

**OMTM, Volume 21**

**Supplemental information**

**Pre-clinical dose-escalation studies**

**establish a therapeutic range for**

**U7snRNA-mediated *DMD* exon 2 skipping**

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## Supplementary Material

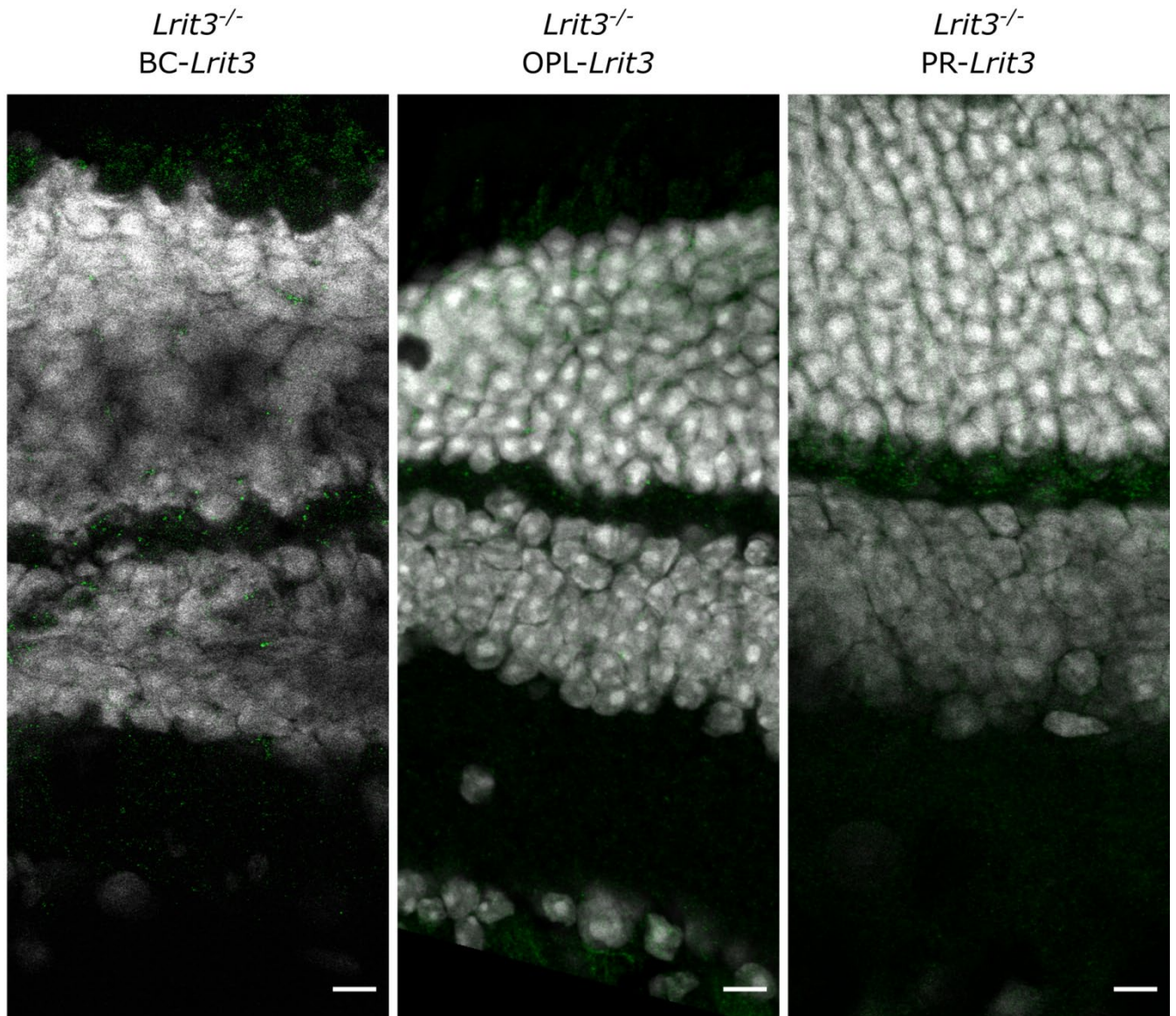


Figure S1: Localization of LRIT3

Representative confocal images of cross-sections of *Lrit3*<sup>-/-</sup>-BC-*Lrit3*, *Lrit3*<sup>-/-</sup>-OPL-*Lrit3* and *Lrit3*<sup>-/-</sup>-PR-*Lrit3* retinas stained with an antibody against LRIT3 (green). Scale bar, 10  $\mu$ m.

