Championing patients every step of the way
The majority of people living with a rare disease have no treatment for their condition.

At Shire, we have long believed we have a unique opportunity to champion these underserved patient communities.

And so we never stop seeking new therapies, exploring new options, and supporting patients every step of the way.
The patient need drives how we discover, develop and deliver potentially breakthrough therapies

A worldwide problem
Rare diseases, most of which are genetic and around half of which begin in childhood, pose a significant medical and economic burden for patients, communities and healthcare systems.

At Shire, our answer has been to expand our rare disease portfolio dramatically in recent years — developing transformative treatments for previously overlooked conditions.

We focus on a core of rare disease programs within the therapeutic areas of hematology, immunology, genetic diseases and oncology. Our knowledge and experience extends to highly specialized conditions in the fields of neuroscience, ophthalmics, gastrointestinal and internal medicine.

The patients’ champion
We see ourselves as committed champions for patients who are struggling to live their lives to the fullest — as well as their families, caregivers and healthcare professionals.

And with our global reach we ensure that our innovative therapies and support systems can reach patients wherever they are in the world.

With nearly 24,000 employees in 65+ countries our therapies are available in more than 100 countries.

The need is staggering and personally felt...

Of the 7,000 known rare diseases only ~5% have a treatment available.¹

Of the known rare diseases, only 50% have a disease-specific foundation or support group.¹

Nearly 50% of rare diseases begin in childhood, and 30% of children with a rare disease will not live to see their fifth birthday.¹

In the U.S. and UK, the average time to obtain a correct diagnosis is 5—7 years, requiring visits to up to eight physicians.²

...and so we invest in innovative patient solutions

Our focus: innovation

Innovation is the lifeblood of our current and future success and it is the only way to develop the new therapies patients need

Our commitment to innovation drives us to develop new therapies, new technologies to personalize patient resources and new methods to improve participation in clinical trials.

Research and development model
Innovation is fundamental to building our strong clinical pipeline — the cornerstone of our organization. Using in-house rare disease R&D capabilities and consistent input from our patient community partners, we aim to find new treatments for patients in our chosen therapeutic areas. Our pipeline has transformed in recent years, and now includes compounds with potential rare disease indications at all stages of development.

Where we do not have expertise, we seek the best complementary science, technology and industry minds to help us. Through collaboration, partnerships and acquisitions, we work to push the boundaries of what is possible.

Innovation in clinical trials
When conducting clinical trials for diseases with small patient populations, it is a challenge to recruit the necessary number of patients. In pediatric trials, a high percentage of participants have not yet even been born. In response to this problem, we have developed new patient-finding methods, using statistical analysis and demographic information. We can even pinpoint future patients and anticipate their locations. By sharing the results of work in clinical trials, we can build a deeper understanding of rare diseases in the broader scientific and healthcare environment.

Working in partnership with other medical and research organizations helps us accelerate the development of new treatments.

We invest in research to develop educational materials and awareness campaigns to help remove the obstacles associated with diagnosis and access to treatment.

Our patient support programs provide online and telephone support — reaching around 60,000 patients each year.
Our focus: partnerships

By connecting with and learning from others who have gone before them, patients with rare diseases have the opportunity to more proactively manage their conditions.

Personalized solutions
Beyond developing new therapies, we are using digital health innovations to develop personalized solutions for patients. Advancements in smart device technologies are helping us improve the delivery and monitoring of treatment to some patients. We are forming partnerships with online communities where people go for help to learn more about their medical condition and become part of an extensive online network that can track and perhaps improve their outcomes. These communities allow researchers to learn more about what is working, what is not, and where the gaps are, so that they can develop new and innovative treatments.

Partnerships for progress
Partnerships are key to building a global environment in which novel treatments can reach those who need them faster. Working closely with hospitals, academic researchers and charitable organizations helps us take innovation to new levels and can accelerate the development of new treatments. This unique approach has helped deliver many cutting-edge therapies.

Partnership with Cincinnati Children’s Hospital Medical Center
In 2015, we established a partnership with the Cincinnati Children’s Hospital Medical Center to discover and develop novel therapies to treat rare diseases. Our collaboration is able to leverage the hospital’s expertise in many fields of research that align with our therapeutic areas of focus, including rare diseases, gastroenterology, nephrology and neurology.
Our focus: knowledge

The rare disease landscape is complex. Building awareness and understanding removes obstacles patients may face.

