

## **1572 Annotation Examples: Enabling Rare Disease Patient Access in a DCT Model**

Wolfram-like syndrome is a rare endocrine disease characterized by the triad of adult-onset diabetes mellitus, progressive hearing loss (usually presenting in the first decade of life and principally of low to moderate frequencies), and/or juvenile-onset optic atrophy. Psychiatric (i.e. anxiety, depression, hallucinations) and sleep disorders, the only neurologic abnormalities observed in this disease, have been reported in rare cases. Unlike Wolfram syndrome, patients with Wolfram-like syndrome do not report endocrine or cardiac findings.

### **OPHT1234: Study of the efficacy and safety of two times daily dosing of OPTHO4 vs placebo in patients with genetically confirmed Wolfram-like syndrome.**

This clinical trial will assess a new oral medication that may slow the progressive loss of vision. Standard visual assessment tests will be used as the primary outcome measure.

- 15 patient study at one research site only, at Washington University in St. Louis, MO
- Initial visit in Dr. Charles Berry, at his research clinic site,
- Follow up visits thru telehealth and local HCP practice for, Visits 3, 5 and 7
- Home health care for visits 2, 4 and 6 (Labs drawn, PE, etc) using a mobile research nurse
- Telemedicine links are available to connect PI to both home health care and HCP

**Principal Investigator:** Charles Berry, Washington University Dept of Ophthalmology, St Louis, MO

- Sub-Investigator: Christina Turner, Washington Univ. Dept of Ophthalmology, St Louis, MO
- Study Coordinator: Michael McDonald, RN

#### **HCP for visits Visits 3, 5 and 7**

Benjamin Folds, MD

Treating Ophthalmologist, licensed in NC

Practicing at Raleigh Eye Center, Durham

Address: 6104 Fayetteville Rd STE 108, Durham, NC 27713

- Nina Simone, HCP Center Nurse

#### **Home Health Care**

Provided by PCM Trials, HQ Address: PCM Trials, 709 N/ Clarkson Street, Denver, CO 80218 USA

- PCM Study nurse in Durham, NC: Jo Baker (RN)

#### **IRB:**

WIRB, 1019 39th Ave SE Ste 120, Puyallup, Washington, 98374, United States

**DTP Pharmacy:** Pharmacist: Ronnie Milsap, RPh- Pharmacist (NC), Marken

1009 Slater Rd Ste 120, Durham, North Carolina, 27703, United States

**Central Pharmacy Distribution direct to patient:**

Almac Group, 25 Fretz Road, Souderton PA 18964 USA