

**ASGCT 2021 Annual Meeting**  
**Oral Presentation**  
**May 11, 2021**  
**6:45 p.m. ET**

**Next Generation AAV Drug Products: Enhanced Stability & Clinical Ease for High Titer Preparations**

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Homology Medicines, Inc.

Homology Medicines has developed a Plug-&-Play Process and Manufacturing Platform to develop and manufacture gene transfer and gene editing therapeutics for rare diseases, which utilizes our proprietary Clade F AAV capsids derived from hematopoietic stem cells (AAVHSCs). As our programs have progressed through development and into the clinic, we have increased focus on the Drug Product sciences which includes the thoughtful development of stabilizing formulations enabling high titers, easing of clinical storage and supply chains, and enhanced long-term stability.

AAV preparations have long held a reputation as challenging not only for production, but for long-term stability at even low concentrations. However, here we demonstrate not only the stability of AAVHSCs in the liquid state, but also the impact of novel formulations on capsid stability. Our AAV preparations achieve titers in excess of  $1E14$  vg/mL and demonstrate stability for a minimum of one year at 2-8°C and more than six months at room temperature. Benefits of vector stability in the liquid state are a reduction in the need for -80°C-storage infrastructure and simplification of the clinical supply chain by enabling 2-8°C storage at the manufacturing site and clinical pharmacy.

This work demonstrates the marked stability of our proprietary AAVHSCs as well as the impact of a well-developed formulation. Furthermore, this effort shows that by bringing a Drug Product focus to AAV product development, an organization can help support clinical and commercial success by providing long product expiries, smaller administration volumes, and 2-8°C supply chains.