Where every patient counts

www.orphan-reach.com
Introducing Orphan Reach
a rare CRO

From blockbuster drugs to personalised medicine

WHO WE ARE
Orphan Reach was established in 2002, initially starting out as a functional service provider for the pharmaceutical industry, then quickly becoming known for rescuing clinical studies from other CROs, where patient recruitment and retention were the main challenges.

Today we are operating in more than 60 countries and tailor our services to support smaller patient populations with rare diseases where often, larger CROs struggle to find the right balance between efficient study execution and patient centricity.

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RARE DISEASE FOCUS
At Orphan Reach we offer global, dedicated and patient centric orphan drug development solutions in support of accelerated access to orphan drugs. We are a boutique service provider solely focused on orphan product candidates, surrounded by a competent team of experts in the field of rare diseases. Our name is our mission.

THE RIGHT SUPPORT AND RESOURCES YOU NEED
Rare diseases are not common business: with a paradigm shift from blockbuster drugs to personalized medicine, many new challenges have arisen, and the biopharmaceutical industry has learnt that patient focus is critical when working in the orphan drug domain. The integration of the broader contribution of patients and patient advocacy groups into developing orphan therapies and making them accessible has become a key success factor. As the industry heavily relies on the collaboration of many parties, selecting the right service providers who recognise the importance of a patient focused approach is crucial.

WHY WE’RE HERE
Our purpose is to expedite the development of orphan drugs and to facilitate patient access to treatments which can improve the quality of life for patients and their families.

By driving best practice, we can provide continued, seamless and high-quality support to biopharmaceutical companies throughout many stages of the orphan product development cycle.

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CLINICAL DEVELOPMENT

A wealth of experience in rare indications combined with an innovative operational model, enables us to provide both global coverage and local expertise across all territories supported by our dedicated and passionate teams.

We locate and include patients into trials from wherever they are needed in the world, and thanks to our bespoke trial execution model we can ensure that all clinical trials are conducted to the highest standards with each individual patient in mind.

Our clinical operations are fully scalable and have proven to work excellently, particularly in trials involving lower patients’ numbers distributed over many countries. Additionally, we have demonstrated and achieved major cost efficiencies for our clients, thus avoiding expensive and unnecessary overheads commonly found in global CROs targeting high volume studies.

PATIENT TRAVEL

Travel to and from the investigational site during a clinical trial can become a major barrier for patients and their supporting families. With many children affected, it becomes crucial to plan and organise properly based on their special needs and requirements.

We are familiar with the confidential co-ordination of patient travel and expense management for clinical studies helping to make the drug approval process seamless. Whether travel, accommodation, ambulance charter or special care and assistance are required, patient needs can be met based on a proven history of providing efficient and effective solutions to reduce sponsor and Investigator administration.

HOME VISITS

Making trials more acceptable for patients will have a positive impact on patient retention especially in a patient population affected by a rare and often debilitating disease. Through our selected vendors Orphan Reach can offer qualified and trained nurses to fulfil this important role. The nurses have the qualification to provide the following services at the patient’s home:

- Blood draws
- PK sampling
- QOL questionnaires
- Physical assessments
- Delivery of medication
- Patient training
- Infusions

ORPHAN STRATEGY

We work with a world-renowned team of independent consultants in the US and Europe to assist our Sponsors in finding the right orphan product strategy.

Navigating in a regulatory and commercial environment that is constantly changing requires a tailored approach that is in tune with our clients’ goals.
Providing Clinical Development Services Across the Globe

Orphan Reach have an established presence in more than 60 countries and provide integrated drug development solutions, from obtaining orphan drug designation through to conducting post marketing surveillance studies. We are familiar with local regulations in all countries across the globe and maintain excellent relationships with local authorities.

- Disease awareness and diagnosis programs
- Prevalence data collection
- Patient organisations, medical networks and KOL identification
- Feasibility studies
- Natural history and observational studies
- Patient registries
- Interventional clinical trials (Phase I-III)
- Outcome surveys and real-world data
- Post marketing surveillance studies
- Patient travel support
- Patient home support
- Pre-EMA/FDA scientific advice meetings
- Orphan drug designations
- PIP development
- DSMB
- Data Management
Our global, patient focused approach differentiates us from other CROs. It allows Sponsors to choose a nimble CRO with attention dedicated to small patient populations without the need to rely on CROs mainly operating in the field of common diseases involving larger patient pools.

