Rare disease

Our expertise to manage challenging trials

Over the last years, we have been working on several projects related to Rare diseases and Orphan Drugs and have created a dedicated team made of clinical and regulatory experts to support Pharma and Biotech during the Orphan Drug Development process.

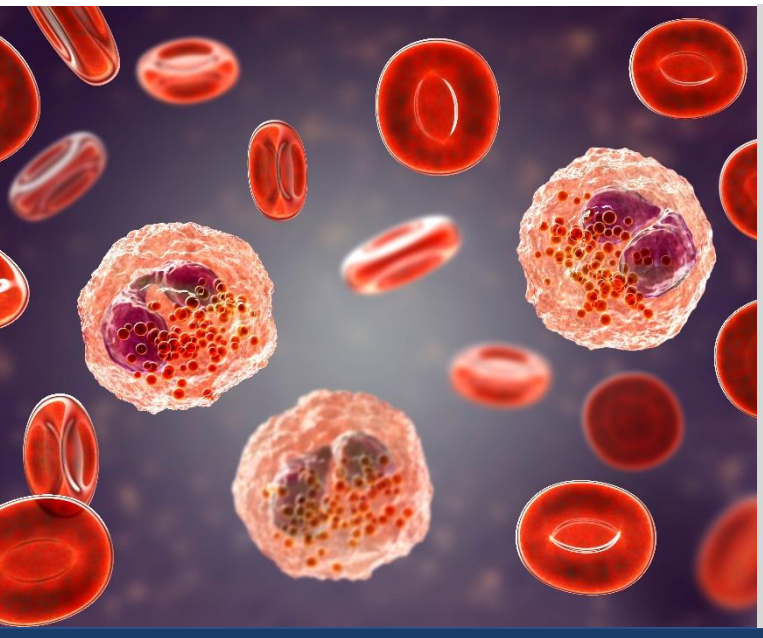
Challenges

- **Small number of patients available**: a rare disease trial usually Involve a small, heterogeneous, and widely dispersed patient population
- **Clinical trial design**: to generate high-quality data the study design for a rare disease should fit small study population. Measuring clinical trial outcomes may be challenging as rare disease patients exhibit huge diversity in their clinical presentation and histories
- **Site and staff suitability:** a larger number of trained and qualified sites is required to conduct rare disease trials.

Our experiencee

Our recent experience on Rare diseases include 20 clinical projects from Phase I to Phase IV and Non-interventional studies. Below is a list of Pathologies we have been working on:

- **Acromegaly –** Phase III
- **Cystic Fibrosis** (x2) – Phase II
- **Fibromyalgia** – Phase II
- **Hemophilia A** - NIS
- **Hemophilia B** - NIS
- **Hereditary angioedema** – Phase II (X2)
- **Idiopathic myelofibrosis** - Phase II
- **Idiopathic Pulmonary Fibrosis** – Phase II
- **Multiple Myeloma** – Phase II, Phase III
- **Myelopfibrosis** – Phase II
- **Ovarian Cancer** – Phase II
- **Pheochromocytomas** – NIS
- **POEMS syndrome** – Phase II
- **Rheumatoid Arthritis** – NIS
- **Sanfilippo B phase syndrome** – Phase I



OUR PLUS

- **Proven clinical and regulatory expertise**
- **20 year experience**
- **Flexible**
- **Global coverage**