



December 14, 2015

Horizon Pharma plc and Fox Chase Cancer Center Temple Health Initiate Phase 1 Study to Evaluate ACTIMMUNE(R) (interferon gamma-1b) in Combination With Nivolumab in Patients With Advanced Solid Tumors

DUBLIN, IRELAND -- (Marketwired) -- 12/14/15 -- Horizon Pharma plc (NASDAQ: HZNP), a biopharmaceutical company focused on improving patients' lives by identifying, developing, acquiring and commercializing differentiated and accessible medicines that address unmet medical needs, today announced that it, in collaboration with Fox Chase Cancer Center Temple Health, has initiated a Phase 1 clinical study to evaluate ACTIMMUNE (interferon gamma-1b) in combination with nivolumab in advanced solid tumors.

"We are eager to explore the safety and efficacy of ACTIMMUNE in combination with nivolumab in multiple, difficult-to-treat cancers that are often inoperable or have become unresponsive to standard therapies," said Jeffrey W. Sherman, M.D., FACP, executive vice president, research and development and chief medical officer, Horizon Pharma plc. "Through this study, we hope to find that the addition of ACTIMMUNE to PD-1 inhibitors, like nivolumab, could potentially lead to better patient outcomes and inform our strategy for future ACTIMMUNE indications to pursue in Phase 2 trials."

The Phase 1, open label study will evaluate the combination of ACTIMMUNE and nivolumab in patients with advanced solid tumors who have progressed on at least one prior systemic therapy, which may include prior immunotherapy. Patients will be treated with a one week induction phase of ACTIMMUNE (starting dose 50 mcg/m² subcutaneously), followed by a combination phase with ACTIMMUNE and nivolumab (3 mg/kg intravenously) for three cycles, followed by a single-agent phase of nivolumab alone for up to one year.

"Nivolumab and other PD-1 inhibitors have demonstrated clinical benefit in multiple trials across many advanced solid tumor types," said Elizabeth R. Plimack, MD, MS, associate professor of medical oncology, and director of Genitourinary Clinical Research at Fox Chase. "However, some patients benefit from these therapies while others do not. Based on work from Fox Chase and other laboratories, we have strong evidence to suggest that ACTIMMUNE may increase the proportion of patients for whom nivolumab is effective. We are excited to test this hypothesis as part of this Phase 1 clinical trial."

The study will primarily assess the safety and tolerability of the combination of ACTIMMUNE and nivolumab. Secondary objectives, including overall response rate, progression free survival and overall survival, will also be assessed, as will various correlative analyses. Initial accrual will occur using a modified 6+6 design, and if endpoints for safety (using dose-limiting toxicity criteria) are met, expansion cohorts in renal cell carcinoma (kidney cancer) and urothelial carcinoma (bladder cancer) are planned for up to 15 patients per cohort.

Additional detail about the study can be found at ClinicalTrials.gov.

About ACTIMMUNE®

ACTIMMUNE (interferon gamma-1b) is a biologically manufactured protein similar to one the body makes naturally to help prevent infection. ACTIMMUNE is currently approved by the U.S. Food and Drug Administration (FDA) for use in two rare diseases. It is indicated to reduce the frequency and severity of serious infections associated with Chronic Granulomatous Disease (CGD), a genetic disorder that affects the functioning of some cells of the immune system. In addition, ACTIMMUNE is indicated to slow the worsening of severe, malignant osteopetrosis (SMO), a genetic disorder that affects normal bone formation. For more information, please see www.ACTIMMUNE.com.

Indications and Usage

Chronic Granulomatous Disease (CGD)

ACTIMMUNE is approved by the US Food and Drug Administration to reduce the frequency and severity of serious infections associated with Chronic Granulomatous Disease. CGD is a genetic disorder that affects the functioning of some cells of the immune system.

Severe, Malignant Osteopetrosis (SMO)

ACTIMMUNE is approved by the US Food and Drug Administration to slow the worsening of severe, malignant osteopetrosis. SMO is also a genetic disorder that affects normal bone formation.

Important Safety Information (ISI)

The most common side effects with ACTIMMUNE are "flu like" symptoms, such as fever, headache, chills, myalgia (muscle pain), or fatigue, which may decrease in severity as treatment continues. Bedtime administration of ACTIMMUNE may minimize these symptoms. Acetaminophen may be helpful in preventing fever and headache.

ACTIMMUNE can cause severe allergic reactions and/or rash. Do not use ACTIMMUNE if you are allergic to interferon-gamma, E. coli-derived products, or any component of the product. (See Full Prescribing Information for a list of components). If you develop a serious reaction to ACTIMMUNE discontinue it immediately and contact your doctor or seek medical help.

