

Inspired Every Day



CHELSEY  
LIVING WITH NMOSD



THE TRENDY BROTHERS  
LIVING WITH LAL-D



AIRA  
LIVING WITH HPP



JOE  
LIVING WITH PNH



RACHEL WITH HER SON  
LIVING WITH gMG

PEOPLE AFFECTED BY RARE DISEASES AND DEVASTATING CONDITIONS  
ARE OUR INSPIRATION AND OUR GUIDING STAR.

Our mission is to transform  
their lives through the development  
and delivery of innovative medicines,  
as well as through supportive  
technologies and healthcare services.  
We believe it is our responsibility  
to listen to, understand,  
and change the lives of patients  
and those who work tirelessly  
to help them.

# Delivering On Our Mission Every Day

Alexion is focused on serving patients and families affected by rare diseases and devastating conditions through the discovery, development and commercialization of life-changing medicines.

When it comes to rare disease, missed or delayed diagnoses are common. Even once properly diagnosed, patients and their families may be left looking for answers. Alexion's proven approach to serving patients with rare diseases focuses on disease awareness, diagnostic initiatives, and patient support.

Our innovation begins with understanding people living with rare diseases, which fuels all of our efforts, beginning with our own medicine discovery efforts, as well as collaboration with external partners. We seek opportunities to better collaborate with everyone involved in the patient journey, including those who provide care and access.

As part of our commitment to the patient communities we serve, Alexion offers patient support programs worldwide. These programs provide education, assistance with access, and treatment support for patients and their caregivers.

We are committed to enhancing patient access to our innovative medicines, and are working with private healthcare organizations, policymakers and governments around the world to develop long-term, sustainable access solutions.

Alexion also partners with patient advocacy organizations and actively listens to patient communities in order to better understand and deliver the support they need.

At Alexion, we believe each of us is accountable to deliver on our commitments to patients, caregivers and families affected by rare diseases and devastating conditions.



TANNER  
LIVING WITH HPP

**50%**  
OF PEOPLE LIVING  
WITH RARE DISEASES  
ARE CHILDREN



TRISTAN  
LIVING WITH LAL-D

**7,000+**  
KNOWN RARE  
DISEASES IN THE WORLD  
BUT LESS THAN

**5%**  
HAVE APPROVED  
TREATMENT  
OPTIONS



**4**  
PRIX GALIEN  
AWARDS



SCIENTISTS AT OUR  
R&D CENTER OF EXCELLENCE

**3,800+**  
TALENTED COLLEAGUES



DONNAN  
LIVING WITH aHUS

SERVING  
PATIENTS IN  
**50+**  
COUNTRIES

“

I feel like tomorrow I can do something, whereas before, I didn't feel like tomorrow was possible.”

**JESSE | LIVING WITH gMG** Growing up, Jesse was very healthy, active and athletic with dreams of becoming an EMT and a firefighter. In his early 30s, he decided to start training to pursue these dreams more seriously. When he started feeling weaker at the gym and experienced double vision and a droopy eyelid, he knew something was wrong. His symptoms continued to worsen – his legs got weaker, he could no longer drive and wasn't able to carry out many of his usual activities of daily living without assistance. He met with a neurologist who diagnosed him with generalized myasthenia gravis (gMG), a debilitating, chronic, and progressive autoimmune neuromuscular disease. Unfortunately, Jesse subsequently experienced a myasthenic crisis so severe that he almost did not survive. He spent a month in the hospital rehabilitating and years being treated with a variety of medications to try and stabilize his gMG. When a treatment option was approved for gMG in 2017, Jesse's neurologist recommended he begin therapy. Jesse remains on treatment today and feels as if he's able to do more of the things he once thought he would have to give up forever.

**10-15% OF gMG PATIENTS FAIL TO RESPOND ADEQUATELY TO, OR CANNOT TOLERATE, MULTIPLE THERAPIES FOR gMG AND CONTINUE TO SUFFER PROFOUND MUSCLE WEAKNESS**



# Building A Better Tomorrow Every Day

Alexion's five highly innovative therapies are approved for the treatment of people living with seven rare diseases and devastating conditions. With the development of our first therapy, Alexion emerged as the global leader in complement science, and now has 30 years of leadership in rare disease.

We developed and deliver medicines for the treatment of complement-mediated diseases – two for people living with paroxysmal nocturnal hemoglobinuria (PNH) and atypical hemolytic uremic syndrome (aHUS), and one for people living with generalized myasthenia gravis (gMG) and neuromyelitis optica spectrum disorder (NMOSD).

