Vision Medicines Overview of VM200:
An aldehyde trap molecule in IND enabling studies for Stargardt disease
VM200 is an aldehyde trap for Stargardt disease

In Stargardt disease, mutations in ABCA4 lead to accumulation of all-trans retinal, a toxic aldehyde that leads to photoreceptor death and is also a precursor to the formation of toxic A2E.

VM200 works by trapping all-trans retinal and thereby preserving retinal structure and function.

Small molecule, administered orally.

Invented by Kris Palczewski Ph.D at Case Western Reserve University.

Supported by co-funding from the Foundation Fighting Blindness.

Currently in IND-enabling studies and expected to enter the clinic in late 2017.
VM200: Trapping toxic all-trans retinal until it can be neutralized

- **Normal state** with intact transporter
- **Disease state** without transporter
- **VM200 neutralizes all-trans retinal (toxic)**
Abca4⁻/⁻ Rdh8⁻/⁻ mouse model replicates human Stargardt Disease

**A2E** in mouse eyes at 3 and 6 months

Retinal autofluorescence (*green*, with intensity in numbers) at 6 months

VM200 prevents retinal degeneration in the mouse model of Stargardt disease

VM200 administered to Abca4<sup>−/−</sup>/Rdh8<sup>−/−</sup> mice 2 hours prior to light damage preserves retinal structure and function in a dose-dependent manner.

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**VM200 preserves retinal structure**

- **No damage**
  - OCT Score: 0
  - Max damage: 4

- **No dysfunction**
  - VM200 Dose (mg): 0, 0.5, 2

**VM200 preserves retinal function**

- **No dysfunction**
  - VM200 Dose (mg): 0.05, 0.2, 0.5, 2

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Source: Unpublished Data from Palcziewski Lab, Case Western Reserve University