



Rare and Orphan Diseases

Synteract Exceeds Mpex Pharmaceuticals Expectations in Rare Disease Trial

Highlights

- Synteract helped to assess site and country viability, meet enrollment goals, ensure quality data, for international study.
- It built close relationships with investigators and sites.
- The study exceeded enrollment goals, was completed earlier than anticipated.

Introduction

Mpex Pharmaceuticals, Inc., a subsidiary of Aptalis, focuses on antibacterial drug development to treat Cystic Fibrosis (CF). Prior to working with Synteract, the Sponsor had worked with another CRO but elected not to continue the collaboration. In 2008 Synteract began working with the sponsor on

Study-204, a blinded, placebo-controlled Phase II study involving over 150 patients at 51 clinical sites internationally. The study was designed to evaluate the efficacy, safety, and tolerability of Aeroquin®, a new aerosolized form of Levofloxacin, to treat CF. Aeroquin was administered in three dosage regimens over 28 days compared to a placebo drug. Trials were conducted in the U.S., Germany, and the Netherlands to assess Aeroquin's effectiveness against *P. aeruginosa* (bacteria in the lungs causing infection), as well as against other organisms that colonize lungs of CF patients.

Challenges

- Recruiting patients: Due to the relatively low number of CF patients who participate in clinical trials and the rigor of enrollment criteria, only about 12% of CF patients in the world were willing and eligible to participate in this clinical trial.
- In addition, there was a significant increase in competition for patients at the time the study was initiated, with multiple pharmaceutical companies also pursuing drug development for CF.

Our Solutions

- **Synteract and the sponsor worked together collaboratively to assess site and country viability, selecting the most appropriate sites to participate in the study, provide quality data, and meet enrollment goals.**

- To differentiate from competing studies, the sponsor and Synteract focused on building close relationships with sites and investigators. With longstanding experience managing clinical trials for small to mid-size biopharmaceutical companies, Synteract provided critical insight on working with research sites to meet study goals and timelines.

Program Success

- **Study-204 was a success. The reduction of P. aeruginosa was statistically significant.** Aeroquin® was well tolerated at all doses and there were strong efficacy results in the treated population.
- The study was completed earlier than anticipated, with the number of patients slightly exceeding enrollment goals.
- Mpex was pleased with the conduct and quality of the study, noting it was “a robust trial with very good study conduct.”
- **Following the completion of Phase I and Phase II (Study-204) trials, Synteract and the sponsor have continued working together on additional non CF trials.** All completed studies were finished on time and within budget, with high-quality data integrity and study conduct.

About Synteract

With employees across 21 countries, Synteract is an innovative, full-service CRO supporting biopharma companies across all phases of drug development to help bring new medicines to market. Synteract has conducted 4,000 studies on six continents and in more than 60 countries, working with more than 26,000 investigative sites and 750,000 patients. It has contributed to more than 240 product approvals. Synteract offers a notable depth of therapeutic expertise in oncology, dermatology, and neuro degenerative indications, as well as rare and orphan, pediatric, and immunotherapy studies.

For more information on how we can support your clinical trial, please visit www.synteract.com/Therapeutic-Expertise/Rare-Orphan-Diseases or ContactUs@synteract.com.

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