

13TH ANNUAL ALICE R. MCPHERSON LECTURE



Optimizing viral gene delivery for the treatment of X-linked retinoschisis

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**THURSDAY
MAY 29th**

**Health Sciences Learning Center
RECEPTION / LECTURE**

3:00PM

Atrium

4:00PM

Room 1345

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Mutations in RS1 are associated with X-linked retinoschisis (XLRs), a common cause of juvenile macular degeneration. The presence of schisis cavities in the central retina of these patients has hampered submacular delivery with conventional Adeno associated viruses (AAVs), and clinical trials employing intravitreally injected AAVs showed lack of efficacy and inflammation. We designed a novel AAV capsid, "AAV.SPR" that efficiently transduces photoreceptors and laterally spreads beyond the margins of the subretinal injection bleb, providing the means to perform peripheral subretinal injection without detaching the macula/fovea and deliver RS1 safely and effectively to the central retina. This talk will summarize nonclinical proof of concept work and early observations from an ongoing Phase 1/2 clinical trial evaluating ATSN-201 for the treatment of XLRs.