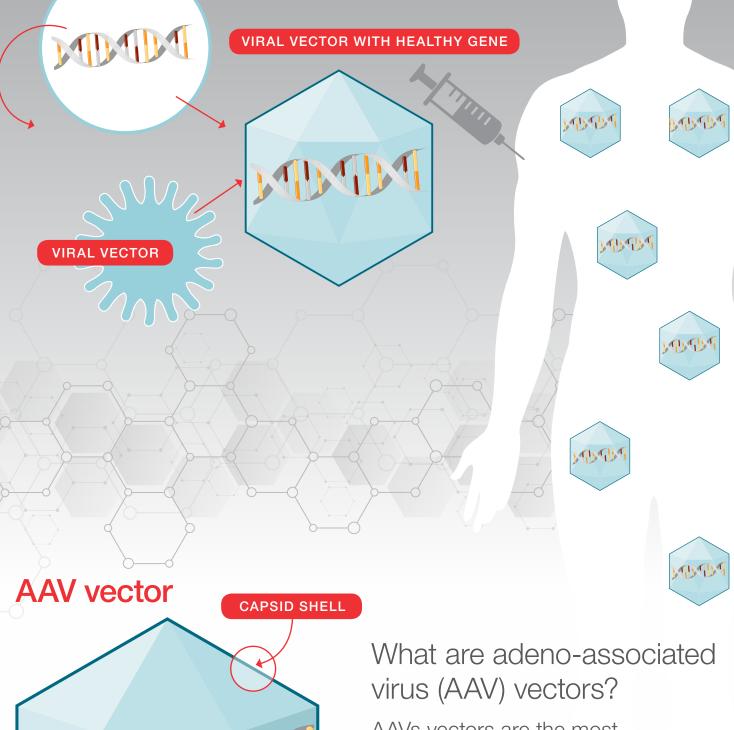
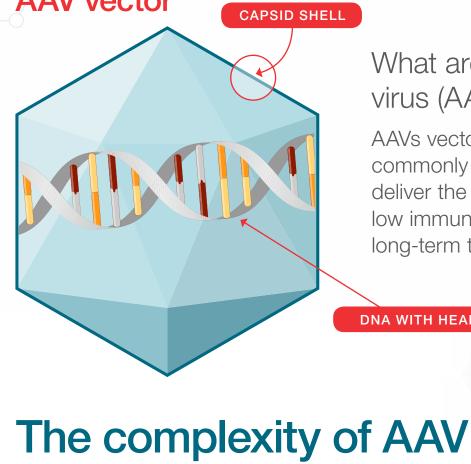
Analytical solutions to accelerate your AAV gene therapy

Gene therapy holds great promise for a wide range of diseases, including cancers, cardiovascular diseases and thousands of rare hereditary diseases caused by gene mutations. **HEALTHY GENE**





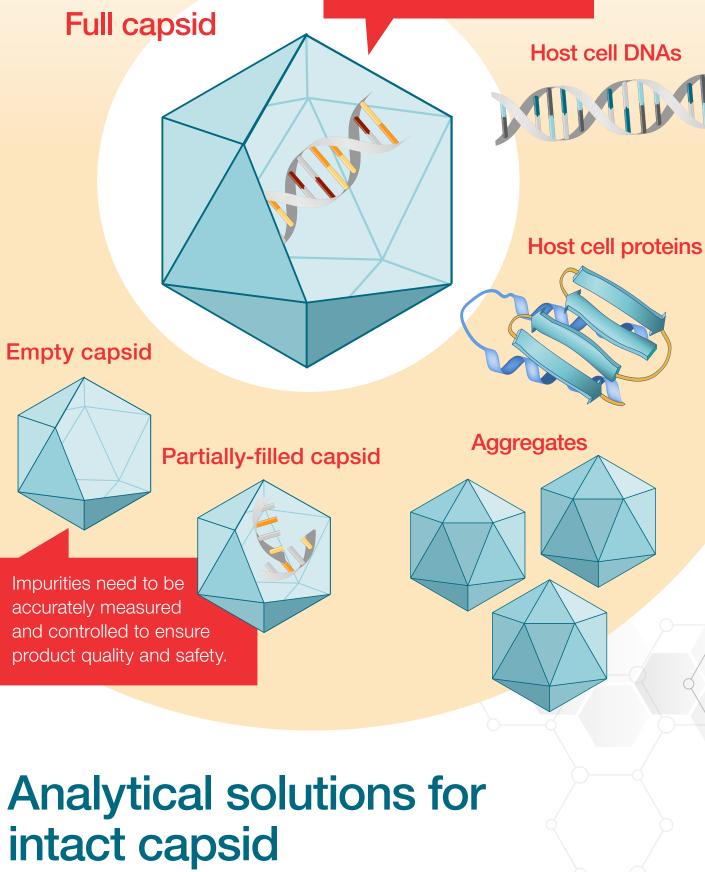
AAVs vectors are the most commonly used viral vectors to deliver the healthy gene, due to their

low immunogenicity, safety and long-term transient expression. **DNA WITH HEALTHY GENE**

AAV based gene therapeutics are far more complex than many traditional biotherapeutics. Besides the full capsid containing the desired gene material, the final product could contain many different types of process and product related impurities.

gene therapy products

The full capsid needs to be carefully characterized to ensure product efficacy.



AAV8 Full + Empty (1:1) **Empty AAV8** Fluorescence (counts) 1.0e7 0.0e0

4.00

5.0e7

AAV6

20

27130.71

by LC-HRMS

no bias for the 25% smaller 3 MDa particle.

