Burden of Illness for Patients With Familial Amyloidotic Polyneuropathy (FAP) Begins Early and Increases With Disease Progression

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Abstract

Background - Familial amyloidotic polyneuropathy (FAP) is an inherited disorder with a high morbidity and mortality. An analysis of an ongoing study for the impact of FAP on healthcare resource utilization, quality of life, employment status, and activities of daily living (ADLs) was completed.

Methods - 28 patients with FAP were included in this analysis. Data was collected for 18 months. From each participant, history of disease manifestations and use of medications and dosing regimens were collected, including TTR genotype, disease disease penetrance and rate of progression. Thirty percent of patients were Stage I, 10% Stage II, 20% Stage IIIB, 20% Stage IIIC, and 10% Stage IV. Disease severity was classified into three levels: mild, moderate, and severe. With healthcare data, quality of life measures, employment status, and ADLs were evaluated. Median survival for Stage I, II, IIIB, IIIC, and IV patients was 5, 15, 40, 120, and 120 months, respectively. The primary endpoint was the Safety and efficacy of Patisiran (siRNA) vs of standard of care (SOC) in patients with FAP and moderate to severe polyneuropathy, enrolled in the APOLLO study.

Results - Median survival for Stage I, II, IIIB, IIIC, and IV patients was 5, 15, 40, 120, and 120 months, respectively. The primary endpoint was the Safety and efficacy of Patisiran (siRNA) vs of standard of care (SOC) in patients with FAP and moderate to severe polyneuropathy, enrolled in the APOLLO study.