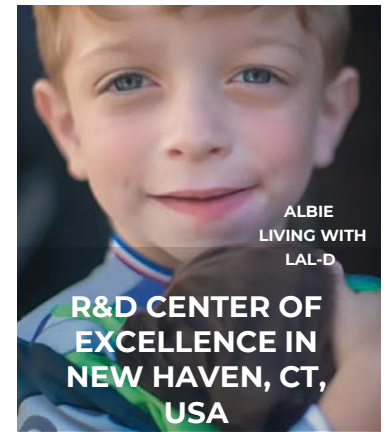


# ABOUT ALEXION UK & IRELAND



\*INCLUDING MANUFACTURING FACILITIES  
IN IRELAND

Our mission is to transform the lives of people living with rare diseases and devastating conditions through the development and delivery of innovative medicines, as well as through supportive technologies and healthcare services

## 5 APPROVED THERAPIES FOR PATIENTS WITH 7 RARE DISEASES & DEVASTATING CONDITIONS\*\*

### Complement inhibitor therapies for the treatment of patients with:

**PNH**  
PAROXYSMAL NOCTURNAL HAEMOGLOBINURIA  
**aHUS**  
ATYPICAL HAEMOLYTIC URAEMIC SYNDROME  
**gMG**  
GENERALISED MYASTHENIA GRAVIS  
**NMOSD**  
NEUROMYELITIS OPTICA SPECTRUM DISORDER

### Enzyme replacement therapies for the treatment of patients with:

**HPP**  
HYPOPHOSPHATASIA  
**LAL-D**  
LYSOSOMAL ACID LIPASE DEFICIENCY

**Factor Xa inhibitor reversal agent when anticoagulant reversal is needed for patients with LIFE-THREATENING OR UNCONTROLLED BLEEDING**

\*\*NOT ALL THERAPIES ARE REIMBURSED OR AVAILABLE IN THE UK & IRELAND.

Our understanding of rare diseases began in our pioneering work in complement biology

**20+**

Years of leadership in complement-mediated diseases

**40,000**

patient-years of trials and real-world data in more than 50 countries

**FIRST PNH THERAPY**

identified and researched in the UK following collaboration between leading UK PNH clinicians and Alexion<sup>1</sup>

Our innovation is driven by understanding people living with rare diseases, which fuels all our efforts

**3.5**

million people are affected by a rare disease in the UK<sup>2</sup>

Rare diseases are often **severe**, chronic and progressive, with **high mortality rates**

**52%**

of patients initially receive an **incorrect diagnosis**<sup>3</sup>

**95%**

of rare diseases do not have a treatment available

Our knowledge allows us to innovate and evolve into new areas where there is great unmet need and opportunity to help patients and families live their best lives

Alexion is developing several early and mid-to-late-stage therapies, such as a copper-binding agent for Wilson's disease, and is expanding its complement technology into novel disease areas including ALS.

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, ophthalmology and acute care.

**10+**

**UK Clinical Trials,**  
including studies in  
PNH, aHUS, HPP and LAL-D,  
and further planned UK trials

We work in partnership with clinical communities in the UK to advance the understanding and management of rare diseases



In the UK & Ireland, Alexion provides quality and specialised manufacturing, with secure end-to-end supply.

Our clinical programme focuses on the unmet needs in complement biology and new disease areas.

In the UK & Ireland, Alexion supports initiatives to reduce the multi-year delays in diagnosis, such as:

- Involvement in the Discovery Forum of the 100,000 Genomes Project to improve earlier diagnosis<sup>4</sup>
- Working across UK hospitals to educate on the importance of considering and testing for rare diseases

Alexion supports education programmes for healthcare professionals (HCPs) in the UK & Ireland, such as sponsorship of Kidney Research UK Fellow's Day, which brings together students, HCPs and researchers.

Alexion raises awareness of rare diseases through partnerships with patient advocacy groups such as Genetic Alliance UK.

Alexion provides education, assistance and support that enhances disease management for patients and caregivers.

In the UK & Ireland, Alexion provides funding for homecare services, enabling PNH and aHUS patients to be treated in the comfort of their own home whilst relieving the burden on hospital resources.

Alexion has a long history of collaborating with the NHS to identify solutions to improve access to our therapies. Example: agreement with NHS to ensure access to treatment for HPP patients in 2017...<sup>5</sup>

*"This innovative deal shows that with goodwill and flexibility from responsible life science companies we can square the circle between offering ground-breaking therapies to NHS patients on the one hand, while driving maximum value on the other." – NHS England Chief Executive Simon Stevens*

People living with rare and devastating diseases are our inspiration and our **Guiding Star**. Every day we strive to live up to their expectations by following our culture values to **serve patients, act with integrity, innovate for solutions** and **empower people**, and earn **trust** within the rare disease community



**QUALITY**

Committed to integrity of our data.

Committed to ensuring our therapies meet the requirements of our regulators, patients and our customers.



**ETHICS & COMPLIANCE**

Committed to conducting business in an ethical, legal and socially responsible manner.



**GIVING BACK**

Dedicated to supporting the communities in which we live and work.



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ALEXION. RARE INSPIRATION. CHANGING LIVES

1. NHS England. 2013. NHS Standard Contract for PNH Service. 2. UK Department of Health and Social Care: UK Strategy for Rare Diseases. 3. Rare Disease UK. 2016. The Rare Reality – an insight into the patient and family experience of rare disease Report. 4. Genomics England. 2019. Industry collaboration already benefiting participants of 100,000 Genomes Project. 5. NHS England. 2017. NHS patients with rare bone disease to benefit from potentially life-transforming drug due to "first of a kind" agreement between NHS England and the manufacturer.