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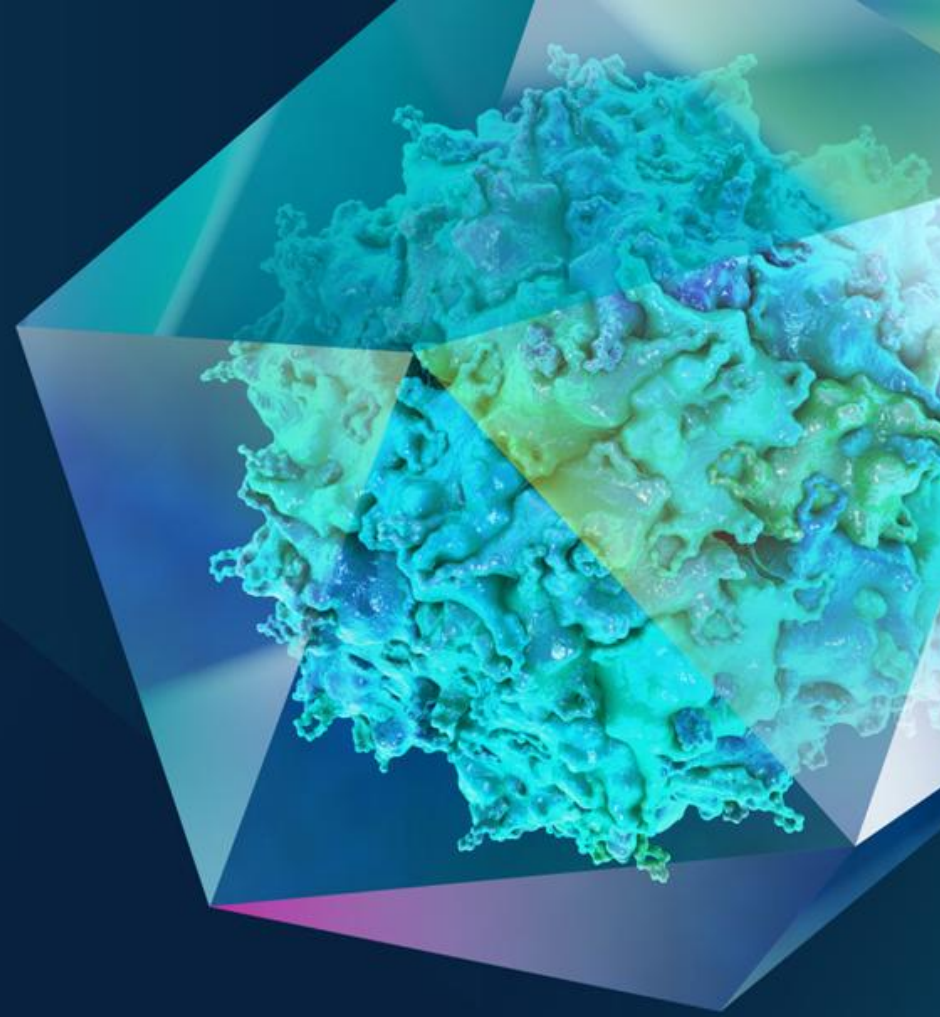


From Platform Knowledge to Commercial Readiness

Ane Quesada Ganuza, MSAT Manager

2026

ALL-IN AAV

Our Origins

A science-driven, AAV-focused gene therapy CMDO founded by AskBio

2017

Founded in 2017 as Joint Venture

2020

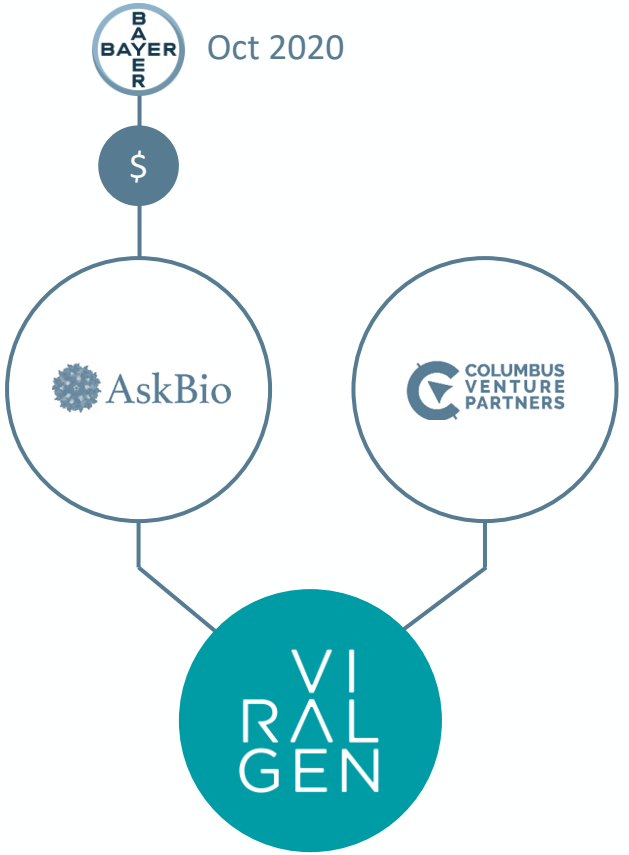
Consolidated through the Bayer's acquisition of AskBio

