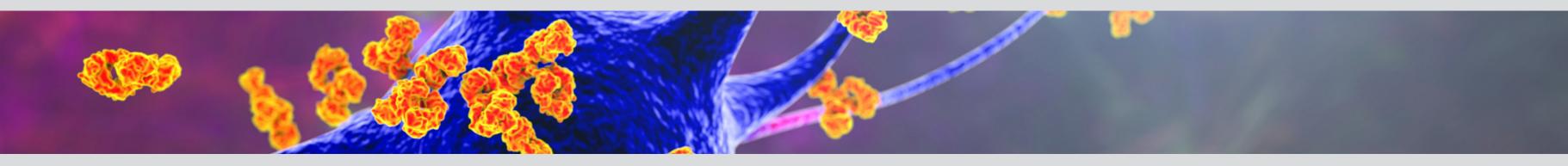
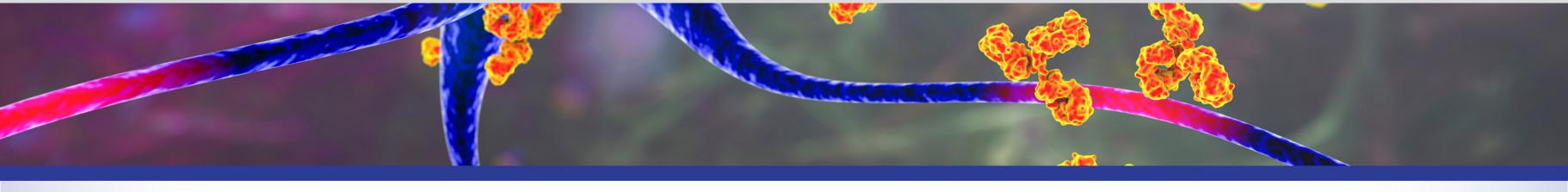
CERTARA



# 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

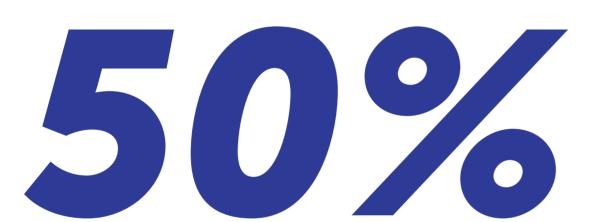


The U.S. has several mechanisms that encourage the development of treatments for rare diseases.

# **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