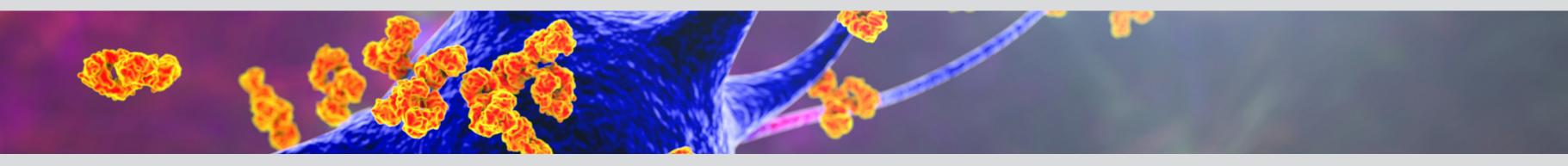
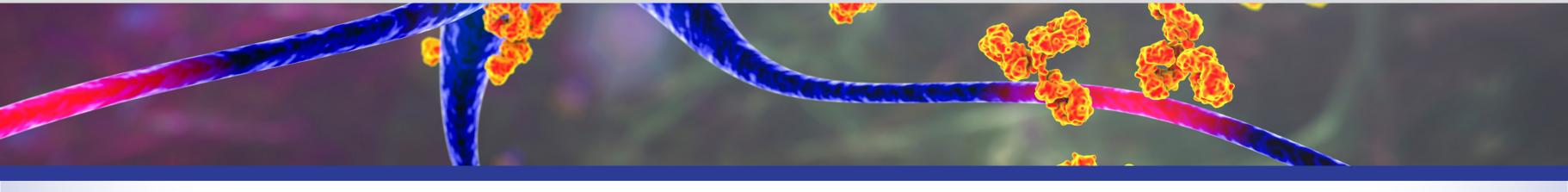
CERTARA



The Tools You Need to **Accelerate Rare Disease Drug Development**



Patients with rare diseases have high unmet medical needs.

Over 7,0000 🖗 **Rare Diseases Identified** 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 |
| | | |

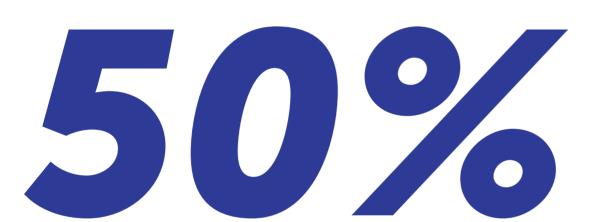


The U.S. has several mechanisms that encourage the development of treatments for rare diseases.

Orphan Drug Act

7 years of market exclusivity Tax incentives Fee exemptions **Priority review vouchers**





Approximately

30%

of People Affected By **Rare Diseases Are Children**

of Children With These **Debilitating Diseases Will Not Live To See Their 5th Birthday**

Approximately

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,

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 Anglodema
- ACE-induced HAE
- Lennox-Gastaut Syndrome
- Familiar Mediterranean Fever
- Gaucher Disease
- Cystic Fibrosis
- Hypophosphatasia
- Chronic Liver Disease
- Huntington's Disease
- Dravet Syndrome
- Thrombocytopenia
- Hereditary Transthyretinmediated Amyloidosis

Antinfectives/Antivirals

- Anthrax
- Orthopoxvirus/Smallpox
- Symptomatic Botulism
- 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:



- Respiratory Syncytial Virus