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CASE STUDY

Securing EMA Approval For First-in-Class Oncology Indication Using Science To Drive Interactions With Regulators

Learn how our Regulatory Scientists helped secure one of the first histology-independent treatments for solid tumors in the EU.

The first European Medicines Agency (EMA) approvals for drugs within a tumor-agnostic indication were granted in 2019. The Regulatory Sciences team within ProPharma was fundamental in securing one of these ground-breaking approvals by successfully navigating the rigid and often inflexible regulatory landscape within the EU and applying scientific rationale to negotiate this landmark approval for an innovative product.

ProPharma provided comprehensive and expert support to successfully navigate the regulatory maze and negotiate approval for one of the first histology-independent cancer treatments in the EU through a sound, scientifically-driven approach.

challenge

In an era of precision medicine, tumor-agnostic therapies have emerged as a revolutionary new approach to cancer treatment. ProPharma was approached to support the EU regulatory activities for a highly specific tyrosine kinase inhibitor targeting any solid tumor expressing a specific gene fusion, and to push the limits of the rigid regulatory framework to accommodate these new pan-tumor approaches.

Most of the client's regulatory activity had been US-focused, with a strategy for filing with the Food & Drug Administration (FDA) already in place. The client was now wishing to implement the same strategy in the EU.

Efficacy and safety data relied on several distinct, single-armed trials, recruiting across all tumor histologies. Such basket-trials are of growing importance in oncology drug development; however, the regulatory framework is built on more traditional clinical trial designs. **The use of early phase, single-arm trials to support a positive benefit-risk profile for the product in limited patient numbers across multiple tumor histologies presented a significant challenge.**

solution

Our involvement began late in the development of the product, but **the Regulatory Sciences team quickly developed and executed a detailed regulatory strategy for the product within the EU.** A timeline for all activities with clear milestones was developed to ensure that a well-defined pathway to filing and approval was identified.

We employed our sound clinical expertise and reviewed all available clinical data at a patient level to build a robust scientific justification for efficacy regardless of tumor type. Detailed patient narratives were prepared to provide further support for the positive benefit-risk profile of this product in a limited patient population.

Full medical writing support was provided to prepare the clinical and nonclinical sections of the submission dossier and accelerated assessment was secured to fast-track the application. Following fi filing, our team project-managed and prepared detailed responses to the clinical review questions raised throughout the MAA procedure. We worked cohesively with the client as a proactive function within their team.

results

ProPharma provided comprehensive and expert support to successfully navigate the regulatory maze and negotiate approval for one of the first histology-independent cancer treatments in the EU using a sound, scientifically-driven, and patient-focused approach.

The services provided by the Regulatory Sciences team were fundamental to the successful registration of the product. Our involvement with the development of this product continues in a post-approval setting. Our client was extremely appreciative of the dedicated support and continues to utilize ProPharma's services for ongoing projects.

Science is developing more quickly than the regulatory framework. Presenting novel science with solid methodology, as well as a clear and balanced benefit-risk discussion will aid the regulatory agencies' assessment and increase likelihood of approval.

ProPharma is able to provide comprehensive support and expertise to develop and execute regulatory strategy, using science as a driver for success.

Contact us to learn how our experienced team can help ensure successful outcomes throughout the product lifecycle.

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