27O-ISMS06 Differential Infection Pattern of AAVs in Mouse Retina

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With an increasing number of causative genes identified, the widespread use of gene therapy becomes feasible. For gene therapy, it is desirable to have a delivery method with both high cell type specificity and high efficiency. The need for cell type specificity and high efficiency is particularly important for treating retinal degenerations, which may result from defects in the pigmented epithelium, in rods, or in cones. Viruses are potent gene delivery vehicles for the nervous system, but they suffer from non-specific infection. To address specificity and efficiency in targeting retinal photoreceptors, we have screened various adeno-associated virus (AAV) serotypes for infection patterns in the mouse retina following subretinal injection. We have identified a serotype that specifically and efficiently infects cone photoreceptors. Or study may provide a useful tool for delivering new genetic information to cones for the purpose of restoring genes involving in degenerations.