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FOR IMMEDIATE RELEASE

A Landmark Global Trial Led from Lebanon: Mitapivat in NTDT Published in The Lancet

In a major advancement for global thalassemia care, the results of a landmark global phase 3 clinical trial of mitapivat in non-transfusion-dependent thalassemia (NTDT) led by the American University of Beirut (AUB) and the Chronic Care Center, the thalassemia center in Lebanon, have been published in *The Lancet*, one of the world's leading medical journals.

The study, titled "Phase 3 trial of mitapivat in non-transfusion-dependent alpha- or beta-thalassemia," led by Dr. Ali Taher, professor of medicine at the Division of Hematology and Oncology, director of the Naef K. Basile Cancer Institute, and consultant at the Chronic Care Center served as both global principal investigator and first and corresponding author. It is the first to demonstrate the efficacy of an oral, disease-modifying therapy for both beta and alpha forms of NTDT, positioning mitapivat as a potential game changer in the treatment of this lifelong blood disorder.

"This publication is more than a scientific milestone," said Dr. Taher. "It's proof that Lebanon, even in its hardest times, can lead groundbreaking medical advances that change global standards of care. I'm proud this trial was conceived, led, and delivered from AUB and the Chronic Care Center."

NTDT is a lifelong inherited blood disorder that results in chronic anemia. Patients with NTDT do not require blood transfusions, but still suffer from chronic anemia and associated complications, including iron overload and reduced quality of life. Until now, therapeutic options for these patients have been limited.

Mitapivat is currently under review by the United States Food and Drug Administration (FDA) for potential marketing authorization. If approved, it would become the first FDA-authorized oral

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disease-modifying treatment for NTDT, offering a transformative option for thousands of patients worldwide.

The phase 3 trial met all of its primary and secondary endpoints, demonstrating statistically and clinically significant improvements in hemoglobin levels, alongside favorable safety and tolerability profiles.

This work represents the culmination of over a decade of research, beginning with redefining the disease and understanding the morbidity and mortality risks of untreated anemia and iron overload. It builds on prior global efforts led from AUB that resulted in the approval of key therapies, including iron chelation through the THALASSA trial and luspatercept through the BEYOND trial. Both studies were led by Dr. Ali Taher, whose vision and leadership have been instrumental in transforming the global approach to thalassemia care. Widely recognized as one of the foremost experts in the field, Dr. Taher has played a central role in advancing treatment standards and improving outcomes for patients worldwide. His long-standing commitment to thalassemia research has not only shaped the current therapeutic landscape, but has also positioned Lebanon and AUB at the forefront of international medical innovation.

This achievement serves as a powerful affirmation of AUB's role as a beacon of academic and clinical excellence, firmly committed to advancing health through research, education, and patient-centered innovation. It also reflects the resilience and determination of Lebanon's medical and scientific communities to contribute meaningfully to global health, despite the formidable challenges faced at home.

ENDS

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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 790 full-time faculty members and a student body of over 9000 students. AUB currently offers more than 140 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 365-bed hospital.

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