Rare disease & orphan designation studies

FACT SHEET

The last two years, 2017 and 2018, have brought the greatest number of orphan drug approvals and orphan drug designations. In 2017, the FDA granted orphan drug designation to over 429 unique drugs under development. In 2017 the FDA approved 46 new orphan drugs and in 2018 approved 59, the most on record.[1]

Over the past five years alone, Parexel has managed more than 1,000 rare disease projects and helped clients successfully obtain Orphan Designation status. We leverage best practices, lessons learned, and our extensive knowledge and provide clients with a holistic team that understands the nature of these debilitating diseases and the emotional component of these studies so that they can focus on the patient and family unit.

A lean and dedicated team

At Parexel, we bring together our medical, scientific and regulatory therapeutic area teams to support your rare and orphan drug development efforts. We provide you with a project leader who acts as a single point of contact, liasing with the other teams as necessary. Having this single point of contact streamlines the process and enables you to more quickly access our comprehensive, worldwide expertise.

Project teams are assembled on the basis of recent and relevant experience with specific indications, familiarity with the class of investigational agents and their mechanisms of action, the phase of study, the type of investigative sites, and knowledge of the key opinion leaders in the field. Our teams also have an in-depth understanding of the competitive environment and global and regional standards of care for specific indications and approaches to therapy.

1. Source: https://www.fda.gov

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Robust and detailed strategy for patient recruitment and retention

Parexel's Patient Innovation Center includes a full-service group with extensive experience in rare disease studies and strategies to aid study design, enrollment and patient awareness. They understand the importance of working closely with patients, caregivers and patient advocacy groups to learn everything they can about the disease and its effect on daily lives.

They work tirelessly to decrease the burden of participation placed on participants, resulting in stronger recruitment and retention rates.

Additionally, our operational plan takes into account leveraging travel, concierge, and home health services to assist patients who may not be located close to the sites where the study will be performed.

Regulatory advice at every step

We have 1,000+ regulatory consultants on staff, of which more than 50 have held positions at regulatory authorities across the world. Our regulatory experts work closely with our clinical and operational teams for an integrated approach, informing your studies every step of the way.

Examples of the rare diseases where Parexel has expertise:

CNS	› Amyotrophic Lateral Sclerosis › Duchenne Muscular Dystrophy › Huntington's disease › Spinal cord injury › Tourette Syndrome › Cervical dystonia › Narcolepsy › Ataxia › Myasthenia gravis › Subtypes of epilepsy
Endocrine Metabolism	› Acromegaly › Fabry Disease › Gaucher's Disease/ Lysosomal storage disorders › Growth Hormone Deficiency › Inborn metabolic diseases: Phenylketonuria (PKU) in children and adults, Morbus Pompe Disease (glycogen storage disorder) and Hunter Syndrome (mucopolysaccharidosis II)
Hematology and Oncology	› Von Willebrand disease › Hemophilia › Sickle Cell Disease › Rare Tumors
Pulmonology	› Cystic Fibrosis
Rheumatology	› Pediatric Systemic Lupus Erythematosus › Juvenile Idiopathic Arthritis

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