SymBio Pharmaceuticals Limited

March 26, 2013
SymBio Pharmaceuticals Limited
Fuminori Yoshida
Representative Director
President and Chief Executive Officer

# SymBio Initiates Japanese Phase I Trial of Oral *rigosertib* (SyB C-1101) in Frontline MDS

TOKYO, Japan, March 26, 2013 -- SymBio Pharmaceuticals Limited (JASDAQ: 4582) announced today that it commenced enrollment of its Phase I clinical trial for oral *rigosertib* (SyB C-1101) in frontline myelodysplastic syndrome (MDS) patients in Japan.

Under the terms of the license agreement concluded with Onconova Therapeutics, Inc. in 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.

MDS patients often require frequent blood transfusions due to the development of severe anemia (decrease in number of red blood cells), with a high rate of progression to acute myelogenous leukemia (AML). There are an estimated 11,000 MDS patients in Japan alone. Currently there is no approved drug for patients who have experienced drug failure after frontline treatment of MDS using hypomethylating agents, and there is an urgent need to develop new therapies for the treatment of both relapsed/refractory (r/r) and frontline MDS.

Onconova Therapeutics, Inc. is currently conducting late-stage clinical trials with *rigosertib* (ON 01910.Na) in the U.S. and Europe for the treatment of hematologic malignancies and solid tumors. A pivotal trial in r/r MDS is underway using the intravenous (IV) formulation of *rigosertib* (SyB L-1101). The U.S. FDA has granted orphan drug designation for the use of *rigosertib* in MDS, and has agreed to a Special Protocol Assessment (SPA) for this Phase III trial.
A pair of Phase II trials are underway in the U.S. for development of the oral formulation of *rigosertib* as frontline treatment for lower-risk MDS.

In addition to SymBio's Phase I trial with oral *rigosertib* in frontline MDS, a Phase I trial in r/r MDS patients using the IV formulation of the drug is also underway in Japan. SymBio is developing *rigosertib* expeditiously, shortening development time and making this innovative drug available to MDS patients in need.

**[Please read the following for additional information on MDS, *rigosertib*, and Onconova ]**

[Contact]
Tomoyuki Watanabe
Director, Head of Corporate Planning Division
Tel: +081(0)3 5472 1125

SymBio Pharmaceuticals Limited

**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-kinase and 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). ONTIME, a multi-site Phase III trial in r/r 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 r/r MDS. The *rigosertib* clinical program in solid tumors is also advancing in ONTRAC, a Phase III adaptive design trial 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. ONTARGET is a Phase II, randomized, two-arm study that will assess the efficacy and safety of oral *rigosertib* in transfusion-dependent low or intermediate-1 MDS patients, based on IPSS classification. Primary endpoints of the study are transfusion independence and erythroid response.

**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 company's 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 r/r 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® (recilisib, 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 disease. The company's lead drug, bendamustine hydrochloride, has been successfully developed and launched in Japan for refractory/relapsed (r/r) low-grade non-Hodgkin's lymphoma (LG-NHL) and mantle cell lymphoma. SymBio is also actively developing bendamustine in frontline LG-NHL, r/r aggressive NHL and multiple myeloma in Japan. The drug has also been launched in Hong Kong, Singapore, Korea and Taiwan. For additional information, please visit our homepage at http://www.symbiopharma.com.