

Shu, Pompe Disease, Japan

SANOFI GENZYME 





## SCIENCE-DRIVEN, PATIENT-FOCUSED

Patients and science are at the center of everything we do, defining and uniting our efforts across rare diseases, rare blood disorders, multiple sclerosis, oncology, and immunology.

### A LEADER IN SPECIALTY CARE

Sanofi Genzyme is the specialty care global business unit of Sanofi, focused on rare diseases, multiple sclerosis, oncology, immunology, and rare blood disorders. We help people with debilitating and complex conditions that are often difficult to diagnose and treat. We are dedicated to discovering and advancing new therapies, providing hope to patients and their families around the world.

Our ambition is to be the industry leader in specialty care. We currently provide more than 25 treatments to patients globally, with many potential new therapies in development.

### OUR APPROACH

Our approach is shaped by a long history of developing highly specialized treatments and forging close relationships with physician and patient communities.

Across each of the therapeutic areas where we focus, we work to identify and address unmet medical needs where patients do not have adequate treatment options. Our commitment to scientific discovery and innovation is foundational to our ability to serve our existing patient populations and offer hope to underserved patient populations in the future.

This potential to improve patients' lives inspires the more than 4,000 Sanofi Genzyme employees who work at the forefront of health care and biotechnology, along with thousands of Sanofi colleagues around the world.

### ABOUT SANOFI

Sanofi, a global healthcare leader, discovers, develops and distributes therapeutic solutions focused on patients' needs. Sanofi is organized into five global business units: Specialty Care (Sanofi Genzyme), Primary Care, China and Emerging Markets, Vaccines (Sanofi Pasteur) and Consumer Healthcare.



# SERVING PATIENTS IN FIVE MEDICAL AREAS



Kaitie | MPS I Disease | Australia

RARE DISEASES



Tucker | Hemophilia B | USA

RARE BLOOD  
DISORDERS



Tiger | Multiple Sclerosis | United Kingdom

MULTIPLE  
SCLEROSIS



James | Prostate Cancer | USA

ONCOLOGY



Hilde | Atopic Dermatitis | Norway

IMMUNOLOGY

# AN INNOVATOR IN RARE DISEASES



Kaitie, MPS I Disease, Australia

For more than 35 years, we have been a pioneer and innovator in rare genetic diseases. Lysosomal storage disorders (LSDs), a group of extremely rare diseases with high unmet need, are an area of expertise for us, particularly Gaucher, Fabry, Pompe and Mucopolysaccharidosis (MPS) I diseases. We are continuing to research and develop new therapies for LSDs while leveraging our expertise to expand our focus to other rare conditions. Our support for patients extends beyond our therapies. We are committed to addressing a significant unmet need that still exists in rare diseases: physician and patient access to tools, knowledge and resources that will lead to timely and appropriate diagnosis.



COMMITTED TO  
RARE BLOOD DISORDERS



Tucker, Hemophilia B, USA

We are committed to helping to improve the health and lives of people with rare blood disorders around the world through groundbreaking science and a deep commitment to the community. We currently have two approved therapies, one for hemophilia A\* and one for hemophilia B\*. We have a third therapy currently under review by the US Food and Drug Administration (FDA) and approved by the European Medicines Agency (EMA) for acquired thrombotic thrombocytopenic purpura (aTTP). In addition to our current portfolio, we have a strong pipeline focusing on areas of unmet need in the rare blood disorder community, including hemophilia, cold agglutinin disease, sickle cell disease, and beta thalassemia.

\*marketed by Sobi in the EU

A LEADER IN  
MULTIPLE SCLEROSIS



Tiger, Multiple Sclerosis, United Kingdom

We have emerged as a leader in multiple sclerosis (MS), an often disabling disease of the central nervous system affecting more than two million people worldwide. Our treatments provide additional therapeutic options for patients with relapsing multiple sclerosis, and we have established partnerships with MS physicians, advocates and researchers. Through internal research and collaborative efforts with outside partners, we are focused on developing novel treatments that seek to go beyond what existing MS therapies offer.



