



# **Our Rare Disease Experience**

- Operational strategies to for long-term studies with small numbers
- Relationships with patient advocacy groups

**Rare diseases by Indication Count** 

Dematology

Cardiology

Hephology ophthalmology

• Global reach to access patients

Inflammation®

Respiratory

60

50 40

30

20

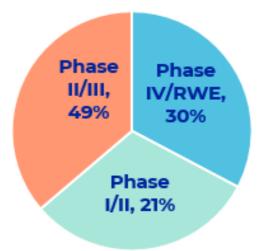
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Oncology

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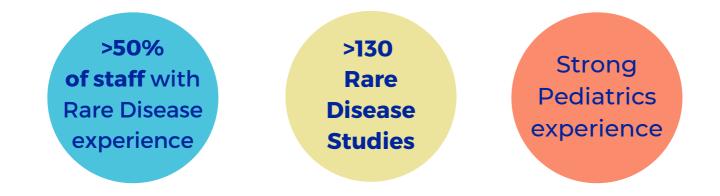
Neurology

Hematology



#### **Specialist Experience**

- 50% of project teams, PMS and LCRAs have Rare and Pediatric experience
- Rare Disease and Pediatrics MD as **Therapeutic Areas Advisor**
- Board certified Pediatricians on Medical Staff



Intections Diseases.

Castloenterobogi

Musculoskeletal

For further information, please contact our sales team: contact@excelya.com



STUDIES UNDERTAKEN :				
•	<b>Cardiovascular</b> (Homozygous familial hypercholesteremia, Transthyretin amyloidosis, Pulmonary Arterial Hypertension)	•	Infectious Diseases Invasive Candidiasis, Acute Bacterial Skin and Skin Structure Infections	
•	<b>Dermatology</b> (Decubitus Ulcer, Hydradenitis Suppurativa, Pemphigus Vulgaris)	•	<b>Neurology</b> Transthyretin (TTR)-Mediated Polyneuropathy, Dystrophinopathy, Multiple sclerosis, Familial Amyloidotic Polyneuropathy, Duchenne Muscular Dystrophy, Supranuclear Palsy, Hereditary Tranthyretin Amyloidosis (hATTR), Spinal Muscular Atrophy, Fabry disease, Tinnitus	
•	<b>Endocrinology</b> (Hyperparathyroidism, Growth hormone deficiency, Cushing Syndrome, Acromegaly)			
•	Hematology (Multiple Myeloma, Hemophilia A, Hemophillia B, Hemophagocytic Lymphohistiocytosis, Thalassemia Major, Waldenström's Macroglobulinemia, T-cell Lymphoma, Follicular lymphoma, β-thalassemia, Myelodysplastic Syndrome, Sickle cell disease, Immune Thrombocytopenic Purpura)	•	<b>Oncology</b> Hepatocellular carcinoma, Renal cell carcinoma, Myelofibrosis, Ovarian cancer, multiple myeloma, Ewing Sarcoma / Osteosarcoma, Glioblastoma , Malignant melanoma, Acute myeloid leukemia, Pancreatic neuroendocrine tumours	
•	<b>Hepatology</b> Intrahepatic cholestasis, NASH, Progressive Familial Intrahepatic Cholestasis, Primary sclerosing cholangitis	•	<b>Ophthalmology</b> Age related macula degeneration, Dry-eye syndrome, von Recklinghausen's disease (NFI) / Neurofibromatosis type 2 (NF2)	
•	<b>Immunology</b> Hereditary angioedema, Pulmonary Alveolar Proteinosis	•	<b>Pneumology/Respiratory</b> Autoimmune Pulmonary Alveolar Proteinosis , Idiopathic pulmonary fibrosis, COPD	

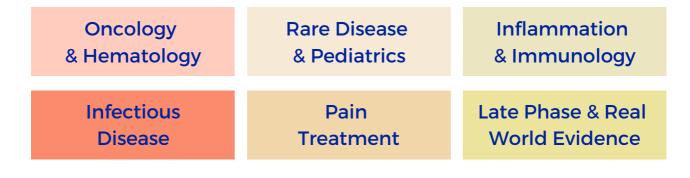
#### Worldwide Coverage

Excelya operates across 28 countries worldwide 25 countries in Europe Established Partner Network for global delivery

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## **6 Therapeutic & Speciality Areas**



### **One-stop Provider: 3 Operating Modes:**



<b>Clinical Operations</b>	Data Management	Statistics & Programming
Pharmacovigilance	Medical Affairs & Writing	eTMF

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