Orbus Therapeutics Announces Positive Outcome of Pre-Planned Interim Futility Analysis for Phase 3 Eflornithine STELLAR Study

- Independent Data Monitoring Committee recommends continuation of STELLAR study in rare brain cancer -

PALO ALTO, Calif., Mar. 2, 2021 – Orbus Therapeutics Inc., a private, late-stage biopharmaceutical company focused on the development and commercialization of therapies that treat rare diseases, today announced a positive outcome of the pre-planned interim analysis for futility in its Phase 3 STELLAR clinical study of eflornithine in patients with recurrent anaplastic astrocytoma.

The pre-planned futility analysis was conducted by Orbus' Independent Data Monitoring Committee (IDMC), an independent panel of experts that periodically monitors the safety and efficacy of the STELLAR study. The study reached a pre-specified number of events required for the futility analysis. For this pre-planned interim analysis for futility, the IDMC's recommendation to continue the study was to be based upon the hazard ratio in the STELLAR study being less than one. Based on its review of the efficacy and safety data, the IDMC recommended that the STELLAR study proceed without any modifications.

"We are very pleased with the STELLAR study successfully passing this important milestone and are looking ahead to significant continued progress over the next 12 to 18 months," said Bob Myers, co-founder and CEO. "We expect to complete full enrollment of patients in the STELLAR study in the second half of 2021, and our next pre-planned interim analysis, which will test superiority, is expected to occur in 2022. This pre-planned interim analysis for superiority will provide the first opportunity in the STELLAR study to determine if the investigational arm, combining eflornithine and lomustine, is out-performing the control arm of lomustine as a single agent."

About the STELLAR study

The STELLAR study, a Phase 3, Randomized, Open-Label **S**tudy **T**o Evaluate the Efficacy and Safety of **E**flornithine and **L**omustine Compared to **L**omustine Alone in Patients with Anaplastic **A**strocytoma That Progress/**R**ecur After Irradiation and Adjuvant Temozolomide Chemotherapy, started in late 2016 and has involved more than 85 leading clinical trial centers in eight countries in North America and Europe. The trial is designed to evaluate the efficacy and safety profile of eflornithine in combination with lomustine compared to lomustine alone in patients with anaplastic astrocytoma that recurs after surgery, irradiation and adjuvant temozolomide chemotherapy.

The Company plans to enroll approximately 340 patients into the STELLAR study. The primary efficacy endpoint in the STELLAR study is the duration of overall survival (OS). Secondary pre-specified efficacy endpoints include OS in isocitrate dehydrogenase (IDH) mutant and wild type sub-populations, progression free survival (PFS) and objective response rate (ORR). Find more information about the STELLAR study here.

About Anaplastic Glioma and Anaplastic Astrocytoma
Several brain tumor types are grouped together under the name glioma which originates in the glial cells that surround and support neurons in the brain. In the United States, greater than 3,600 new cases of anaplastic glioma, one of two categories of malignant glioma, are diagnosed each year with a median survival of just over three years despite treatment with surgery, radiation and chemotherapy. The prevalence of anaplastic astrocytoma in the United States is estimated to be just over 9,000 people. Anaplastic astrocytoma is the largest subset of anaplastic glioma, and represents approximately 75 percent of anaplastic glioma patients. Anaplastic astrocytomas typically require aggressive treatment and, due to tentacle-like projections that grow into surrounding tissue, are difficult to completely remove during surgery. It is estimated that there are more than 3,300 new anaplastic astrocytoma cases diagnosed in the United States each year.

About Eflornithine
Eflornithine is a novel cytostatic agent that irreversibly inhibits ornithine decarboxylase, a key enzyme in mammalian polyamine biosynthesis that is up-regulated in certain types of cancer. In controlled, randomized and single arm clinical studies, eflornithine has shown an increase in overall survival of patients with newly diagnosed or recurrent anaplastic astrocytoma.

Eflornithine has been granted Orphan Drug Designation and Breakthrough Therapy Designation for the treatment of patients with anaplastic glioma by the U.S. Food and Drug Administration (FDA), and has also been granted Orphan Medicinal Product status for the treatment of glioma by the Committee for Medicinal Products for Human Use (CHMP) at the European Medicines Agency (EMA).

About Orbus Therapeutics
Orbus Therapeutics Inc. is a late-stage, private biopharmaceutical company that is dedicated to developing products that treat rare diseases for which there are few, if any, effective therapies. The Company's lead product candidate, eflornithine, is being evaluated in a pivotal Phase 3 clinical trial in patients with recurrent anaplastic astrocytoma, a rare form of central nervous system cancer. For more information, please visit the Company's website at http://www.orbustherapeutics.com

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