Astellas announces isavuconazole orphan drug designation by U.S. FDA

Tokyo, May 28, 2012 – Astellas Pharma Inc. (Tokyo:4503) announced today that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation to isavuconazole for the treatment of invasive aspergillosis.

An FDA orphan drug designation provides several benefits to the sponsor including a seven-year market exclusivity from product approval in the U.S. Isavuconazole has been previously granted FDA fast track status designed to facilitate the development and expedite the review of drugs to treat serious diseases and fill an unmet medical need in order to get important new drugs to patients earlier.

"Invasive fungal infections are life-threatening diseases primarily afflicting immunocompromised patients Preclinical and early clinical data suggest that isavuconazole may provide an alternative treatment for patients suffering from these conditions" said Bernie Zeiher, Immunology/Infectious Disease/Transplant Therapeutic Area Head of Astellas Pharma Global Development Inc.

Topline data from two isavuconazole phase 3 studies are expected in the second half of 2013. These include the SECURE phase 3 registration study, evaluating safety and efficacy of once-daily isavuconazole versus twice-daily voriconazole for the primary treatment of invasive fungal disease caused by *Aspergillus* species and from the open-label VITAL study investigating isavuconazole for the treatment of patients with invasive life-threatening fungal disease caused by emerging fungi and the treatment of aspergillosis patients with pre-existing renal impairment.

The isavuconazole ACTIVE phase 3 study, evaluating the use of isavuconazole i.v. and oral versus caspofungin i.v. followed by oral voriconazole for the treatment of invasive Candida infections, will continue to recruit into 2014.

Invasive aspergillosis is estimated to occur in 5-13 % of recipients of bone marrow transplants, 5-25 % of patients who have received heart or lung transplants, and 10-20 % of patients who are receiving intensive chemotherapy for leukemia.[1] Mortality rates for transplant patients with invasive aspergillosis have been reported to be between 34 and 58 %.[2]

About isavuconazole

Isavuconazole is an investigational intravenous and oral broad-spectrum antifungal. In collaboration with Basilea Pharmaceutica Ltd., isavuconazole is being investigated in phase 3 clinical studies for the treatment of severe invasive fungal infections. The drug demonstrated *in-vitro* and *in-vivo* coverage of a broad range of yeasts (such as *Candida* species) and molds (such as *Aspergillus* species) as well as *in-vitro* activity against less prevalent but often fatal molds including those that cause mucormycosis. In clinical studies to date, isavuconazole achieved predictable drug levels supporting reliable dosing and a switch from intravenous administration to a once-daily oral dose. The intravenous formulation of isavuconazole does not contain potentially kidney damaging solubilizers and has the potential to be given also to patients with pre-existing renal impairment.

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For inquiries or additional information
Astellas Pharma Inc. Corporate Communications TEL: +81-3-3244-3201, FAX:+81-3-5201-7473 http://www.astellas.com/en

References

1 Harman EM, Medscape Reference, Drugs, Diseases & Procedures, Aspergillosis Clinical Presentation, http://emedicine.medscape.com/article/296052-overview

2 Baddley JW et al. Factors Associated with Mortality in Transplant Patients with Invasive Aspergillosis. Clinical Infectious Disease 2010 (50),1559-1567