Current Agreements

Dealdoc

Licensing agreement for KYNAMRO (mipomersen sodium) injection

Ionis Pharmaceuticals
Kastle Therapeutics

May 03 2016

Licensing agreement for KYNAMRO (mipomersen sodium) injection

Companies:	Ionis Pharmaceuticals Kastle Therapeutics
Announcement date:	May 03 2016
Deal value, US$m:	95 : sum of upfront and milestone payments

Details

Announcement date:	May 03 2016
Industry sectors:	Pharmaceutical
Brand name:	Kynamro
Asset type:	Product
Therapy areas:	Cardiovascular » Hypercholesterolemia
Technology types:	Drug delivery » Parenteral » Injectable Small molecules
Deal components:	Development Licensing Marketing
Stages of development:	Marketed
Geographic focus:	Worldwide

Financials

Deal value, US$m:	95 : sum of upfront and milestone payments
Upfront, US$m:	15 : upfront payment
Milestones, US$m:	10 : milestone payment 70 : sale milestone
Royalty rates, %:	n/d : on global sales
Semi-quant royalties:	Low teens Mid teens
Equity, US$m:	n/d : common equity position Ionis will receive a 10 percent common equity position in Kastle's parent company.
More details:	Sanofi Genzyme will earn a 3 percent royalty on sales of Kynamro and 3 percent of the cash payments Ionis receives from Kastle.

Termsheet

Ionis Pharmaceuticals and Kastle Therapeuticsb announced that Kastle has acquired global rights to develop and commercialize KYNAMRO (mipomersen sodium) injection.

KYNAMRO is approved in the United States for use in patients with homozygous familial hypercholesterolemia (HoFH) to reduce low density lipoprotein-cholesterol (LDL-C), apolipoprotein B (apoB), total cholesterol (TC) and non-high density lipoprotein-cholesterol (non-HDL-C) as an adjunct to lipid lowering medications and diet.

Under the terms of the agreement, Ionis is eligible to receive up to $95 million, which includes a $15 million up-front payment, a $10 million payment three years from today and up to $70 million in sales milestones.

Beginning in 2017, Ionis will earn royalties on global sales of KYNAMRO in the mid to low teens.

In addition, Ionis will receive a 10 percent common equity position in Kastle's parent company.

Sanofi Genzyme will earn a 3 percent royalty on sales of Kynamro and 3 percent of the cash payments Ionis receives from Kastle.

KYNAMRO is an oligonucleotide inhibitor of apolipoprotein B-100 synthesis.

KYNAMRO is indicated as 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).

Press Release

CARLSBAD, Calif. and CHICAGO, May 3, 2016 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (NASDAQ: IONS) and Kastle Therapeutics, LLC today announced that Kastle has acquired global rights to develop and commercialize KYNAMRO (mipomersen sodium) injection. KYNAMRO is approved in the United States for use in patients with homozygous familial hypercholesterolemia (HoFH) to reduce low density lipoprotein-cholesterol (LDL-C), apolipoprotein B (apoB), total cholesterol (TC) and non-high density lipoprotein-cholesterol (non-HDL-C) as an adjunct to lipid lowering medications and diet.

Under the terms of the agreement, Ionis is eligible to receive up to $95 million, which includes a $15 million up-front payment, a $10 million payment three years from today and up to $70 million in sales milestones. Beginning in 2017, Ionis will earn royalties on global sales of KYNAMRO in the mid to low teens. In addition, Ionis will receive a 10 percent common equity position in Kastle's parent company. Sanofi Genzyme will earn a 3 percent royalty on sales of Kynamro and 3 percent of the cash payments Ionis receives from Kastle.

