

THE AMERICAN UNIVERSITY OF BEIRUT MEDICAL CENTER

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The American University of Beirut Medical Center leads on groundbreaking research marking significant advancement in treatment of thalassemia

Dr. Ali Taher serves as lead investigator driving success of breakthrough study

The American University of Beirut Medical Center (AUBMC) has played a pivotal role in a significant milestone in thalassemia research, offering new hope for patients with non-transfusion dependent (NTD) thalassemia. As the institution's leading expert, Dr. Ali Taher, professor of medicine at the Division of Hematology and Oncology, Department of Internal Medicine; director of the Naef K. Basile Cancer Institute; and consultant at the Chronic Care Center (the thalassemia center in Lebanon), spearheaded the successful completion of the global Phase 3 ENERGIZE study of a new oral medication called mitapivat. The groundbreaking study, conducted by Agios Pharmaceuticals, Inc., in collaboration with the Chronic Care Center, represents the first clinical trial in NTD thalassemia patients of all genotypes to demonstrate benefits on anemia caused by an increase in hemoglobin levels, and improved quality of life, as measured by the FACIT-Fatigue Score

These compelling results underscore the potential of mitapivat to emerge as the first oral therapy for all NTD thalassemia patients, encompassing individuals with both alpha- and beta-thalassemia. Notably, there are currently no approved oral treatments available for NTD thalassemia patients. The prospect of mitapivat as an oral therapeutic option holds significant promise in addressing the unmet medical needs of this patient population, marking a substantial advancement in the pursuit of effective and accessible treatments for individuals living with thalassemia.

“The results of the ENERGIZE study support the potential of mitapivat to be the first oral therapy for all NTD thalassemia patients, including those with alpha- or beta-thalassemia,” says Dr. Taher. “For NTD thalassemia patients across the globe, there are currently no approved oral treatments, and NTD thalassemia has consistently been associated with morbidity and mortality if left untreated. NTD thalassemia represents over half of clinically significant forms of thalassemia, so there is a tremendous unmet need. Based on the data reported to date, mitapivat has the potential to be a foundational treatment option for the thalassemia community.”

Echoing the same sentiment, Dr. Sarah Gheuens, MD, PhD, chief medical officer and head of R&D at Agios, stated, “The results of the Phase 3 ENERGIZE study underscore the potential of mitapivat to be a meaningful treatment option for adults with NTD alpha- or beta-thalassemia. All subgroup analyses favored the mitapivat treatment arm compared to placebo. We are grateful to all the patients who participated in this trial, our collaborators, study investigators, and advisors in the patient and clinical communities for their partnership in achieving this milestone. These data bring us one step closer to a treatment for all thalassemia patients, and we look forward to the ENERGIZE-T readout mid-year.”

Dr. Taher's expertise and leadership have been instrumental in driving the success of this pivotal study. His ongoing contributions to AUBMC have played a significant role in advancing the understanding and treatment of thalassemia, reflecting the institution's commitment to groundbreaking and globally-relevant research.

The collaboration between AUBMC and Agios Pharmaceuticals, Inc. continues to yield significant advancements in thalassemia research, as the outcomes of this study hold promise for addressing the unmet medical needs of patients with NTD thalassemia.

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About AUBMC

Since 1902, AUBMC has been providing the highest standards of care to patients across Lebanon and the region. It is also the teaching hospital for the Faculty of Medicine at AUB (established in 1867), which has trained generations of medical students and physicians, and whose graduates can be found at leading institutions around the world. AUBMC is the only medical institution in the Middle East to have earned the five international accreditations of JCI, Magnet, CAP, ACGME-I and JACIE attesting to its superior standards in patient-centered care, nursing, pathology/laboratory services and graduate medical education.

The Faculty of Medicine has graduated over 4,000 medical students and physicians; the Rafic Hariri School of Nursing provides excellent education for the nursing staff, and the Medical Center meets the healthcare needs of over 360,000 patient visits annually.

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