Pfizer’s RAPAMUNE® (sirolimus) Becomes First FDA-Approved Treatment for Lymphangioleiomyomatosis (LAM), A Rare Progressive Lung Disease

Friday, May 29, 2015 - 04:30am

Pfizer Inc. (NYSE: PFE) announced today that the U.S. Food and Drug Administration (FDA) has approved RAPAMUNE® (sirolimus) for the treatment of lymphangioleiomyomatosis (LAM), a rare, progressive disease that affects the lungs, kidneys and the lymphatic system. This is the first approved treatment that helps stabilize lung function in patients with LAM.

“Pfizer is proud to gain approval for RAPAMUNE as the first treatment for patients with LAM, through our work with the FDA, the clinical investigation team and the LAM Foundation,” said Rory O’Connor, MD, senior vice president and head of Global Medical Affairs, Global Innovative Pharmaceuticals Business, Pfizer Inc. “This type of cooperative effort creates opportunities for innovation in developing therapies for patients with rare diseases.”

LAM is a rare progressive lung disease that usually affects women during their childbearing years and can result in abnormal growth of smooth muscle cells in the lung. Over time, the muscle growth can cause airway obstructions and limit the delivery of oxygen to the body. Approximately 800 patients in the U.S. are currently diagnosed with LAM, which is a rare disease and is often fatal. RAPAMUNE is also approved in the U.S. as an immunosuppressive agent for the prophylaxis of organ rejection in kidney transplant patients aged 13 years and older.

The FDA approval is based on the results from the Multicenter International Lymphangioleiomyomatosis Efficacy of Sirolimus or MILES Trial. The MILES Trial included 89 LAM patients with moderate lung impairment and showed that those treated with RAPAMUNE for one year experienced stabilization of lung function measured by forced expiratory volume in one second (FEV1). The adverse drug reactions observed in this trial were consistent with the known safety profile for renal transplant patients receiving RAPAMUNE, with the addition of weight decreased, which was reported at a greater incidence with RAPAMUNE versus placebo. Serious adverse events were reported more frequently during the treatment period in patients receiving RAPAMUNE compared to the placebo group.

“I am thrilled for families living with LAM,” said Dr. Francis X. McCormack, director of Pulmonary, Critical Care and Sleep Medicine at the University of Cincinnati College of Medicine and lead investigator of the MILES Trial. “The courage of the women who enrolled in the MILES trial made this possible. I am proud of the 200 investigators, coordinators and nurses who participated in the MILES trial that enabled FDA approval, and I sincerely thank all who supported the study.”

Pfizer worked with the FDA, Dr. McCormack and the LAM Foundation to evaluate RAPAMUNE as a treatment option for LAM in the United States. The MILES trial was conducted by Dr. McCormack and conducted within the National Institutes of Health 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.

“This approval is a landmark breakthrough for LAM patients to have access to this important treatment option," said Susan E. Sherman, executive director of the LAM Foundation. "It is the result of decades of work by researchers and women of the LAM community who volunteered for this pivotal clinical trial.”

About RAPAMUNE

RAPAMUNE is an immunosuppressive agent indicated in the United States for the prophylaxis of organ rejection in patients aged 13 years and older receiving kidney transplants. It is prescribed to kidney transplant patients to help prevent their natural immune system from rejecting the transplanted kidney. RAPAMUNE is also indicated for the treatment of patients with LAM. Therapeutic drug monitoring is recommended for all patients receiving RAPAMUNE. RAPAMUNE has a boxed warning for infections and certain cancers. Please see the full prescribing information, including boxed warning and full

indications at www.pfizer.com/products/product-detail/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.

The most common side effects of RAPAMUNE in people with renal transplant 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), and high blood sugar (diabetes).

The most common side effects of RAPAMUNE in people with LAM include mouth sores, diarrhea, stomach pain, nausea, sore throat, acne, chest pain, upper respiratory tract infection, headache, dizziness, and sore muscles.

Tell your doctor if you have any side effect that bothers you or that does not go away.

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 more than 20 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 May 29, 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 RAPAMUNE and about an indication in the U.S. for the treatment of lymphangioleiomyomatosis (the "Indication"), including their potential benefits, 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 regarding the commercial success of RAPAMUNE and the 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, 2014 and in its subsequent reports on Form 10-Q, including in the sections thereof captioned "Risk Factors" and "Forward-Looking Information and Factors 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 andwww.pfizer.com.

Media:Steven DanehyO: 212-733-1538E: Steven.Danehy@pfizer.comorInvestor:Ryan CroweO: 212-733-8160E: Ryan.Crowe@pfizer.com