At high doses, ACTIMMUNE can cause (flu-like) symptoms, which may worsen some pre-existing heart conditions. Tell your doctor if you have a cardiac condition, such as irregular heartbeat, heart failure, or decreased blood flow to your heart.

ACTIMMUNE may cause reversible changes to your nervous system, including decreased mental status, walking disturbances, and dizziness. Tell your doctor if you have a history of seizures or other neurological disorders.

Bone marrow function may be suppressed with ACTIMMUNE and decreased production of cells important to the body may occur. This effect, which can be severe, is usually reversible when the drug is discontinued or the dose is reduced. Tell your doctor if you have, or have had reduced bone marrow function. Your doctor will monitor these cells with blood tests at the beginning of therapy and at 3 month intervals thereafter.

Taking ACTIMMUNE may cause reversible changes to your liver function, particularly in patients less than one year old. Your doctor will monitor your liver function at the beginning of therapy and at 3 month intervals thereafter.

If you are pregnant or plan to become pregnant or plan to nurse you should consult your physician.

If you are receiving ACTIMMUNE at home, your doctor will provide to you or your caregiver appropriate instructions on the administration of the drug and disposal of the container, needles and syringes.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch, or call 1-800-FDA-1088.

This information is not intended to replace discussions with your doctor. For additional information about ACTIMMUNE, please consult the Full Prescribing Information and the Information for the Patient/Caregiver and talk to your doctor. ACTIMMUNE is available by prescription only.

Visit www.ACTIMMUNE.com to download a copy of the ACTIMMUNE Full Prescribing Information.

About Horizon Pharma plc

Horizon Pharma plc is a biopharmaceutical company focused on improving patients' lives by identifying, developing, acquiring and commercializing differentiated and accessible medicines that address unmet medical needs. The Company markets seven medicines through its orphan, primary care and specialty business units. Horizon's global headquarters are in Dublin, Ireland. For more information, please visit www.horizonpharma.com. Follow [@HZNPplc](https://twitter.com/HZNPplc) on Twitter or view careers on our [LinkedIn](#) page.

About Fox Chase Cancer Center

Fox Chase Cancer Center, part of the Temple University Health System, is one of the leading cancer research and treatment centers in the United States. Founded in 1904 in Philadelphia as one of the nation's first cancer hospitals, Fox Chase was also among the first institutions to be designated a National Cancer Institute Comprehensive Cancer Center in 1974. Fox Chase researchers have won the highest awards in their fields, including two Nobel Prizes. Fox Chase physicians are also routinely recognized in national rankings, and the Center's nursing program has received the Magnet recognition for excellence four consecutive times. Today, Fox Chase conducts a broad array of nationally competitive basic, translational, and clinical research, with special programs in cancer prevention, detection, survivorship, and community outreach. For more information, call 1-888-FOX CHASE or (1-888-369-2427).

Forward Looking Statements

This press release contains forward-looking statements, including statements regarding the scope and design of the Phase 1 clinical evaluating ACTIMMUNE in solid tumors, potential initiation of expansion cohorts evaluating ACTIMMUNE in urothelial carcinoma (bladder cancer) and renal cell carcinoma, and the potential benefits of ACTIMMUNE in combination with PD-1 inhibitors. Forward-looking statements speak only as of the date of this press release and Horizon does not undertake any obligation to update or revise these statements, except as may be required by law. These forward-looking statements are based on management's expectations and assumptions as of the date of this press release and actual

results may differ materially from those in these forward-looking statements as a result of various factors. These factors include, but are not limited to, risks regarding whether Horizon will be able to continue its collaboration with Fox Chase Cancer Center, whether the parties will comply with their obligations under the collaboration, whether the Phase 1 clinical trial will proceed as anticipated or whether any expansion cohorts will be initiated, whether Horizon will conduct further studies of ACTIMMUNE or have the financial resources to do so, and the risks associated with pre-clinical and clinical development of drug candidates, including unexpected safety and efficacy results. For a further description of these and other risks facing Horizon, please see the risk factors described in Horizon's filings with the United States Securities and Exchange Commission, including those factors discussed under the caption "Risk Factors" in those filings. Forward-looking statements speak only as of the date of this press release and Horizon undertakes no obligation to update or revise these statements, except as may be required by law.

Contacts:

Investors:

John Thomas
Executive Vice President, Strategy and Investor Relations
investor-relations@horizonpharma.com

Tina Ventura
Vice President, Investor Relations
investor-relations@horizonpharma.com

U.S. Media:

Geoff Curtis
Group Vice President, Corporate Communications
gcurtis@horizonpharma.com

Ireland Media:

Ray Gordon
Gordon MRM
ray@gordonmrm.ie

Source: Horizon Pharma plc

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