## ADVANCING OUR PIPELINE IN ONCOLOGY



James, Prostate Cancer, USA

Our ambition is to be a leader in oncology, driven by our cutting-edge science, promising and diverse pipeline, and determination to find better solutions for patients. Today we have a significant presence in oncology and provide important treatment options to patients with prostate cancer, colon cancer, and several other types of cancer. We also have a therapy for advanced cutaneous squamous cell carcinoma that is currently under review by the European Medicines Agency (EMA) and is approved in the United States. We continue to battle cancer through multiple research approaches including a special focus on the field of immuno-oncology in which a patient's immune system is used to fight cancer cells. Every day, we take bold steps to advance our pipeline and find solutions for people with difficult-to-treat cancers.

## RESEARCHING NEW TREATMENTS IN IMMUNOLOGY



Hilde, Atopic Dermatitis, Norway

With two treatments on the market and a robust pipeline, immunology is a strong area of growth for us and one in which we hope to make a significant impact on the lives of patients. In collaboration with Regeneron, we launched a first in class treatment for adults with moderate-to-severe atopic dermatitis and adults and adolescents with moderate-to-severe asthma, two chronic and difficult to treat diseases driven by underlying inflammation. Our second treatment is for adults with moderate-to-severe rheumatoid arthritis (RA), a chronic and painful inflammatory disorder that damages the joints. Our pipeline includes other allergic and respiratory diseases, including nasal polyposis, eosinophilic esophagitis, COPD, and food allergies, as well as programs focused on juvenile idiopathic arthritis, giant cell arteritis and, polymyalgia rheumatica.

# SCIENCE

Sanofi has a strong pipeline with a significant portion of its R&D spending concentrated on specialty care. Sanofi Genzyme's presence in Cambridge, Massachusetts — the international center for biotechnology and innovation — has helped Sanofi expand its capacity to collaborate with world-class scientists in universities, research hospitals, and other companies to accelerate treatment development.

The agents mentioned here are investigational and have not been approved by the US Food and Drug Administration (FDA) or any other regulatory agency worldwide for the uses under investigation.

## R&D PIPELINE HIGHLIGHTS

Disease	Program	Phase
<b>RARE DISEASES</b>		
POMPE DISEASE	Avalglucosidase alfa (neoGAA)	Phase 3
GAUCHER DISEASE TYPE 1 (children, switch from ERT)	Eliglustat	Phase 3
AUTOSOMAL DOMINANT POLYCYSTIC KIDNEY DISEASE (ADPKD)	Venglustat	Phase 2/3
ACID SPHINGOMYELINASE DEFICIENCY (ASMD)	Olipudase alfa	Phase 2/3
FABRY DISEASE	Venglustat	Phase 2
GAUCHER DISEASE TYPE 3	Venglustat	Phase 2
PARKINSON'S DISEASE WITH A GBA MUTATION	Venglustat	Phase 2
ALPORT SYNDROME	SAR339375	Phase 2

Disease	Program	Phase
<b>RARE BLOOD DISORDERS</b>		
HEMOPHILIA A&B	Fitusiran*	Phase 3
COLD AGGLUTININ DISEASE	Sutimlimab	Phase 3
HEMOPHILIA A	BIVV001	Phase 1/2
TRANSFUSION-DEPENDENT β-THALASSEMIA (TDT)	ST-400∞	Phase 1/2

Disease	Program	Phase
<b>MULTIPLE SCLEROSIS</b>		
MULTIPLE SCLEROSIS	SAR442168*	Phase 1

Disease	Program	Phase
<b>ONCOLOGY</b>		
RELAPSING REFRACTORY MULTIPLE MYELOMA	Isatuximab (IKEMA)	Phase 3
NON-SMALL CELL LONG CANCER (FIRST LINE)	Cemiplimab^ +Ipilimumab	Phase 3
CERVICAL CANCER (SECOND LINE)	Cemiplimab^	Phase 3
NEWLY DIAGNOSED MULTIPLE MYELOMA	Isatuximab	Phase 3
NEWLY DIAGNOSED MULTIPLE MYELOMA (FIRST LINE)	Isatuximab (IMROZ)	Phase 3
RELAPSING REFRACTORY MULTIPLE MYELOMA	Isatuximab + Cemiplimab^	Phase 2
NON-SMALL CELL LUNG CANCER (FIRST LINE)	Cemiplimab^	Phase 3
NON-SMALL CELL LUNG CANCER (SECOND LINE)	Cemiplimab^	Phase 2
ADVANCED MALIGNANCIES	Isatuximab + Cemiplimab^	Phase 2
ADVANCED MALIGNANCIES	Isatuximab + Atezolizumab	Phase 2
ADVANCED BASAL CELL CARCINOMA	Cemiplimab^	Phase 2
ADVANCED SOLID TUMORS	SAR439459 (anti-TGFB)	Phase 1
SOLID TUMORS	SAR408701 (CEACAM5)	Phase 1