We evaluate the current literature and available epidemiological data. We also identify appropriate thought leaders and interact with patient groups for the purpose of natural history studies, which are key initial steps to inform the later clinical development. We design and execute NH studies and at the end of the process we publish the findings from natural history and epidemiological studies.

We nurture excellent global contacts with Clinical Research Networks, indication specific Investigator networks and Patient Advocacy Groups. We also engage referral sites, pre-identify patients at site and use relationship marketing experts. Combining the various measures in an effective way helps us to meet or exceed patient inclusion timelines.

We focus on the specific requirements of each study and the relevant patient groups. If children are involved in clinical trials, our team of pediatric specialists is very familiar with the strict regulatory requirements and ensures that these requirements are met, to ensure that the interests of this vulnerable patient group are kept at the forefront of all trial considerations. Our paediatrics rare disease experience includes:

- A proven track record of 42 studies
- 13 pediatric rare disease indications
- Sites in Europe, North America, Middle East, Australia, Latin America and Africa.

With very few patients, the integrity and completeness of data from each patient assumes even greater importance. We employ many methods to retain patients and maintain compliance. Focussed training of sites and patients is provided to increase retention and compliance supported by experienced Senior CRAs at a site level. Engaging patient advocacy groups, providing home healthcare by trained nurses and patient & family travel support are all additional efforts to ensure a smooth data collection according to the planned study timelines.

We are engaged and communicate at a local and national level to many of the patient advocacy groups in multitude of rare disease areas. This way we obtain valuable advice on practical considerations from a patient’s perspective (e.g. protocol feasibility, drug application etc.).
OPRHAN INDICATION EXPERTISE

- AA Amyloidosis
- Acromegaly
- Adrenoleukodystrophy
- ATTRV30M Amyloidosis
- Atypical Hemolytic-Uremic Syndrome
- Calciphylaxis
- Central Precocious Puberty
- Ch. Thromboembolic Pulm. Hypertension
- Congenital Fibrinogen Deficiency
- Cushing’s Syndrome
- Cystic Fibrosis
- Duchenne Muscular Dystrophy
- Dysfibrinogenemia
- Erythropoietic Protoporphyria
- Fabry’s Disease
- Gaucher’s Disease
- Glanzmann Thrombasthenia
- Haemophilia
- Hairy Cell Leukaemia
- Hereditary Angioedema
- Hereditary ATTR amyloidosis
- Homozygous Familial Hypercholesterolemia
- Huntington’s Disease
- Hypofibrinogenemia
- Idiopathic Thrombocytopenic Purpura
- Lamellar ichthyosis
- McArdle’s Disease
- Mastocytosis
- Mucocutaneous candidiasis
- Mucopolysaccharidosis Type I
- Paroxysmal Nocturnal Hemoglobinuria
- POMC, PCSK1 or LEPR deficiency obesity
- Prader-Willi Syndrome
- Primary Biliary Cirrhosis
- Primary Mitochondrial Myopathy
- Rett Syndrome
- Small Bowel Syndrome
- Von Willebrand Disease

WHY ORPHAN REACH

Making a difference is important to us. In a world of more than 7,000 rare diseases for which only 400 treatments are available there is still a lot to do.

Extraordinary concerted efforts from many different stakeholders are required to accelerate the process of bringing urgently needed new medicines to the market.

Healthcare companies with rare disease focus are relying on outsourcing like any other company involved in the product development and commercialization of new medicines. Whereas today biopharmaceutical companies operating in the common disease field can refer to a broad base of full-service providers, it becomes difficult to select appropriate providers that can address the particular challenges that rare diseases entail.

With Orphan Reach our clients obtain a comprehensive orphan product development solution including clinical support and integrated patient management.

We invite you to collaborate with us, the first of its kind CRO, focused exclusively on orphan drugs and rare diseases.

Please contact our Client Relationship team to discuss your company’s needs:
info@orphan-reach.com