



# Notable Experience in Rare and Orphan Diseases

Highlights in the past 5 years:

136+ RARE & ORPHAN DISEASES CLINICAL TRIALS

50+ INDICATIONS

3,000 SITES

15,000 SUBJECTS

## Advancing Complex Rare and Orphan Clinical Trials

Demand for trials in rare and orphan indications has been growing as scientific advances are made, legislation incentivizes rare and orphan research and most importantly because patients are waiting for a treatment.

## Addressing Unique Challenges Rare and Orphan Trials Pose

We understand that both rare and orphan indications most often come with unique characteristics. Recruitment can be challenging, because a high number of study sites are required to enroll a very small number of patients. Outcome measures are more complex, and there may be scientific limitations. Statistical significance may be limited as well, so analysis may hinge on individual patients and historical comparison. Working with regulatory authorities throughout the process is critical.

Synteract draws on a strong history of working with and guiding investigative sites, sponsors, and patients through unique scenarios. We frequently work on studies that have never been done before.

At Synteract, we leverage our operational expertise and regulatory acumen in building a study program that integrates patients, sites, and advocacy groups to bring your clinical trials to life and new medicines to market! Our proactive recruitment, mental agility, strong relationships with patient advocacy groups, and international reach are critical to identifying hard-to-find patient populations.

## Rare/Orphan Indications Experience

- A - Oncology: 41%
- B - Respiratory: 15%
- C - Hematology: 13%
- D - Metabolic: 11%
- E - Neurology: 9%
- F - Gastroenterology: 5%
- G - Endocrinology: 2%
- H - Ophthalmology: 2%
- I - Rheumatology: 1%
- J - Transplantation: 1%



## Syneract Exceeds Mpex Pharmaceuticals Expectations in Rare Disease Trial for Cystic Fibrosis

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

- Syneract 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 Syneract focused on building close relationships with sites and investigators. With longstanding experience managing clinical trials for small to mid-size biopharmaceutical companies, Syneract 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, Syneract 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 Syneract:

With employees across 21 countries, Syneract is an innovative, full-service CRO supporting biopharma companies across all phases of drug development to help bring new medicines to market. Syneract 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. Syneract 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.syneract.com/Therapeutic-Expertise/Rare-Orphan-Diseases](http://www.syneract.com/Therapeutic-Expertise/Rare-Orphan-Diseases) or [ContactUs@syneract.com](mailto:ContactUs@syneract.com).

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