

## **DEVELOPMENT OF A SCALABLE PRODUCTION PLATFORM FOR RECOMBINANT ADENO-ASSOCIATED VIRUS VECTORS IN SUSPENSION HEK293 CELLS**

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Recombinant Adeno Associated Viral vectors (rAAV) have emerged as a powerful tool for *in vivo* gene delivery owing to their unique characteristics, including a high safety profile and ability to drive long-term gene expression in humans. Current successes in AAV-mediated clinical trials have prompted pharmaceutical companies to quickly move into the AAV gene therapy market and resulted in growing demand for clinical-grade rAAV vectors. In order to address these requirements, Oxford Genetics has developed a scalable manufacturing platform based on optimized expression plasmids and a clonal cGMP-compliant suspension HEK293 cell line. While this platform facilitates representative material generation for early stage clinical trials, it also allows the transition to packaging and producer cell lines based on the same underlying genetic components, cell line background and process/media system as vector requirements increase. Stable cell lines enable the development of rapid, robust and high-productivity processes, with reduced cost of goods and simplified supply chain. Here we present an update on characterization of these cell lines and internal process development activities to demonstrate associated product quality assurance and scale-up.