For Immediate Release

Beirut: 2-4-2020

Dr. Ali Taher from AUB:

"Breakthrough clinical trial with global health impact"

Results from a clinical trial by American University of Beirut (AUB) Professor of Medicine, Hematology, and Oncology, Dr. Ali Taher and a multicenter international team has recently been published in the New England Journal of Medicine. The publication reports the benefits of Luspatercept for patients with transfusion-dependent β-thalassemia (TDT), which subsequently led to the FDA approval of this breakthrough treatment.

"Transfusion-dependent β-thalassemia patients require lifelong regular transfusion therapy for survival" explained Dr. Ali Taher, director of the Naef K. Basile Cancer Institute at AUBMC; vice chair of research at AUBMC's Department of Internal Medicine; and founding director of the Fellowship and Residents Research Program at AUB's Faculty of Medicine. "Although the disease was historically confined to the Mediterranean Basin, Sub-Saharan Africa, and the Middle East, continued migration translated into a higher number of patients in the US and Europe. Thalassemia is now considered a global health burden, owing to its associated healthcare needs and costs."

Dr. Taher further explained that thalassemia is an inherited blood disorder in which the body makes a significantly reduced amount of hemoglobin. In severe forms of thalassemia, patients become transfusion-dependent since early childhood and cannot thrive or survive without lifelong regular transfusions which are usually administered on a monthly basis, thus posing a significant public health concern.

The trial evaluated the ability of Luspatercept—a recombinant fusion protein that binds transforming growth factor β superfamily ligands—in reducing transfusion requirement in TDT patients. The aim behind the trial was to improve the quality of life and mitigate the risks of chronic transfusion therapy, including secondary iron overload and associated morbidity and

mortality. In the larger picture, this would also address the ongoing global challenge of providing blood products, especially in resource-poor countries.

The study included a total of 336 patients assigned in a 2:1 ratio into Luspatercept or placebo groups over approximately 64 weeks. The trial met its primary endpoint showing that Luspatercept is superior to placebo in reduction of transfusion requirement, with a proportion of patients becoming transfusion-independent.

Dr. Taher, one of the main authors and highest recruiting investigators in the trial, has spent decades of research trying to improve outcomes in this patient population by optimizing standards of care through leadership of international management guidelines as well as large-scale multicenter trials investigating novel therapies that address the underlying pathophysiology. Several of his trials led to drug approvals that transformed the patient journey and the way we understand the disease today.

The clinical development of Luspatercept is still ongoing, as it is now being evaluated for its ability to raise haemoglobin level and improve patient-reported outcomes even in patients who are non-transfusion-dependent. Results from the randomized, multicenter trial are awaited, with Dr. Taher as the principal investigator.

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Note to Editors

About AUB

Founded in 1866, the American University of Beirut bases its educational philosophy, standards, and practices on the American liberal arts model of higher education. A teaching-centered research university, AUB has more than 900 full-time faculty members and a student body of about 9,100 students. AUB currently offers more than 120 programs leading to bachelor's, master's, MD, and PhD degrees. It provides medical education and training to students from throughout the region at its Medical Center that includes a full-service 420-bed hospital.

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