Disease	Program	Phase
<b>IMMUNOLOGY</b>		
ATOPIC DERMATITIS (6 mo - 5 years old, 6-11 years old, 12-17 years old)	Dupilumab^	Phase 3
NASAL POLYPS	Dupilumab^	Phase 3
ASTHMA (6-11 years old)	Dupilumab^	Phase 3
EOSINOPHILIC ESOPHAGITIS	Dupilumab^	Phase 3
GIANT CELL ARTERITIS	Sarilumab^	Phase 3
POLYMYALGIA RHEUMATICA	Sarilumab^	Phase 3
ASTHMA	SAR440340^	Phase 2
CHRONIC OBSTRUCTIVE PULMONARY DISEASE	SAR440340^	Phase 2
POLYARTICULAR JUVENILE IDIOPATHIC ARTHRITIS	Sarilumab^	Phase 2
SYSTEMIC JUVENILE ARTHRITIS	Sarilumab^	Phase 2
SYSTEMIC SCLERODERMA	SAR156597	Phase 2
GRASS IMMUNOTHERAPY	Dupilumab^	Phase 2
PEANUT ALLERGY PEDIATRIC	Dupilumab^ + AR101-CODIT	Phase 2
PEANUT ALLERGY	SAR439794^	Phase 1

^ being developed with Regeneron  
+ being developed with Principia Biopharma

\* being developed with Alnylam  
∞ being developed with Sangamo Therapeutics



## COMMUNITY

We believe in having a lasting, positive impact where our employees live and work. Through a combination of financial contributions and employee involvement, we build sustainable partnerships with schools and nonprofit organizations. We develop and fund innovative science education programs for people of all ages. We also fund community-based, nonprofit organizations dedicated to health and increasing access to health care, and other critical community needs.



Universal Access  
Community Boating,  
Boston, MA, USA

## RESPONSIBILITY

Our dedication to patients extends beyond the development of medicines. We support access to treatment through humanitarian programs to help ensure that appropriate treatment reaches all those who need it.

### HUMANITARIAN PROGRAM FOR LYSOSOMAL STORAGE DISORDERS

The primary goal of our Humanitarian program is to deliver therapies, to the best of our ability, to patients with lysosomal storage disorders who have a demonstrated need in certain circumstances where treatment access is limited. Established in 1991, in our first year of having a commercial product, the program has evolved and expanded to support five different lysosomal storage disorder communities across six continents. While more than 250 patients have been on treatment through the program for over 10 years, our program frequently services as a bridge while countries work to establish a long term patient care support systems. It has played a key role in helping build sustainable healthcare systems that are able to provide support and holistic care for patients globally. More than 2,500 patients in over 80 countries have received access to therapy since then including more than 700 patients in 60 countries on therapy today.

### HUMANITARIAN AID IN HEMOPHILIA

Our vision is to help transform the way hemophilia care is delivered in the developing world, where the vast majority of people with hemophilia have limited or no access to diagnosis and treatment. That's why in 2014, together with Sobi, we pledged to donate up to one billion IUs of clotting factor over 10 years, including up to 500 million IUs to the World Federation of Hemophilia (WFH) Humanitarian Aid Program over a period up to five years. Since shipments began, our medicines have touched the lives of more than 16,000 people in 40 developing world countries, treated over 100,000 acute bleeds and enabled over 1,800 surgeries through the WFH Humanitarian Aid Program. This donation, the single largest of its kind, is an important first step towards providing those most in need with a predictable and sustainable supply of therapy.



Ailin,  
Gaucher Disease,  
Cuba



Hakob,  
Hemophilia,  
Armenia



*"We have to be focused on the science, focused on the patients – that is the core of what we do. If we do that right, we can ultimately be successful."*

**BILL SIBOLD**  
Executive Vice President  
and Head of Sanofi  
Genzyme

## ABOUT SANOFI

- > Based in Paris, France
- > Present in more than 100 countries
- > 100,000+ employees globally
- > Revenues of €35B
- > €6B annual investment in R&D

## ABOUT SANOFI GENZYME

- > Based in Cambridge, MA, USA
- > 4500+ employees globally
- > Revenues of €5B



