We take rare diseases personally
Orphan Europe, part of the Recordati group, is a unique pharmaceutical company. Since 1990 we have used big thinking and extensive know-how on behalf of people affected by rare diseases. Patients and their families are central to our planning, our thinking and our actions.

Ten facts that tell the story of Orphan Europe:

1. Our team shares the conviction that each person with a rare disease has the right to the best treatment.
2. We have nearly 25 years experience in bringing orphan drugs to market for rare diseases:
3. We help to create, and work alongside, global networks of patients, patient groups, experts, healthcare professionals, scientists, policy makers and regulators – see pages 6–7
4. We have over 130 staff in 15 countries, and have plans to expand further around the world – see pages 12–13
5. We have unparalleled experience around the development of clinical trials for orphan drugs and in specialist regulatory requirements – see pages 8–11
6. Our infrastructure has been developed for the unique needs of producing, packaging and distributing very small numbers of specialist products to people around the world – see pages 8–9
7. Our Orphan Drug Specialists are highly qualified and trained, and work closely with key opinion leaders – see pages 10–11
8. We are at the forefront of rare disease policy. We are members of many rare disease organisations.
9. Orphan Europe is committed to improving the diagnosis and management of rare diseases through our educational work and the Recordati Rare Diseases Foundation (fondation d’entreprise) – see pages 20–21
10. Everyone who works for Orphan Europe is encouraged to take part in our staff volunteering programme. Staff demonstrate their commitment to people living with a rare condition and their families by volunteering at recreational therapeutic programmes and holiday camps – see pages 22–23

Rare diseases affect many people

In Europe, a rare disease is defined as a condition that affects less than 5 per 10,000 inhabitants, and is fatal or severely debilitating. There are about 7,000 such diseases. While each disease is rare, when considered together they affect over 25 million Europeans or almost 1 in 10 people. There are certain challenges that all patients and families affected by rare diseases share.

Source: European Medicines Agency
Her diagnosis was difficult.

We make sure her treatment isn’t
Jean-Luc, Director of Production and Manufacturing for Orphan Europe, received a call from a doctor from a university hospital in Switzerland. The doctor explained that a two-year old girl had been diagnosed with NAGS (N-acetylglutamate synthase) deficiency. Without treatment the child would die within a few days.

After the doctor's call, our manufacturing manager started to organise the required supplies along with Swiss labelling and packaging. By 12pm the same day, Jean-Luc, carrying the supply of Carbaglu®, jumped onto a train at Gare de l'Est in Paris just as the doors were closing.

Jean-Luc’s story is just one of the many interconnecting stories that thread together to form the picture of Orphan Europe

At 10am on 21 August 2012
Jean-Luc met with Kurt, Orphan Europe’s manager in Switzerland. Kurt had to organise the necessary customs clearance that the border authorities required before 5pm to gain entry of Carbaglu® into Switzerland that day.

At about 6pm

Kurt handed the package to the hospital pharmacist and the little girl received her first dose. The child is now thriving – she’s one of about 40 patients with NAGS (N-acetylglutamate synthase) deficiency to receive this treatment worldwide.

This was Jean-Luc’s third month working for Orphan Europe. Colleagues described his involvement in the delivery as his “Orphanisation” or initiation into the company. Such special deliveries and personal involvement are not unusual for us – they are all part of the Orphan Europe story.
At Orphan Europe, we see our approach as a joint effort. We work alongside and help bring together networks of stakeholders. We work with interconnecting networks of patients and their families, patient groups, scientific researchers, academics, key opinion leaders, healthcare professionals, policy makers and regulators.
the importance of networks
We have developed our infrastructure to meet the needs of producing, packaging and distributing very small quantities of specialist products to people around the world. Producing, packaging and distributing orphan drugs demands flexibility and cost-effectiveness while maintaining pharmaceutical industry quality standards. All our production stages follow Good Manufacturing Practice (GMP) standards. Products are produced in country-specific packaging.

We believe that patients should have access to treatment as soon as a diagnosis is made, therefore we work closely with the quickest worldwide transport services to ensure prompt and safe delivery. Orphan Europe makes over 15,000 shipments per year to Europe, the Middle East, Africa, Asia and the Americas, and all product boxes are traceable. We also provide emergency deliveries when necessary.

