TITLE:

A PROSPECTIVE, MULTICENTER, OPEN LABEL EXTENSION STUDY TO EVALUATE THE LONG TERM SAFETY OF OTX-TP (SUSTAINED RELEASE TRAVOPROST INTRACANALICULAR INSERT)

NCT#: NCT04061044

DATE: MAY 26, 2020

Study Title A Prospective, Multicenter, Open Label Extension Study to Evaluate the Long Term Safety of OTX-TP (sustained release travoprost) Intracanalicular Insert

STATISTICAL ANALYSIS

Study Populations

Safety: The Safety population will include all subjects who receive any investigational study medication (OTX-TP).

Unit of Analysis

The unit of analysis in this study will be any eye that received any investigation study medication. Summaries will be completed including all treated eyes.

Ocular (occurring in any treated eye) and non-ocular adverse events will be presented at the subject level.

Hypotheses

There are no formal hypotheses to be tested in this single arm safety study.

Determination of Sample Size

One-hundred (100) subjects treated for 1 year yields at least 95% probability of observing adverse events that occur at a true subject rate of 3% or greater. That is, if an adverse event of a given type is not observed in 100 subjects, then, with 95% one-sided confidence, the true rate of the adverse event will be <3%.

Methods of Analyses

Summaries for continuous variables will include the sample size, mean, standard deviation, median, minimum, and maximum. Minima and maxima will be reported with the same precision as the raw values; means, standard deviations, and medians will be presented to one additional decimal place than reported in the raw values. Summaries for discrete variables will include frequencies and percentages. All percentages will be rounded to one decimal place (i.e., XX.X%).

Change from baseline will be calculated as follow-up visit minus baseline. The baseline visit will be defined as the last non-missing measure prior to initiation of OTX-TP (either in this study or in OTX-16-002), with the exception of OCT (which was not measured in OTX-16-002), for which baseline will be defined as the last non-missing measure prior to initiation of OTX-TP during this open label extension study.

All summaries will be presented where appropriate by visit, summarizing data from all subjects at a given visit in this open label extension study, regardless of whether subjects received OTX-TP in OTX-16-002. Additional analyses may be performed including the OTX-16-002 treatment data for subjects receiving OTX-TP in OTX-16-002.

Summaries will be presented by separately for subjects/eyes who received OTX-TP during OTX-16-002, subjects/eyes who received OTX-PV during OTX-16-002, and over all subjects/eyes.

Demographics and Baseline Data

Subject demographics: gender, ethnicity, race, age category (< 65 years and ≥ 65 years), and iris color will be presented using discrete summary statistics. Age will also be presented using continuous summary statistics.

Non-ocular and ocular medical history will be summarized by treatment group using discrete summaries.

Subject disposition will be presented, including the number of subjects screened, enrolled and treated. The number of subjects who completed the study and reasons for discontinuation will be summarized.

Safety Analyses

The primary safety analysis will summarize treatment emergent ocular AEs (TEAE) using discrete summaries at the subject (occurring in any treated eye) and event level by system organ class and preferred term for each treatment group. A TEAE will be defined as occurring after initiation of treatment with OTX-TP this open label extension study. Treatment emergent non-ocular AEs will be summarized using discrete summaries at the subject and event level by system organ class and preferred term for each treatment group. Treatment related treatment emergent ocular and non-ocular AEs and serious treatment emergent adverse events will be summarized similarly. Treatment emergent ocular and non-ocular AEs will also be summarized by severity. Adverse events will be coded using the Medical Dictionary for Regulatory Activities (MedDRA) by system organ class and preferred term.

Intraocular pressure data will be summarized using continuous summaries, including change from baseline.

Slit lamp biomicroscopy and dilated fundoscopy measures will be summarized using discrete summary statistics.

Visual acuity data will be summarized using both continuous summaries, including change from baseline, and discrete summaries, including change from baseline in the number of lines and the proportion of subjects with a worsening of ≥ 3 lines from baseline.

Ocular hyperemia will be summarized using discrete summary statistics.

Ocular comfort will be summarized using continuous summary statistics.

Optical Coherence Tomography of Optic Nerve on Nerve Fiber Layer data will be summarized using continuous and discrete summary statistics, including change from baseline, as appropriate.

Summaries of other safety related outcomes will be provided.

Other Analyses

Visualization of the intracanalicular insert by the Investigator and by the subject will be summarized using discrete summary statistics.