

Alexion, AstraZeneca Rare Disease, enters agreement with Pfizer to acquire a portfolio of preclinical rare disease gene therapies

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Agreement furthers Alexion's ambition to transform patient outcomes with genomic medicine and provides synergistic opportunities across AstraZeneca

Portfolio includes preclinical gene therapy programmes and enabling technologies with potential across several therapeutic areas

Alexion, AstraZeneca Rare Disease, today announced that it has entered a definitive purchase and licence agreement for a portfolio of preclinical gene therapy programmes and enabling technologies from Pfizer Inc. (Pfizer). The agreement furthers Alexion and AstraZeneca's commitment to advancing next-generation genomic medicines with the addition of complementary pipeline assets and innovative technologies.

As part of the agreement, the transaction will bring to Alexion a number of novel adeno-associated virus (AAV) capsids. AAV capsids have been shown to be an effective mechanism for delivering therapeutic gene cargos for gene therapy and gene editing.¹ These new resources build on Alexion and AstraZeneca's combined capabilities in genomic medicine, recently strengthened with the acquisition of LogicBio, with the objective to develop new genetic therapies with improved safety and efficacy profiles. Additionally, Alexion will seek to welcome talent from Pfizer associated with the portfolio.

Marc Dunoyer, Chief Executive Officer, Alexion, AstraZeneca Rare Disease, said: "Today's announcement represents another major step forward in Alexion and AstraZeneca's ambition to be an industry leader in genomic medicine, which has potential to be transformative and even curative for patients with devastating diseases. We look forward to continuing our work to develop enhanced platforms and technologies with broad therapeutic application while integrating best-in-class expertise to accelerate promising therapeutics into the clinic."

There are more than 7,000 known rare diseases, and around 80% of rare diseases are believed to be caused by a genetic mutation.^{2,3} Genomic medicines are designed to treat or cure these diseases by addressing the malfunctioning gene. This can be done through addition, alteration or inactivation of the gene to help the body fight the disease.⁴

Financial considerations

Under the agreement, Alexion will purchase and licence the assets of Pfizer's early-stage rare disease gene therapy portfolio for a total consideration of up to \$1bn, plus tiered royalties on sales.

Alexion plans to close the transaction in Q3 2023, subject to the satisfaction of closing conditions.

Notes

Alexion

Alexion, AstraZeneca Rare Disease, is the group within AstraZeneca focused on rare diseases, created following the 2021 acquisition of Alexion Pharmaceuticals, Inc. As a leader in rare diseases for nearly 30 years, Alexion is focused on serving patients and families affected by rare diseases and devastating conditions through the discovery, development and commercialisation of life-changing medicines. Alexion focuses its research efforts on novel molecules and targets in the complement cascade and its development efforts on haematology, nephrology, neurology, metabolic disorders, cardiology and ophthalmology. Headquartered in Boston, Massachusetts, Alexion has offices around the globe and serves patients in more than 50 countries.

AstraZeneca

AstraZeneca (LSE/STO/Nasdaq: AZN) is a global, science-led biopharmaceutical company that focuses on the discovery, development, and commercialisation of prescription medicines in Oncology, Rare Diseases, and BioPharmaceuticals, including Cardiovascular, Renal & Metabolism, and Respiratory & Immunology. Based in Cambridge, UK, AstraZeneca operates in over 100 countries and its innovative medicines are used by millions of patients worldwide. Please visit [astrazeneca.com](https://www.astrazeneca.com) and follow the Company on Twitter @[AstraZeneca](https://twitter.com/AstraZeneca).

Contacts

For details on how to contact the Investor Relations Team, please click [here](#). For Media contacts, click [here](#).

References

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