Alexion also has two highly innovative enzyme replacement therapies for people living with life-threatening and ultra-rare metabolic disorders – hypophosphatasia (HPP) and lysosomal acid lipase deficiency (LAL-D).

In addition, Alexion's portfolio includes a prescription medicine to treat neurofibromatosis type 1 (NF1) plexiform neurofibromas (PN).

We continue to deepen our understanding of rare disease, which began with our pioneering work in complement biology. This knowledge allows us to innovate and evolve into new areas, where there is great unmet need and opportunity to help patients and families fully live their best lives.

## COMMITMENT TO QUALITY

- Delivering safe and effective medicines that meet or exceed the requirements of our patients and our customers
- Complying with all applicable regulatory requirements
- Operating a Quality Management System and improving our systems and processes
- Ensuring the integrity of our data
- Upholding our individual and collective accountability for quality
- We have a dedicated Global Product Security Team that provides a clear line of sight, communication, accountability and subject matter expertise in relation to product security



BUILDING FRANCHISES  
IN  
HAEMATOLOGY, NEPHROLOGY,  
NEUROLOGY, METABOLICS, BONE METABOLISM  
CARDIOLOGY, OPHTHALMOLOGY




ADVANCING CLINICAL  
STAGE THERAPIES THROUGH  
COLLABORATIONS WITH:



neurimmune





“ I don’t define myself as having a rare disease. It’s part of my life but it’s certainly not who I am. ”

**CHELSEY | LIVING WITH NMOSD** In 2009, Chelsey woke up with severe eye pain and noticed that her eyesight had been significantly impacted. Prior to this, Chelsey had spent nearly a year visiting different specialists to identify the cause of the symptoms she had been experiencing: vomiting, numbness, tingling in her limbs, headaches, and disabling fatigue. After being admitted to the hospital for the eye pain, Chelsey was diagnosed with multiple sclerosis (MS). During the ten years that followed, Chelsey continued to experience those symptoms and new ones, along with 10 episodes of optic neuritis. She began to wonder if she truly had MS or if there had been a mistake. In early 2019, after spending a week in the hospital, Chelsey woke up with eye pain that was unmistakable. Her neurologist began to explore neuromyelitis optica spectrum disorder (NMOSD), and formally diagnosed her after a series of tests and scans.

**FOR PEOPLE LIVING WITH NMOSD, EACH INDIVIDUAL ATTACK RESULTS IN CUMULATIVE DISABILITY INCLUDING BLINDNESS AND PARALYSIS, AND SOMETIMES PREMATURE DEATH.**

“

PNH is a disease that requires medical attention and needs to be taken seriously, but it's also important to me to remain positive. It's a scary journey and there are unknowns. But from my perspective, I still have to live my life.

”

**JASON | LIVING WITH PNH** In the winter of 2018, Jason began experiencing shortness of breath. He visited both a lung doctor and a heart doctor but neither could find anything wrong. His heart doctor scheduled him for a CT scan, but when the nurse put the IV into his arm, his vein collapsed, he passed out and his heart rate dropped. He was rushed to urgent care and spent three days in the hospital. Still, the doctors could not determine what was wrong. After being released from the hospital, Jason was referred to a hematologist who ran several tests and eventually diagnosed Jason with paroxysmal nocturnal hemoglobinuria (PNH), an ultra-rare blood disorder in which uncontrolled activation of complement, a component of the normal immune system, leads to chronic hemolysis (destruction of red blood cells). Upon his diagnosis, Jason discussed treatment options with his doctor and learned that a therapy was expected to receive FDA approval in the coming months. He worked with his doctor to manage his PNH in the near-term and began treatment when the therapy finally became available. Jason remains on treatment today and is active within the PNH patient community.

**BEFORE CURRENT TREATMENTS, 1 IN 3 PATIENTS WITH PNH DIED WITHIN 5 YEARS OF DIAGNOSIS**



# We Strengthen Our Impact Every Day

We invest in and value people who believe in the importance of our purpose and understand what it takes to deliver on it. In everything we do, we are empowered and committed to speak up and perform at our personal best to accelerate our collective impact for people living with rare diseases and devastating conditions.

There is an inherent connection between the experience of our employees and the experience of the patients we serve. With a focus on developing world-class leadership at every level of the company, Alexion is able to deliver world-class innovation to patients and their caregivers while creating meaningful and fulfilling work for its employees.

Our culture is rooted integrity, inclusiveness, and our dedication to joining and supporting the communities in which we live and work. It guides our success, allowing us to better serve patients, deliver value to our stakeholders, and make Alexion the most rewarding company to work for.



