SymBio Pharmaceuticals Limited

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SymBio Pharmaceuticals Limited
Fuminori Yoshida
Representative Director
President and Chief Executive Officer

Onconova Presents Phase 2 Data for Oral Rigosertib at 2013 American Society of Hematology Annual Meeting

- Oral Rigosertib (ON 01910.Na) in Transfusion-Dependent Lower Risk Myelodysplastic Syndromes (MDS)-

TOKYO, Japan, December 10, 2013 -- SymBio Pharmaceuticals Limited (Headquarters: Tokyo, "SymBio") announces that Onconova Therapeutics, Inc. (Headquarters: Newtown, PA and Pennington, NJ, "Onconova") presented favorable data from its US ONTARGET trial in transfusion-dependent lower risk myelodysplastic syndromes (MDS) patients treated with oral rigosertib (ON 01910.Na) at the 2013 American Society of Hematology (ASH) Annual Meeting (ASH Abstract #2745) in New Orleans, LA on December 8, 2013. This Phase 2 study follows the positive findings of a Phase I dose escalation study in MDS patients treated with oral rigosertib. SymBio signed a license agreement with Onconova in July, 2011, for the exclusive right to develop and commercialize both the intravenous and oral forms of rigosertib in Japan and Korea.

Azra Raza, MD, Director, MDS Center, Columbia University Medical Center, New York, NY presented study results from sixty patients enrolled in the ONTARGET study. ONTARGET is a Phase 2 study of oral rigosertib in transfusion-dependent, lower risk MDS patients. Transfusion-dependent patients must have received at least four units of red blood cells (RBC) transfusions over eight weeks prior to entering the study.

In the ONTARGET study, a combined response rate of 53% according to International Working Group criteria (IWG) was observed in 36 evaluable patients receiving the intermittent dosing schedule of rigosertib. Of the 36 evaluable patients, 14 (39%) achieved transfusion independence defined as no RBC transfusions for at least eight consecutive weeks. In these patients, rigosertib induced transfusion independence when employed as a single agent or when used in combination with erythropoiesis-stimulating agents (ESA).

The major adverse events were related to bladder toxicity and included dysuria, frequency of urination, and hematuria/cystitis. No treatment emergent myelosuppression was noted in this study. To address urinary adverse events, a modified dosing regimen with 560 mg in the morning and 280 mg in the

afternoon was tested in a cohort. Although the follow-up of this cohort is still limited, of the 13 patients receiving the new regimen only one patient reported a Grade 2+ urinary event (8%).

Employing a whole genome scan, a methylation signature comprising 50 loci was identified. This signature helped to relate transfusion independence with methylation profile in the 32 patients analyzed. A confirmation cohort of 20 additional patients is now being enrolled to further explore this potential prognostic tool.

Also, Shyamala Navada, MD, Assistant Professor, Icahn School of Medicine at Mount Sinai et al., presented a poster entitled, "Predictors of Response to Rigosertib In Patients with a Myelodysplastic Syndrome (MDS) or Acute Myeloid Leukemia (AML) Relapsed or Refractory to Hypomethylating Agents." The poster summarized results from a Phase 1/2 study of rigosertib in patients with MDS and AML who had failed treatment with hypomethylating agents.

All 22 patients enrolled in this study had been previously treated with hypomethylating agents. Thirteen had AML, eight had MDS and one had chronic myelomonocytic leukemia (CMML). Of the 19 evaluable patients in this trial, 10 (53%) demonstrated either a reduction/stabilization in bone marrow blasts or improvement in their peripheral blood counts. Further, the median overall survival of these patients was 9.6 months versus 1.7 months for non-responders (p=0.001), thus suggesting a clear correlation between bone marrow blast response and increased overall survival following treatment with rigosertib. Overall, IV rigosertib was well tolerated with urinary events reported in 9 of 19 patients.
(Please visit to Onconova's website http://www.onconova.com to get more detail information)

Rigosertib is being developed in both oral and intravenous forms. Onconova is conducting late-stage clinical trials with rigosertib in the U.S., Europe and India for the treatment of MDS and solid tumors. Onconova announced on May 9, 2013, that it had reached its enrollment goal of 270 for its randomized, controlled pivotal Phase 3 ONTIME trial of rigosertib in patients with myelodysplastic syndromes (MDS) who have failed prior therapy with the hypomethylating agents azacitidine or decitabine. Onconova is also evaluating intravenous rigosertib in a Phase 3 trial (ONTRAC) for first-line treatment in combination with gemcitabine for patients with metastatic pancreatic cancer who have not previously received any chemotherapy.

In Japan, two Phase 1 trials are currently underway by SymBio Pharmaceuticals in relapsed or refractory high-risk MDS patients using the intravenous formulation of the drug (SyB L-1101), and in frontline low-risk MDS patients using the oral formulation of rigosertib (SyB C-1101). SymBio is striving to ensure that this much-needed drug is made available to MDS patients in Japan as soon as possible.

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

SymBio Pharmaceuticals Limited

[Contact]
Takashi Shimomura
Executive Vice President, CFO, and Head of Corporate Div.
Tel: +081(0)3 5472 1125

Note to Editors

About Myelodysplastic Syndromes (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 is an inhibitor of two important cellular signaling pathways, phosphoinositide 3-kinase, or PI3K, and polo-like kinase, or PLK, both of which are frequently activated in cancer cells. Rigosertib is being developed in both oral and intravenous forms as a treatment for hematological diseases and solid tumors. Onconova recently announced reaching the enrollment goal in its randomized, controlled ONTIME Phase 3 Trial for intravenous rigosertib in adult patients with myelodysplastic syndromes whose disease has failed azacitidine or decitabine therapy. Rigosertib is also being evaluated in a Phase 3 trial for first-line treatment in combination with gemcitabine for patients with metastatic pancreatic cancer who had not previously received any chemotherapy. The oral form of rigosertib is currently being studied in Phase 2 trials in patients with transfusion-dependent lower risk myelodysplastic syndromes and in patients with head and neck cancer. Rigosertib has been granted orphan drug status for MDS in both the United States and Europe, as well as orphan drug status for pancreatic cancer in the United States. Rigosertib is being developed in partnership with Baxter International (commercialization rights in Europe) and SymBio Pharmaceuticals (Japan and Korea). Onconova has retained all other territories for commercialization.

About Onconova Therapeutics, Inc.

Onconova Therapeutics is a clinical-stage biopharmaceutical company focused on discovering and developing novel products to treat cancer. Onconova's clinical and pre-clinical stage drug development candidates are derived from its extensive proprietary chemical library and are designed to work against specific cellular pathways that promote cancer while causing minimal damage to normal cells. In addition to rigosertib, the Company’s most advanced product candidate, two other candidates are in clinical trials, and several candidates are in pre-clinical stages. For more information, please visit http://www.onconova.com.

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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 main 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 chronic lymphocytic leukemia 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.