

Leading in Tenacity, Grit and Curiosity to Deliver Rare Medicines

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Every day, people living with rare diseases face uncertainty with bravery and an unyielding determination to live their lives to the fullest. These individuals inspire us to approach our research and development (R&D) with the same level of [tenacity, grit and curiosity](#) in order to push the bounds of science and deliver transformative medicines.

Science is hard – and this is especially true in rare diseases where we are often among the first to address an unmet need. Assays, endpoints, patient registries, regulatory precedent, and many other sources of information that are available for more common conditions do not exist for most rare diseases. It falls to us to lay the groundwork upon which medicines can be made.

For three decades, our R&D colleagues have embraced this challenge. While we have experienced many successes that have changed the treatment landscape for certain rare diseases, failure is also a reality of working in a complex and challenging field. Although failure is never the desired outcome, the experience gives us an opportunity to learn and improve. When we encounter a setback, we assess what went wrong, share those learnings with others in the scientific community, and apply them to our future research. We press on in our mission to help transform patient lives.

Sharon Barr, SVP, Head of Research & Product Development, further explains how our determination and curiosity makes Alexion unique: “Our researchers are unafraid to step into new territory, and we refuse to wait for others to pave a path for us. Being the first to take on a scientific challenge takes a great deal of courage, resiliency, and a thirst for knowledge. We embrace this mindset across every stage of R&D because we know that people living with rare diseases are counting on us.”

Building on our earliest days as pioneers in complement research, we continue to fearlessly follow the science. The work is deeply rewarding for our many scientists who have a passion for discovery and are relentless in their work to find new solutions for patients. Collaboration is critical in this effort, and our scientists are constantly sharing ideas and learnings to help move our research forward. This interconnectivity enables us to continuously evolve how we approach R&D, identify targets to pursue, and tackle the most urgent rare disease challenges.

Learn more about our pipeline and how we're advancing rare disease R&D to help more people living with rare diseases and their families by visiting <https://alexion.com/our-research/pipeline>.