

Leading In Rare Disease Clinical Trials

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Alexion's journey to bring life-changing therapies to people living with rare diseases has been as innovative as the medicines themselves. For three decades, our researchers and scientific leaders have been pioneering new pathways for research and development (R&D) in rare disease with unyielding perseverance. We continue to [lead the way in rare disease clinical research](#) by tailoring core elements of the clinical trial process to meet the unique needs of the rare disease patients we aim to serve.

As compared to more common diseases, studying rare diseases poses unique challenges due to the low numbers of people living with a specific condition. Rare diseases are often not well understood, and few diagnostic tools exist to detect and guide the development of effective therapies. Without a well-defined clinical development roadmap to follow, our teams must continuously think outside the box to identify endpoints and design robust clinical trials to ensure we generate meaningful results.

Our close ties to the rare disease community and our patient-centric approach to clinical trials are the keys to our success. Every stage of the Alexion R&D process is infused with direct insights from patients, caregivers, and advocates who help improve our understanding of the diseases we set out to treat. These inputs are used to tailor trial protocols, define what success looks like, and, ultimately, ensure that our work is a true reflection of the people we serve and will have a meaningful impact on their lives.

"Pushing the boundaries of rare disease research requires smart, inventive, and fearless thinking. I'm proud to be part of a company leading through innovation to bring hope to rare disease communities. Alexion has advanced groundbreaking science over the past 30 years, providing us with unmatched rare disease expertise that we continue building upon to help us deliver new medicines to more patients." – Gianluca Pirozzi, SVP, Head of Development and Safety

These bold and creative approaches have been essential to delivering transformational medicines for diseases where few to no options existed.

[Learn more](#) about our research and how we're blazing a trail in rare disease clinical development.