430+

Employees



Based in
San Sebastian, Spain



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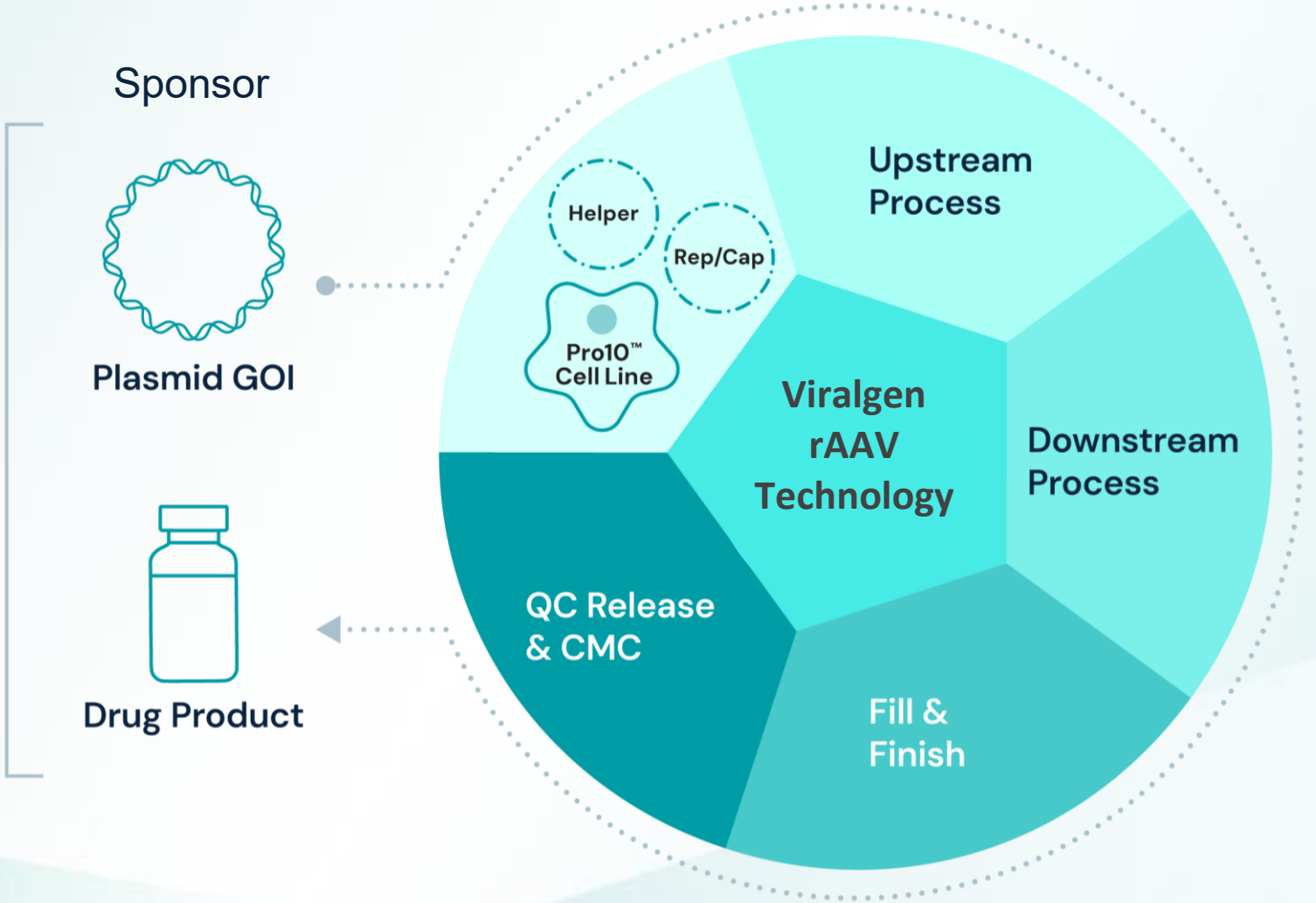
Our rAAV Technology: The Aava™ Platform



