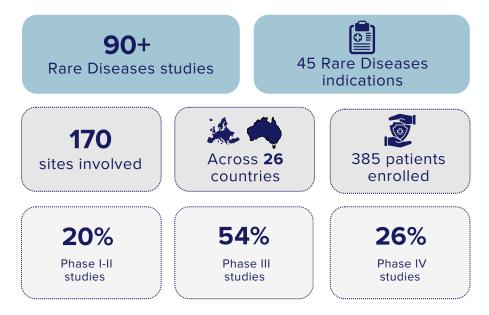
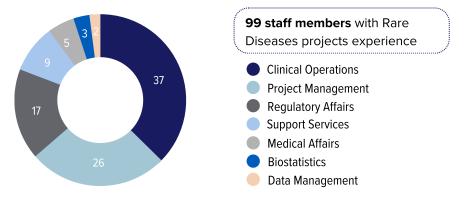


RARE DISEASES EXPERIENCE AND EXPERTISE

Optimapharm Rare Diseases Clinical Studies Overview



Optimapharm Staff Experience in Rare Diseases Clinical Studies





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RARE DISEASE FACTSHEET



Distribution of Rare Diseases Studies

Optimapharm has extensive experience in Rare Disease studies, with 90+ studies. The chart below shows the distribution of patient population in Rare Disease studies.

38% Paediatric

62% Adult population

Rare Diseases Experience by Indications

Optimapharm has conducted clinical studies in over 45 Rare Diseases indications, including those featured in the table below:

Cystic fibrosis	Niemann-Pick type C
Hemophilia A	Crohn's disease
Idiopatic Pulmonary Fibrosis	AA Amyloidosis
Cushing's syndrome	Epidermolysis Bullosa
Fragile X Syndrome	Mitochondrial diseases
Muckle-Wells syndrome	Eisenmenger Syndrome
Fabry Disease	Gaucher Disease
Immune Thrombocytopenic Purpura	Large-scale mtDNA Deletion Syndromes
Hypercholesterolemia	Huntington's Disease
Myelofibrosis	Von Willebrand Disease

How Optimapharm can support your next Rare Diseases study?

Clinical Operations

Project Management
Medical Affairs

Regulatory Affairs Safety & Pharmacovigilance Data Management & Biostatistics



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