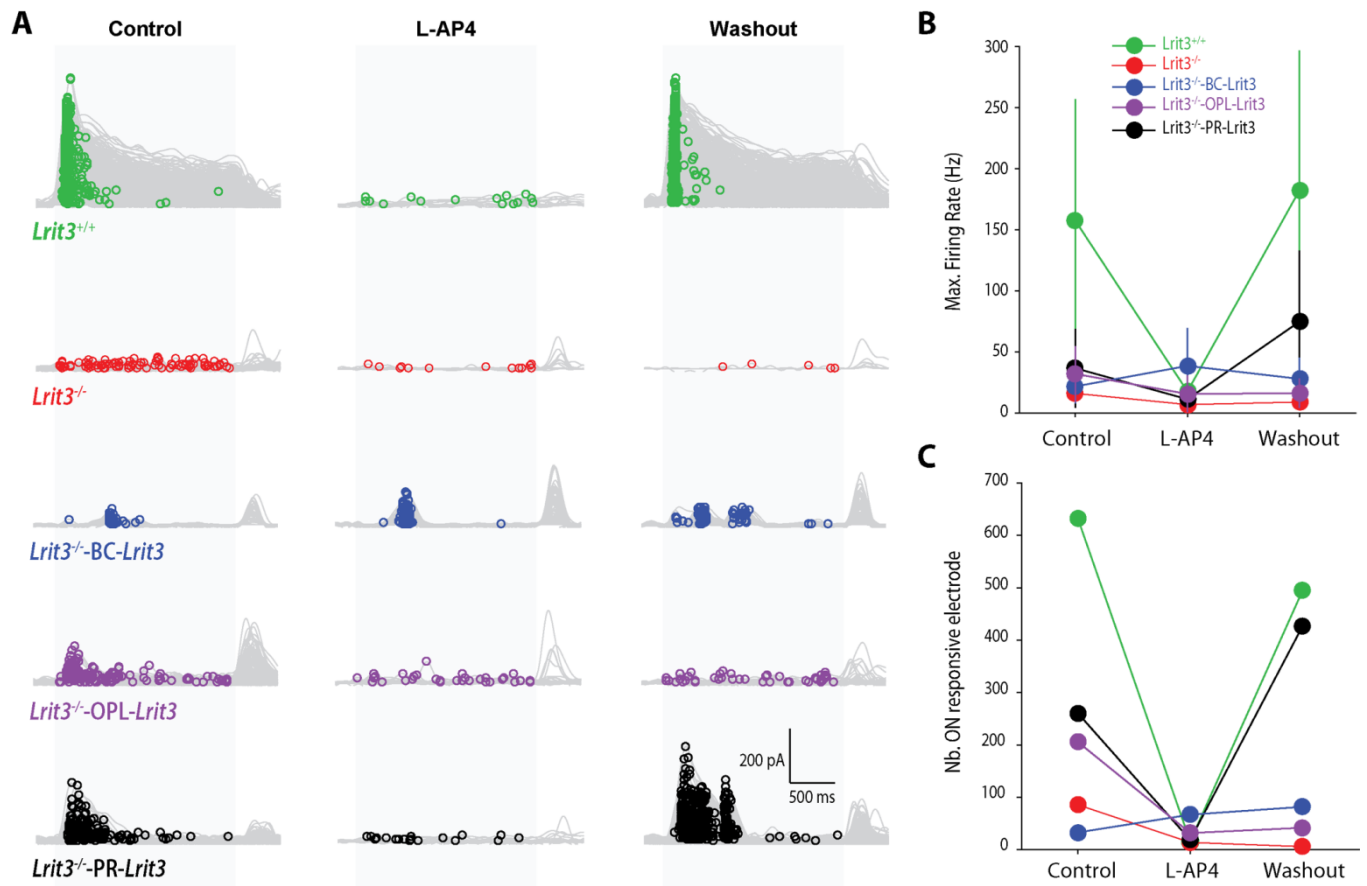


Figure S2: L-AP4 treatment reduce restored ON responses.

A) Spike density function for all recorded electrodes with ON responses (grey), before (control), during (L-AP4), and after (washout) perfusion of L-AP4. Peak indicated on individual traces during the light stimulation (light grey area). B) Peak firing rate for the different condition during the pharmacological experiment. C) Evolution of the number of electrodes with an ON response with the addition and removal of L-AP4 in the solution.

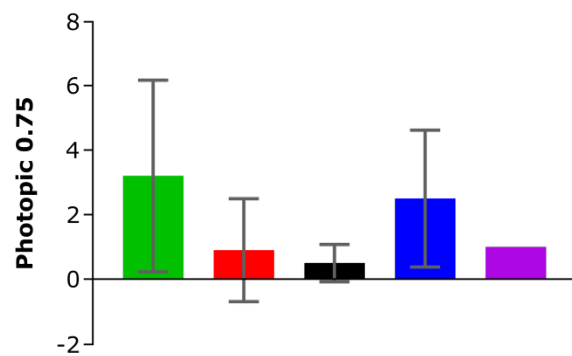
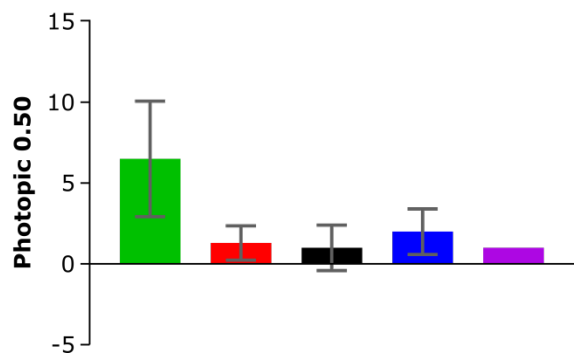
