

Shu, Pompe Disease, Japan

SANOFI GENZYME 



PATIENTS AND SCIENCE ARE AT THE CENTER OF EVERYTHING WE DO

Leading with science and keeping patients at the center of our work defines and unites 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, rare blood disorders, multiple sclerosis, oncology and immunology. 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 4800+ 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 three core global business units: Specialty Care (Sanofi Genzyme), Vaccines (Sanofi Pasteur), and General Medicines. Consumer Healthcare is a standalone business unit.

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



Ron | Cutaneous Squamous Cell Carcinoma | 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 for hemophilia, one for hemophilia A* and one for hemophilia B*. We have a third therapy approved 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



Ron, Cutaneous Squamous Cell Carcinoma, USA

We are building on a rich legacy in oncology with a rejuvenated pipeline and a renewed commitment to bring novel treatment options to patients with unmet medical needs. Today, we have a portfolio of ten medicines treating numerous types of cancer, including skin, prostate, lung, colon, breast, and multiple myeloma. We are determined to provide better treatment options for patients through a commitment to drug discovery and development rooted in strong science and innovative technologies. Our pipeline in oncology includes more than a dozen compounds in all phases of clinical trials. We work every day on behalf of patients to advance our pipeline and find solutions that address unmet medical needs.

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, adolescents and children ages 6-11 with moderate-to-severe atopic dermatitis; and adults and adolescents with 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 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. Our presence in Cambridge, Massachusetts – the international center for biotechnology and innovation – affords us tremendous opportunity 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
RARE DISEASES		
GAUCHER TYPE 1, ERT SWITCH PEDIATRIC	Eliglustat	Phase 3
POMPE DISEASE	Avalglucosidase alfa (neoGAA)	Phase 3
GM2 GANGLIOSIDOSIS	Venglustat	Phase 3
AUTOSOMAL DOMINANT POLYCYSTICKIDNEY DISEASE (ADPKD)	Venglustat	Phase 3
ACID SPHINGOMYELINASE DEFICIENCY (ASMD) Adult and Pediatric	Olipudase alfa	Phase 2
FABRY DISEASE	Venglustat	Phase 2
GAUCHER DISEASE TYPE 3	Venglustat	Phase 2
ALPORT SYNDROME	SAR339375	Phase 2

Disease	Program	Phase
RARE BLOOD DISORDERS		
HEMOPHILIA A&B	Fitusiran	Phase 3
HEMOPHILIA A & B PEDIATRIC	Fitusiran	Phase 3
COLD AGGLUTININ DISEASE	Sutimlimab	Phase 3
HEMOPHILIA A	BIVV001 ^o	Phase 2
β-THALASSEMIA (TDT)	ST-400 ^{oo}	Phase 1/2
SICKLE CELL DISEASE	BIVV003 ^{oo}	Phase 1/2
IMMUNE THROMBOCYTOPENIC PURPURA (ITP)	Sutimlimab	Phase 1
IMMUNE THROMBOCYTOPENIC PURPURA	BIVV020	Phase 1

^{oo} being developed with Sangamo Therapeutics ^obeing developed with Sobi

Disease	Program*	Phase
MULTIPLE SCLEROSIS, NEUROLOGY, OPHTHALMOLOGY		
RELAPSING MULTIPLE SCLEROSIS PEDIATRIC	Teriflunomide	Phase 3
RELAPSING MULTIPLE SCLEROSIS PEDIATRIC	Alemtuzumab	Phase 3
MULTIPLE SCLEROSIS	SAR442168*	Phase 2
PARKINSON'S DISEASE WITH A GBA MUTATION	Venglustat	Phase 2
AMYOTROPHIC LATERAL SCLEROSIS (ALS)	SAR443060 [†]	Phase 1
MULTIPLE SCLEROSIS	SAR443060 [†]	Phase 1
MULTIPLE SCLEROSIS	SAR441344 ^{**}	Phase 1

*being developed with Principia Biopharma [†] being developed with Denali ^{**} being developed with Immunext