+60
Global Sponsors

+2000
Batches

+45
Clinical Trial Authorizations

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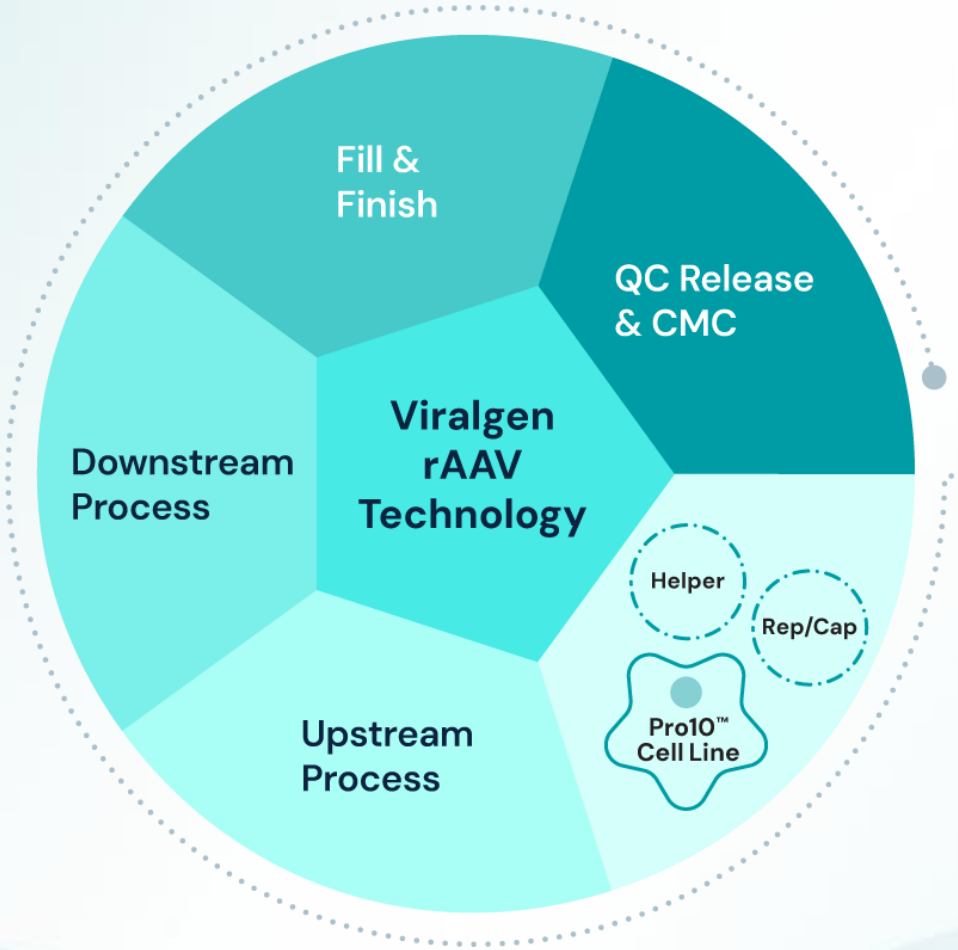
Consistent Processes to Drive Consistent Outcomes



Same Production System

- Master/Working Cell Bank
- Rep-Cap and helper DNA material for a given serotype
- Raw materials and its release specifications
- Facilities and equipment
- Operational parameters
- In-Process panel
- Quality attributes for drug substance (DS) and drug product (DP) release

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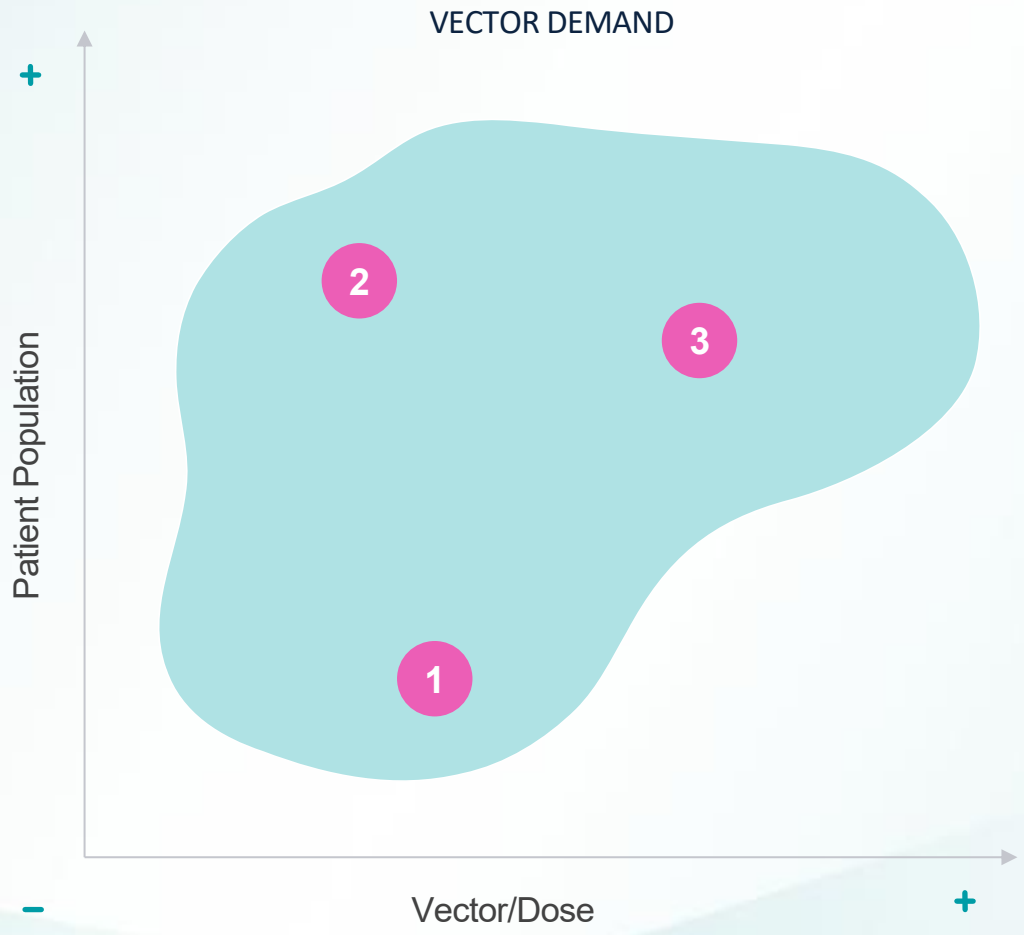
AAV Commercialization Presents Multiple Unique Challenges



1. Rare and ultrarare diseases
2. Diseases requiring low doses for treatment
3. Prevalent diseases

SINGLE DOSE Therapy

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Pre-PPQ Constraints in Rare-Disease Programs

01

Limited material

Small patient populations and costly vector constrain the material available for characterization.

