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ASIA EDITION

Takeda presents Phase III Trial results in patients with amyloidosis

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TOURMALINE-AL1 is a randomized phase III clinical trial that evaluates the effect of NINLARO TM (ixazomib) in combination with dexamethasone in patients with recurrent or resistant systemic light chain (AL) amyloidosis

Japan based Takeda Pharmaceutical Company Limited announced the presentation of the results of tourmaline-AL1 trial in an oral session at the 61 to annual meeting of the American Society of Hematology (ASH). TOURMALINE-AL1 is a randomized phase III clinical trial that evaluates the effect of NINLARO TM (ixazomib) in combination with dexamethasone in patients with recurrent or resistant systemic light chain (AL) amyloidosis.

The TOURMALINE-AL1 trial did not meet the two main criteria for assessing significant improvement in the overall hematological response, as reported in June 2019. Hematologic responses were observed in 53% versus 51% of patients who received NINLARO more dexamethasone versus the doctor's choice (probability ratio of 1.10 [95% CI 0.60-2.01], p = 0.762) according to the evaluation of the Adjudication Committee (CA). The second main assessment criterion of vital organ deterioration or death at two years was not developed at the time of analysis. Other assessment criteria studied that included progression-free survival (PFS) of vital organs, hematological PFS, Time to treatment failure and time to subsequent therapy were numerically superior in the NINLARO plus dexamethasone group compared to the physician's group of choice. Takeda is committed to providing access to data for researchers in order to continue the investigation of this disease. NINLARO is not approved for the treatment of AL amyloidosis.

“AL amyloidosis is a rare disease, for which diagnosis and patient outcomes are scarce. Often, current treatments are updated with therapies that are used for multiple myeloma, ”said Angela Dispenzieri, MD, Mayo Clinic, and principal investigator and lead author of the trial. “To be a phase III study that did not meet the main assessment criteria, this essay provides interesting information for this community and future studies. It is essential to continue research and development to investigate potential treatment options for this unattended patient population. ”

"We have expectations about this opportunity to share the data from the TOURMALINE-AL1 trial," said Phil Rowlands, head of Clinical Oncology Development and Research at Takeda. "We are confident that sharing our findings with the rest of the community will help drive conversations about the need for ongoing research to address the needs that still exist in this patient population."

“There are serious unmet needs for people living with amyloidosis. AL amyloidosis is a progressive and fatal disease; Many patients receive a late diagnosis, which significantly impacts life expectancy. The challenges associated with the development of drugs for this disease make research and development of treatments essential, ”said Isabelle Lousada,

founder, president and executive director of the Amyloidosis Research Consortium. “TOURMALINE-AL1 data provide valuable insight to researchers in the selection of assessment criteria for future studies on amyloidosis and the knowledge that will provide context in future drug reviews and approvals, which will eventually help provide treatment options to patients. "