.

Scheduled shipments

Shipments were timed to reach doctors preparing to see specific patients in their offices, in hospitals or in hospices. In Argentina, one desperate patient went to our clinical supply distribution facility in Buenos Aires to ask the Client Services Manager when the medication he was awaiting would reach his doctor.

As with many cancer treatments, the investigational drug had to be shipped in the cold-chain to make sure it was 100 percent temperature-compliant, so that when the supplies arrived, they could be used immediately.

Regulatory compliance

While speed and coordination were a mandate, no shortcuts were permitted in fulfilling NPP and expanded access requests. Every regulatory requirement pertaining to clinical trials had to be met. And since individual countries have their own requirements governing clinical trials, it was impossible for the Thermo Fisher Scientifics team to implement a single process for fulfilling requests.

Behind the scenes, our personnel worked closely with country authorities under just-in-time conditions to expedite delivery of investigational drug to doctors. In some cases, that required efforts that went above and beyond.

Take Qatar for example. This Middle Eastern country required a Certificate of Origin for the investigational drug that had to be stamped by the German regulatory authority, a process that typically takes one week by mail. To save precious time, a Basel-based distribution project manager volunteered to drop off and pick up the documents at the authority during his work commute. In Brazil, which has a three month timeline for the granting of an import license, our team collaborated with authorities to obtain the license in days.

Reducing timelines

These and other efforts enabled reducing the average request-to-delivery timeline of 90 days to 20 days, or the equivalent of 14 working days. In all, about 500 patients received the investigational drug through NPPs and expanded access programs. Importantly, the experience of doctors and patients in these settings helped push the drug past the finish line to early licensure in the U.S.

Early licensure

Physicians participating in expanded access programs in the U.S. reported that the drug significantly improved the condition of their patients to the U.S. Food and Drug Administration (FDA). On the basis of those reports and clinical trial results, the FDA fast-tracked the investigational drug by granting it breakthrough status.

This led to an early 2017 licensure of the drug under the accelerated approval process. The approval occurred in record time, less than four years from the start of clinical trials. Today, the drug is being evaluated in more than 50 protocols for 16 other types of cancer. Thermo Fisher Scientific is managing the clinical supply chain for all of those studies.

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