

## Understanding & Overcoming the Challenges in Rare Disease Clinical Trials

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Why is conducting clinical trials for rare diseases difficult? Because studying a rare disease poses unique challenges and requires thinking differently.

Advancing innovative science into a new medicine for a rare disease is complicated by many factors, including:

- Rare diseases are often not well understood, compared to more common diseases. The low numbers of people living with each rare disease makes it difficult to study.
- For many rare diseases, established endpoints, or defined clinical measures, don't exist.
- Because many have no approved therapies, rare diseases often lack a clearly defined clinical trial roadmap.

Science is hard, and this is especially true for rare diseases where awareness, assays, endpoints, patient registries, regulatory precedent, and many other sources of information often do not exist.

### **Understanding the unique challenges and meeting patient needs**

Alexion's approach to rare disease clinical research is guided by the insights and perspectives we gain from listening to and learning from the rare disease communities we aim to serve. This is why Alexion continues to lead the way in helping to address the unique challenges of designing, recruiting, and conducting clinical trials for rare diseases.

### **Pioneering Innovation in Rare Disease Research and Development (R&D)**

Our ability to deliver transformative therapies for people living with rare diseases stems from a deep understanding of patients' lived experiences and close relationships with the rare disease community. In addition to designing research frameworks and systems to inform rigorous clinical trials, we're also conducting research aimed at shortening the diagnostic odyssey, which can take as long as five years for many rare diseases.

Alexion's bold and creative approaches are essential to delivering transformational medicines for diseases where few to no options exist. Where there are no well-defined clinical development roadmaps or regulatory pathways to follow, our teams continuously think outside the box to incorporate patient and caregiver insights and real-world evidence into our research programs. This helps to ensure that our clinical trials are designed to represent patients' unique needs, demonstrate the impact of what is often a first-of-its-kind medicine, and comply with global regulatory requirements.

Fearlessly pursuing today's most pressing rare diseases requires researchers to think differently about R&D – and we remain committed to leading the way. At Alexion, our deep understanding of the patient journey informs our decisions about trial design, including considerations on how to reduce the burden for clinical trial participants and their caregivers.

We continue to pioneer new approaches to accelerate the discovery and development of transformative medicine where scientific progress has stalled or there are limited or no treatment options – because we know the need is great, and people are counting on us.

Learn about our work in [research and development](#).