

## **Genzyme and Isis Receive Corporate Award from the National Organization for Rare Disorders (NORD) for the Development of KYNAMRO®**

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CAMBRIDGE, Mass. & CARLSBAD, Calif.--(BUSINESS WIRE)--

Genzyme, a Sanofi company (EURONEXT: SAN and NYSE: SNY), and Isis Pharmaceuticals Inc. ([ISIS](#)), today announced that they have received the 2014 Partners in Progress Corporate Award from NORD in recognition of KYNAMRO® (mipomersen sodium) injection. KYNAMRO, selected for being a very important orphan therapy to reach the market in the United States, is an adjunct to lipid-lowering medications and diet to reduce low density lipoprotein-cholesterol (LDL-C), apolipoprotein B (Apo B), total cholesterol (TC), and non-high density lipoprotein-cholesterol (non HDL-C) in patients with homozygous familial hypercholesterolemia (HoFH).

“There are over 7,000 rare diseases, of which, only 300 currently have a treatment option,” said NORD President and CEO Peter L. Saltonstall. “Each therapy brought to market for one rare disease provides hope to the many others that are still in need. We are pleased to recognize Genzyme and Isis for their dedication and commitment to improving the lives of patients with a rare disease.”

This award marks the third time that Genzyme has been recognized by NORD for its contributions and ongoing commitment to improving the lives of those affected by rare diseases. The award will be presented tonight at NORD’s “Portraits of Courage Celebration” in Washington, DC.

“It is an honor to be recognized by NORD and we hope this award serves to highlight the considerable unmet need that still exists in helping patients with rare diseases,” said Genzyme President and CEO, David Meeker, M.D. “We are proud that we are able to bring our rare disease expertise to patients living with HoFH, who have exhausted conventional medications and are still in need of additional treatment options.”

The NORD Gala is an annual event at which researchers and others are honored for improving the lives of people with rare diseases. Each year NORD honors organizations that have made a positive contribution to further the needs of the patient community, and have inspired the public to do so as well.

“KYNAMRO is the first systemic antisense drug to reach the market and is the culmination of two decades of work to create a new, more efficient drug technology platform,” said Stanley T. Croke, M.D., Ph.D., Chairman of the Board and CEO of Isis. “We are incredibly grateful to NORD for its recognition of the work to bring this much needed therapy to patients living with HoFH.”

NORD is a nonprofit organization established in 1983 to represent all individuals and families affected by rare diseases. It provides advocacy, education, support for research, and patient assistance programs to improve the lives of the 30 million Americans living with rare diseases.

### **About KYNAMRO (mipomersen sodium) injection**

KYNAMRO is a first-in-class, oligonucleotide inhibitor, of apolipoprotein B-100 synthesis. KYNAMRO (mipomersen sodium) injection 200 mg/mL is an adjunct to lipid-lowering medications and diet to reduce low density lipoprotein-cholesterol (LDL-C), apolipoprotein B (apo B), total cholesterol (TC), and non-high density lipoprotein-cholesterol (non HDL-C) in patients with homozygous familial hypercholesterolemia (HoFH). KYNAMRO reduces LDL-C by preventing the formation of atherogenic lipoproteins, the particles that carry cholesterol through the bloodstream. KYNAMRO acts by blocking the production of apo B, the protein that provides the structural core for these atherogenic particles, including LDL. KYNAMRO can cause elevations in transaminases and increases in hepatic fat. Because of the risk of hepatotoxicity, KYNAMRO is available only through a restricted program under a Risk Evaluation and Mitigation Strategy (REMS) called KYNAMRO REMS. For more information, please visit [www.kynamro.com](http://www.kynamro.com).

### **About Homozygous Familial Hypercholesterolemia (HoFH)**

HoFH is a rare genetic disease characterized by extreme cholesterol levels. People with HoFH have inherited mutations that limit the body's ability to clear cholesterol. HoFH is extremely rare: it is believed to occur in only one out of every one million persons. As with other rare diseases, the true prevalence of HoFH may be underestimated because of inadequate data and under-diagnosis. Today, it is estimated that HoFH affects about 6,000 people globally. Medical literature includes different criteria for marking an HoFH diagnosis. HoFH may be diagnosed by clinical or genetic parameters, and may be considered in cases of unusually high LDL-C, such as greater than 500 mg/dL without treatment, or 300 mg/dL after taking cholesterol-lowering medication. Because HoFH is genetic, it is important that all family members of people with HoFH know their cholesterol levels, regardless of their age.

### **About Genzyme, a Sanofi Company**

Genzyme has pioneered the development and delivery of transformative therapies for patients affected by rare and debilitating diseases for over 30 years. We accomplish our goals through world-class research and with the compassion and commitment of our employees. 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. That goal guides and inspires us every day. Genzyme's portfolio of transformative therapies, which are marketed in countries around the world, represents groundbreaking and life-saving advances in medicine. 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. Learn more at [www.genzyme.com](http://www.genzyme.com).

### **About Sanofi**

Sanofi, a global and diversified healthcare leader, discovers, develops and distributes therapeutic solutions focused on patients' needs. Sanofi has core strengths in the field of healthcare with seven growth platforms: diabetes solutions, human vaccines, innovative drugs, consumer healthcare, emerging markets, animal health and the new Genzyme. Sanofi is listed in Paris ([SAN](#)) and in New York ([SNY](#)).

### **About Isis Pharmaceuticals, Inc.**

Isis is exploiting its leadership position in antisense technology to discover and develop novel drugs for its product pipeline and for its partners. Isis' broad pipeline consists of 28 drugs to treat a wide variety of diseases with an emphasis on cardiovascular, metabolic, severe and rare diseases, and cancer. Isis' partner, Genzyme, is commercializing Isis' lead product, KYNAMRO®, in the United States for the treatment of patients with HoFH. Genzyme is also pursuing marketing approval of KYNAMRO in other markets. Isis' patents provide strong and extensive protection for its drugs and technology. Additional information about Isis is available at [www.isispharm.com](http://www.isispharm.com).

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Contact:

#### **Genzyme Media Relations**

Ingrid Mitchell, 617-768-6699

[Ingrid.Mitchell@genzyme.com](mailto:Ingrid.Mitchell@genzyme.com)

or

#### **Isis Media Relations**

Amy Blackley, Ph.D., 760-603-2772 (Media)

[ABlackley@isisph.com](mailto:ABlackley@isisph.com)

or

#### **Isis Investor Relations**

D. Wade Walke, Ph.D., 760-603-2741 (Investors)

[WWalke@isisph.com](mailto:WWalke@isisph.com)