02

Evolving process

Manufacturing and analytical methods mature alongside the clinical program.

03

Compressed timelines

Prior knowledge can focus new studies where uncertainty and product risk remain.

Platform knowledge informs the scope. Product-specific justification supports each decision.

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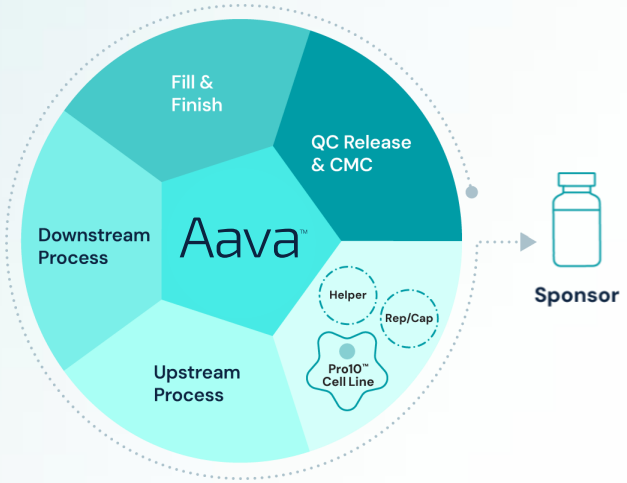
Fundamentals of Commercial Readiness





QTPP: Quality Target Product Profile

CQA: Critical Quality Attributes Qualification

PFMEA: Process Failure Mode & Effects Analysis

SDM: Scale Down Model

PPQ: Process Performance Qualification

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Platform Scope across the Manufacturing Process

DNA Provision	Upstream Processes	Downstream Processes
Helper	Cell Line Development and Banking	Clarification
Rep/Cap	Cell Thaw and Expansion	Affinity Chromatography
GOI	AAV production and Triple Transfection	Ultracentrifugation
	Cell Lysis	AEX
		TFF
		DS Formulation

Platform | Serotype Specific | Product Specific

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Prior Knowledge by Unit Operation

Proposed Approach from Viralgen

Organize evidence by unit operation

Link batch trends and characterization reports to the CQA assessment, FMEA and qualified small-scale model.

Tag each record by scope, serotype, product, scale and process version. Record the source, owner and applicability limits.

Maintain a controlled core package and a product-specific gap assessment

Platform

Cell expansion, lysis, AEX: reuse applicable process evidence and risk assessments.

Serotype Specific

Clarification and affinity chromatography: use matching serotype data and assess differences.

Product Specific

Transfection, TFF and DS formulation: reuse study designs and test remaining product risks.

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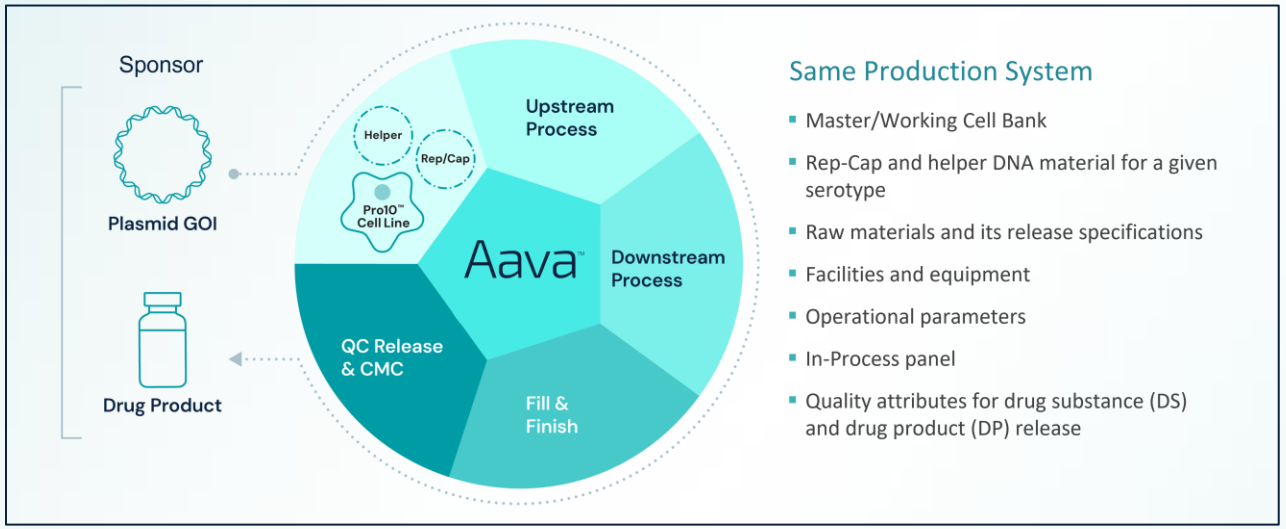
Completed Platform Work Accelerates Client Adaptation



Already completed at platform level

CQA risk assessment (RA)

Digitalized Process FMEA

Small-scale model qualification (SSMQ)

Adapted to each client's process

Update for product differences and close the remaining evidence gaps.

Source: CATT briefing, October 2024, Figure 1. Platform scope depends on the operation, serotype and product.

