

LEADING 30 YEARS
IN RARE DISEASES FOR

RARE DISEASE REGULATORY PATHWAYS

Today, just 5% of the 7,000 rare diseases that exist have approved therapies. Alexion is working to change this by following the science to bring new treatments to people living with rare diseases. Our rich history of developing medicines for patients who have few or no treatment options includes our leadership in [pioneering rare disease regulatory pathways](#), which continues to guide many innovators today.

Presenting potential rare disease therapies for regulatory review poses unique challenges because many of these conditions are not well understood and often lack regulatory roadmaps. When working on a rare disease that has no existing treatment options, one key challenge is the lack of pre-defined disease measurement standards that experts and regulators agree will demonstrate the impact of a potential therapeutic for that condition. Through a robust understanding of the science and rare disease patient experience, and early engagement with health authorities, Alexion identified innovative ways to demonstrate the impact of its medicines while satisfying regulatory requirements.

Our expertise and experience in rare diseases has taught us to think creatively about how to capture and convey meaningful data. For example, Alexion was one of the first companies to use video evidence in regulatory submissions to demonstrate the impact of an outcome measure for a metabolic rare disease, an approach that is now considered standard practice. We've also utilized real-world evidence from registries to provide new and complementary information for regulatory submissions in an effort to accelerate sorely needed advances for rare patient communities.

"Alexion's tenacity, scientific expertise and collaborative approach with regulatory agencies and patient communities help us to bring new or improved treatments to people living with rare diseases as quickly as possible. Our innovations over the last three decades drive us forward as we continue our work to help transform the lives of more patients around the world." - Martine Zimmermann, SVP, Head of Regulatory & Quality Affairs, Alexion.

By listening to and learning from the communities we serve, Alexion has been able to work together with regulatory decision makers to define and establish meaningful outcome measures, creating bespoke pathways for certain rare disease medicines to bring hope to more patients in need.

Visit our [Research & Development](#) page to learn more about Alexion's leadership in rare disease R&D.