



SOL, ARGENTINA
POMPE DISEASE

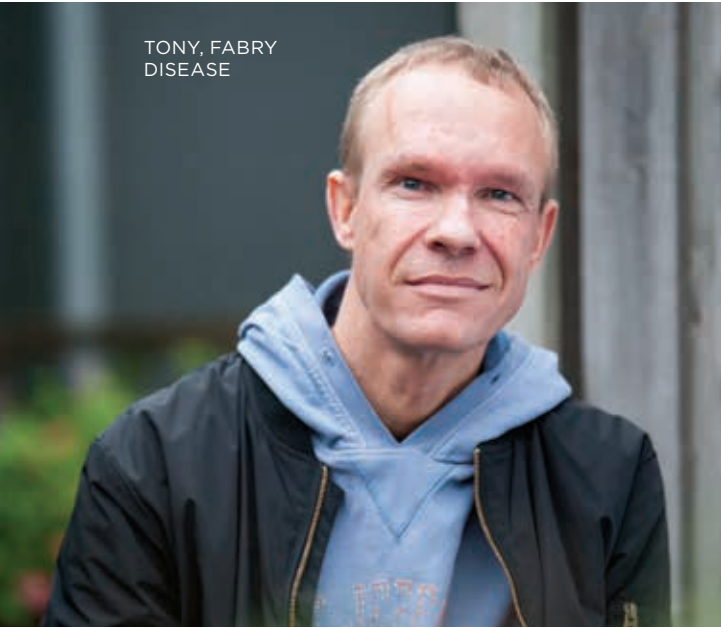
This is Genzyme

genzyme
A SANOFI COMPANY



MARIA, GAUCHER DISEASE

TONY, FABRY DISEASE



MICHELLE, MULTIPLE SCLEROSIS



BRANT, THYROID CANCER



JIMENA, MPS I

Genzyme has pioneered the development and delivery of transformative therapies for patients affected by rare and debilitating diseases for over 30 years.

With a focus on rare diseases and multiple sclerosis, we are dedicated to making a positive impact on the lives of the patients and families we serve. As a Sanofi company, Genzyme benefits from the reach and resources of one of the world's largest pharmaceutical companies, with a shared commitment to improving the lives of patients.

GENZYME IN THE UNITED KINGDOM AND IRELAND

Genzyme is a significant force in the biotechnology sector in the UK and Ireland focused on improving the lives of patients with rare genetic diseases and multiple sclerosis.

First established in the UK in 1981, Genzyme employs almost 100 people working out of our commercial base in Oxford, marketing products for the treatment of patients with chronic debilitating diseases. These include genetic diseases such as lysosomal storage disorders (LSDs), characterised by a lack of enzymes essential to healthy biological processes, and multiple sclerosis, a complex disease affecting the central nervous system. Our portfolio also includes a therapy which aids in the management of thyroid cancer.

Genzyme has operated a biotechnology campus in Waterford, Ireland since 2001. With 550 people working at a €500m-plus facility, Genzyme products are supplied from Waterford to patients in more than 70 countries worldwide.

Additional Genzyme and Sanofi new product introductions are planned at the Waterford site which continues to expand its mandate across multiple platforms. Genzyme Waterford has been recognised as one of the country's best workplaces by Great Place to Work Ireland.

A legacy of LEADERSHIP.

Genzyme
was first to...

- Successfully bring an enzyme replacement therapy into clinical use
- Develop a treatment for type 1 Gaucher disease
- Develop a treatment for MPS I
- Develop a treatment for Pompe disease
- Develop an adjuvant therapy for thyroid cancer
- Register a treatment for Fabry disease in the U.S.
- Launch two NICE approved, reimbursed treatments for relapsing remitting multiple sclerosis (RRMS), in a new therapy area, in the same year

We accomplish our goals through world-class research, collaboration with the global patient community, and with the compassion and commitment of our employees. Our research and development is focused on delivering breakthrough therapies for patients who might otherwise have few or no treatment options.

We continue to invest in a global pipeline of innovative therapies...

- ▶ Investigational oral compounds for Gaucher disease and Fabry disease
- ▶ A potential treatment for familial amyloid polyneuropathy, co-developed with Alnylam
- ▶ A potential enzyme replacement therapy for Niemann-Pick disease type B
- ▶ A second generation enzyme replacement therapy for Pompe disease
- ▶ Research in Parkinson's disease and age-related macular degeneration

RARE DISEASES

Genzyme has long been a leader in the development of targeted therapies for rare diseases. We put patients at the heart of what we do. By combining this patient focus with our deep knowledge of disease biology, our groundbreaking science, and our relationships with medical specialists, we distinguish Genzyme from new entries to the rare disease field.

