Functional Recovery for Photoreceptor Degenerative Diseases with UGX202, a Novel Optogenetic Therapy

Lingjie Cui, Zhen Yu, Meng Li, Xueting Luo, Kai Wu
UgeneX Therapeutics Co., Ltd. Shanghai, China.
Contact: luoxueting@u-genex.com

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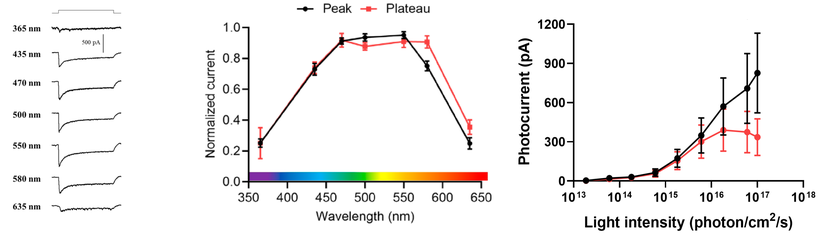
INTRODUCTION

Progressive and irreversible photoreceptor degeneration contributes to vision impairment in retinal diseases such as retinitis pigmentosa and geographic atrophy. Currently effective treatment is limited to restore visual function for late-stage patients suffering from these diseases. Optogenetic therapy holds potential to reconstruct photoreception relying on spared retinal neurons of intrinsic phototransduction pathway. Herein, we evaluated pharmacodynamic effects of a novel optogenetic gene therapy UGX202 in animal models for photoreceptor degeneration.

Objective

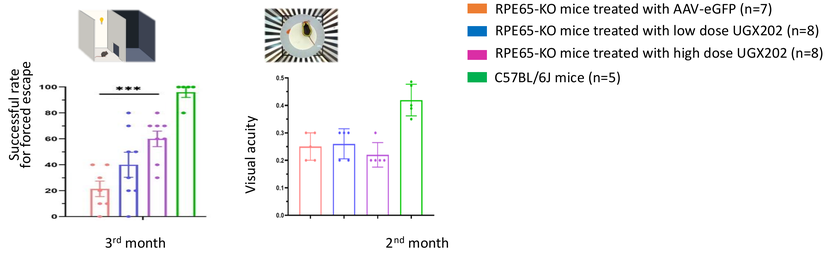
To evaluate pharmacodynamic efficacy of intravitreal treatment with UGX202 in restoring visual function across multiple animal models of photoreceptor degeneration.

1. UGX202 opsin features wide spectrum and high sensitivity.



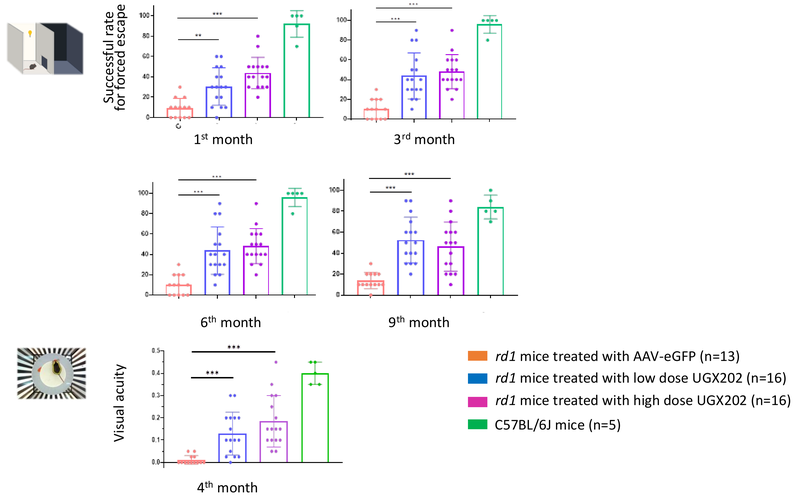
Analysis of excitatory response evoked by RGCs at different wavelengths or intensities of light to assess the spectral properties of UGX202. Representative traces of light-evoked currents of RGC in voltage-clamp recording mode (Left). Spectral curves of peak versus plateau currents normalized to a fixed wavelength (Middle, Mean±SEM, n=6-9). Statistics of peak and plateau current amplitude changes under different light intensity stimuli (Right,Mean±SEM, n=6-9). Peak represents the peak currents, and Plateau represents the plateau currents.

2. Visual function recovery in RPE65-KO mice after UGX202 treatment.



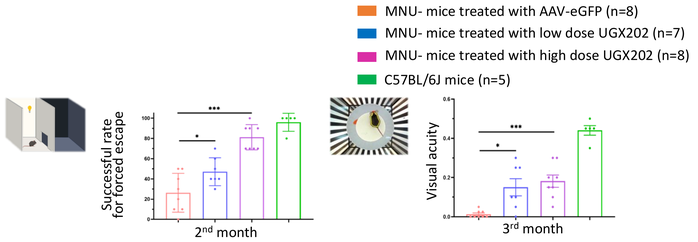
Visual function of RPE65-KO mice was assessed by light/dark Box (left) and optomotor response (right) after UGX202 treatment. Kruskal-Wallis test was applied to calculate statistical significance between groups. ***p<0.001.

3. Visual function recovery in *rd1* mice after UGX202 treatment.



Visual function of *rd1* mice was assessed by light/dark Box (left) and optomotor response (right) after UGX202 treatment. Kruskal-Wallis test was applied to calculate statistical significance between groups. **p<0.01, ***p<0.001.

4. Visual function recovery in mice with MNU-induced photoreceptor degeneration after UGX202 treatment.



Visual function of MNU-treated mice was assessed by light/dark Box (left) and optomotor response (right) after UGX202 treatment. Kruskal-Wallis test was applied to calculate statistical significance between groups. *p<0.05, ***p<0.001.

SUMMARY

- UGX202 treatment significantly improved visual function and vision-guided behaviors in three animal models for photoreceptor degeneration.
- These findings support clinical translation of UGX202 for photoreceptor degenerative diseases.

RECENT PROGRESS

- Investigator-initiated Clinical Trial in China is ongoing to explore safety and efficacy of UGX202 in patients with retinitis pigmentosa .

Disclosures

- L. Cui, Z. Yu, M. Li, X. Luo and K. Wu are employees of UgeneX Therapeutics. This study was supported by UgeneX.
- AviadoBio has an exclusive option and license agreement for the development and commercialization of UGX-202 outside of China and select territories.

Abbreviations:
RGC, retinal ganglion cell, MNU, N-methyl-N-nitrosourea.