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G-011 - GLOBAL OPEN-LABEL EXTENSIÓN: 24-MONTH DATA OF PATISIRAN IN PATIENTS WITH HEREDITARY TRANSTHYRETIN-MEDIATED AMYLOIDOSIS

I. Losada[1], D. Adams[2], A. González-Duarte[3], E. Mauricio[4], T. Brannagan[5], T. Coelho[6], J. Wixner[7] and H. Schmidt[8] E. Berber, M. Sweetser, M. White, J.J. Wang and M. Polydefkis en representación del Grupo de Trabajo Alnylam Global OLE Group

[1]Hospital Son Llàtzer, Palma (Illes Balears). [2]National Reference Center for FAP (NNERF)/APHP/INSERM U 1195/CHU Bicêtre, Le Kremlin Bicêtre (France). [3]Instituto Nacional de Ciencias Médicas y Nutrición, Salvador Zubirán, Mexico City (Mexico). [4]Mayo Clinic, Jacksonville, FL (USA). [5]Department of Neurology, Columbia University, New York City, NY (USA). [6]Centro Hospitalar Universitário do Porto (Portugal). [7]Department of Public Health and Clinical Medicine, Umeå University, Umeå (Sweden). [8]University Hospital Muenster, Muenster (Germany).

Resumen

Objectives: Hereditary transthyretin-mediated (hATTR) amyloidosis, is a progressive, life-threatening disease; majority of patients develop a mixed phenotype including polyneuropathy and cardiomyopathy. Patisiran's efficacy/safety have been demonstrated in prior studies in patients with hATTR amyloidosis with polyneuropathy. Interim 24-month efficacy/safety analyses of the Global OLE study are described.

Methods: International, OLE study (NCT02510261) in patients who completed parent studies: Phase 3 APOLLO randomized to placebo (APOLLO/placebo, n = 49) or patisiran (APOLLO/patisiran, n = 137) and Phase 2 OLE (n = 25) receiving patisiran.

Results: 178/211 patients had at least 24 months of exposure as of 7Oct2019. Safety profile remained consistent. After 24 months of additional patisiran treatment in the Global OLE, durable improvement was seen for mNIS+7 (mean change [¬SEM]) in APOLLO/patisiran (-4.9 [2.1]) and Phase 2 OLE (-5.9 [2.1]) groups compared to their parent study baselines. Norfolk QOL-DN continued to show durable improvement in APOLLO/patisiran patients (-2.4 [2.4]) following 24-months treatment in OLE. In the Global OLE, APOLLO/placebo patients experienced halting of disease progression and quality of life (QOL) improvement compared to Global OLE baseline after 24 months of patisiran (mNIS+7: +0.1 [3.3], Norfolk QOL-DN: -4.1 [3.3]), although they had progressed relative to APOLLO baseline (mNIS+7: +26.3 [5.0], Norfolk QOL-DN: +15.8 [4.5]) given the progression while on placebo in APOLLO.

Discussion: Patients with long-term exposure to patisiran continue to demonstrate durability of efficacy. Despite marked progression on placebo during APOLLO, previously untreated patients continue to exhibit halting of disease progression and QOL improvement following 24 months of patisiran.

Conclusions: Patisiran demonstrates a positive benefit:risk.