THERAPIES FOR RARE DISEASES IN THE UK AND IRELAND

GENETIC DISEASES

Type 1 Gaucher disease, Fabry disease, Pompe disease, MPS I disease

ENDOCRINOLOGY

Facilitating thyroid cancer treatment and testing

.....
EVA AND HER SON,
ALBERTO
FABRY DISEASE



INNOVATIVE solutions for rare diseases

Lysosomal Storage Disorders (LSDs), a group of rare conditions caused by enzyme deficiencies, are a cornerstone of Genzyme's business, and the medical area for which we are most well-known. In 1984, we pioneered the first enzyme replacement therapy to treat Gaucher disease, which has transformed the lives of patients.

Our breakthrough work in genetic engineering and recombinant protein manufacturing has made possible the large-scale production of enzyme replacement therapies for several previously untreatable LSDs, including Gaucher disease, Fabry disease and Pompe disease.

Almost all of our enzyme replacement therapies were the first — and in some cases are still the only — treatments available to patients. We remain committed to finding new life-changing therapeutic solutions for patients who may have little or no other alternatives.

We continue to innovate. We have completed our phase 3 trials for an oral therapy for patients with type 1 Gaucher disease, which is approved in the United States, has received marketing authorisation in Europe and has been filed for approval in other countries. We also contribute our scientific expertise in the area of diagnostic testing for LSDs.

We are applying our genetic expertise, built through our work on LSDs, to other rare and personalised medicine areas, including endocrinology, often partnering to bring new solutions to our patients.

Our recombinant form of thyroid stimulating hormone (TSH) helps thyroid cancer patients avoid hypothyroidism, while allowing ablation of thyroid remnants. It also enhances reliable diagnostic testing for the recurrence of thyroid cancer.

MULTIPLE SCLEROSIS

We are committed to becoming a long-term partner to the multiple sclerosis (MS) community by expanding on our heritage of revolutionising treatment for patients with rare and debilitating diseases. We are working to deliver scientific advancements that will make a positive difference in the lives of patients with MS and look forward to continuing to enhance our portfolio and serving patients' needs.

THEAPIES FOR RELAPSING MS IN THE UK AND IRELAND

Oral immunomodulator that inhibits the proliferation of stimulated B & T lymphocytes

Infused monoclonal antibody that selectively targets the cell surface protein CD-52

.....
TIGER,
MULTIPLE SCLEROSIS



STRIVING to address unmet needs in MS

For over a decade, Genzyme and Sanofi have each been working to develop novel treatments for MS, a debilitating, chronic disease in which the immune system attacks the central nervous system, causing symptoms ranging from numbness in the limbs or forgetfulness to paralysis or loss of vision. As part of the process of merging the two companies, we have integrated these programmes within Genzyme to position ourselves as a leader in MS. We are approaching the MS community with the commitment, engagement, transparency and focus on patients that define Genzyme. We recognise that an individualised approach to living well with MS involves developing relationships with patients through patient organisations and foundations and care partners, and health care providers.

We collaborate with physicians in research and healthcare improvement while we continue to push the boundaries of MS research in our

own laboratories. We further support MS nurse specialists and patient groups in their work to foster close connections with patients and their families, to best understand their needs and to develop and improve healthcare delivery.

Our MS leadership comprises experienced professionals with a strong foundation in MS. We have an exciting opportunity to draw on the diverse experiences of our MS team to forge a unique identity for Genzyme within the MS community.

Our MS therapeutic portfolio includes a once-daily, oral therapy for relapsing remitting multiple sclerosis (RRMS) and a novel infusion therapy for RRMS that is given as two annual treatment courses. Both therapies are available on the NHS in England, Scotland, Wales, Northern Ireland, and in the Republic of Ireland.

*Prix Galien
Highly Commended 2014*

MONOCLONAL ANTIBODIES: BREAKTHROUGH DISCOVERY BY UK SCIENTISTS IN THE 70S

The science of monoclonal antibodies goes back to the seventies when two scientists, César Milstein and George Köhler, from Cambridge's MRC Laboratory of Molecular Biology invented the technology for making large quantities of an antibody targeted at one specificity, so-called monoclonal antibodies; for which they subsequently received the Nobel Prize for Physiology or Medicine in 1984. Building on this research, Professor Herman Waldmann and colleagues

at the University of Cambridge produced the first monoclonal antibody for potential use as a medicine, Campath-1H (Cambridge Pathology 1st Human). In 1990, Professors Waldmann and Alastair Compston began discussions over the use of Campath-1H in multiple sclerosis. Subsequently renamed alemtuzumab, it was first used in 1991 at the University of Cambridge for treating patients with secondary progressive multiple sclerosis.

SCIENCE is at the core of what we do.

GLOBAL RESEARCH AREAS:

- ▶ Rare Genetic Diseases
- ▶ Neurologic Diseases
- ▶ Cardiovascular Diseases
- ▶ Endocrinology
- ▶ Immunology

Research at Genzyme is a core part of our commitment to improve the lives of patients and their carers and is concentrated in medical areas where new therapies can have a significant impact, including our primary areas of focus — rare diseases, immunologic and neurologic disorders.