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The Platform CQA Risk Assessment is Already Complete



Reuse the assessment and update it for the client's product

Client-specific QTPP

Adapt the existing template

Attribute	Target (example)
Clinical Setting (e.g. Intended use in clinical setting, route of administration, delivery systems, number of doses)	Clinical setting (e.g. Intended use in clinical setting, route of administration, dosage form, number of doses, delivery systems various routes of administration such as intravenous, ocular, and via the central nervous system.)
Therapeutic Indication	Product specific
Mechanism of Action	Product specific
Shelf-life/Stability	≥X years at intended storage conditions.
Storage, handling, and transportation including stability	Formulation stable as a (formulation) at (temperature) °C or alternately frozen at -(temperature) °C or ≤-(temperature) °C for (duration).
Container /Container closure system / Packaging material	Selected to support product compatibility and stability and to ensure sterility. Common primary containers for gene therapies may include vial, bag, sterile sealed container.
Drug product quality criteria (e.g. appearance, purity, sterility, drug release, identity, potency)	It shall comply with all Pharmacopeia requirements and fit the geographic scope of the product. Microbial controls and testing based on the nature of the product (i.e., gene therapy products must be sterile), Use of an analytical test to determine the chemical and biochemical identity of a material. (i.e., presence of gene of interest), Measure of the active product received by the patient, (i.e., in vivo bioassay), Tests to assess the purity of the product, considering the product (e.g., empty vs full, process residuals, host cell protein, host cell DNA)
Delivery volume/Dosage Form/Strength	Variable based on patient weight. Particles per unit. Liquid suspension.
Safety & Efficacy	(required for Risk Ranking against this attribute) Biological Activity/Potency: The efficacy/potency of the product shall be ensured. Pharmacology: The Impact on Pharmacokinetics shall be controlled to ensure proper distribution and half-life of the drug. Immunogenicity: The product shall not lead to the formation of ADAs or any other adverse events in regard of immunogenicity. Safety: Product needs to be safe for intended use. Attributes related to the safety, efficacy, and quality of the product. These attributes are further assessed for level of criticality (determination of CQAs)

Completed platform CQA risk assessment

Completed literature assessment

- 4,500 papers screened by Bayer
- 417 abstracts screened by MSAT Viralgen
- ~150 papers identified
- 57 citations used for assessment of CQAs

Criticality	Attributes
Obligatory CQA	23
CQA	11
Potential CQA	15
Non-CQA	3

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Platform CQA RA and FMEA Shorten Client Preparation

Existing assessments focus the client-specific update

01 CLIENT QTPP

QTPP
Quality Target Product Profile

Define the client product

02 PLATFORM CQA RA

CQA
Critical Quality Attributes

Update the existing assessment

03 PLATFORM FMEA

PFMEA

Update with sponsor input

Reported WP 1–2 preparation: 5–6 months to 8 weeks

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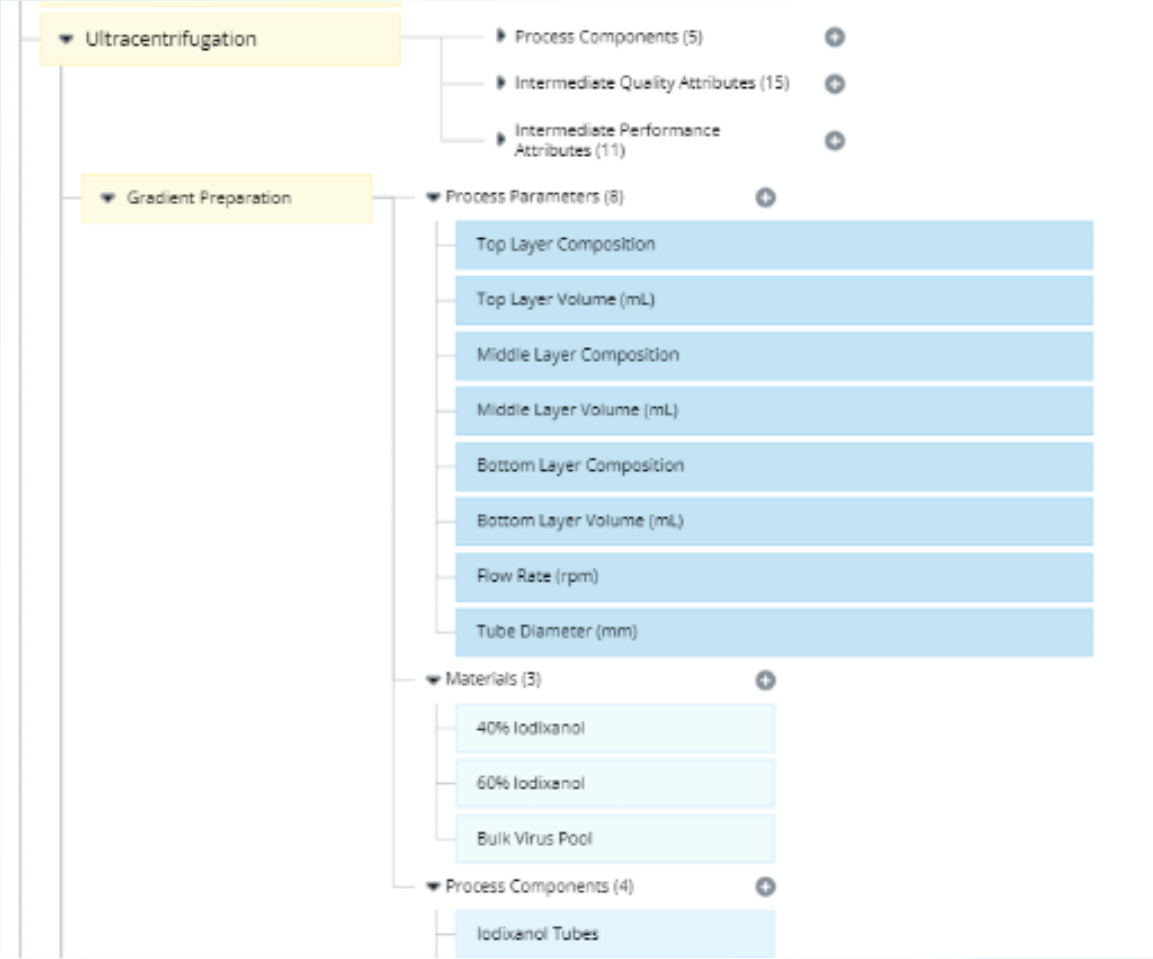
A One-week Workshop Adapts the Platform FMEA