Novel clinical trial methods

Orphan drugs undergo the same review processes as other pharmaceuticals and are required to meet the same high standards of quality, safety and efficacy.

The small numbers of patients affected by a rare disease and the variability of any inherited trait (the so-called high phenotypic heterogeneity) means that randomised clinical trials are not always feasible for an orphan drug. In addition, the use of placebo-controlled trials may be unethical and standardised treatments are often not available for comparison.

We work closely with physicians, centres of expertise, regulators and patient organisations to design tailor-made clinical programmes that define the key evaluation criteria for each drug. We have developed a unique external network of experts who support and cooperate closely with us from a very early stage of development through to market authorisation and post-marketing registries.

Tailor-made production, packaging and distribution

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“Late in the afternoon we had an emergency call that a baby was in a life-threatening condition in a hospital in the Spanish Balaeric Islands. We mobilised our team to find a solution logistically and that solved particular export problems. An emergency shipment was co-ordinated and it arrived before 9am the next day. The child survived and it was so rewarding to receive thanks from the parents. The child is doing well today. We wouldn’t achieve what we achieve if we didn’t have such a strong personal commitment.”

Phillippe, Production Assistant, France

“We were contacted by a physician who had a patient in urgent need of medication for acute intermittent porphyria. I personally transferred the medicine from one hospital to another. I will always remember the face of the patient’s parents waiting for me. That’s what we call ‘taking rare diseases personally’.”

Marc, Marketing Manager, Belgium
The people in our marketing and sales teams have a personal commitment to improving the lives of people who have a rare disease. We recognise that only through working collaboratively with all stakeholders we are able to tackle the challenges of the orphan drug and rare disease field. These challenges include disease awareness, education and training, and the development of consensus around diagnostic and clinical care guidelines.

Our Orphan Drug Specialists (ODS) are highly trained experts working through Europe, Russia, the Middle East and beyond. ODS work closely with patient organisations, healthcare professionals, and regional key opinion leaders, to understand the needs of local markets, develop solutions around improving the diagnosis of rare conditions and access to treatments.

Each of our ODS has experiences of delivering life-saving products in the most extreme and time-sensitive circumstances. When a diagnosis is made, particularly in a newborn baby, treatment can be needed within just a few hours to save a life. ODS are regularly involved in such situations – either personally delivering, or helping to organise the delivery of treatment, often in logistically challenging locations. We call this “doing it the Orphan Europe way”.

Specialist regulatory affairs expertise

Orphan Europe has extensive experience of the specialist regulatory affairs required to bring an orphan drug to market. Building a marketing authorisation dossier based on data from small numbers of patients requires specific know how, and a good working relationship with the regulatory authorities. We have nearly 25 years experience in:

- Orphan drug designations
- Marketing authorisation applications – central applications with the European Medicines Agency (EMA) as well as mutual recognition procedures, US Food and Drug Administration (FDA) procedures including New Drug Applications (NDA), and applications with the Japanese PMDA, Health Canada and other regulatory agencies around the world
- Post-approval safety surveillance
- Named-patient use

Highly qualified & focused market access, marketing and sales teams

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“The focus of the company purely on rare diseases means I have met most of the key clinicians and other workers in this area. I do admire their dedication and concern.”

– Antoine, Country Manager, France

“My role is a strong mixture of education and sales, which I always hope will make a difference to someone, somewhere, even if I never know.”

– Richard, Orphan Drug Specialist, UK

“My job is compelling because I work with all the professionals: physicians, laboratory researchers, pharmacologists, pharmacists, public administrators and patient organisations... All have different needs and I have to know what these are and address them.”

– Roberto, Orphan Drug Specialist, Italy

“Working as an Orphan Drug Specialist means I keep well informed and updated about the pathology of the rare diseases in Orphan Europe’s portfolio. It also means being in close contact with the hospital specialists in my region that are involved in the diagnosis, management and treatment of those diseases.”

– Elvira, Orphan Drug Specialist, Spain

“By sensitive co-operation with key clinicians at the forefront of metabolic disorders, I have been able to go beyond simple sales interviews and become personally involved and concerned about many patients and the issues they face.”

– Richard, Orphan Drug Specialist, UK
Emergency supply delivered to Martinique from Paris on Christmas Day

Orphan Europe has a growing international presence. From our headquarters in Paris, our network of operations spreads through Europe, the Middle East, North Africa, Central and Eastern Europe, Russia, Asia-Pacific and Latin America. Our market activities are also expanding into other regions around the world. Recordati is present in the USA through its company Recordati Rare Diseases.