The impact of being misunderstood
The prevalent lack of awareness surrounding rare diseases means that they are unrecognized and often under-diagnosed. On average, it takes five to seven years for a patient in the U.S. and UK to receive an accurate diagnosis. It is estimated that 41 percent of people may be misdiagnosed at least once.1 This delay can often cause a prolonged period of uncertainty and burden for many patients and their families through increased medical costs or incorrect treatments.

Opening the door
With so many patients unable to get the treatment they so desperately need, we are exploring targeted diagnostic approaches early in clinical development to help improve the pathway to diagnosis. We also work with policymakers around the world to find ways to improve the availability and affordability of treatments.

Fostering awareness
Building the knowledge and understanding of rare diseases is a precursor to future innovation and treatments. It is why we have built relationships with more than 180 patient advocacy groups and organizations globally. Working together, we can help patients and their doctors by raising awareness of the conditions, offering education about their disease and sharing information about relevant clinical trials.

Developing a medicine is far from the end of the story. Many patients need our help accessing medicines or support in managing their treatment.

**Long-term support**
To Shire, a patient’s well-being is a bigger picture than the medicine alone. Before launching any new treatment, we invest time in thinking about the types of support that will be most beneficial to patients. That could mean anything from designing a model to improve diagnosis, to creating a dedicated website that provides patient resources. We also devise personalized lifelong programs to help improve a patient’s quality of life.

**Promoting communication**
To help patients share experiences and lessen the sense of isolation, we are building patient networks to create connections. By supporting online communities, we encourage people to learn from each other. And these forums are just as useful for researchers, who learn about what is working, what is not, and where the gaps in treatment exist.

**Shire patient assistance programs**
Our patient assistance programs are set up to provide help and support for our patients. This includes online and telephone services to give product support, explain insurance and coverage options, provide educational resources and facilitate contact with healthcare professionals and pharmacists. These programs provide support to around 60,000 patients annually.
Our people are our greatest asset — talented, focused and committed. We all believe that where there is a will, there is always a way.

Today’s employees want to be associated with a company that helps them feel good about what they are doing to address society’s issues and problems.

At Shire, we are building a culture that ensures we all come to work with a common purpose: to transform the treatment of rare diseases.

We know our employees are committed to a better world, and patients feel the benefit of a global team focusing on their conditions. We also know that great teams are made up of outstanding individuals. We work hard to attract and develop the best talent, create a diverse and inclusive workforce, and provide a safe and healthy workplace.

Genetic fellowships
In response to the severe shortfall in numbers of medical geneticists, we are contributing to support 10 genetic fellowships at the American College of Medical Genetics and Genomics. It’s a long-term investment that will ultimately help shape the future of rare disease treatments for patients worldwide.
Patients tell us they believe in our ability to deliver the medicines they need. To keep their trust, we must also run our business responsibly.

Responsibility is deeply embedded within our organization. When it comes to making sure we are accountable for our behaviors and our social, economic and environmental impacts, our employees lead the way.

Our commitment to doing business with high standards of ethics and integrity comes from an instinctive sense of responsibility, but it also earns and maintains the trust of the patients and communities we serve — and that is invaluable.

We take great care in managing the environmental and social impacts of our operations, products, and sourcing, and ensure the products we deliver are of the highest quality. The same rigor applies to our value chain. We encourage our business partners, suppliers and contractors to adopt equally responsible and sustainable practices.

SeriousFun camps
SeriousFun is a global organization operating summer camps for children with rare diseases. As its partner, we’ve pledged a $3 million gift over three years to help fund 16 camps and provide hundreds of campers with the opportunity to attend free of charge. Shire employees have also given a total of over 5,000 hours of their time to help out at camps around the world.
At Shire, we know that speed matters, especially to the patients who are waiting for treatments.

That’s why we’ve built a fast-paced, entrepreneurial, international culture where we give people freedom and opportunity to excel while also setting a high bar for being ethical and responsible.

Our teams look forward to championing patients today, tomorrow and through every step of their journey.
Shire is the global leader in serving patients with rare diseases. We strive to develop best-in-class therapies across a core of rare disease areas, supplemented by diversified capabilities in highly specialized conditions.

We feel a strong sense of urgency to address the high unmet medical needs of these patient communities.