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VERISTAT

Data-Driven Strategies to Advance Development and Approval of Rare/Ultra-Rare Disease Therapies

Getting Your Novel Therapies to Patients Everywhere

At Veristat, we see facilitating success for our clients of rare disease therapy clinical trials as a fundamental responsibility. As a data-driven CRO, we focus on turning complex evidence into clear, well-supported decisions across development. We never assume a situation can be addressed the way it's always been done before.

From regulatory pathway selection, to patient recruitment and site selection, to collecting and cleaning the data, we plan your trial with the utmost precision and innovative approaches. Our teams integrate data capture, data quality, and analytical insight to reduce uncertainty and support confident decision-making. Our knowledgeable experts account for your highly compromised study participants, leveraging the power of biostatistics to meet your trial's objectives, regardless of the complexities.

Supporting the development of rare disease therapies accounts for **25%** of Veristat's work

In the past 5 years

Nearly

400

Rare Disease Clinical Trials & Consulting Projects

Including:

46 cell and gene therapy projects

27 US and European marketing applications (NDAs/BLAs/MAAs, etc.) prepared

Unparalleled Rare Disease Expertise from IND/CTA to Successful Outcomes

Strategic and Regulatory Advice

We help determine if your study qualifies for an accelerated regulatory approval pathway and we'll represent you at US Food & Drug Administration (FDA) and European Medicines Agency (EMA) meetings.

Clinical Trial Design and Delivery

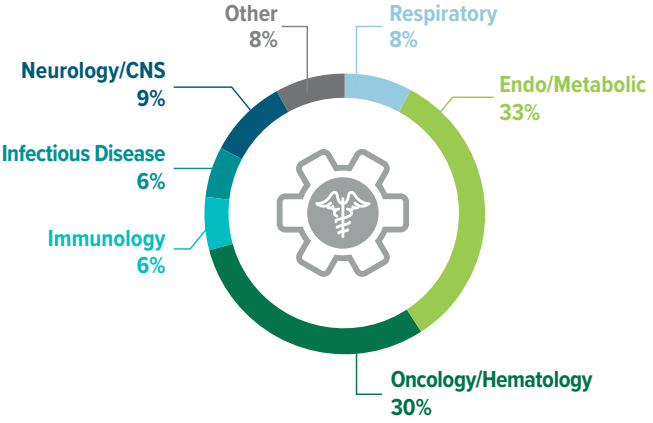
Veristat ensures that your clinical trial or program design supports your regulatory strategy, whether you plan to run a single pivotal trial or multiple trials. We offer a range of solutions – including decentralized trials, natural history studies, and a central site model – to keep your program on track and enable better-supported paths to approval.

Marketing Application Preparation

When your next milestone is to get your NDA, BLA, MAA, NDS or jNDA submitted on-time, trust our integrated team that has prepared more than 184 marketing applications to successful outcomes.

The work of Veristat's innovative regulatory, statistical, and medical writing experts was vital in supporting 27 regulatory approvals for rare disease therapies in the past five years.

Tailored, trusted expertise across a broad range of therapeutic areas



> *"Advancing complex therapies for challenging diseases is what motivates us every day. What distinguishes Veristat is the ability of our multi-disciplinary project teams to draw the appropriate connections amongst the fundamental constructs of the science, the practical and operational requirements of the clinical program, and the regulatory pathway. Those combined provide the evidentiary basis for product approval."*
>
> – John Balser, Ph.D., Co-founder, President & Chief Statistical Officer, Veristat

Meet Veristat

Rare Disease Therapy Expertise That Accelerates Success

If you've struggled with missed deadlines or regulatory uncertainty, talk to Veristat. We have deep experience supporting rare disease and gene therapy development programs from first-in-human trials through global approvals. With specialized expertise and a proven track record, our team accelerates complex clinical development without compromising quality. It's not just business for Veristat—it's personal.

veristat.com ›