

Small Scale AAV Feasibility Studies & QC Release Testing

Pharmaron's well established, multi-serotype adeno-associate virus (AAV) small scale feasibility studies are a rapid and cost-effective way to assess your viral vector product's performance within Pharmaron's scalable AAV platform process, without having to commit to larger scale programs, long lead times or high investments early on in your product development lifecycle.



Key Features

- Platform Process AMBR 250mL
- Chromatography-based purification
- Platform Analytics
- HEK293 cell line, pHelper Plasmid, RepCap Plasmid (all serotypes) from Pharmaron or customer



Ideal Requirements

- Customer transgene (RCB vial or small quantity for Pharmaron to amplify)

Note: If only sequence is available, Pharmaron can construct and produce transgene plasmid (timelines may extend)



Performance Outputs

- Process and analytical data for process steps
- Titre and Empty:Full capsid ratio for drug substance
- Performance of transgene in platform and recommendations for targeted process optimisation



Key Benefits

- Rapid Study (typically, 6-8 weeks)
- **Diagnosis** of areas for **optimisation**
- **Fast track** development and scaling for **clinical supply**
- **Data** can support presentations to **investors**



Additional Opportunities

Candidate not selected yet? Serotype not selected yet?

A Feasibility study can be configured to test potential candidates, side-by-side to aid selection of preferred candidate.



Small Volume Analytics

Analytical Testing for Small Scale Feasibility Studies

To support Pharmaron’s well established, multi-serotype adeno-associate virus (AAV) small scale feasibility studies, Pharmaron have invested in state-of-the-art methods and technologies to ensure studies can be well-characterised through use of small volume analytics. By improving detection methods, deploying new technologies and optimising sample preparations Pharmaron can **do more** testing **with less** customer product.

Key Assays for Small Scale Feasibility Studies

- Viral protein titre by Capsid ELISA
- Viral genome titre by droplet digital PCR
- Empty: Full capsid ratio by Mass Photometry
- Residual Host Cell impurity assays
- Empty: Full capsid ratio by Analytical Ultra Centrifugation (AUC)

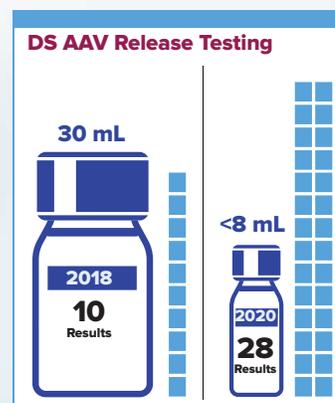


How Small Volume Analytics Translates into Larger Scale Programs

To support Quality Control release testing and stability studies of drug substance (DS) and drug product (DP), Pharmaron have extended their small volume analytics into their platform release testing portfolio. Small volumes enable the conservation of the AAV product, which ensures increased amounts of batches are readily available for important studies including comparability studies and clinical studies.

Key Assays Adapted for AAV QC Release Testing

- Colour, clarity and visible particles, E.P. appearance method modified from 7mL to 1mL
- Sub-visible particle analysis, HIAC volume reduced from 6mL to 1.5mL
- Rapid MCS bacterial endotoxin analysis, volume reduced from 600µL to 150µL
- Highly sensitive UV & fluorescence detectors for multiple attributes, e.g., titre, aggregation, impurities



Reduction in sample volume for batch release and stability

Maximised assay sensitivity for AAV impurity analysis

Higher proportion of batch available for clinical studies



Laboratory Services



Chemistry, Manufacturing & Control



Clinical Development



Biologics & CGT

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