Supporting Gene Therapy Development with Safer, More Efficacious AAV Vectors

SIRION
BIOTECH
a PerkinElmer company

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SIRION provides a 360° solution from early R&D up to the late preclinical AAV vector space to develop safe and efficacious AAV-based drug products.

SIRION KEY Advantages

12 years in-house AAV vector expertise
de-risking the development of AAV-based therapies

13 **Proactive development**
addressing key requirements as early as possible

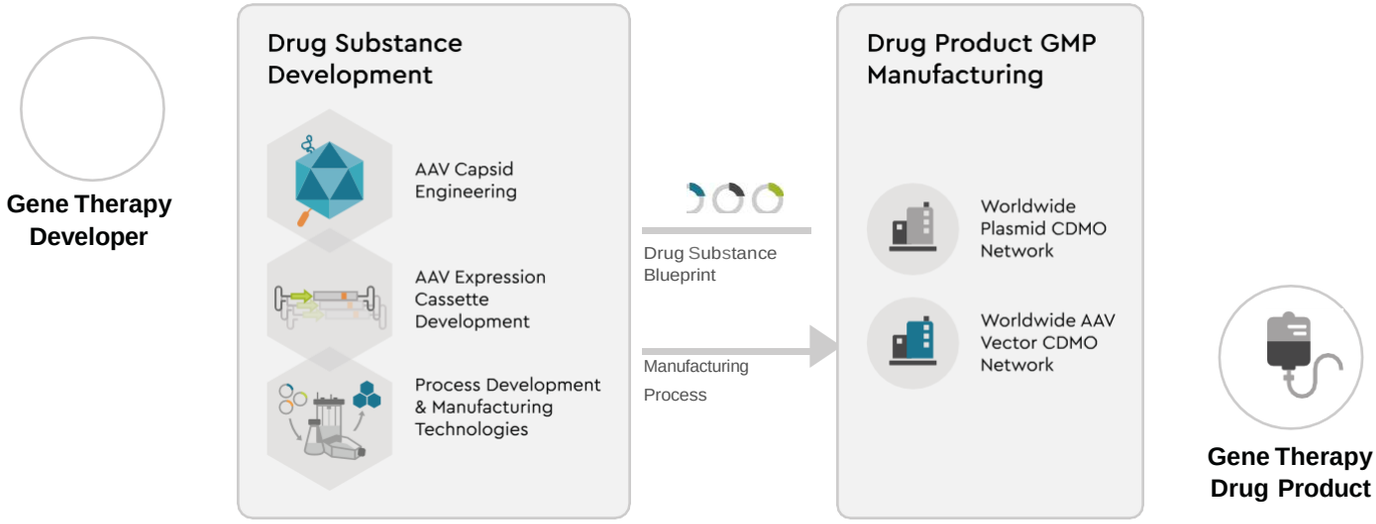
Focus on dient needs
in a cost- and time-effective manner

Integrated discovery programs
enabling development of all therapeutic vector key elements

Established worldwide CDMO network
for GMP manufacturing of AAV vector drug products

SIRION Capabilities at a Glance

SIRION Biotech supports clients developing viral vectors for safer and more efficacious drug products. At SIRION, we combine our technology, experience, and capabilities, with external partners to provide an accelerated gene therapy vector development program.



SIR/ON supports gene therapy developers in all key aspects of drug substance development: AAVCapsid Engineering, AAV Expression Cassette Development, as weil as Process and Manufacturing Technologies. To support swift progression to Jirst clinical phases, SIR/ON can transfer drug substance blueprints and manufacturing processes to our worldwide CDMO network.

How can SIRION Biotech support you to develop AAV vectors for safer and more efficacious AAV gene therapies?

Gene Therapy Developer

Customized AAV vector engineering

Manufacturing plasmids

Process optimization

CDMO forGMP manufacturing

Drug substance

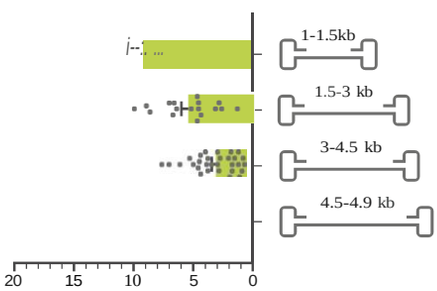
AAV-based Drug Product

Development of cutting edge AAV vector components

Lead vector design, including the AAV capsid and the AAV therapeutic transgene expression cassette, is the basis for successful gene therapy development.

AAV Capsid Engineering

SIRION's innovative key AAV technologies enable directed evolution of AAV vectors via DNA shuffling and peptide insertion as weil as AAV re-targeting via cell-specific nanobodies. Aiming at dose reduction and mitigation of potential side effects, the AAV capsid structure is optimized to improve tropism and target cell specificity.



Therapeutic Expression Cassette Development

Safety, efficacy and manufacturability are the criteria driving the development of transgene expression cassettes and elements such as promoters and transgene optimization.

AAV vector cassette *size and impact on p/asmid DNA impurities. Shown* as *percentage of vector preparation and determined by qPCRfor ORI amplicon.*

Manufacturing Technologies

SIRION Biotech's platform process paired with our manufacturing technologies enable cost-effective manufacturing of high quality material. To accelerate translation of AAV vectors, SIRION developed an AAV plasmid backbone optimized for clinical manufacturing.

USP and DSP process development to meet customized quality attributes

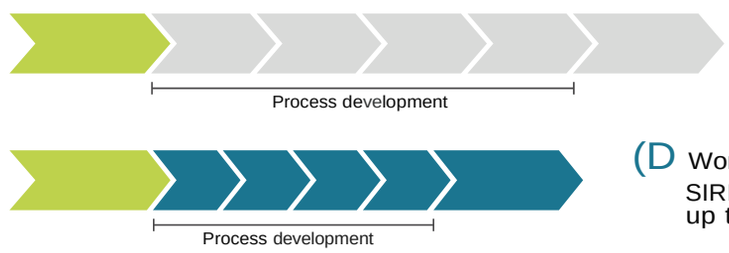
Determine the most cost-effective upstream and downstream processes to meet client-specified quality attributes, e.g., cross-packaging of non-AAV cassette material and empty/full capsid ratios.

SIRION offers extensive QCs and production of preclinical batches of **up to 50 L.**

Transfer to GMP CDMO network

Process Transfer Case Study

Gtv1P optimized processes can be transferred to our worldwide CDtv1O network, saving up to 8 months of precious time on project set up and process development.



(D Working with SIRION can save up to 8 months