Key

- Our main territories
- Other territories

25–30 million people in the USA have a rare disease
Source: Food and Drug Administration

Emergency supply delivered to Martinique from Paris on Christmas Day

Over 15,000 shipments to over 60 countries in Europe, the Middle East, North Africa, Russia, Asia and Latin America
Product flown within a few hours of a request to a patient in a sudden coma in Beirut, Lebanon

Emergency deliveries to China and India

The first Asia Pacific Recordati Rare Diseases Foundation course: Tokyo 2014

Support to the Middle East metabolic group

30 million people in the EU have a rare disease

Source: European Medicines Agency

Supporting the European Porphyria Network
We are currently working in the following rare disease areas:

**Acute porphyria:**
- The porphyrias are a group of inherited disorders in which substances called porphyrins and porphyrin precursors accumulate in the body
- There are three types of acute porphyria: acute intermittent porphyria, variegate porphyria and hereditary coproporphyria
- Causes acute, potentially life-threatening, attacks. Non-specific symptoms make diagnosis difficult
- Triggers can include certain medicines, alcohol, hormonal changes, stress, infection or a strict diet
- Largely affects women aged 15–45. Estimated incidence in most European countries is 1 case per 75,000 people
- Orphan Europe supports the European Porphyria Network in developing information for patients and families

**Homocystinuria:**
- An inherited metabolic disorder involving the processing of the amino acid methionine. There are multiple forms of homocystinuria
- Typically develops within the first year of life, although a mild form can develop later
- Orphan Europe collaborates with the European registry and network for homocystinuria and methylation defects (E-HOD)

**Hyperammonaemia due to NAGS deficiency or organic acidaemia:**
- **NAGS deficiency:**
  - NAGS deficiency is one of the urea cycle disorders - inherited conditions in which the body lacks, or is deficient in, the enzymes necessary to remove ammonia (a waste product of protein breakdown) from the blood stream, transform it into urea and eliminate it through urine.
  - Results in hyperammonaemia

- **Organic acidaemias (IVA, MMA or PA):**
  - Genetic disorders caused by a genetic mutation that disrupts amino acid metabolism, causing the build up of harmful products
  - Symptoms include hyperammonaemia and vary from individual to individual, and may present at different ages
  - Orphan Europe supports the European registry and network for intoxication type metabolic disorders (E-IMD) in the development of information for patients and families

**Nephropathic cystinosis:**
- A genetic disorder in which the amino acid cystine accumulates throughout the body, notably in the kidneys and eyes
- Estimated incidence: 1 case per 1–200,000 live births
- Orphan Europe has supported the development of information for patients and families

**Patent ductus arteriosus (PDA):**
- In a developing foetus, the ductus arteriosus (DA) is a shunt connecting the pulmonary artery to the aortic arch. It allows the blood from the right ventricle to bypass the lungs
- In normal newborns, the DA closes within a few hours of birth. In preterm babies the DA can remain open causing blood to flow back from the aorta to the pulmonary artery
- PDA affects the perfusion of the lungs and other organs
- Orphan Europe supports the NeonatologyForParents website

**Severe combined immunodeficiency (SCID) - adenosine deaminase (ADA) deficiency:**
- SCID refers to a group of disorders caused by various genetic defects
- SCID ADA is caused by a deficiency of the ADA enzyme - an enzyme normally present in all tissues and that’s used to break down a toxin involved in regular cell division
- A lack of ADA allows the accumulation of toxins, impacting the production of lymphocytes (both T and B)
- Causes severe and recurrent infections, failure to thrive and metabolic abnormalities
- Can be fatal within the first months if left untreated
- Estimated incidence: 1 case per 1,000,000 births
- Can be detected also by newborn screening programs in certain countries
Vitamin E deficiency in chronic cholestasis:
• Chronic cholestasis is a symptom resulting from various conditions, where the flow or the formation of bile is impaired.
• Bile salts and pigments accumulate in the bloodstream instead of being eliminated from the body.
• Affects the liver and intestine, but can involve all organs.
• Patients with chronic cholestasis cannot absorb fat-soluble vitamins such as vitamin E.

