

## Leading in Rare Diseases for 30 Years: Pioneering Complement Science Then and Now

March 16, 2022



As we celebrate Alexion's 30th anniversary this year, we are reflecting on our history of scientific firsts, showcasing our innovations, and reaffirming our commitment to continue leading in rare disease research and development to support more patients. Persevering despite many challenges, our "[complement pioneers](#)" discovered and advanced a new class of medicines and set the tone that continues to drive our work to help more people living with rare diseases reclaim their lives.

Alexion's legacy in rare diseases is rooted in being the first to translate the complex biology of the [complement system](#), a foundational part of the immune system, into transformative medicines. When working properly, the complement system is a strong and powerful tool that helps keep the body healthy. But when the system is thrown out of balance, or dysregulated, it can attack cells and tissues, resulting in harmful inflammation and destruction of healthy cells.

In the early 1990s, the concept of complement inhibition — targeting specific proteins in the complement system to prevent the destruction of cells — was met with skepticism by the scientific community. At the time, there was speculation that it would not address a key driver of disease. But Alexion's scientists led the way by following the science and ultimately delivered the first targeted complement inhibitor. Our perseverance helped transform the lives of patients living with a rare disease for which there were previously no treatment options, and that was just the beginning.

Today, Alexion's research in complement biology has led to transformative medicines for certain diseases spanning hematology, nephrology, and neurology. Our pioneering spirit persists in our ongoing efforts to continue unlocking the potential of the complement system, which may be the key to accelerating the discovery and development of new life-changing therapies for even more patients.

"Complement is the cornerstone of our portfolio and I'm proud of our organization's deep expertise. We honor the grit and tenacity of Alexion's early scientists by remaining bold, determined, and focused on our goal of transforming the lives of people living with rare diseases." — Sharon Barr, SVP, Head of Research and Product Development

[Learn more](#) about Alexion's science and hear directly from those who played a role in translating complement biology into transformational medicines for rare diseases.