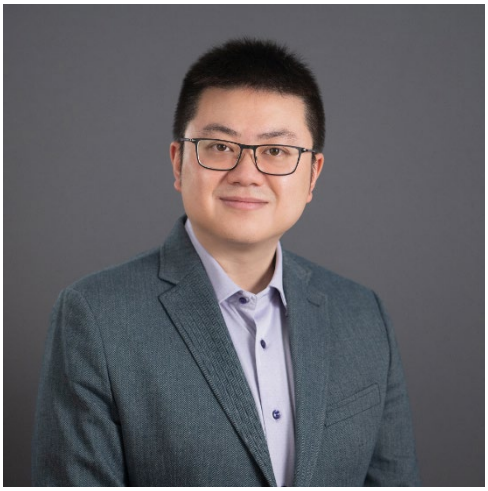
COLLEGE OF PHARMACY
PHARMACEUTICAL SCIENCES
UNIVERSITY OF MICHIGAN

Pharmaceutical Sciences Seminar

Wednesday, April 19, 2023
4:00pm
NCRC Building 10 Research Auditorium or Zoom

"Clinical Pharmacology Considerations for Recombinant Adeno-Associated Virus (AAV) Gene Therapy"

Presented by:



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Abstract: Recombinant adeno-associated virus (AAV) is currently the most widely used platform for in vivo gene therapy. Clinical pharmacology is a central field for AAV gene therapy, represented by the pillars of pharmacokinetics (PK), pharmacodynamics (PD) / efficacy, and safety. In this seminar, a comprehensive summary on clinical pharmacology considerations for recombinant AAV is provided. The main topics covered are biodistribution & shedding, dose – exposure – response relationship, safety, immune and stress response, and clinical dose selection strategies. The seminar highlights how the cumulative knowledge on AAV gene therapy could help with guiding clinical trial design, assessing and mitigating risks, as well as planning and executing PK/PD/safety data analyses. In addition, the major gaps and areas of growth in clinical pharmacology understandings of recombinant AAV are discussed. These include the mechanisms of durability of treatment response and variability in biodistribution, transduction, and immunogenicity, as well as potential influence on AAV's safety and efficacy profiles by drug product characteristics and patient intrinsic / extrinsic factors.

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