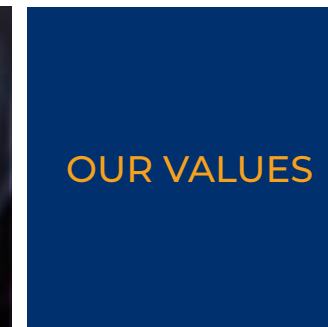
**TO TRANSFORM THE PATIENT EXPERIENCE WE MUST GO BEYOND DELIVERING A MEDICINE. WE MUST ACTIVELY LISTEN TO PATIENTS, THEIR CAREGIVERS AND THEIR HEALTHCARE PROVIDERS TO DELIVER SERVICES AND SUPPORT TO ADDRESS THEIR NEEDS.**



**We Put  
Patients First**



**We are  
Entrepreneurial**



**OUR VALUES**



**We Do the  
Right Thing**



**We Play  
to Win**



**We Follow  
the Science**



“

**I remember the day I was diagnosed. It was relieving because not only was there a diagnosis but there was an answer. That was the most relieving part about it.**

”

**JULIA | LIVING WITH aHUS** Julia's symptoms began when she was in eighth grade. She became tired and nauseous, had trouble focusing, and developed a piercing pain in her abdomen. Eventually, her parents brought her to the emergency room where she was treated for a suspected bacterial infection. Over the next year and a half, Julia was admitted to the hospital several more times, saw countless physicians and underwent many tests and medical procedures, including a bone marrow biopsy, renal biopsy and multiple platelet and blood infusions. Ultimately, Julia was diagnosed with atypical hemolytic uremic syndrome (aHUS), an ultra-rare, genetic, chronic and life-threatening disease that progressively damages vital organs and can lead to stroke, heart attack, kidney failure, and death. Once confirming this diagnosis, Julia's doctors were able to begin to manage her aHUS. Julia is now in college and studying medicine, with hopes of going to law school.

**2 IN 3 aHUS PATIENTS WITH THE MOST COMMON MUTATION REQUIRED KIDNEY DIALYSIS, HAD PERMANENT KIDNEY DAMAGE, OR DIED WITHIN THE FIRST YEAR AFTER DIAGNOSIS**



“

**I just feel proud. He's fought for himself, he's been strong.**”

– CHARLOTTE, ALBIE'S MOM

**ALBIE | LIVING WITH LAL-D** When Albie was born, he was seemingly okay. However, two weeks after birth he started to have intestinal problems. His doctor thought he might be lactose intolerant or have reflux. He continued getting sicker, developed a large abdomen and groin, and wasn't gaining significant weight. At two months of age he had only gained one pound since birth. After numerous tests, physicians and hospitals, it was confirmed that Albie had a genetic and progressive ultra-rare metabolic disease called lysosomal acid lipase deficiency (LAL-D). At the time there were no treatments for LAL-D, but there was a clinical trial for an enzyme replacement therapy. After several weeks of treatment, Albie gradually began to put on weight and improve. He continues to receive the treatment today.

**WITHOUT TREATMENT, THE MEDIAN AGE OF DEATH IS 3.7 MONTHS IN LAL-D PATIENTS WHO EXPERIENCE SYMPTOMS IN INFANCY**



“

When you have a child with a disability, you just have to do what you can to give them as much quality of life as you can.

– KATE, MORGAN'S MOM

”

**MORGAN | LIVING WITH HPP** Kate was four months pregnant when she went to the doctor for a routine ultrasound and received devastating news. A test of her amniotic fluid determined that her baby girl, Morgan, had hypophosphatasia (HPP), an inherited, progressive, ultra-rare metabolic disease that is characterized by low alkaline phosphatase activity and defective bone mineralization that can lead to destruction and deformity of bones and other skeletal abnormalities. HPP can also lead to systemic complications such as muscle weakness and respiratory failure leading to premature death in infants. Morgan was born with short limbs, bilateral cleft feet and femurs that were curved like old-style telephone receivers. At the time, there were no approved therapies for the treatment of HPP. She spent the first few years of her life in a series of casts and went through intense physical therapy. Learning to walk was a struggle and she lost her teeth almost as soon as they grew in. When Morgan was four, she was accepted into a clinical trial for an enzyme replacement therapy, giving the family hope for a normal life. Within weeks of starting the drug, many of Morgan's symptoms began to improve.

**WITHOUT TREATMENT, 73% OF INFANTS WITH HPP SYMPTOM ONSET IN THE FIRST 6 MONTHS WILL NOT SURVIVE BEYOND 3.5 YEARS**



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