Platform FMEA already digitalized

Update with the client

Link product specific process adaptations differences to the product's CQA RA.

Review pCPPs and pKPPs

Review CQA impact, occurrence and controls in a product specific manner.

Define follow-up actions

Set characterization scope and control updates.

Sources: attached "One week FMEA workshop with client" slide and existing Xtalks process-description figure.

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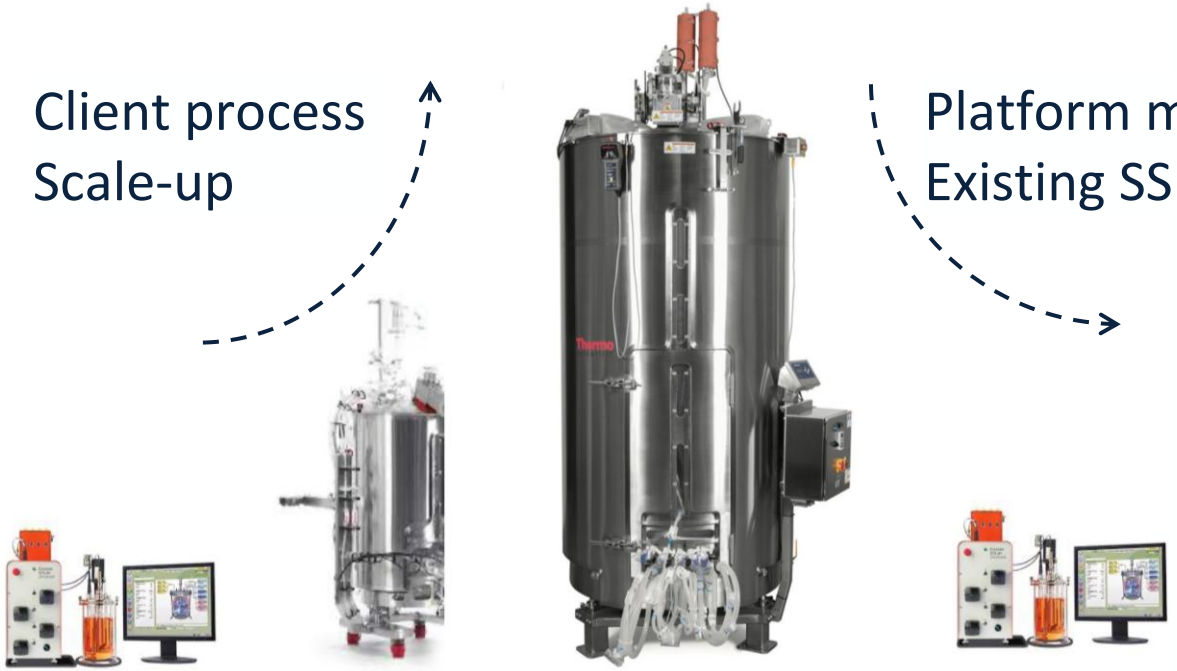
Qualified Platform SSMQ De-risks Product Specific Qualification

3 Adapt the qualified model to the client's process

Existing platform model and qualification

Client process
Scale-up



Platform model
Existing SSMQ

Commercial
Scale

Product-specific adaptation

Review unit-operation differences

Confirm relevant analytical responses

Assess model limits and acceptance criteria

Address remaining qualification gaps

Reuse platform SSMQ evidence to reduce repeat work and shorten preparation.

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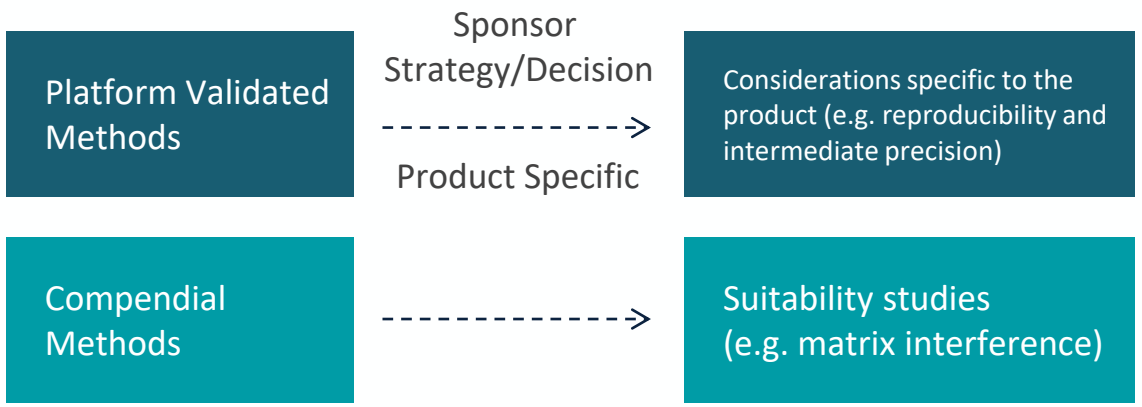
Stages of Commercialization Design: WP 4