Wilson’s disease:
• A genetic disorder in which copper accumulates, initially within the liver, and then the brain and other tissues.
• Pre-symptomatic disease is followed by neurological symptoms and liver disease. Develops most commonly in teenage years or the 20s but can affect young children.
• Requires life-long treatment and monitoring.

Acute lymphoblastic leukaemia (ALL):
• A cancer that affects the blood and the bone marrow.
• Immature white blood cells (lymphoblasts) multiply in the bone marrow and replace normal blood cells, resulting in anaemia, thrombocytopenia, and neutropenia.
• The most common malignancy in children – causes 30% of all cancers and 80% of leukaemias.
• Peak incidence is at 2–5 years of age.
• Uncommon in adults – about 10,000 new adult cases diagnosed in Europe each year.

Wilms’ tumor (Nephroblastoma):
• An embryonal malignancy of the kidney, the most common renal tumor of childhood.
• Usually presents as an abdominal mass in an otherwise apparently healthy child.
• It occurs most commonly among children younger than 5 years of age, very low incidence for 10-14 and 15-19 year olds population.
• Incidence is 7/1000 000 for children under 15 years.
• It represents 5% from all paediatric cancers.

Childhood rhabdomyosarcoma:
• Rhabdomyosarcoma is the most common soft tissue tumor found in children and adolescents.
• It is a cancer made up of cells that normally develop into skeletal muscles.
• Incidence is 4.5/1000 000 in children under 19 years.
• It accounts for 3% of childhood cancers.

Ewing’s sarcoma:
• It is a malignant small round cell bone tumor with strong metastatic potential.
• It is the second most frequent primary malignant bone cancer found in young people after osteosarcoma.
• The first symptom is usually pain at the tumor site, sometimes along with a mass or swelling.
• It occurs between the ages of 5 and 30 with a peak of incidence between ages 12 and 18. The median age of patients with Ewing’s sarcoma is 15 years.
• The incidence is 3/1000 000 in children under 15 years.

Gestational trophoblastic neoplasia:
• Gestational trophoblastic tumors (GTT) or Gestational trophoblastic neoplasia is a group of tumors that grow from the tissue that forms in the womb during pregnancy.
• Develops from the cells that would normally develop into the placenta during pregnancy.
• GTT include invasive mole, choriocarcinoma, placenta site trophoblastic tumor and epithelioid throphoblastic tumor (very rare).
• Choriocarcinoma is the most aggressive form of GTT and can quickly spread to the lungs, brain, liver, and other organs. The incidence is 1 per 20 000- 40 000 pregnancies.

For more information, please contact us.
...as told by his father, François

About six months after Geoffrey’s birth we realised something was wrong: he was a very quiet baby, and didn’t move or play as you would expect. We had difficulties getting access to a specialist and therefore to a diagnosis; but as soon as we were seen by a paediatric nephrologist at a hospital in Montpellier, Geoffrey was diagnosed with cystinosis (see page 15), and his treatment started.

At the time of diagnosis, we were unable to obtain answers to our questions concerning Geoffrey’s future. I think the doctors were afraid of our reaction, and so they said very little.

“Dad, just once I would like to be someone else and not the kid with a disease.”

Since becoming a teenager, Geoffrey regularly says, “Dad, I would like to be someone else and not the kid with a disease.” It’s so hard to hear your child say those words. For Geoffrey, the problem is not so much the medication or the regular hospital visits, but the fact that he is constantly reminded of his disease. Like most teenagers, Geoffrey wants to be like everyone else and be part of a group. Unfortunately, parents like me are part of the incessant reminder of the condition, by making sure the child or young adult has taken their medication. This can place parents in a state of permanent conflict with their child, which becomes more problematic during the teenage years. It can be very difficult to find the right balance.

At times Geoffrey wants to believe that he has a future, and at other times he can’t see the light, and it’s very difficult to communicate. He thinks that we don’t always tell him the truth, even though Geoffrey often comes with me to congresses (see right). We do our best to persuade Geoffrey that a normal life is possible.

Because of Geoffrey I’ve learnt to enjoy the best moments in life – moments I might not have noticed in the past. I see the importance of not what time we have left, but how we live this time together.

