Inspired by patients.
Driven by science.

Rare Diseases

There are between 27 and 36 million patients affected by rare diseases in the EU alone[1], an additional 30 million in the USA and an estimated 400 million people globally.

About 8 out of 10 of these conditions are genetic and around 75% present symptoms at birth or during early childhood, but some do not present until adulthood. Sadly, most rare diseases remain without a cure and living with one has a considerable life-long impact on both patients and their families.

The often long journey to diagnosis can be a major hurdle and one which presents a huge burden on patients, both physically and mentally. Even once diagnosed, unfortunately there are often no treatments available. This also represents a major challenge for healthcare systems.

UCB supports the aims of the UN Resolution on "Addressing the challenges of persons living with a rare disease and their families" which emphasizes the need to take action to tackle rare diseases globally, and highlights that addressing the needs of people living with rare diseases is crucial to advancing the 2030 Agenda for Sustainable Development. Fortunately, new technologies and advanced therapies are increasingly providing hope for some patients living with rare diseases.

UCB's Values

Our goal is that by 2030, all patients who need our medicines in countries where we operate have access to them. Patients are at the heart of everything we do and we believe rare disease patients have as much right to effective healthcare as everyone else. We are driven to help those we can live the best life possible and relieve the challenges and uncertainty of disease.

At UCB, we do not just see patients or population size, we see people in need. We build on our science led heritage to deliver real value for patients, focusing on improving patient outcomes, experience and access to treatments, especially in areas of unmet need including rare diseases. This is driven by UCB's Patient Value Strategy which underpins our strategic priorities and R&D processes.

UCB's Approach

UCB is a scientific leader with global impact, and we are determined to leverage our expertise and knowledge to make rapid advances in the development of treatments for rare diseases. We are increasing the scientific understanding of how diseases change the way our bodies work, known as pathophysiology, to radically accelerate treatment options.

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[1] European Commission. (2016). Rare diseases. [online] Available **here**.

No one size fits all, and this is even more evident in rare disease. We are working towards improving the lived experience of each individual, ensuring they have sustainable access to our treatments. To ensure a truly holistic approach, we work side-by-side with patient communities and embed their perspectives throughout the life cycle, from research to patient support programs.

UCB's Research Focus

UCB works closely with partners to strengthen our ability to find solutions for patients living with rare diseases. For example, we are strengthening our neurology and immunology efforts and advance progress in treating a broad population of people living with Myasthenia Gravis (MG), a rare, chronic neuromuscular disease which affects around 700,000 people worldwide. We are expanding our research capabilities and accelerating the development of new medicines, with the aim of making a bigger impact within the MG community.

Our commitment to rare disease communities expands beyond our own research. We are also convening a global network of rare disease experts and believe this community will lead the evolution of care while serving patients living with rare diseases.

As we continue to work to find solutions for rare disease patients, UCB's research is expanding into sectors such as gene therapy, with a commitment to build a ground-breaking new gene therapy and clinical manufacturing plant in Belgium.

UCB's Commitment to a Global Approach

Treating each rare disease requires its own unique combination of health interventions and support structures, however many rare disease patients face common issues and challenges. We are committed to do our part and work closely in partnership with the global rare disease community to:

- Ensure research is targeted at areas of unmet need. UCB's pipeline focuses on innovative therapies which offer new hope for patients for conditions such as rare epilepsies
- Ensure that patients have timely access to the latest treatment innovations through modernized technology assessment processes and value assessments
- Improve early and accurate diagnosis of rare diseases
- Support health systems to properly recognize and understand newly discovered rare diseases.