Analytical Methods in a Platform Strategy

AAV CQAs		
Safety Assays	**Purity Assays**	**Identity Assays**
Mycoplasma	General Purity	Capsid Protein Identity
Mycobacteria	Full/Empty Vector Particle	Genomic Identity
Adventitious Virus	Residual Host Cell DNA (18S)	
Bioburden	Residual Host Cell DNA (E1A)	**Strength Assays**
Replication Competent AAV	Residual Plasmid DNA	Vector Genome Titer vg/mL
Endotoxin	Residual Host Cell Protein	Infectious Particle Titer
Sterility	Residual Immunoaffinity Ligand	
Subvisible Particles	Residual Chemicals	**General Quality**
Appearance (Coloration/Clarity)		Packaging (Primary Container)
Appearance (Visible Particles)		Extractable Volume
CCIT		Osmolality
		pH
		Particle Size Distribution

1. Validations methods strategy/timelines
2. Data strategy impacts in-process testing complexity
3. Comparability studies
4. Reference materials
5. Bridging studies
6. Sampling plan/volume for method validation



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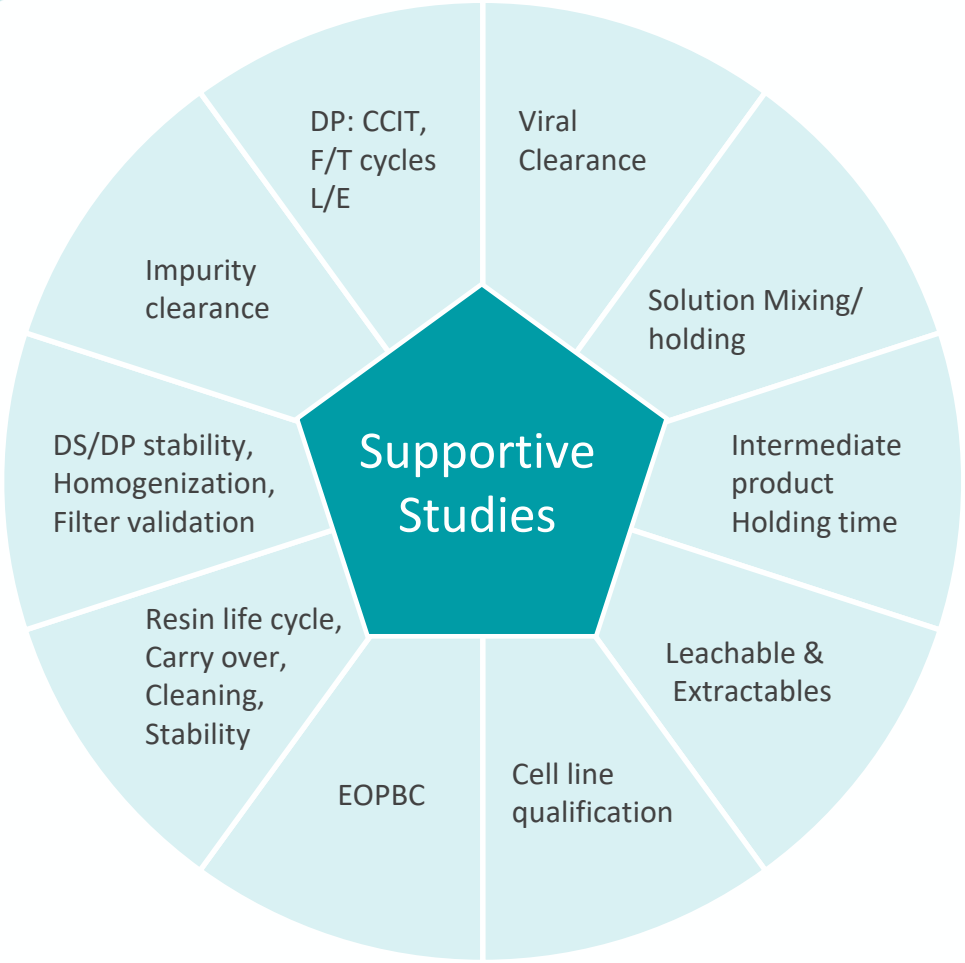
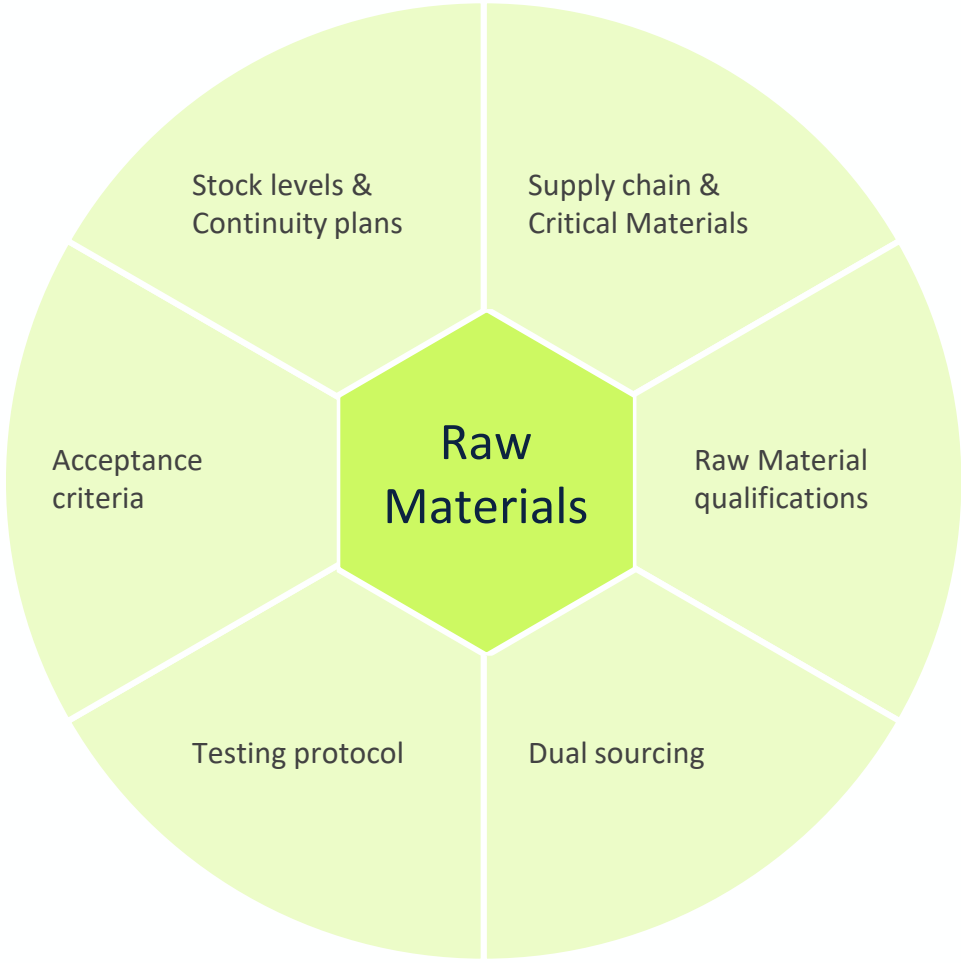
Stages of Commercialization Design: WP 5