My partnership with Orphan Europe goes back a long way. It has allowed us to share information and our experience of the difficulties we face, and to not feel alone against the disease. As a parent and through my work with the AIRG (see right) we all work together for the future of our children. I think Orphan Europe has the human touch that all pharmaceuticals companies would like to have.
Geoffrey has cystinosis.
To us Geoffrey is not an ordinary patient

From parent to charity board member

I started to work for the AIRG France (Association for Information and Research on Genetic Renal Diseases) about 18 years ago. I became involved to help others but also to help me— it’s given me the will to go on. At the moment, my goal is to bring together the different European cystinosis communities to be more effective in the future.

Through my work with the AIRG, I am constantly learning how to enhance the quality of life of patients and families. One of my roles is to teach families how they can live with the disease but also how they can forget it. We need to prevent what I call a “family explosion”: where the mother and the father don’t speak about cystinosis, the grandparents feel responsible for passing on the disease, the brothers and sisters feel guilty because they don’t have it, and finally the child with cystinosis feels guilty because he or she feels responsible for bringing the focus of the family onto the disease.
At Orphan Europe, we’re committed to improving the diagnosis and management of rare diseases. Fundamental to this is our collaboration with expert disease networks, registries and clinical databases and also our work through the Recordati Rare Diseases Foundation – see pages 20–21.

Orphan Europe has partnered with academia in several expert networks, registers and clinical databases.

Our goal in these collaborations is to help improve and disseminate knowledge of rare diseases and their management internationally, as well as improving and providing information for patients about conditions, treatment options and where to obtain care.
We collaborate or have collaborated on the following projects:

**E-IMD**
www.e-imd.org

Orphan Europe is an associate partner of E-IMD – the European registry and network for intoxication type metabolic diseases. It aims to promote health for people affected with an organic aciduria (OAD) or with a urea cycle defect (UCD). E-IMD has been funded by the European Commission through its Public Health and Consumer Protection Directorate (DG SANCO) PHEA programme.

The network has two major activities:
- To establish a European patient registry
- To provide European evidence-based consensus care protocols

**European Porphyria Network (EPNET)**
www.porphyria-europe.org

The mission of EPNET is to present an up-to-date approach to the understanding of porphyria, focusing on the prevention and treatment of acute attacks, to provide information and support to families affected by porphyria, and to support and encourage medical research. EPNET expert centres share data in a European patient registry.

**E-HOD**
www.e-hod.org

E-HOD is the European registry and network for homocystinurias and methylation defects. E-HOD has been funded by the European Commission through its Public Health and Consumer Protection Directorate (DG SANCO) PHEA programme. The aim of E-HOD is to reduce variation in the diagnosis and care of homocystinuria and methylation defects between countries and allow patients to access expertise and services.

E-HOD has three specific objectives:
- Improving knowledge on homocystinurias and methylation defects through the collection of clinical data into a registry
- Developing diagnosis and clinical care recommendations
- Evaluating newborn screening with recommendations.

Orphan Europe is an associate partner.

**EuroWilson**
www.eurowilson.org

EuroWilson is a network and registry for Wilson’s disease. EuroWilson has partners from over 20 countries and continues to follow an important cohort of Wilson’s disease patients in the European registry. EuroWilson supports European patient organisations, participates in laboratory external quality schemes and supports a network of Wilson’s disease expert centres with annual meetings.

**Urea Cycle Disorders Consortium**
http://rarediseasesnetwork.epi.usf.edu/ucdc/index.htm

The Urea Cycle Disorders Consortium is a team of doctors, nurses, research coordinators, and research labs throughout the USA, working together to improve the lives of people with Urea Cycle Disorders. Orphan Europe works with this consortium to collect post marketing surveillance study data.

**Eunefron**
www.eunefron.org

EUNEFRON was a consortium focusing on orphan nephropathies and which acquired EU funding through Research Framework Programme 7. The project ended in May 2012. One deliverable was to gather data on a rare renal disease, cystinosis, into a European registry. The registry is hosted in France by Cemara. Orphan Europe continues to support this registry.
The Orphan Europe Academy was founded in the year 2000 and gained a reputation for its high-quality, innovative scientific education. On 5th October 2013, the Academy was granted foundation status under French law and is now known as Recordati Rare Diseases Foundation (Fondation d’entreprise).

The Foundation (Fondation d’entreprise) continues the unique mission established by the Orphan Europe Academy. The Foundation promotes the accurate diagnosis and care of patients affected by a rare disease. To achieve this, the Foundation develops and delivers tailored solutions in training and education in rare diseases. These are conditions for which there are few continuing medical education options, and most centres are unable to gain expertise as patient numbers are very small.

