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LIFE SCIENCES

# FACET PROVIDES LASER FOCUS ON CLINICAL DEVELOPMENT

## ONE SCIENTIST AND A NOVEL COMBINATION DRUG IN SEARCH OF AN INDICATION

## BACKGROUND

A pharmaceutical company with one employee based in the United States was developing a novel drug combination product. Since 1995, a considerable amount of theoretical research on the product had been completed and dozens of publications were generated. The combination product showed promise in several animal models for serious diseases, some with unmet medical need. These results triggered several investigator-initiated clinical trials (using compounded drug product) with the goal of demonstrating proof-of-concept in several different disease states.

## BUSINESS CHALLENGE

Though the research was promising, the company was struggling to make progress in moving the product toward market authorization in the United States and Europe.

## SOLUTION

The company reached out to Facet Life Sciences for assistance in focusing their development approach. The Facet Expert Development Services Team reviewed the available nonclinical and clinical data as well as the existing published literature on the individual components and the combination product. Using this information, Facet prepared a regulatory strategy that:

- Identified where the team was in the regulatory drug development process ("You are Here" report)
- Evaluated the regulatory and medical landscape, clinical development requirements, and competitive market for 5 potential indications
- Provided a clear, comprehensive project plan to move several indications forward in the United States and Europe

## RESULTS

Facet's Development and Regulatory experts helped the Sponsor secure a Type A meeting with FDA and two subsequent teleconferences to establish a path forward in the company's stated lead indication. In these meetings, the Facet Life Sciences team was able to reach agreement with FDA on an acceptable clinical trial design and clinical endpoints for a proof-of-concept study in a novel orphan indication.

Within the span of two years, Facet's team of experts:

- Helped the Sponsor secure orphan drug status in 4 indications in the U and 1 indication in Europe
- Championed the move of this company's chemistry, manufacturing, and controls (CMC) activities from compounding pharmacies into a GMP-certified commercial manufacturer. This move revealed a critical complexity in the drug product manufacturing requiring a specialized manufacturing method for the combination product to maintain product integrity and stability.
- Designed several new proof-of-concept clinical studies using the GMP product, provided estimates of timelines and cost for execution, and provided priority recommendations for development to the Sponsor that aligned with their corporate objectives for the product.

The Facet team provided expert guidance in developing a strategy and partnered with the client to ensure successful execution of a plan that simultaneously supported their corporate and regulatory goals.

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