VERISTAT

Rare & Orphan Disease Clinical Development Expertise

Veristat is passionate about helping clients accelerate their rare disease therapies for unmet medical needs through the clinical development process. With more than 350 million rare disease sufferers worldwide today[1], the urgency for more treatments is paramount.

30% of all clinical trials run by Veristat are for rare diseases

Our teams are passionate and work tirelessly to learn and become experts in every unique and rare disease indication we work in. To date, we've worked on more than 300 clinical projects and have prepared more than 35 regulatory submissions for rare disease therapies.

>300
Rare Disease Trials

Including:

>75
Genetic Disease Studies

>135
Rare Cancer Studies

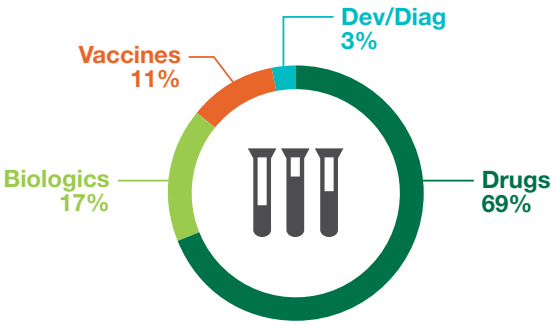
Overall Rare Disease Experience				
✓ Allergies	✓ Blood disorders	✓ Endocrine disorders	✓ Genetic disorders	✓ Infectious diseases
✓ Auto-immune diseases	✓ Cardiovascular diseases	✓ Gastrointestinal disorders	✓ Lymphomas/ Leukemias	✓ Solid tumors

Unparalleled Rare Disease Expertise From Consultation to Submission

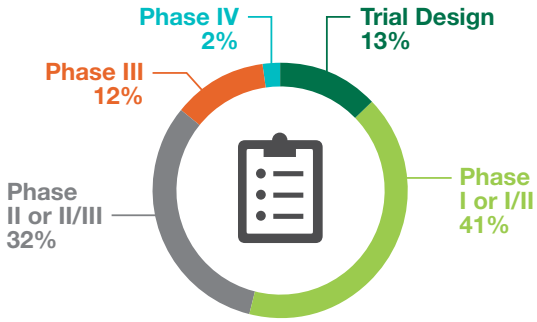
Rare Disease trials present additional unique challenges and opportunities. We help determine if your study qualifies for an accelerated regulatory approval pathway – including Orphan Drug Designation, Fast Track Status or Priority Review – and we'll represent you at US Food & Drug Administration (FDA) and European Medicines Agency (EMA) meetings.

Veristat ensures that your clinical trial or program design supports your regulatory strategy, particularly if you will only run a single pivotal trial or will use supplemental data from earlier run trials, disease progression analytical models or previous adult trials. Our experts can help you explore and simulate adaptive trial designs. Then, we find, recruit and engage the patients and sites through to study completion. Lastly, we analyze the data and prepare your submission for regulatory agency review.

Rare Disease Experience by Product Type



Rare Disease Experience by Study Phase



Critical Expertise We Devote to Rare Disease Trials

1. REGULATORY STRATEGY
2. CLINICAL TRIAL/ PROGRAM PLANNING
3. FEASIBILITY & PATIENT RECRUITMENT
4. SITE ENGAGEMENT THROUGHOUT STUDY CONDUCT
5. DATA ANALYSIS & REGULATORY SUBMISSION

Accelerated Pathways for Rare Disease Submissions

Of the total rare disease submission projects that Veristat has supported, the following have an accelerated pathway:

77%	>30%	>30%	11%	11%
Orphan Disease Designation	Fast Track Designation	Priority Review	First-In-Class	Breakthrough Designation

Making informed decisions for selection of the right regulatory pathway at the start of the orphan drug development process helps mitigate risk and ensure the fastest path to approval. We specialize in rare disease submissions – more than **55% of the submission projects** we've worked on were for rare disease indications.

Rare Disease Indications

Rare Cancers	Vaccines
Advanced NRAS-mutant Melanoma	Pancreatic Cancer
Gastro-Entero-Hepatic Neuro-Endocrine Tumors (NET)	Smallpox
Renal Cell Cancer	Thyroid Cancer

Blood Cancers	Others
Cutaneous T-cell Lymphoma	Blastic Plasmacytoid Dendritic Cell Neoplasm (BPDCN)
Mantle Cell Lymphoma	Endocarditis
Peripheral T-cell Lymphoma	Familial Chylomicronemia Syndrome (FCS)
Relapsed / Refractory Acute Myelogenous Leukemia	Homozygous Familial Hypercholesterolemia (HoFH)
Relapsed / Refractory Chronic Lymphocytic Leukemia	LAL Deficiency
Relapsed / Refractory Multiple Myeloma	Tardive Dyskinesia

"I am grateful for the dedication, excitement, and clinical trial expertise that the Veristat team has given Alnylam since the start of this program. Veristat accepted our challenge and their collaboration has been critical to the successful clinical trials that led to the U.S. FDA approval of ONPATTRO."

Akshay Vaishnaw, MD, PhD, President of R&D at Alnylam

[1] https://rareundiagnosed.org/rare-disease-facts/

CASE STUDY

Regulatory Submission Support for First-in-Class Approval

Long-term collaboration leads to a drug approval for hATTR amyloidosis, a rare disorder.

Situation: Alnylam, a leading innovator of RNA interference (RNAi) therapies, hired Veristat nearly 11 years ago to help design the studies and prepare the IND submission for patisiran.

Solution: Beyond study design and IND preparation, our collaboration continued to include a phase II study, a phase III pivotal study, and a phase III open-label extension study. Veristat teams provided biostatistics and programming, data analysis and conversion, DSMB coordination, medical writing and project management support for these trials.

Impact: ONPATTRO™ (patisiran), a lipid complex injection for treatment of the polyneuropathy of hATTR amyloidosis in adults, was approved as a first-in-class treatment by the FDA in late 2018. Veristat is currently assisting with the ongoing open-label extension and preparation of patisiran regulatory submissions for PMDA in Japan and other health agencies.

Contact Veristat Today

Learn more about Veristat and how we can assist you with your rare disease trial development, execution, and regulatory submission preparation.

www.veristat.com