FDA Accepts Supplemental New Drug Application for Priority Review of RAPAMUNE® (sirolimus) for Treatment of Lymphangioleiomyomatosis (LAM)

Friday, February 20, 2015 - 05:30am

Pfizer Inc. (NYSE: PFE) announced today that the U.S. Food and Drug Administration (FDA) has accepted for priority review a supplemental New Drug Application (sNDA)for RAPAMUNE® (sirolimus) for the treatment of lymphangioleiomyomatosis (LAM), a rare, progressive lung disease in women of childbearing age that is often fatal. With the Priority Review designation for the sNDA, we anticipate a decision in June of 2015 based on the anticipated Prescription Drug User Fee Act (PDUFA) action date.

“If approved, RAPAMUNE would be the first FDA approved treatment option for patients living with LAM,” said Steve Romano, MD, SVP and head of Global Medicines Development at Pfizer’s Global Innovative Pharmaceuticals Business. “We look forward to continuing to work closely with the FDA throughout the review process.”

The sNDA is based on results from the Multicenter International Lymphangioleiomyomatosis Efficacy and Safety of Sirolimus (MILES) Trial. The MILES Trial included 89 LAM patients with moderate lung impairment who were randomized to receive RAPAMUNE (dose adjusted to 5-15 ng/mL) or placebo for 12 months, followed by a 12 month observation period. In the trial, those treated with RAPAMUNE for one year experienced stabilization of lung function as measured by forced expiratory volume in one second (FEV1). Full results of the MILES Trial were published in the New England Journal of Medicine. The most common adverse events reported during the study were

mucositis, diarrhea, nausea, hypercholesterolemia, acneiform rash and swelling in the lower extremities. The adverse drug reactions observed were consistent with the known safety profile of RAPAMUNE in renal transplant patients, with the exception of weight decreased, which was reported at a greater incidence with RAPAMUNE compared to placebo.

“The results of the MILES Trial demonstrated that RAPAMUNE has the potential to stabilize lung decline in patients suffering from LAM,” said Dr. Francis X. McCormack, Director of Pulmonary, Critical Care and Sleep Medicine at the University Of Cincinnati School of Medicine and the lead investigator of the MILES Trial. “We are excited about the FDA’s review of RAPAMUNE and the potential to make this medication available to LAM patients.”

The MILES trial was conducted by Dr. McCormack and conducted within the NIH Rare Lung Diseases Consortium. Pfizer provided study drug and a portion of the funding but had no involvement in the design or conduct of the study. The LAM Foundation assisted with the recruitment of patients and logistics for the study.

“For 20 years, the LAM Foundation has been committed to seeking treatment options for LAM and we are thrilled about the possibility of getting a therapy approved to treat this rare and potentially deadly disease,” said Susan E. Sherman, Executive Director of the LAM Foundation.

About RAPAMUNE

RAPAMUNE is indicated for the prevention of organ rejection in kidney transplant patients aged 13 years or older. Blood levels of sirolimus should be checked in all patients taking RAPAMUNE. Please see the full prescribing information including, boxed warning and full indications at www.RAPAMUNE.com.

RAPAMUNE Important Safety Information

There is an increased risk of developing infections or certain cancers, especially lymphoma and skin cancers. RAPAMUNE (sirolimus) has not been shown to be safe and effective in people who have had liver or lung transplants. Serious complications and death may happen in people who take RAPAMUNE after a liver or lung transplant. You should not take RAPAMUNE if you have had a liver or lung transplant without talking with your doctor.

Do not take RAPAMUNE if you know you are allergic to sirolimus or any of the other ingredients in RAPAMUNE. Symptoms of an allergic reaction include swelling of your face, eyes, or mouth; trouble breathing or wheezing; throat tightness; chest pain or tightness; feeling dizzy or faint; and rash or peeling of your skin.

Before taking RAPAMUNE, tell your doctor if you have liver problems, skin cancer or it runs in your family, high cholesterol or triglycerides, are breastfeeding or plan to breastfeed, and are pregnant or plan to become pregnant. Women of childbearing potential should use effective birth control before therapy, during therapy, and for 12 weeks after RAPAMUNE therapy has been stopped. RAPAMUNE may interact with other medicines. Make sure that your doctor is aware of all prescription and over-the-counter drugs that you are taking, including vitamins, herbs, and nutritional supplements.

RAPAMUNE may cause swelling in your hands, feet, and in various tissues of your body. Call your doctor if you have trouble breathing.

