CERTARA

The Tools You Need to
Accelerate Rare Disease Drug Development

Patients with rare diseases have high unmet medical needs.

Over **7,000**
Rare Diseases Identified

Only **5%**
Of Rare Diseases Have an FDA Approved Drug

What makes something a "rare or a neglected disease"? There is no universally accepted definition as the prevalence criteria varies by region/country.

Regulatory Agency	Prevalence Criteria	Reference
U.S. FDA	200,000	Orphan Drug Act, 1983
EMA	5/10,000	EC 141/2000
Japan PMDA	50,000	JPMA, 2008
Australia	2000	Therapeutic Goods Act, 1989

Rare Disease Affects More Than
350
Million People Worldwide

The U.S. has several mechanisms that encourage the development of treatments for rare diseases.

Orphan Drug Act
7 years of market exclusivity
Tax incentives
Fee exemptions
Priority review vouchers

FDA
Fast-track status
Accelerated approval
Priority review

50%
of People Affected By Rare Diseases Are Children

Approximately
30%
of Children With These Debilitating Diseases Will Not Live To See Their 5th Birthday

Approximately
80%
Of Rare Diseases Are Genetic

While rare disease drug development is challenging, the experts at Certara can help!

Small patient pools
We use clinical pharmacology expertise in combination with MIDD tools to discern effects in small patient populations.

Sensitive patient populations
We model the impact of a new drug on other disease states or untested populations.

Unique regulatory landscape
We provide end-to-end regulatory support and medical writing services: from meetings with health authorities to preparation and submission of marketing applications.

Reimbursement challenges
We develop a quantitative multi-criteria decision analysis-based framework adapted to the very specific issues in rare disease development and access including quality of evidence, disease severity, ethical considerations, population-level factors, economic impact of the disease, and specific budgetary impact.

We have broad therapeutic expertise in rare disease drug development

Genetic & Hereditary
- Fabry Disease
- Pompe Disease
- Hunter's Disease
- Metachromatic Leukodystrophy Disease
- Transthyretin-mediated Amyloidosis
- Urea Cycle Disorder
- Hereditary Angiodema
- ACE-induced HAE
- Lennox-Gastaut Syndrome
- Familiar Mediterranean Fever
- Gaucher Disease
- Cystic Fibrosis
- Hypophosphatasia
- Chronic Liver Disease
- Huntington's Disease
- Dravet Syndrome
- Thrombocytopenia
- Hereditary Transthyretin-mediated Amyloidosis

Antinfectives/Antivirals
- Anthrax
- Orthopoxvirus/Smallpox
- Symptomatic Botulism
- Respiratory Syncytial Virus

Gastrointestinal
- Short Bowel Syndrome
- Pediatric Crohn's Disease
- Carcinoid Syndrome

Oncology
- Acute Lymphoblastic Leukemia
- Metastatic Myeloma
- Multiple Myeloma
- Acute Myeloid Leukemia
- Urinary Bladder Cancer (Photodynamic Therapy)
- BRAF Mutation
- Blood Cancer
- Ovarian Cancer

CNS
- Myasthenia Gravis
- Restless Leg Syndrome
- Spasticity due to Multiple Sclerosis
- PKAN (Pantothenate Kinase-associated Neurodegeneration)
- Friedreich's Ataxia

Blood Disorders
- Paroxysmal Nocturnal Hemoglobinuria
- Atypical Haemolytic Uremic Syndrome (aHUS)
- Beta-thalassemia (Sickle Cell Beta-thalassemia)

Bone & Muscle Disease
- X-linked Hypophosphatemia
- Duchenne Muscular Dystrophy
- Hypophosphatasia

Other
- Radiation Poisoning
- Hypoparathyroidism
- Molybdenum Deficiency
- Peyronie's Disease
- Hyperuricemia in Patients with Gout Refractory
- Hepatic Veno-occlusive Disease
- Onchocerciasis
- Neglected Tropical Diseases

Ready to learn more about how our software and services supported the approval of over 100 orphan drugs, including over 90% of new novel drug approvals with orphan designation by the U.S. FDA since 2014? Read our white paper:

SCAN ME

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