

## ADDRESSING THE NEEDS OF RARE DISEASE PATIENTS AROUND THE WORLD

### GRI Standards :

N/A

### EXECUTIVE SUMMARY

Our global Specialty Care business unit, Sanofi Genzyme, has been a pioneer in rare diseases for more than 35 years by developing enzyme replacement therapies for the treatment of lysosomal storage disorders. Sanofi Genzyme has recently broadened its focus to include treatments for people with rare blood disorders such as hemophilia.

Sanofi Genzyme also has a responsibility to help patients access these unique therapies, regardless of their location and financial situation, by working closely with national health services, government agencies and private insurers, as well as patient associations, to build sustainable health systems and make a difference in patients' lives.

# TABLE OF CONTENTS

- 1. BACKGROUND .....3
- 2. BRIDGING THE GAP TO ACCESS.....4
- 3. HUMANITARIAN PARTNERSHIPS .....4
- 4. EDUCATION AND OUTREACH.....5

## 1. BACKGROUND

---

Our global specialty care business unit, Sanofi Genzyme, has been a pioneer in rare diseases for more than 35 years by developing enzyme replacement therapies for the treatment of lysosomal storage disorders. Sanofi Genzyme has recently broadened its focus to include treatments for people with rare blood disorders such as hemophilia. Sanofi Genzyme has also launched a number of managed access programs, often referred to as compassionate use programs and named patient programs, also in rare blood disorders like acquired thrombotic thrombocytopenic purpura and cold agglutinin disease. We continue to focus on being transformative in rare diseases and rare blood disorders: developing therapies, supporting research and offering innovative solutions that have the potential to offer meaningful change to those impacted by these conditions. Our commitment to provide patients, including those in emerging markets, with healthcare solutions, goes beyond products to offering services and support that complement our therapies.

Sanofi Genzyme's sense of responsibility is grounded in our commitment to patients. We were founded to address the needs of patients with rare diseases who previously had no available treatment options, and we remain committed to advancing the understanding of these diseases and developing new therapies for unmet medical needs. Our patient focus is about much more than developing and delivering products, though. We also take responsibility for helping patients obtain access to our unique therapies, regardless of their location, financial circumstances or other obstacles. We establish patient assistance and humanitarian programs, help build sustainable healthcare systems, partner with advocacy groups on disease awareness and patient support and provide a broad range of services.

Sanofi Genzyme's commitment to helping improve access to healthcare is multi-faceted, focusing on working with national health services, government agencies and private insurers, as well as patient organizations, to establish or improve patient access to, and coverage for, our products.

The current rare disease portfolio covers :

- **Lysosomal storage disorders** : Gaucher disease, Fabry disease, Pompe disease, Niemann-Pick disease (Acid sphingomyelinase deficient disease) and MPS I and MPS II disease.
- **Rare Blood Disorders** : Hemophilia, acquired thrombotic thrombocytopenic purpura and cold agglutinin disease.
- **Oncology** : Medullary thyroid cancer.

Sanofi Genzyme works closely with governments to facilitate approval of our treatments. In countries with established healthcare systems, we work with public and private insurers to secure coverage and reimbursement. In developing countries and emerging markets, we lend our experience to patient organizations, physicians and local authorities to help build sustainable healthcare networks that can deliver and pay for critical treatment. Around the world, Sanofi Genzyme's employees collaborate with healthcare providers, humanitarian organizations, advocacy groups and other knowledgeable local partners to overcome barriers to treatment.

Where commercial coverage of our therapies is not available, we strive to :

- Facilitate treatment access.
- Ensure a sustainable treatment environment.
- Help patients find alternative funding.
- Help countries establish sustainable healthcare systems.

*For more information, please refer to [Sanofi Genzyme website](#).*

## 2. BRIDGING THE GAP TO ACCESS

---

Even in countries with established health systems, patients can face delayed or limited coverage, reimbursement difficulties, or other circumstances that prevent their access to treatment. For such cases, Sanofi Genzyme has established several programs to provide our lysosomal storage disorder therapies free of charge, while also working with governments and other local entities to help identify sustainable, long-term financial resources for treatment.