The foundation facilitates the organisation of independent training and education in rare diseases, organised by healthcare professionals for healthcare professionals. It does this by:

1. Providing up-to-date training on when to suspect a rare disease, diagnostics, clinical presentation, best care and current treatment strategies in order to improve rare disease recognition and management in the medical setting
2. Sharing experience in the management and outcome of rare diseases where individual experience is limited
3. Improving dialogue between different medical specialities, particularly in multi system-disease
4. Strengthening scientific collaboration.

“The course has provided me with a platform to consolidate my previous experiences and access the relevant literature meaningfully. It has lent familiarity, first hand, with world experts in the field... invaluable. Ultimately, I hope it will help me and others to save children and their families from devastating illness. Thank you so much for your part in that.

Recordati Rare Diseases Foundation participant

For any information, please visit the Recordati Rare Diseases Foundation website: www.RRD-foundation.org
This is not acceptable. We’re striving to improve the diagnosis of rare diseases.
Everyone who works for Orphan Europe is encouraged to take part in our staff volunteering programme – to take time out to work at holiday camps and recreational therapeutic programmes with children who are living with a rare condition and their families. Staff members are given an extra five days holiday and we cover their travel costs.

The staff volunteering programme is just one part of a wider collaboration being co-ordinated by Orphan Europe, Eurordis and the Association of Hole in the Wall Camps to recruit volunteers.

The Association of Hole in the Wall Camps is a growing global network of innovative camps and programmes that provide life-changing experiences to children with serious medical conditions, free of charge. The central focus is to enable children with serious illnesses to regain their independence, to foster optimism and personal growth, and give them the opportunity to celebrate simply being a child and to feel normal. Activities are designed to inspire children to realise their full potential, encourage teamwork and social interaction, and to boost self-esteem and confidence.

Taking part in the staff programme is just one way Orphan Europe staff demonstrate their personal commitment to people living with a rare condition. The impact of the experiences gained is brought back into the daily working lives of our staff and resonates at the heart of our company philosophy and strategy.
“As part of the Orphan Europe staff volunteering programme, in July 2012 I spent ten days volunteering at the Dynamo Camp in Tuscany, Italy. It was an experience that will stay with me.

Dynamo Camp is the first recreational therapy summer camp in Italy specifically for children aged 7 to 16 who have a serious or chronic illness and who are in therapy or convalescing after hospital treatment. The majority of the children have either a rare condition, a haematological cancer, neurological disease, or spina bifida.

After a selection process I was accepted to be a camp volunteer. Volunteers are key to the running of the camp. With three other volunteers and a group leader we were responsible for ten teenagers: nine of whom are independent and one who uses a wheelchair.

The activities included: swimming, archery, climbing, horse riding, circus activities, theatre, camping, dance, painting, helping with the camp radio station, table football and table tennis.

For many of the children the camp is somewhere they can find peace of mind. They don’t feel they have to explain their condition as they are mixing with others who have been through similar experiences.

Each day was full, with a 7am start, running through to midnight! It was physically demanding but this was repaid by what I got back emotionally from the children. The experience made me think about my life priorities and that the concept of happiness is often linked with material things, while for the children it is linked with their health status. Overall I’d recommend the experience to anyone.”
Partnership and collaboration are central to our philosophy. We consider all those who make up the different networks of stakeholders we work alongside as our partners.

Orphan Europe has its own strong portfolio of products, but another important way to bring orphan drugs to market is through our partnerships with other companies.

We have developed successful partnerships with a number of well-established pharmaceutical and biotechnology companies and research institutes. These partnerships have involved co-development, clinical studies, regulatory applications, production, marketing and worldwide distribution.

We would like to hear from you if you are looking for a partner in the development and commercialisation of an orphan drug.
He is 1 of 40 patients globally receiving treatment for his condition.

To us, Juan is not an ordinary patient.
A shared conviction

Every one of our staff members shares a personal conviction that each person with a rare condition has the right to the best possible treatment. We all pledge to pursue treatment options for people with rare disorders, because for us, it’s personal.

We would like to hear from you

Whether you are living with a rare disorder or caring for someone, or if you are a healthcare professional, academic, someone interested in working with us or a company looking to explore partnership possibilities, do get in touch.

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