SymBio Pharmaceuticals Limited

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SymBio Pharmaceuticals Limited
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# Clinical Trial Notification Accepted by Japanese Regulatory Agency for SymBio's Phase I Oral Rigosertib (SyB C-1101) Trial in Frontline MDS

TOKYO, Japan, December 27, 2012 – SymBio Pharmaceuticals Limited (JASDAQ: 4582) announced that clinical trial notification (CTN) has been accepted by the Japanese Pharmaceuticals and Medical Devices Agency (PMDA) for its Phase I oral *rigosertib* (SyB C-1101) clinical trial in frontline myelodysplastic syndrome (MDS) patients. This CTN enables SymBio to proceed with clinical trials and is comparable to an Investigational New Drug (IND) application in the U.S.

Under the terms of a license agreement with Onconova Therapeutics, Inc. signed July, 2011, SymBio has an exclusive license to develop and commercialize *rigosertib* in Japan and Korea. *Rigosertib* is a novel, patent protected inhibitor of PI3-Kinase and PLK pathways in cancer cells.

Patients with MDS often have severe anemia (decrease in number of red blood cells) or low platelet counts, and have a high rate of progression to acute myelogenous leukemia (AML). The prevalence of MDS is estimated to be 11,000 patients in Japan alone. Currently, there is no approved therapy for MDS patients after they fail frontline therapy with hypomethylating agents. As such, there is an urgent need to develop therapies to treat refractory/relapsed and frontline MDS.

Onconova Therapeutics Inc. is currently conducting late-stage clinical trials with *rigosertib* (ON 01910.Na) in the U.S., Europe and India for the treatment of hematologic malignancies and solid tumors. A pivotal trial in refractory/relapsed MDS is underway using the intravenous (IV) formulation of *rigosertib*. The U.S. FDA has granted orphan drug designation for *rigosertib* in MDS and has agreed to a Special Protocol Assessment (SPA) for this Phase III trial. Onconova is also developing an oral formulation of *rigosertib* as a frontline treatment for MDS.

In addition to the Phase I trial with oral *rigosertib* (SyB C-1101) in frontline MDS, a Phase I trial in refractory/relapsed MDS patients using the IV formulation of the drug is underway in Japan. SymBio is developing *rigosertib* expeditiously in order to make this innovative drug available to patients in need.

**[Please see the following for further information on MDS, *Rigosertib*, and Onconova ]**

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**Note to Editors**

**About Myelodysplastic Syndrome (MDS)**

MDS represents a group of diverse myeloid (bone marrow) stem cell disorders that gradually affect the ability of bone marrow to produce normal red blood cells, white blood cells, and platelets. Blood stem cells fail to mature into healthy blood cells; and the immature blood cells, called blasts, do not function normally and either die in the bone marrow or enter the blood. A higher percent of blasts is linked to a higher likelihood of developing leukemia and poorer overall prognosis. The risk of MDS increases with age and the disease commonly affects the elderly.

**About *Rigosertib***

Rigosertib (ON 01910.Na) is a small molecule inhibitor that targets the PI3- kinases and the PLK mitotic pathways, critical pathways to the growth and survival of cancer cells. Phase I-III studies with rigosertib have been conducted at leading institutions in more than 800 patients with solid tumors and hematological cancers, including myelodysplastic syndrome (MDS) and acute myeloid leukemia (AML). A multi-site Phase III trial (ONTIME) in MDS patients is being conducted under a Special Protocol Assessment (SPA) from the U.S. Food and Drug Administration (FDA) and is being supported by an award from the Therapeutics Acceleration Program of The Leukemia and Lymphoma Society. Both the FDA and European Medicines Agency have granted Orphan Drug Designation for the use of rigosertib in MDS. The rigosertib clinical program in solid tumors is also advancing in a Phase III adaptive design trial (ONTRAC) for pancreatic cancer. ONTRAC is a Phase III, multicenter, randomized, controlled study (with an interim analysis for futility) that compares the efficacy and safety of gemcitabine alone vs. rigosertib combined with gemcitabine in patients with previously untreated metastatic pancreatic cancer.

**About Onconova Therapeutics, Inc.**

Onconova Therapeutics, based in Newtown, PA and Pennington, NJ, discovers and develops novel small molecule therapeutics directed against targets involved in signal transduction, cell-cycle, and DNA repair. The most advanced product, rigosertib (ON 01910.Na), is now in a pivotal trial being conducted under a Special Protocol Assessment (SPA) from the U.S. Food and Drug Administration (FDA) for myelodysplastic syndrome (MDS), as well as in a Phase III trial for pancreatic cancer. In addition to rigosertib, Onconova is developing two other clinical stage products: Ex-RAD® (a radioprotectant) and ON 013105 (a novel anti-cancer agent initially directed toward refractory lymphoma, including mantle cell lymphoma). For additional information, please visit http://www.onconova.com.

**About SymBio Pharmaceuticals Limited**

SymBio Pharmaceuticals Limited, based in Tokyo, Japan, was established in March, 2005, by Fuminori Yoshida, who previously served concurrently as Corporate VP of Amgen Inc. and founding President of Amgen Japan. The company's underlying corporate mission is to "deliver hope to patients in need" as it

aspires to be a leading specialty pharma in Asia Pacific dedicated to addressing underserved medical needs with focus in the areas of oncology, hematology and autoimmune. The company's lead drug candidate, bendamustine hydrochloride, has been successfully developed and launched in Japan for refractory/relapsed indolent non-Hodgkin's lymphoma (NHL) and mantle cell lymphoma. SymBio is also actively developing bendamustine in frontline indolent NHL, refractory/relapsed aggressive NHL and multiple myeloma in Japan. The product has been launched in Hong Kong, Singapore, Korea and Taiwan. For additional information, please visit our homepage at http://www.symbiopharma.com.