ORION-2

Pilot Homozygous Familial Hypercholesterolemia (HoFH)

(NCT02963311)

COMPLETE
October 2018
Phase 2 Study

ORION-2 is a completed, Phase 2 pilot study to assess the safety, tolerability and efficacy of a single 300-mg dose of inclisiran sodium administered subcutaneously in participants with genetically confirmed HoFH initially, with participants receiving a second 300-mg dose of inclisiran sodium at day 90 (three months) based on proprotein convertase subtilisin-kexin type 9 (PCSK9) levels. The primary endpoint of the trial was percentage change in LDL cholesterol (LDL-C) from baseline from day one to day 90 (three months) and percentage change in LDL-C from day one to day 180 (six months) or final physician visit.

PATIENT POPULATION

Four participants 12 years of age or older with a diagnosis of HoFH by genetic confirmation or a clinical diagnosis.

LOCATIONS

Three sites in three countries

STUDY TIMEFRAME

Dec. 2016 – Oct. 2018

Data from ORION-2 were first presented at the 87th European Atherosclerosis Society Congress in May 2019.

TOPLINE RESULTS

HoFH patients who received 300 mg of inclisiran sodium met the primary efficacy endpoint of durable LDL-C lowering up to day 180 (six months), with maximum LDL-C lowering of up to 43%. All patients achieved reductions in PCSK9 and apolipoprotein B.

Inclisiran sodium was reported to be well-tolerated, with no drug-related adverse events. A larger Phase 3 study of inclisiran in HoFH (ORION-5) is ongoing.

Inclisiran is an investigational drug not yet approved for use by the FDA or any other regulatory authority.

Updated August 2019.