RAPAMUNE may cause your wounds to heal slowly or not heal well resulting in redness, drainage, or opening of the wound.

RAPAMUNE may increase the levels of cholesterol and triglycerides (lipids or fat) in your blood. Your doctor should do blood tests to check your lipids during treatment with RAPAMUNE. Your doctor may recommend treatment if your lipid levels become too high. Your lipid levels may remain high even if you follow your prescribed treatment plan.

In patients taking RAPAMUNE with cyclosporine, decreased kidney function has been observed. Your doctor will regularly check your kidney function.

RAPAMUNE may increase protein in your urine. Your doctor may monitor you for abnormal protein in your urine from time to time.

RAPAMUNE may increase your risk for viral infections. Certain viruses can live in your body and cause active infections when your immune system is weak. One of these viruses, BK virus, can affect how your kidney works and cause your transplanted kidney to fail. A certain virus can cause a rare serious brain infection called Progressive Multifocal Leukoencephalopathy causing death or severe disability.

RAPAMUNE may cause potentially life-threatening lung or breathing problems. Symptoms may include coughing, shortness of breath, or difficulty breathing.

When RAPAMUNE is taken with cyclosporine or tacrolimus, you may develop a blood clotting problem resulting in unexplained bleeding or bruising.

Common side effects associated with RAPAMUNE include high blood pressure, pain (including stomach and joint pain), diarrhea, headache, fever, urinary tract infection, low red blood cell count (anemia), nausea, and low platelet count (cells that help blood to clot). If you experience any side effects, contact your doctor.

About Lymphangioleiomyomatosis (LAM)

Lymphangioleiomyomatosis (LAM) is a rare progressive lung disease that usually affects women during their childbearing years and results from abnormal growth of smooth muscle-like cells. Over time, the abnormal growth of these cells can cause airway obstruction and limit the delivery of oxygen to the body.

Pfizer and Rare Diseases

Rare diseases are among the most serious of all illnesses and impact millions of patients worldwide, representing an opportunity to apply our knowledge and expertise to help make a significant impact in addressing unmet medical needs. The Pfizer focus on rare diseases builds on more than a decade of experience and a global portfolio of 22 medicines approved worldwide that treat rare diseases in the areas of hematology, neuroscience, inherited metabolic disorders, pulmonology, and oncology.

Pfizer Inc.: Working together for a healthier world®

At Pfizer, we apply science and our global resources to bring therapies to people that extend and significantly improve their lives. We strive to set the standard for quality, safety and value in the discovery, development and manufacture of health care products. Our global portfolio includes medicines and vaccines as well as many of the world's best-known consumer health care products. Every day, Pfizer colleagues work across developed and emerging markets to advance wellness, prevention, treatments and cures that challenge the most feared diseases of our time. Consistent with our responsibility as one of the world's premier innovative biopharmaceutical companies, we collaborate with health care providers, governments and local communities to support and expand access to reliable, affordable health care around the world. For more than 150 years, Pfizer has worked to make a difference for all who rely on us. To learn more, please visit us at www.pfizer.com.

DISCLOSURE NOTICE: The information contained in this release is as of February 20, 2015. Pfizer assumes no obligation to update forward-looking statements contained in this release as the result of new information or future events or developments.

This release contains forward-looking information about a potential new indication for RAPAMUNE® (sirolimus) for the treatment of lymphangioleiomyomatosis, including its potential benefits and the potential timing of a decision by the FDA, that involves substantial risks and uncertainties that could cause actual results to differ materially from those expressed or implied by such statements. Risks and uncertainties include, among other things, the uncertainties inherent in research and development; whether and when the supplemental new drug application for the potential new indication for RAPAMUNE will be approved by the FDA, which will depend on the assessment by the FDA of the benefit-risk profile suggested by the totality of the efficacy and safety information submitted; decisions by the FDA regarding labeling and other matters that could affect the availability or commercial potential of the potential new indication; and competitive developments.

A further description of risks and uncertainties can be found in Pfizer's Annual Report on Form 10-K for the fiscal year ended December 31, 2013 and in its subsequent reports on Form 10-Q, including in the sections thereof captioned "Risk Factors" and "Forward-Looking Information That May Affect Future Results," as well as in its subsequent reports on Form 8-K, all of which are filed with the SEC and available at www.sec.gov and www.pfizer.com.

Pfizer Inc. Media: Steven Danehy, 212-733-1538 Steven.Danehy@pfizer.com or Investor: Chuck Triano, 212-733-3901 charles.e.triano@pfizer.com