**microRNA translational research: Developing eyedrop to treat myopia**

**Background**

Myopia is a common eye disease and also a major cause of blindness. Asians have the highest risk for myopia with a prevalence of 85% in developed regions, while the white adults also have a prevalence of 50%. Myopia is primarily caused by abnormal elongation of eyeball axial length. Myopia power increases by -3D with an increase of 1 mm axial length. High myopia (worse than -5 diopter) significantly increases the risk for severe eye diseases including macular damage, retinal detachment, blindness etc.

**Current management of myopia**

Myopia progression typically happens in the schoolchildren. Once the children reach age around 18 years, myopia progression and elongation of axial length significantly slow down. Accordingly, the target population for myopia control is schoolchildren. There are two way to handle myopia:

1. Eye drops for myopia treatment. Unfortunately, there is ONE effective eye drop, Atropine, to treat myopia. Atropine can prevent/reduce eyeball elongation leading to treat myopia and to prevent myopia-induced eye diseases. However, Atropine can dilate pupil up to 7 days which not only causes photophobia, but also has a concern of photo-damage.
2. Although myopia-induced poor vision can be corrected by glasses or LASIK, these methods do not prevent myopia progression or eyeball elongation. In other words, these methods cannot prevent myopia-induced serious eye diseases.

Accordingly, there is unmet medical need for myopia.

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We identified microRNA-328 (miR-328) as a risk factor for myopia through genetic studies. Overexpression of miR-328 can affect several myopia-related genes including collagen1A1, fibromodulin, PAX6 etc. To suppress the excess miR-328, we designed an oligonucleotide antis-sense. Using this oligonucleotide in the eye drop form, we successfully reduce excessive miR-328 in the mouse eyes. Furthermore, this eye drop effectively treated myopia in mice and rabbits. More importantly, our eye drop is more effective than 0.1% atropine in animal studies and our eye drop did not dilate the pupils. No adverse effects were noticed in the GLP-graded toxicology studies using both rabbits, rats and dogs. With plenty exciting results, we plan to initiate a phase I clinical trial in 2021 to test its safety in human subjects.