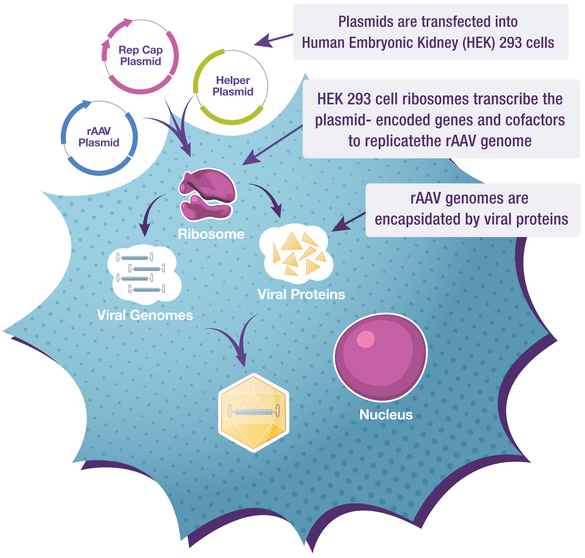
HOW CAN AN EXPERT GMP/CMC LAB SUPPORT YOUR RAAV MANUFACTURING PROCESS?

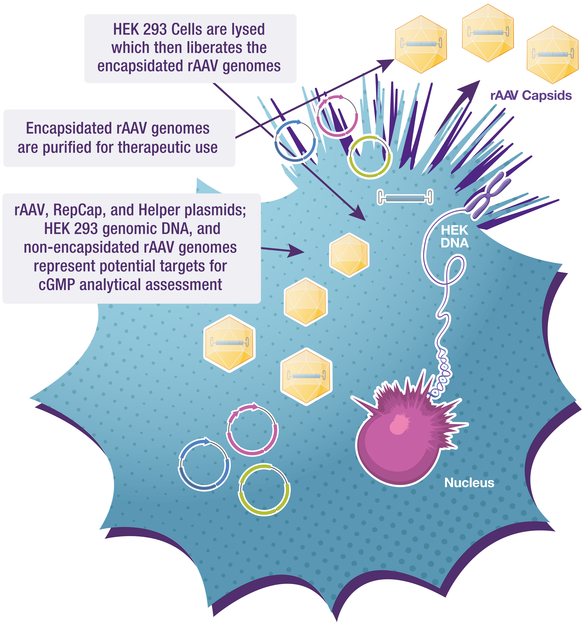
Gene therapy research continues to expand due in part to new gene delivery vectors. Adeno-associated virus (AAV), a non-enveloped virus that can be engineered to deliver DNA to target cells, has attracted a significant amount of attention. Generating recombinant AAV (rAAV) particles lacking any viral genes and containing DNA sequences of interest for various therapeutic applications has proven to be one of the safest strategies for gene therapies.

Explore this infographic to learn one way rAAV products are created and the laboratory assays required to support their development and manufacturing.

EXPRESSION & ASSEMBLY



LYSIS & PURIFICATION



FIVE CMC ASSAYS YOU'LL NEED TO ENSURE THE QUALITY OF YOUR RAAV

1. Encapsidated rAAV genomes are quantified and characterized by molecular methods such as quantitative real-time PCR (qPCR) or droplet digital PCR (ddPCR) assays to ensure adequate quantity of active particles are included in the final product. Potential contaminants in the purified rAAV product must be detected and quantified.
2. Empty capsids (RepCap) can be quantified by methods like ultra-centrifugation.
3. Helper plasmids can be quantified by ddPCR or qPCR using specific probes and primers.
4. Residual HEK 293 DNA can be quantified by targeting the EIA gene with specific probes and primers using ddPCR or qPCR.
5. Non-encapsidated rAAV genomes can be quantified by applying various pre-treatment steps to samples prior to the PCR process.

PPD™ Laboratory services GMP lab has helped customers deepen their understanding of more than 45 rAAV products and refine their manufacturing process to ensure the safety and effectiveness of their gene therapy product. Our experience and expertise span the design and validation of qPCR, ddPCR, DNA sequencing and many other assays under cGMP regulations that meet USP standards and comply with 21 CFR Part 11.

Learn more at: *www.ppd.com/our-solutions/ppd-laboratories/gmp-lab*

Put our expertise and experience to work for you on your next project.