



## **Precision genomic optimization of producer cell lines**

### **Selected Literature**

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## 1 General literature on rAAV mediated gene targeting

- Human gene targeting by viral vectors  
*Russel et al., Nature Genetics 1998, v18, p325*
  - Improved methods for the generation of human gene knockout and knockin cell lines  
*Topaloglu et al., Nucleic Acids Research 2005, v33, e158*
  - Safety and tolerability of gene therapy with an adeno-associated virus (AAV) borne GAD gene for Parkinson's disease: an open label, phase I trial  
*Kaplitt et al., The Lancet 2007, v369, p2097*
  - Engineering of Human Pluripotent Stem Cells by AAV-mediated Gene Targeting  
*Khan et al., American Society of Gene & Cell Therapy 2010, v18, p1192*
  - AAV Vector Delivery to Cells in Culture  
*Smith et al., Methods in Molecular Biology, v246, p167*
  - Adeno-associated virus (AAV)-3-based vectors transduce haematopoietic cells not susceptible to transduction with AAV-2 based vectors  
*Handa et al., Journal of General Virology 2000, v81, p2077*
  - Adeno-associated virus-targeted disruption of the CFTR gene in cloned ferrets  
*Sun et al., Journal of Clinical Investigation 2008, v118, p1578*
  - Production of CFTR-null and CFTR- $\Delta$ F508 heterozygous pigs by adeno-associated virus-mediated gene targeting and somatic cell nuclear transfer  
*Rogers et al., Journal of Clinical Investigation 2008, v118, p1571*
  - Efficient targeting of expressed and silent genes in human ESCs and iPSCs using zinc-finger nucleases  
*Hockemeyer et al., Nature Biotechnology 2009, v27, p851*
  - Frequent Endonuclease Cleavage at Off-target Locations *In Vivo*  
*Patek et al., The American Society of Gene & Cell Therapy 2010, v18, p983*
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## 2 rAAV & bioprocessing

- GMP Production of Self-Complementary Serotype 8 AAV Vector for a Hemophilia B Clinical Trial.  
*Allay et al., Human Gene Therapy 2010*
  - Highly Purified Recombinant Adeno-Associated Virus Vectors Are Biologically Active and Free of Detectable Helper and Wild-Type Viruses.  
*Clark et al., Human Gene Therapy 1999, v10, p1031*
  - Adeno-associated Virus as a Gene Therapy Vector: Vector Development, Production and Clinical Applications.  
*Grieger et al., Adv biochem Engin/Biotechnol 2005, v99, p119*
  - High-titer, serum-free production of adeno-associated virus vectors by polyethyleneimine-mediated plasmid transfection in mammalian suspension cells.  
*Hildinger et al., Biotechnol Lett 2007, v29, p1713*
  - High-Fidelity Correction of Mutations at Multiple Chromosomal Positions by Adeno-Associated Virus Vectors.  
*Inoue et al., Journal of Virology 1999, v73, p7376*
  - Recombinant Adeno-Associated Virus As Delivery for Gene Therapy – A Review  
*Lu., Stem Cell And Development 2004, v13, p133*
  - AAV-mediated factor IX gene transfer to skeletal muscle in patients with severe hemophilia B.  
*Manno et al., Blood 2003, v101, p2963*
  - Successful transduction of liver in hemophilia by AAV-Factor IX and limitations imposed by the host immune response.  
*Manno et al., Nature 2006, v12, p342*
  - Recombinant adeno-associated virus purification using novel methods improves infectious titer and yield.  
*Zolotukhin et al., Gene Therapy 1999, v6, p973*
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