APCER LIFE SCIENCES
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CASE STUDY

Collaborative Expertise Secures Successful FDA Meeting for Phase 2b Oncology Trial

Therapeutic area: Oncology

Product type: Drugs

Geography: Unites States

Product life cycle stage: Clinical Trial Phase 2b

About the Client

This case study focuses on a pioneering United States (US)-based biopharmaceutical company at the forefront of developing novel treatments for a rare and serious blood disorder. The client is currently pursuing an orphan drug designation for a Phase II clinical trial with the US Food and Drug Administration (FDA).

Business Challenge

A US-based biopharmaceutical company (the Sponsor) contacted APCER to prepare a briefing document for a **Type C meeting** with the FDA for their investigational drug. This crucial meeting aimed to obtain the Agency's guidance on the development strategy for an investigational new drug (IND) targeting an oncology indication. The Sponsor faced the following challenges:

- **Uncertainty around Phase 2b trial design:** The Sponsor needed guidance on designing their Phase 2b clinical trial, specifically the endpoints and sample size.
- **Securing accelerated approval:** They aimed at getting an approval for the drug as an orphan treatment for a specific oncology indication. However, they lacked clarity on whether the chosen endpoints and sample size would be sufficient to support this accelerated approval pathway.
- **Navigating IND development challenges:** Facing a complex regulatory landscape for their investigational oncology drug, the Sponsor sought APCER's support.

Overview - Briefing Documents

Briefing documents serve as essential information packages for regulatory agencies. They equip the FDA to prepare effectively for pre-scheduled meetings. These documents typically present the drug candidate, summarize key pre-clinical/clinical data, and outline specific questions for the agency. By providing a clear overview, briefing documents promote efficient communication and successful regulatory interactions.

A **Type C meeting** is any meeting other than a Type A, Type B, Type B (EOP), Type D, or INitial Targeted Engagement for Regulatory Advice on CBER ProducTs (INTERACT) meeting regarding the development and review of a product, including meetings to facilitate early consultations on the use of a biomarker as a new surrogate endpoint that has never been previously used as the primary basis for product approval in the proposed context of use.

A sponsor may outsource briefing documents preparation activities to leverage the expertise of service providers in producing high-quality content with a quick turnaround as a cost-effective solution.

Solution

Working in tandem, our Medical Writing (MW) and Regulatory Affairs (RA) Teams ensured a well-coordinated effort that addressed the Sponsor's goals:

Drafting targeted questions: The MW Team collaborated with the Sponsor to formulate well-defined questions for the FDA, focusing on:

- Adequacy of endpoints and sample size for the Phase 2b study.
- Feasibility of accelerated approval for the orphan indication.

Comprehensive literature review: The MW Team conducted a thorough review of relevant scientific literature to strengthen the background section of the briefing document submitted to the FDA.

Supporting protocol and Investigator's Brochure (IB) development: The MW Team provided valuable assistance in authoring and finalizing the study protocol and IB, ensuring clarity, consistency, and comprehensiveness across these documents.

Communication and document management: The RA Team efficiently managed all communication with the FDA, ensuring timely publishing and submission of the briefing package.

Collaborative meeting participation: Alongside the Sponsor, the MW and RA Teams at APCER actively participated in the PDUFA (Prescription Drug User Fee Act) meeting with the FDA, ensuring all parties were well-represented and prepared.

Outcome

By fostering a collaborative environment where MW and RA Teams work in unison, APCER supports sponsors to navigate through the complexities of IND development with confidence. This integrated approach proved to be instrumental in securing a successful Type C meeting for the Sponsor, paving the way for a more streamlined IND path for their promising oncology treatment.

The Agency provided valuable feedback on the study design, endpoints, and sample size, leading to:

- **Increased potential for success:** The Agency's guidance significantly enhanced the project's overall viability and potential for success.
- **Boost sponsor's confidence:** The positive outcome of the meeting instilled confidence in the Sponsor, providing them a clear direction for their ongoing clinical development program.

APCER's Integrated Regulatory Affairs and Medical Writing Services

APCER comprises a team of highly qualified healthcare professionals having extensive experience in regulatory writing including both **CMC** (chemistry, manufacturing, and controls) **and MW**, providing end-to-end support to clients throughout the product life cycle.

- **High-quality deliverables** consistently exceed client expectations and comply with regulatory authorities' requirements.

- **Publishing and submission** of electronic dossiers in different countries such the US, the European Union, the United Kingdom, and Canada.

- **Customized and fully compliant** solutions to meet the ever-changing regulatory landscape.

From meticulously crafted documents to navigating regulatory hurdles, APCER's interwoven MW and RA teams have become an extension to the client's team. This powerhouse synergy dismantles the complexities of drug development, empowering sponsors to bring life-saving treatments to patients faster.

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APCER Life Sciences is committed to improving health in partnership with its clients. We bring together safety, medical, regulatory and technology resources to ensure that patients receive the safest, most effective therapies possible.

We are an ISO 9001:2015, ISO 27001:2022, and ISO 27701:2019 certified company.

Learn more at www.apcerls.com or contact us at one of our global offices: Americas: (+1) 609 455 1600 • Europe: (+49) 2409 725 6120 • UK: (+44) 208 326 3220 • Asia: (+91) 79 6677 8600

For Business enquiries, please email at: marketing@apcerls.com
For General enquiries, please email at: info@apcerls.com