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Takeda receives U.S. FDA Breakthrough Therapy Designation for Pevonedistat

30 July 2020 | News

First Novel Treatment Option for the Treatment of Patients with Higher-Risk Myelodysplastic Syndromes (HR-MDS) after a decade

Takeda Pharmaceutical Company Limited on 30 July 2020 announced that the U.S. Food and Drug Administration (FDA) granted Breakthrough Therapy Designation for its investigational drug pevonedistat for the treatment of patients with higher-risk myelodysplastic syndromes (HR-MDS). Pevonedistat, a first in class NEDD8-activating enzyme (NAE) inhibitor, could be the first novel treatment for HR-MDS patients in more than a decade, expanding treatment options that have so far been limited to hypomethylating agent (HMA) monotherapy alone. Even with current treatment options, outcomes for people living with HR-MDS remain poor.

The Breakthrough Therapy Designation is based on the final analysis of the Pevonedistat-2001 Phase 2 study, which evaluated pevonedistat plus azacitidine versus azacitidine alone in patients with rare leukemias, including HR-MDS. The FDA considered a number of endpoints, including overall survival (OS), event-free survival (EFS), complete remission (CR) and transfusion independence, as well as the adverse event profile. This designation signals a potential advancement in addressing the needs of people living with HR-MDS, for whom few therapies exist and the benefits are limited.

“Higher-risk MDS is associated with poor prognosis, diminished quality of life and a higher chance of transformation to acute myeloid leukemia, another aggressive cancer. The combination of pevonedistat and azacitidine is a promising therapeutic approach with the potential to be the first novel treatment advancement for higher-risk MDS in more than 10 years,” said Christopher Arendt, Head, Oncology Therapeutic Area Unit, Takeda. “We thank the FDA for recognizing pevonedistat, and the urgency to develop innovative therapies that address critical treatment needs for higher-risk MDS, a patient population with few options.”

Breakthrough Therapy Designation from the U.S. FDA is granted to accelerate the development and regulatory review of investigational drugs that are intended to treat serious or life-threatening ailments. Agents with this designation have shown preliminary clinical evidence that indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints.

Takeda presented results of the Pevonedistat-2001 trial during oral sessions at the virtual 56th American Society of Clinical Oncology (ASCO) Annual Meeting and virtual 25th European Hematology Association (EHA) Annual Congress.