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# Fast and reliable neutralization antibodies assay for AAV gene therapy (2020-121)

An assay to measure neutralization antibodies for adeno-associated viral vectors in gene therapy, to screen patients for clinical trials and treatment.

Technology No. 2020-121

**IP Status:** US Patent Pending **Application #:** 17/921,752

## Applications

- Clinical trials: determine patient AAV neutralizing antibody status
- Therapeutic screening
- Detect seroconversion in patients treated with AAV vectors

## Key Benefits & Differentiators

- **Sensitive assay to assess neutralization of AAVs:** A major strength of the assay is its enhanced dynamic range, facilitating sensitive detection of AAV neutralizing antibodies.
- **Simple assay with replicable results:** The AAV neutralization antibody assay uses readily available reagents and well know techniques, relieving the need for a high level of expertise and increasing reproducibility.
- **Fast and economical:** Assay can be completed in 24 hours without the requiring costly reagents.

## AAV vectors in gene therapies

Adeno-associated virus (AAV) vectors are a common platform for in vivo gene therapy applications and have been used successfully in clinical trials in patients with blood diseases and blindness. The presence of pre-existing neutralizing antibodies in about 50% of patients significantly decreased the clinical utility of AAV vectors. The prevailing assays used to screen for AAV neutralizing antibodies are not standardized, and can produce unreliable data depending on the AAV serotype. Drs. George Aslanidi and Karina Krotova at the University of

Minnesota have developed a fast and reliable method to evaluate pre-existing AAV neutralizing antibodies for most of the commonly used AAV serotypes. The resulting assay can be used with virtually all AAV serotypes, requires small amounts of AAV and can produce reliable data in 24 hours.

## Reliably measuring AAV neutralizing antibodies

The technology leverages a drug, Dorsomorphin (also known as Compound C), that drastically increases infection of HEK293 cells by AAV vectors. Using AAV vectors containing expression cassettes for luciferase, the increased infection rate provides a broad, measurable dynamic range to assess the infection rates of cells. This sensitized infection-rate assay facilitates reliable determination of the AAV-neutralization titers or various antibodies and/or serum. The research team optimized the assay and has shown it to be effective for measuring neutralization of three clinically relevant AAV serotypes in micel.

## Phase of Development

**TRL: 3-4**
Assay tested with ten different clinically relevant AAV serotypes in a mouse model.

## Desired Partnerships

This technology is now available for:

- License
- Sponsored research
- Co-development

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## Researchers


- **George Aslanidi, Ph.D.** Associate Professor, The Hormel Institute
- **Karina Krotova** Research Assistant Professor, The Hormel Institute


## References


Karina Krotova and George Aslanidi, https://doi.org/10.1089/hum.2020.074, Human Gene Therapy

Kuoch H, Krotova K, Graham ML, Brantly ML, Aslanidi G(2023 Feb 11), https://doi.org/10.3390/biomedicines11020523, https://www.mdpi.com/2227-9059/11/2/523, 11(2), 523

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