Inspired by the potential to improve patients' lives, Genzyme's scientists work at the forefront of healthcare research and technology. Our scientists are part of a broader Sanofi research organisation located out of Boston, U.S. Beyond our own labs, we collaborate with investigators at universities, research institutions, private companies, and government organisations to discover and develop treatments for patients in need around the world.

Genzyme's groundbreaking research in enzyme replacement therapies for lysosomal storage disorders (LSDs) was the foundation of our business. Today, we are still widely recognised as the global leader in LSD research and have expanded our efforts. We are developing a therapy to treat Niemann-Pick disease type B, a new therapy for Pompe disease, and exploring targets for cystic fibrosis and myotonic dystrophy. We are applying our expertise to develop new forms of treatment delivery that are more convenient for patients than intravenous enzyme infusions — such as our oral therapy in preclinical development for Fabry disease and our oral

therapy for type 1 Gaucher disease, which has completed phase 3 trials.

Continued research in the field of multiple sclerosis (MS) seeks to develop well tolerated and effective treatments that are accessible to everyone living with MS. A key focus for our scientists is to develop a treatment that is aimed at repairing the myelin sheath.

We have a robust research effort in the area of immunologic and neurologic diseases, expanding our research into neuropathic LSDs, lupus, spinal muscular atrophy and Huntington's disease. We also have one of the industry's longest standing and largest research programmes in the field of gene therapy, including a research collaboration with the University of Florida and University of Pennsylvania to develop a gene therapy for the treatment of a rare genetic disease that causes childhood blindness. We are exploring targets in several genetic diseases and have clinical trials to advance treatments in Parkinson's disease and age-related macular degeneration.

Ensuring Access

We continue to expand our manufacturing capacity and we remain committed to delivering quality product to patients. From 2012-2015, approximately £620m (\$1B) will be invested in our global manufacturing operations.



Our new Framingham, Massachusetts, U.S. facility (above) joins our robust manufacturing network which includes Geel, Belgium, Waterford, Ireland (left) and Allston, Massachusetts, U.S.

Genzyme has locations in more than 40 countries and our products are available in nearly 100 countries.



RESPONSIBILITY Our dedication to patients extends beyond the development and manufacture of products. We have initiated numerous free-drug, payment-assistance, and humanitarian programmes to help ensure that treatment reaches all those who need it, regardless of their ability to pay. We are also committed to responsible corporate citizenship: we lead the industry in environmental initiatives and support local communities through grants and volunteering initiatives.

CHARITABLE PROGRAMMES

We believe that developing lifesaving therapies carries with it the responsibility to increase access to these therapies for patients around the world through free-drug programmes, humanitarian initiatives, education and advocacy. Genzyme sponsors a range of programmes to help ensure that patients have access to the treatments they need. These programmes include the International Charitable Access Programme (ICAP) which provides, to the best of our ability, our four enzyme replacement therapies, focused on specific regions, such as Eastern Europe, India, and China.

COMMUNITY RELATIONS

We partner with schools, nonprofit agencies and grassroots organisations in our communities. Through a combination of grants, employee involvement and in-kind contributions, we build sustainable relationships that have a lasting positive impact.

We develop and fund innovative science education programmes for young people, and we also fund community-based, nonprofit organisations dedicated to health-related issues.

ENVIRONMENTAL INITIATIVES

We are among corporate leaders in environmental management and performance.

As part of the Sanofi Group commitment to promoting a sustainable future we are reducing our CO2 emissions in business travel, company car use and general energy consumption. There are also separate initiatives to increase recycling and dramatically reduce water consumption across our manufacturing facilities.

We will continue to explore new ways to improve our environmental performance.

Patients are people like us. We all laugh and cry. We hope and dream. We have family and friends who love us. We want to lead normal lives. This is why we do what we do.

ONE PATIENT'S JOURNEY

Brian Berman
Gaucher disease

“My mother played an important role in the early development of Gaucher disease treatment and she was just tremendous, and an amazing role model for me. I know the doctors told her that I was going to die but her perseverance, dedication and ability to work closely with Genzyme and search around the world to develop a treatment was amazing. There was tremendous excitement being part of the development of a new medicine.

Today, my health is very good. I am a father of small children. I lead a very normal, very active, healthy life. My thoughts and wishes for the future concerning rare diseases are that there's hope. Today, I would say to other people living with a rare disease, don't give up. There will be new treatments for rare diseases which don't currently have any treatments.

I believe people like us with rare diseases can live normal lives.”



1983



1991



2001



2012



DAVID MEEKER, MD, PRESIDENT AND CEO

We talk a lot about the fact that we are a company with a purpose: our extraordinary commitment to understanding and meeting the needs of patients and their families. It is that sense of purpose that will allow us to be successful as we look toward our future.

Our mission is to discover and deliver transformative therapies for patients with rare and special unmet medical needs, providing hope where there was none before.

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