

## Leading In Continued Innovation for Rare Diseases

December 1, 2022



Over the past year, we marked 30 years of leadership in rare diseases by celebrating our pioneering legacy in complement science, rich history of scientific firsts and unyielding commitment to patients and their families. Our achievements, including delivering the first targeted complement inhibitor and first approved treatments in multiple rare diseases, are a testament to our tenacity and relentless pursuit of innovation. But there is much more work to be done to support rare disease communities across the world, and as we look toward the next 30 years, we will [continue to lead the way](#).

As part of AstraZeneca, we have more opportunities for innovation than ever before. Combining AstraZeneca's capabilities in precision medicine and our expertise in rare disease development and commercialization enables us to further develop a portfolio of medicines addressing the large unmet needs of patients with rare diseases. New capabilities, including cutting-edge genomic medicine and artificial intelligence technology, as well as tools that enhance the way we evaluate molecules are opening up new research pathways and accelerating the pace of discovery.

In addition, we remain committed to expanding access to our medicines for more rare disease patients around the world. Leveraging AstraZeneca's far-reaching global footprint, we have an opportunity to bring our clinical trial programs to new regions and deliver our medicines to more patients, many of whom have limited or no treatment options available to them today. Geographical expansion is an essential part of our commitment to innovate for the small, but mighty, diverse population of people living with rare diseases worldwide.

Gianluca Pirozzi, SVP, Head of Development and Safety, reinforces why the future is bright: "We are incredibly proud of what we have accomplished together with the rare disease community over the past three decades, but we see today as just the beginning. Our unmatched rare disease expertise combined with AstraZeneca's expansive reach and scientific capabilities positions us to continue to take on the tough challenges, follow the science and expand the reach of our medicines into new areas where there is great need. We are excited to continue leading at the forefront of innovation for patients."

Learn more about our leadership in rare disease R&D, and our commitment to listening to, understanding and responding to the patients we serve by visiting <https://alexion.com/our-research/research-and-development>.