Supportive Studies





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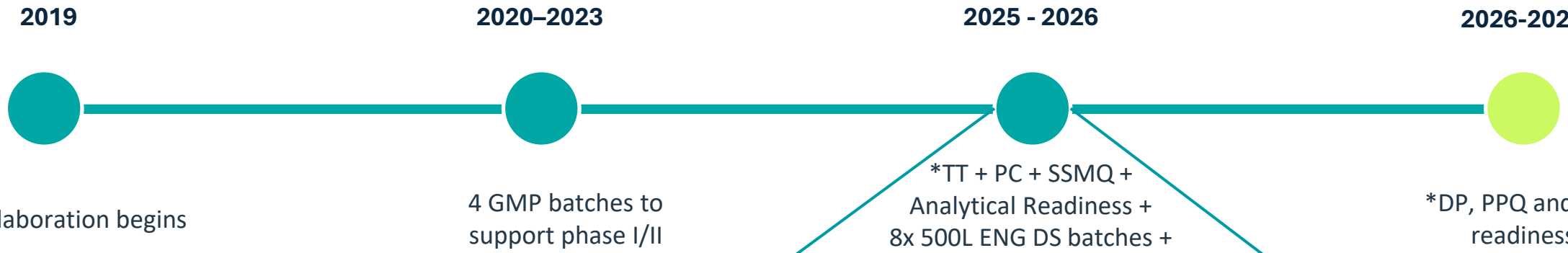
Case Study

A Customer's Experience with a Platform Approach to Commercialization

In Practice, Leveraging Prior Knowledge has had Measurable Impact



Achievements Thus Far:

- Lower Failure Risks (Informed by Prior Knowledge)
- Time Savings (>5 months)
- Cost Savings (~2500L of Production Avoided)
- Direction Support by FDA

One Year Towards Commercialization:

1 Process Characterization & Small-Scale Model Qualification			2 New Processes Implemented		3 Material Generation & Manufacturing Readiness
78	5	+500	4	32	8
2L Batches (PC & SSMQ)	50L batches	QC tests performed	Surrogate batches	QC Dev & Val / Verifications	500L ENG batches

* Acronyms: Technology Transfer (TT); Process Characterization (PC); Small Scale Model Qualification (SSMQ); Engineering (ENG); Biologics License Application (BLA)

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Key Learnings from our Platform Strategy



- Process history is derived from over 2,000 batches carried out using our **Aava™ platform.**
- Performance of a detailed risk assessment identifies and prioritizes Key and Critical Process Parameters (KPPs & CPPs).
- The insights gained from our platform process provide a comprehensive understanding of how process parameters relate to each CQA
- Different studies regarding commercialization readiness can be incorporated into distinct sponsor CMC documentation.
- By establishing a clear execution plan between the sponsor and our platform strategy, it is possible to accelerate the timeline to PPQ.

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Thank You

Questions?