This is Genzyme
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.
A legacy of LEADERSHIP.

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.

Genzyme was first to...

- Successfully bring an enzyme replacement therapy into clinical use
- Develop a treatment for Gaucher disease type 1
- Develop a treatment for Fabry disease
- Develop a treatment for MPS I
- Develop a treatment for Pompe disease
- Develop an adjuvant therapy for thyroid cancer

Our innovation continues with...

- Two promising late-stage investigational compounds for multiple sclerosis
- An investigational compound for familial hypercholesterolemia
- Investigational oral compounds for Gaucher and Fabry diseases
- An investigational enzyme replacement for Niemann-Pick disease type B
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.

GENETIC DISEASES
- Gaucher disease type 1
- Fabry disease
- Pompe disease
- MPS I disease

ENDOCRINOLOGY
- Facilitating thyroid cancer treatment and testing

CARDIOVASCULAR
- Late-stage treatment for familial hypercholesterolemia (FH)

INNOVATIVE solutions for rare diseases

Lyosomal 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 transformed the lives of patients and established the standard for Gaucher disease treatment.

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 treatments for Gaucher disease, Fabry disease, and Pompe disease.

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 no other alternatives.

We have applied the genetic expertise we built through our work on LSDs to other rare and personalized medicine areas, including cardiovascular and endocrinology, often partnering to bring the best solutions to our patients.

An established leader in endocrinology, our recombinant form of thyroid stimulating hormone (TSH) helps thyroid cancer patients avoid hypothyroidism while still allowing their physician to successfully ablate the thyroid remnant as well as obtain reliable diagnostic test results for the recurrence of thyroid cancer. Today, our partnership with Veracyte allows us to further this commitment by improving diagnosis of thyroid nodules, and potentially reducing the number of unnecessary thyroidectomies.

In partnership with Isis Pharmaceuticals, we are completing development of a lipid lowering drug candidate, mipomersen, as a potential treatment for familial hypercholesterolemia (FH). FH is a genetic, severe form of high cholesterol which often does not respond to existing treatments. FH can lead to early cardiovascular complications, potential need for apheresis (lipid dialysis) and can be life-threatening at a young age.

SANDRA
FABRY DISEASE

Sandra is passionate about the health and care of her two children who, like her, also have Fabry disease. They are an active family that enjoys playing and supporting local sports teams and spending time with their large extended family.

We continue to innovate. We are completing our phase 3 trials for an investigational oral therapy for patients with Gaucher disease type 1, eliglustat tartrate. We also contribute our scientific expertise in the area of newborn screening for LSDs.
MULTIPLE SCLEROSIS

We are committed to becoming a long-term partner to the multiple sclerosis (MS) community by expanding on our heritage of revolutionizing treatment for patients with rare diseases. We are working to deliver scientific advancements that will have a significant impact on the unmet needs of people living with MS. Our late-stage clinical pipeline has promise for MS patients.

INVESTIGATIONAL THERAPIES FOR RELAPSING MS

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

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

STRIVING to address unmet needs in MS

For over a decade, Genzyme and Sanofi have each been working to develop novel treatments for MS. We have integrated these programs within Genzyme to establish 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 recognize that an individualized approach to living well with MS involves developing relationships with patients and care partners, health care providers, and disease organizations and foundations.

We offer physicians the highest level of expertise and will continue to support and collaborate with them on research and to push the boundaries of MS research in our own labs. We aim to foster close connections with patients and their families to best understand their needs and to deliver services that have a positive impact.

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 clinical pipeline includes both teriflunomide and alemtuzumab:

Alemtuzumab clinical studies researched a unique dosing regimen for relapsing MS. Alemtuzumab showed promise in head to head clinical studies against an approved MS therapy and could make a very important contribution to the treatment landscape. Alemtuzumab has completed phase 3 trials.

Teriflunomide is being developed as a once-daily oral treatment for relapsing forms of MS. The teriflunomide clinical development program is one of the largest and broadest of any MS therapy currently in development. Teriflunomide is currently under review by regulatory agencies.

Alemtuzumab and teriflunomide may hold promise for people living with MS.

JANNAN, ALEMTUZUMAB IN MS CLINICAL TRIAL
Jannan enjoys taking care of her young daughter and exploring new hobbies such as photography. She was diagnosed with MS in her early twenties and participated in a clinical trial for alemtuzumab.

MICHELLE, TERIFLUNOMIDE IN MS CLINICAL TRIAL
Michelle was diagnosed with MS in her late twenties. She is part of a clinical trial for teriflunomide, an investigational once-daily oral immunomodulatory, disease-modifying therapy for relapsing MS.
Inspired by the potential to improve patients’ lives, Genzyme’s scientists work at the forefront of health care research and technology. Our scientists are part of a broader Sanofi research organization known as the Boston R&D Hub. Beyond our own labs, we collaborate with investigators at universities, research institutions, private companies, and government organizations, 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 recognized 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 more potent 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 phase 3 oral therapy trials for eliglustat tartrate for Gaucher disease type 1.

Genzyme has been consistently ranked among the top places to work for scientists. Our research is concentrated in medical areas where new therapies can have a significant impact, including our primary areas of focus — rare diseases and neuroimmunological disorders such as multiple sclerosis.

RESEARCH AREAS:
- Rare Genetic Diseases
- Neurologic Diseases
- Cardiovascular Diseases
- Endocrinology
- Immunology

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We have a robust research effort in the area of immunologic and neurologic diseases, expanding our research into neuropathic LSDs, lupus, and Huntington’s disease. We also have one of the industry’s longest standing and largest research programs in the field of gene therapy. We are exploring targets in several genetic diseases and have clinical trials underway 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 $1B will be invested in our global manufacturing operations.

We believe that developing life-saving therapies carries with it the responsibility to increase access to these therapies for patients around the world through free-drug programs, humanitarian initiatives, education and advocacy. Genzyme sponsors a range of programs to help ensure that patients have access to the treatments they need including the International Charitable Access Program (ICAP) which provides our four enzyme replacement therapies at no cost to eligible patients who live outside of the United States. Donations are subject to product availability and medical criteria.

RESPONSIBILITY

Our dedication to patients extends beyond the development of products. We have initiated numerous free-drug, payment-assistance, and humanitarian programs to 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 PROGRAMS

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COMMUNITY RELATIONS

We partner with public schools, nonprofit agencies and grassroots organizations 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 programs for young people, and we also fund community-based, nonprofit organizations dedicated to health-related issues.

ENVIRONMENTAL INITIATIVES

We are among the world’s corporate leaders in environmental management and performance. Ten of our facilities have received certification from the U.S. Green Building Council’s Leadership in Energy and Environmental Design (LEED®) Green Building Rating System.

In addition to facility design, Genzyme oversees global initiatives to reduce the company’s impact on the environment. In 2007, we implemented an aggressive goal to reduce our greenhouse gas emissions by 25% per dollar of revenue by the end of 2012. We met our goal two years ahead of schedule and we continue to explore new ways to improve our environmental performance.

Genzyme has locations in more than 40 countries and the company’s products are available in nearly 100 countries.

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


We talk a lot about the fact that we’re 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.