Rare Disease

Annual Report 2023 Fact Sheet

AstraZeneca

After more than two full years as Alexion, AstraZeneca Rare Disease, our medicines are approved in 70 countries.

As we expand the reach of our medicines, our growing pipeline of investigational molecules represents continued innovation on behalf of rare disease patients. Our mission remains to transform the lives of people affected by rare diseases through the development and delivery of innovative medicines as well as supportive technologies and healthcare services.

Unmet need

400m

people around the world are living with a rare disease.

>10,000

estimated number of rare diseases; fewer than 10% have approved treatment options.

Alexion, AstraZeneca Rare Disease in 2023

Total Revenue

$7,764m

Up 10%

(12% at CER)

Therapy area world market

(MAT Q3-23)

$158.4bn

Annual worldwide market value

Source: IQVIA.
AstraZeneca focuses on specific segments within this overall therapy area market.

Sustained leadership in complement

Alexion was the first company to translate the complement system into transformative medicines. We are continuing that legacy of leadership across multiple disease areas, leveraging AstraZeneca's established footprint and expanding our global presence through Centres of Excellence to reach patients with high unmet medical need.

Pursuing new indications

We are developing a broad portfolio of potential medicines that target various components of the complement system, with opportunities to pursue indications across a wide range of therapeutic areas of interest, including haematology, nephrology, neurology and ophthalmology.

Factor D inhibition

We have a robust portfolio of investigational medicines that inhibit the complement protein Factor D, including small molecule oral assets with potential broad application across several disease areas.

Expanding beyond complement

We have continued to expand our rare disease focus with novel assets for non-complement mediated diseases.

Amyloidosis is a group of complex rare diseases, with varying types and severities. Alexion and AstraZeneca are advancing the industry's largest amyloidosis pipeline, across a broad range of modalities, to address the spectrum of patient need across multiple disease subtypes.

Genomic medicine

Thousands of diseases – including 80% of known rare diseases – are believed to be caused by a genetic mutation. Genomic medicines are designed to treat or cure these diseases through the addition, alteration or inactivation of the malfunctioning gene.

Supported by recent strategic acquisitions, investments and collaborations, Alexion and AstraZeneca are uniquely positioned to advance an industry-leading suite of next-generation genomic medicines and platforms, with the objective to develop innovative therapies with improved safety and efficacy profiles.

Source of content: 2023 AstraZeneca Annual Report.