Disease	Program	Phase
ONCOLOGY		
RELAPSING REFRACTORY MULTIPLE MYELOMA (THIRD LINE) (ICARIA)	Isatuximab	Phase 3
RELAPSING REFRACTORY MULTIPLE MYELOMA (SECOND LINE) (IKEMA)	Isatuximab	Phase 3
NEWLY DIAGNOSED MULTIPLE MYELOMA Tt ⁽³⁾ (FIRST LINE) (IMROZ)	Isatuximab	Phase 3
NEWLY DIAGNOSED MULTIPLE MYELOMA Te ⁽²⁾ (GMMG)	Isatuximab	Phase 3
SMOLDERING MULTIPLE MYELOMA	Isatuximab	Phase 3
CERVICAL CANCER (SECOND LINE)	Cemiplimab [^]	Phase 3
ADJUVANT IN CUTANEOUS SQUAMOUS CELL CARCINOMA (CSCC)	Cemiplimab [^]	Phase 3
NON-SMALL CELL LUNG CANCER (FIRST LINE)	Cemiplimab [^]	Phase 3
NON-SMALL CELL LUNG CANCER (FIRST LINE)	Cemiplimab [^] + chemotherapy	Phase 3
NON SMALL CELL LUNG CANCER (SECOND LINE + THIRD LINE)	SAR408701	Phase 3
RELAPSED REFRACTORY MULTIPLE MYELOMA	Isatuximab + Cemiplimab [^]	Phase 2
LYMPHOMA	Isatuximab + Cemiplimab [^]	Phase 2
METASTATIC COLORECTAL CANCER	Isatuximab + Atezolizumab [†]	Phase 2
SOLID TUMORS	Isatuximab + Atezolizumab [†]	Phase 2
PATIENTS AWAITING KIDNEY TRANSPLANTATION	Isatuximab	Phase 2
ACUTE MYELOID LEUKEMIA (AML) / ACUTE LYMPHOPBLASTIC LEUKEMIA (ALL) PEDIATRICS (FIRST LINE + SECOND LINE)	Isatuximab	Phase 2
BASAL CELL CARCINOMA (SECOND LINE)	Cemiplimab [^]	Phase 2
BREAST CANCER ADJUVANT	SAR439859	Phase 2
METASTATIC BREAST CANCER (SECOND LINE + THIRD LINE)	SAR439859	Phase 2
RELAPSED REFRACTORY MULTIPLE MYELOMA	REGN5458 ⁽⁴⁾	Phase 1
OVARIAN CANCER	REGN4018 ⁽⁴⁾	Phase 1
SOLID TUMORS	SAR442720 ^o	Phase 1
LEUKEMIA	SAR440234	Phase 1
SOLID TUMORS	SAR441000 ^o , mono & with PD1	Phase 1
ADVANCED SOLID TUMORS	SAR439459, mono & with cemiplimab [^]	Phase 1
METASTATIC BREAST CANCER	SAR439859 + palbociclib	Phase 1
OVARIAN CANCER	Cemiplimab + REGN4018 ^{^ (4)}	Phase 1
MULTIPLE MYELOMA	SAR442085	Phase 1
RELAPSING REFRACTORY MULTIPLE MYELOMA	REGN5459 ⁽⁴⁾	Phase 1
RELAPSING REFRACTORY SOLID TUMORS	SAR442720 ^{^ ^} + cobimetinib	Phase 1
SOLID TUMORS	SAR444245 ⁽⁴⁾ (THOR-707), mono & combo	Phase 1

⁽²⁾ Transplant eligible ⁽³⁾ Transplant ineligible ⁽⁴⁾ Opt-in rights products for which rights have not been exercised yet
^o being developed with Regeneron [†] being developed with Roche [^] being developed with Revolution Medicines [^] being developed with Regeneron ^{**} being developed with Revolution Medicines. Cobimetinib is a Genentech product

Disease	Program	Phase
IMMUNOLOGY		
ATOPIC DERMATITIS (6 mo - 5 years)	Dupilumab [^]	Phase 3
ASTHMA (6-11 years old)	Dupilumab [^]	Phase 3
EOSINOPHILIC ESOPHAGITIS	Dupilumab [^]	Phase 3
BULLOUS PEMPFIGOID	Dupilumab [^]	Phase 3
PRURIGO NODULARIS	Dupilumab [^]	Phase 3
CHRONIC SPONTANEOUS URTICARIA	Dupilumab [^]	Phase 3
GIANT CELL ARTERITIS	Sarilumab [^]	Phase 3
POLYMYALGIA RHEUMATICA	Sarilumab [^]	Phase 3
CHRONIC OBSTRUCTIVE PULMONARY DISEASE (COPD)	Dupilumab [^]	Phase 3
GRASS POLLEN ALLERGY	Dupilumab [^]	Phase 2
PEANUT ALLERGY	Dupilumab [^]	Phase 2
ASTHMA	SAR440340 [^]	Phase 2
CHRONIC OBSTRUCTIVE PULMONARY DISEASE (COPD)	SAR440340 [^]	Phase 2
POLYARTICULAR JUVENILE IDIOPATHIC ARTHRITIS	Sarilumab [^]	Phase 2
SYSTEMIC JUVENILE ARTHRITIS	Sarilumab [^]	Phase 2
SYSTEMIC SCLERODERMA	Romilkimab	Phase 2
PSORIASIS	SAR441169 ^o	Phase 1
HIV	SAR441236	Phase 1
INFLAMMATORY INDICATIONS	SAR443122 [†]	Phase 1

[^] being developed with Regeneron ^o being developed with Lead Pharma [†] being developed with Denali

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.

For over 10 years more than 300 patients have been on treatment through the program, which frequently serves as a bridge while countries work to establish 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 3,000 patients in over 90 countries have received access to therapy including more than 750 patients in 65 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 we are continuing to support, together with Sobi, the 2014 pledge to donate up to one billion international units (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 17,200 people in 40 developing world countries, treated over 160,000 acute bleeds and enabled nearly 2,300 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.





"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
- > 4,800+ employees globally
- > Revenues of €7B