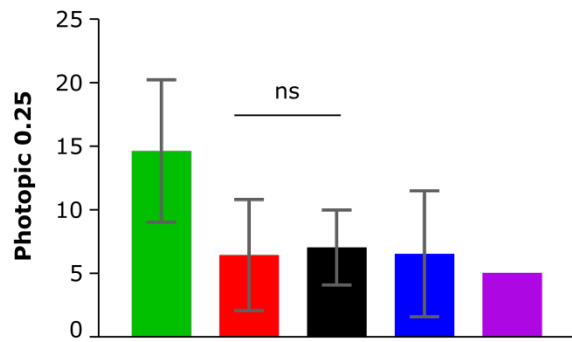
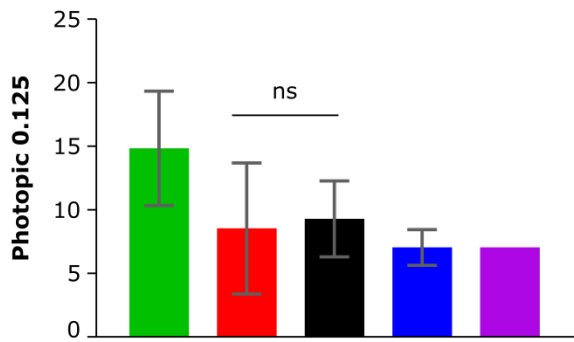
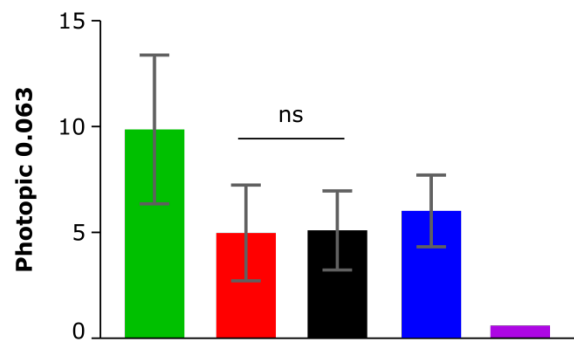
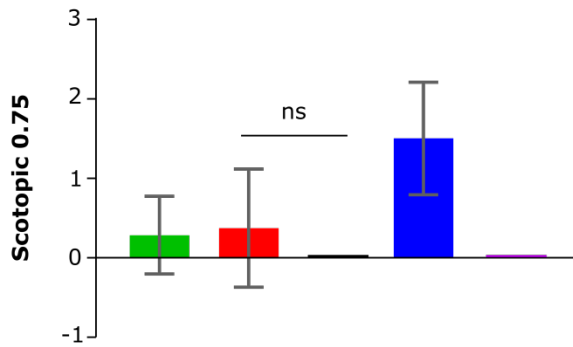
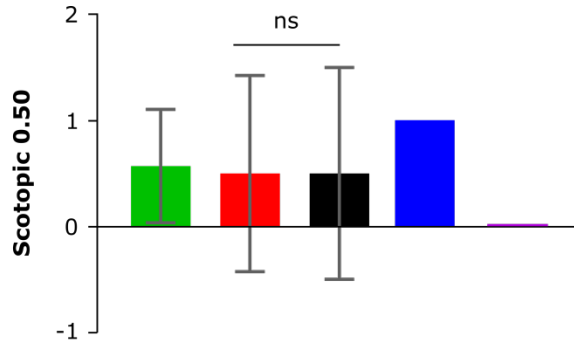
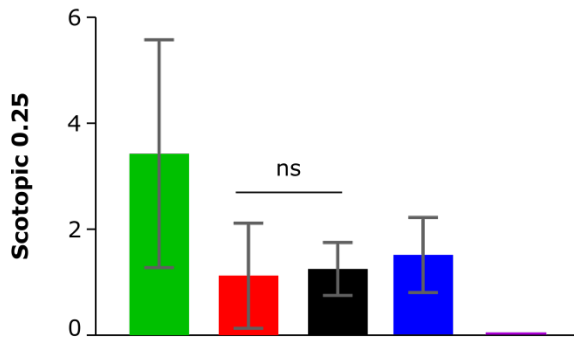


Figure S3: Measurements of optomotor responses under both scotopic and photopic conditions

A) Histogram representation of the number of head movements per minute which was obtained under scotopic conditions with spatial frequencies from 0.25 to 0.75 cycles/degree for *Lrit3*<sup>-/-</sup>-BC-*Lrit3* (blue), *Lrit3*<sup>-/-</sup>-OPL-*Lrit3* (purple) and *Lrit3*<sup>-/-</sup>-PR-*Lrit3* (black) mice and compared using Mann-Whitney statistical test with representative *Lrit3*<sup>+/+</sup> (green) and *Lrit3*<sup>-/-</sup> (red) mice. B) Histogram representation of the number of head movements per minute which was obtained under photopic conditions with spatial frequencies from 0.063 to 0.75 cycles/degree for *Lrit3*<sup>-/-</sup>-BC-*Lrit3* (blue), *Lrit3*<sup>-/-</sup>-OPL-*Lrit3* (purple) and *Lrit3*<sup>-/-</sup>-PR-*Lrit3* (black) mice and compared using Mann-Whitney statistical test with representative *Lrit3*<sup>+/+</sup> (green) and *Lrit3*<sup>-/-</sup> (red) mice.