

August 25, 2020  
SymBio Pharmaceuticals Limited  
Fuminori Yoshida  
Representative Director  
President and Chief Executive Officer  
(Securities Code: 4582)

## **Onconova Therapeutics Announces Topline Results from the Pivotal Phase 3 INSPIRE Trial for Higher-Risk Myelodysplastic Syndromes (HR-MDS)**

TOKYO, Japan, August 25, 2020 -- SymBio Pharmaceuticals Limited (Headquarters: Tokyo, "SymBio") today announced that its U.S. licensor of rigosertib, Onconova Therapeutics, Inc. (Headquarters: Newtown, PA, "Onconova"), a Phase 3 stage biopharmaceutical company focused on discovering and developing novel products to treat cancer, announced on August 24, 2020 (EST) that INSPIRE, the pivotal Phase 3 study assessing the efficacy and safety of IV rigosertib in higher-risk MDS (HR-MDS) patients, did not meet its primary endpoint of improved survival.

The primary endpoint of the trial was overall survival, comparing IV rigosertib plus best supportive care to physician's choice (PC) plus best supportive care in patients who had progressed on, failed to respond to, or relapsed after previous treatment with a hypomethylating agent (HMA) within nine cycles over the course of one year after initiation of HMA treatment. A pre-specified analysis in the very high risk (VHR-MDS) patient subgroup was also conducted.

Results of INSPIRE demonstrated that in the intent-to-treat analysis patients randomized to IV rigosertib achieved overall survival of 6.4 months, versus 6.3 months for PC ( $p=0.33$ ) in the overall HR-MDS population. Overall survival in the pre-specified VHR-MDS subgroup of patients was also not significantly different between the two study arms. There was an increase in overall survival in the PC arm post-interim analysis that was unexpected. Onconova is conducting additional analyses.

Safety analysis indicates that IV rigosertib was generally well tolerated, with reported adverse events similar to those observed in previous clinical studies with IV rigosertib in MDS. Serious adverse events (SAEs) were uncommon, with a similar profile of SAEs in both study arms.

Statement from Fuminori Yoshida, President and Chief Executive Officer of SymBio: "While the results of the INSPIRE study are disappointing, we believe that rigosertib as a RAS pathway inhibitor has potential applications in many other oncological areas beyond hematological diseases, and we look forward to using the findings from the genomic analysis of the INSPIRE study in the

future development of rigosertib. In addition to the ongoing Phase 1/2a trial in KRAS+ lung adenocarcinoma, we look forward to continuing to expand the study to other solid tumors."

SymBio does not expect the information presented herein to have any material impact on its financial outlook for the fiscal year ending December 2020.

### **About Rigosertib**

Rigosertib is a small molecule inhibitor that has a new mechanism of action: it inhibits the activation of RAS as an oncogene-related product, thereby blocking the action of multikinases, including PI3K, and inhibits cellular signaling in cancer cells necessary for their survival and proliferation, thus killing cancer cells. SymBio obtained the development and commercialization licensing rights for Rigosertib from Onconova in July 2011 for Japan and Korea.

### **About Onconova Therapeutics, Inc.**

Onconova Therapeutics, Inc. is a Phase 3-stage biopharmaceutical company focused on discovering and developing novel drugs to treat cancer. Onconova has a pipeline of proprietary targeted agents designed to work against specific cellular pathways that are important in cancer cells. Clinical trials with Onconova's compound rigosertib are aimed at what Onconova believes are unmet medical needs of patients with cancer. For more information, please visit <https://www.onconova.com>.

### **About SymBio Pharmaceuticals Limited**

SymBio Pharmaceuticals Limited was established in March 2005 by Fuminori Yoshida who previously served concurrently as Corporate VP of Amgen Inc. and founding President of Amgen Japan. In May 2016, the Company incorporated its wholly-owned subsidiary in the U.S., SymBio Pharma USA, Inc. (Headquarters: Menlo Park, California, President: Mr. Fuminori Yoshida). The Company's underlying corporate mission is to "deliver hope to patients in need" as it aspires to be a leading global specialty biopharmaceutical company dedicated to addressing underserved medical needs.