Kastle was formed in 2015 to focus on acquiring, developing and commercializing pharmaceuticals targeted toward diseases with high unmet medical needs. "As Kastle's first acquisition, KYNAMRO fits our strategy perfectly," said Bryan Stuart, president of Kastle. "KYNAMRO is an innovative and important therapy for patients with HoFH, and we are fully committed to both the product and the patients it serves. This acquisition marks a major milestone in Kastle's evolution as an emerging biopharmaceutical company. With KYNAMRO, Kastle aims to bring an established rare disease therapy to more patients in the U.S. and other regions through the pursuit of additional indications and regulatory approvals worldwide."

"We believe Kastle Therapeutics has the expertise, financial resources and initiative to maximize the commercial value of KYNAMRO. Despite the emergence of new therapies that also lower LDL-cholesterol, there remains a significant unmet medical need for HoFH patients," said Sarah Boyce, chief business officer at Ionis Pharmaceuticals. "We feel that Kastle's management team brings expertise in marketing orphan drugs for rare diseases which, combined with its ability to be nimble and focused, has the potential to greatly enhance the KYNAMRO brand. Already Kastle has commenced initiatives to identify new patients to bring onto therapy in the United States and plans to pursue marketing approval in other countries."

"KYNAMRO has demonstrated consistent and sustained LDL reductions across five randomized placebo controlled phase 3 clinical trials," said Richard Geary, Ph.D., senior vice president of development at Ionis Pharmaceuticals. "Ionis is proud of the robust clinical program supporting Kynamro."

"Kastle's acquisition of KYNAMRO is significant news for patients with HoFH who are in continued need of additional treatment options for this rare and often under-diagnosed disease," said Alan MacKenzie, Executive Chairman of Kastle. "Our outstanding commercial and business development teams were able to work quickly to negotiate a transaction with Ionis and develop a transition plan to maintain market continuity for this important product. Going forward, we will continue to look for partnerships and investments where we can help support underserved patient populations."

About KYNAMRO® (mipomersen sodium) injection KYNAMRO is an oligonucleotide inhibitor of apolipoprotein B-100 synthesis. KYNAMRO is indicated as 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.

Limitations of Use:

The safety and effectiveness of KYNAMRO have not been established in patients with hypercholesterolemia who do not have HoFH. The effect of KYNAMRO on cardiovascular morbidity and mortality has not been determined. The use of KYNAMRO as an adjunct to LDL apheresis is not recommended. 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. The true prevalence

of HoFH may be underestimated because of inadequate data and under-diagnosis. Medical literature includes different criteria for making an HoFH diagnosis. HoFH may be diagnosed by clinical or genetic parameters, and may be considered in cases of unusually high LDL-C. Because HoFH is genetic, it is important that all family members of people with HoFH know their cholesterol levels, regardless of their age.

ABOUT IONIS PHARMACEUTICALS, INC. Ionis is the leading company in RNA-targeted drug discovery and development focused on developing drugs for patients who have the highest unmet medical needs, such as those patients with severe and rare diseases. Using its proprietary antisense technology, Ionis has created a large pipeline of first-in-class and/or best-in-class drugs, with over a dozen drugs in mid- to late-stage development. Drugs currently in Phase 3 development include volanesorsen, a drug Ionis is developing and, if approved, plans to commercialize through its wholly owned subsidiary, Akcea Therapeutics, to treat patients with familial chylomicronemia syndrome and familial partial lipodystrophy; IONIS-TTRRx, a drug Ionis is developing with GSK to treat patients with all forms of TTR amyloidosis; and nusinersen, a drug Ionis is developing with Biogen to treat infants and children with spinal muscular atrophy. Ionis' patents provide strong and extensive protection for its drugs and technology. Additional information about Ionis is available at www.ionispharma.com.

ABOUT KASTLE THERAPEUTICS Kastle Therapeutics is a biopharmaceutical company focused on developing and commercializing pharmaceuticals for diseases with high unmet medical needs. Kastle Therapeutics is partnered with Flexpoint Ford, a private equity firm with $2.3 billion under management dedicated to the healthcare and financial services sectors. Kastle Therapeutics is headquartered in Chicago. For more information, please visit www.kastletherapeutics.com.

Filing Data

Not available.

Contract

Not available.