Ionis statement about results from an analysis of exploratory cardiac endpoints in the Phase 3 NEURO-TTRansform study of eplontersen

On Oct. 7, Ionis announced new results from a 66-week analysis of exploratory cardiac endpoints in the Phase 3 NEURO-TTRansform study of eplontersen, an investigational treatment for hereditary transthyretin-mediated amyloid polyneuropathy (ATTRv-PN). In a pre-defined cardiac subpopulation of ATTRv-PN patients, treatment with eplontersen showed stabilization or improvement in cardiac function and structure relative to external placebo, including levels of N-terminal prohormone of brain natriuretic peptide (NT-proBNP), a measure of cardiac stress, and a trend towards improvement in echocardiographic parameters. The results were presented during a rapid-fire oral session at the 2023 Heart Failure Society of America Annual Scientific Meeting.

Transthyretin-mediated amyloid cardiomyopathy (ATTR-CM) is a systemic, progressive and fatal condition that typically leads to progressive heart failure and often death within three-to-five years from disease onset. With more than 1,400 patients enrolled, CARDIO-TTRansform (NCT04136171) is the largest, most comprehensive ATTR-CM study ever conducted. Data from the study are expected as early as the first half of 2025.

As part of a global development and commercialization agreement, Ionis and AstraZeneca are seeking regulatory approval for eplontersen for the treatment of ATTRv-PN in the U.S. and plan to seek regulatory approval in Europe and other parts of the world. This agreement was recently expanded to include exclusive rights for AstraZeneca to commercialize eplontersen in Latin America and all other countries outside the US. Eplontersen was granted Orphan Drug Designation in the U.S. for the treatment of transthyretin-mediated amyloidosis (ATTR). The U.S. Food and Drug Administration (FDA) granted a PDUFA action date of Dec. 22, 2023.

*A link to the data will be added when available.