Our first Charitable Access Program was started in 1991 in the United States, within a year of having a commercial product.

In 2003, we expanded beyond the US by establishing the International Charitable Access Program. Over the years, other targeted charitable access programs have focused on specific countries, such as Egypt, India and China.

As of today :

- More than 900 patients are currently receiving free therapy through Sanofi Genzyme Humanitarian programs.
- More than 3,200 patients in 100 countries have received free therapy since these programs were introduced.
- More than 350 patients have been receiving free therapy for 10 years or longer through these programs.

## 3. HUMANITARIAN PARTNERSHIPS

---

In developing countries, government and private health coverage may be non-existent, or barely able to address basic medical care. For patients who require specialized treatment, care can be financially and logistically beyond their means. Living with a rare disease or rare blood disorder is often overwhelming for families and having to advocate for your own care is an additional burden. Sanofi Genzyme has long provided several of our therapies for free to people in these situations, often by partnering with humanitarian organizations.

We began in 1999 by establishing the Gaucher Initiative, a humanitarian partnership with Project HOPE, to provide Cerezyme® (imiglucerase for injection) to Gaucher disease patients in China and Egypt. Over time, as we developed new therapies for other diseases, we evolved similar programs to distribute those treatments. In select countries, we work with NGO organizational partners — which include Project HOPE, the World Federation of Hemophilia (WFH), Global Health Partners, ANERA and Direct Relief — for their local expertise and relationships, which help us navigate the challenges of ensuring that the product reaches the patients.

One specific example is in hemophilia, a rare blood disorder that affects more than 400,000 people worldwide, 75 % of which have limited or no access to treatment. Together with Sobi, we continue to deliver on the 2014 pledge to donate up to one billion international units (IUs) of clotting factor for humanitarian use over ten years, including up to 500 million IUs of factor to the WFH Humanitarian Aid Program over five years. This unprecedented donation is an important first step in providing those most in need with a predictable and sustainable supply of hemophilia therapy, Sanofi Genzyme and Sobi collaborate on the development and commercialization of our extended half-life factor replacement therapies.

Since donations to the WFH Humanitarian Aid Program began in 2015, the impact has been far reaching :

- Nearly 450 million IUs of factor therapy have been donated.
- More than 17,200 people in 40 countries have been treated with our medicines.
- Over 160,000 acute bleeds have been treated.
- Nearly 2,300 surgeries have been performed, including life- and limb-saving.
- The percentage of children receiving treatment has increased from 14 % to 37 %.

#### 4. EDUCATION AND OUTREACH

---

Sanofi Genzyme's business was founded on treating diseases so rare that they are often unfamiliar even to many healthcare professionals. We are therefore acutely aware of how important education and outreach are to identifying and effectively treating patients. Through a variety of programs, initiatives and partnerships, we have long worked to raise awareness of the diseases we treat, educate both patients and the medical community, and advance research and quality of care. For example :

- As one of the world's leading authorities on the group of rare genetic diseases called lysosomal storage disorders (LSDs), Sanofi Genzyme develops educational materials for both patients and professionals to increase understanding of these diseases.
- In 2018, Sanofi Genzyme launched The Rare Diseases University (RDU), a physician-need-oriented, interactive and virtual educational initiative designed for physicians who are committed to advancing their knowledge and leadership in the field of rare lysosomal storage disorders. The RDU aims to develop top-level expertise in rare diseases through education on key aspects encompassing comprehensive scientific knowledge and more clinically challenging concepts and topics.
- By establishing LSD registries — large, often multinational databases to which physicians contribute clinical data on patients — we help pool knowledge on rare diseases that would otherwise be difficult to study.
- We sponsor independent investigator research and provide grants for continuing medical education and research fellowships in our areas of focus.
- Our Medical Information team is staffed across all regions to answer questions about our treatments.
- We collaborate with local patient organizations/advocacy groups around the world to make a meaningful difference to the patients we serve.