**Introduction**

- **CEP290** protein localizes to connecting cilium of photoreceptors and is essential for ciliary assembly and protein trafficking.
- **CEP290** mutations frequently cause Leber congenital amaurosis type 10 (LCA10), a retinal degenerative condition resulting in severe visual impairment.

The most common **CEP290** mutation causing LCA10 is CEP290-IVS26, which results in a nonfunctional CEP290 protein (Figure 1A)2.

**Methods**

**EDIT-101 utilizes the photoreceptor-tropic adeno-associated virus type 5 (AAV5) to deliver DNA encoding Cas9-based gene editing medicine that specifically removes the CEP290-IVS26 mutation to restore photoreceptor function.**

**Sensitivity and linear range assessment:**

- Assay sensitivity in all blood, tears, and nasal mucosa was determined over 5 runs using a double standard EDIT-101 viral vector Dil. Limit of detection (LoD) and limit of quantification (LoQ) were determined to be 10 and 25 copies per ml, respectively.

**Matrix effect assessment:**

- Matrix effect was determined for EDIT-101 at 50 and 100 times the LoQ, respectively, in blood, tears, and nasal mucosa. Normalized mean intensity values were calculated for each matrix.

**Stability of EDIT-101 in matrices:**

- Stability was determined in tears, blood, and nasal mucosa. No correlation between EDIT-101 doses and viral shedding levels was observed. Taken together, these data suggest a favorable safety profile of EDIT-101, a First-in-Human Therapy for CEP290-Related Retinal Degeneration.