Abstract Submission for Digital Event: Workshop on Systemic Immunogenicity Considerations for AAV-mediated Gene Therapy

The virtual Workshop on Systemic Immunogenicity Considerations for AAV-mediated Gene Therapy will take place on November 30th and December 1st, 2020.

For general meeting information, visit ncats.nih.gov/events#aav.

Email this form to Deanna Portero (deanna.portero@nih.gov) with the subject line: Abstract Submission for Digital AAV Immunogenicity Workshop.

*Required

Email address*_____

November 30th – December 1st, 2020

Submission Deadline
October 1, 2020

Submitters Notified by
October 15, 2020

Please select the session topic(s) that your abstract should be considered for.*

Please only select the session topics for which your abstract has direct relevance.

- ☐ **Immunogenicity on the Cellular Level**—How do the adaptive immune system and innate immune system (including the complement system) respond to systemically administered AAV gene therapies and contribute to immune response symptoms? Which responses are transgene-related and which responses are related to the AAV capsid? How do cellular responses vary in response to CpG sequences? This session will feature in-depth background on the immunological processes underlying innate and adaptive immunity to AAV gene therapies.
- ☐ **Clinical Manifestations of Immunogenicity**—This session will provide an overview of immunogenic responses to systemically and locally administered AAV gene therapies. What is the spectrum of response symptoms across organ systems? How do innate and adaptive immune responses to the AAV capsid and transgene impact the short-term and long-term efficacy of gene therapies? What is the impact of patients null or mutation in transgene and CRIM status on the immune response (examples from hemophilia)?
- ☐ **Monitoring Patients Who Receive AAV-mediated Gene Therapies**—Best practices for assays in immunomonitoring pre-and post-treatment, e.g. monitoring anti-drug-antibodies (ADA) and neutralizing antibodies (nAb), correlation between binding and neutralizing antibodies, identifying neutralizing factors in the serum that are not ADA-related, and methods to monitor cellular immune responses.
- ☐ **Considerations of Acquired Immunity to AAV for Clinical Trial Eligibility**—Currently, patients who develop AAV antibodies above certain thresholds are effectively excluded from receiving AAV-mediated gene therapies. Further, patients who receive AAV gene therapies may shed the virus for a period following administration. This session will review the implications of those facts for patients, patient communities and the researchers who design gene therapy clinical trials. Topics will include preclinical best practices related to determining antibody thresholds, the likelihood of acquiring AAV immunity over the course of daily life, whether that likelihood could increase with the growing number of AAV gene therapy products, what prospective consumers of a future gene therapy can and should do to protect against acquisition of AAV antibodies, the role of patient organizations in educational efforts on this subject, and the challenges and opportunities of implementing sibling protocols.
- ☐ **Circumventing Immunogenic Response to AAV Vectors**—Less immunogenic AAV gene therapies would be desirable for many reasons, including simpler and safer clinical management, and the ability to redose patients. This session will feature a selection of technologies and methods to prevent or reduce adaptive and/or innate immune responses to systemically administered AAV gene therapies. This session will also feature discussion of the adequacy of preclinical models to support innovation in this space.
- ☐ **Circumventing Immunogenic Response to Transgene Product and Transgene Tolerizing Strategies**—Non-immunogenic transgene would be desirable for many reasons, including preventing autoimmunity, simpler and safer clinical management, and the ability to redose patients. This session will feature a selection of technologies and methods to prevent or reduce adaptive and/or innate immune responses to systemically administered AAV gene therapies. This session will also feature discussion of the adequacy of preclinical models to support innovation in this space.

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*Required

Would you like this submission to be considered for an oral presentation, poster presentation or both?*

- ◯ Consider this submission for poster presentation and oral presentations
- ◯ Consider this submission for a poster presentation only
- ◯ Consider this submission for oral presentation only

What is the full name of the presenting author?*

If selected for poster or oral presentation, this response will be included in meeting materials.

Your Answer

What is the title of the abstract?*

If selected for poster or oral presentation, this response will be included in meeting materials.

Your Answer

What is/are the institutional affiliations of the presenting author?*

If selected for poster or oral presentation, this response will be included in meeting materials. The first institutional affiliation listed may be presented singularly in certain contexts.

Your Answer

What is the full list of authors and their corresponding affiliations?*

If selected for poster or oral presentation, this response may be included in meeting materials. Please use the following format: Author (Institutional affiliations), Author (Institutional affiliations) & Author (Institutional affiliations). If a singular author, use the same format.

Your Answer

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Abstract (Maximum 250 words)*

If selected for poster or oral presentation, this response may be included in meeting materials. Please use word count functionality to confirm this submission is under 250 words.

Your Answer

Additional Questions or Comments

If you have any additional questions or comments about this abstract submission, you can leave that information here. Please expect a response within approximately 2 business days.

Your Answer

By submitting this form, you give meeting organizers permission to import the information you've provided into meeting materials (e.g. a meeting app). If you wish to revoke that permission or update any of the information submitted, you can email contact Deanna Portero at deanna.portero@nih.gov and expect response within 2 business days.*

☐ Yes, I understand and agree