Serotype confirmation by

• Accurate molecular weight determination

Complete sequence verification

intact protein analysis

of capsid proteins at <10 ppm

Capsid characterization

2.00

(stunoo) 3.0e7 4.0e7

> 2.0e7 1.0e7 0.0e0

Accurate measurement of empty/full ratio

Empty

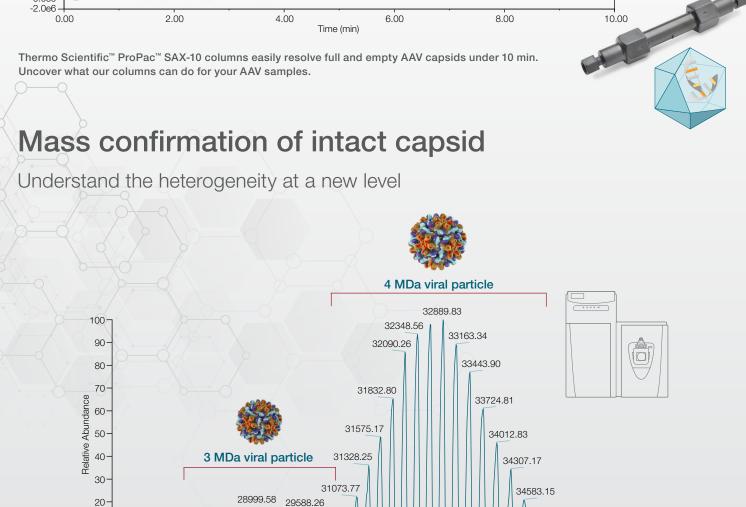
(~)

35205.44

VP3

VP2

36186.02

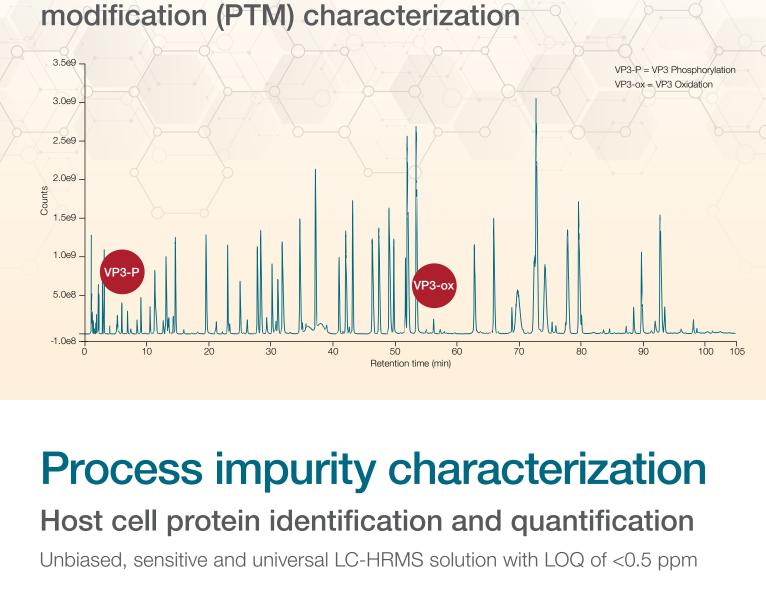


Mixtures of 3 MDa and 4 MDa virus capsids analyzed under charge reducing conditions span a wide mass range between 27,000 and 36,000 m/z. The spectrum contains well-resolved charge states for both particles and shows

 Confident identification of low abundant degradants at ng level through top-down sequencing

and sensitive peptide and post translational

50000



Host cell proteins

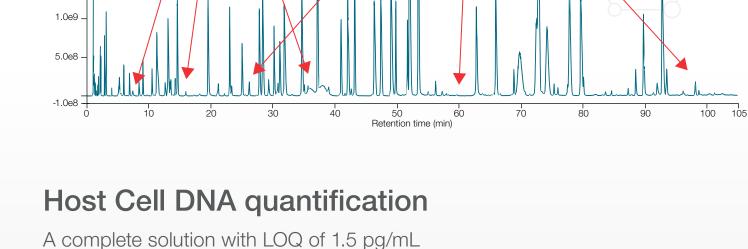
3.5e9

3.0e9

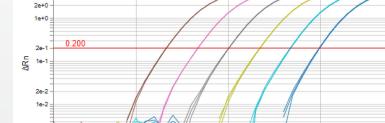
2.5e9

2.0e9

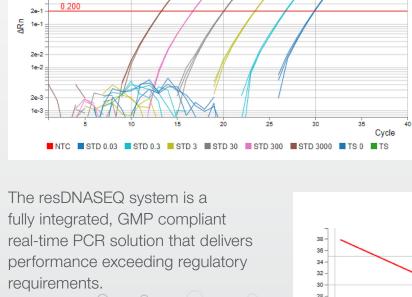
1.5e9

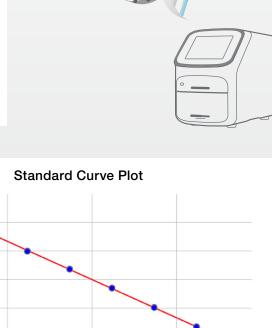


20 -18



Amplification Plot





Target: CHO, Slope: -3.3309, R^2: 0.9997, Y-Intercept: 24.9702, Efficiency: 99.63 StdError: 0.0120

Host cell DNAs

TUTT

Find out more at

thermofisher.com/genetherapyanalysis

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