

Alexion Pharmaceuticals Sets New Benchmarks in Rare Disease Innovation and Financial Growth in 2024

04 September 2024 | Analysis

Alexion Pharmaceuticals, a key player in AstraZeneca's Rare Disease unit, has demonstrated remarkable progress in both clinical research and financial performance, reinforcing its leadership in the treatment of rare diseases. The company's recent achievements reflect a robust commitment to advancing innovative therapies and delivering impactful results for patients globally.



Clinically, Alexion has made substantial strides in the treatment of rare and debilitating conditions. At the 2024 International Symposium on Amyloidosis, the company presented 14 studies focused on amyloidosis therapies, underscoring its dedication to addressing this complex disease. Among the highlights was the Phase 3 NEURO-TTRansform study on WAINUA™ (eplontersen), which showcased promising results. Additionally, Alexion introduced investigational treatments like ALXN2220 and anselamimab, further expanding its pipeline of potential therapies for amyloidosis.

In another major advancement, Alexion unveiled new clinical data at the 2024 American Academy of Neurology (AAN) Annual Meeting, spotlighting the efficacy of ULTOMIRIS® and SOLIRIS® in treating generalized myasthenia gravis (gMG) and neuromyelitis optica spectrum disorder (NMOSD). Notably, ULTOMIRIS® received FDA approval for NMOSD, demonstrating a groundbreaking 98.6% reduction in relapse risk, positioning it as a leading treatment option for this serious condition.

Further cementing its position as an innovator in rare disease treatments, Alexion secured FDA approval for VOYDEYA™ (danicopan), a first-in-class oral Factor D inhibitor, as an add-on therapy for adults with paroxysmal nocturnal hemoglobinuria (PNH). This approval marks a significant step forward in providing more accessible treatment options for patients with PNH.

On the financial front, Alexion's performance in the first half (H1) and second quarter (Q2) of 2024 has been equally impressive. The company reported a robust 18% year-over-year growth in total revenue, reaching \$25.6 billion. This growth was driven in large part by the strong demand for Ultomiris, which saw a remarkable 36% increase in sales. Conversely, Soliris experienced an 8% decline in sales, reflecting the ongoing conversion of patients to Ultomiris, a testament to the newer therapy's effectiveness and acceptance.

Reflecting on its strong performance, Alexion has upgraded its full-year 2024 guidance, now anticipating mid-teens percentage growth in Total Revenue and Core Earnings Per Share (EPS). This positive outlook underscores the momentum Alexion has gained through its strategic focus on clinical advancements and market performance, setting the

stage for continued success in the rare disease sector.

Alexion Pharmaceuticals' ongoing commitment to innovation, coupled with its strategic financial management, positions the company as a leader in the treatment of rare diseases. With a robust pipeline and a track record of delivering transformative therapies, Alexion is poised to make a lasting impact on the lives of patients worldwide.