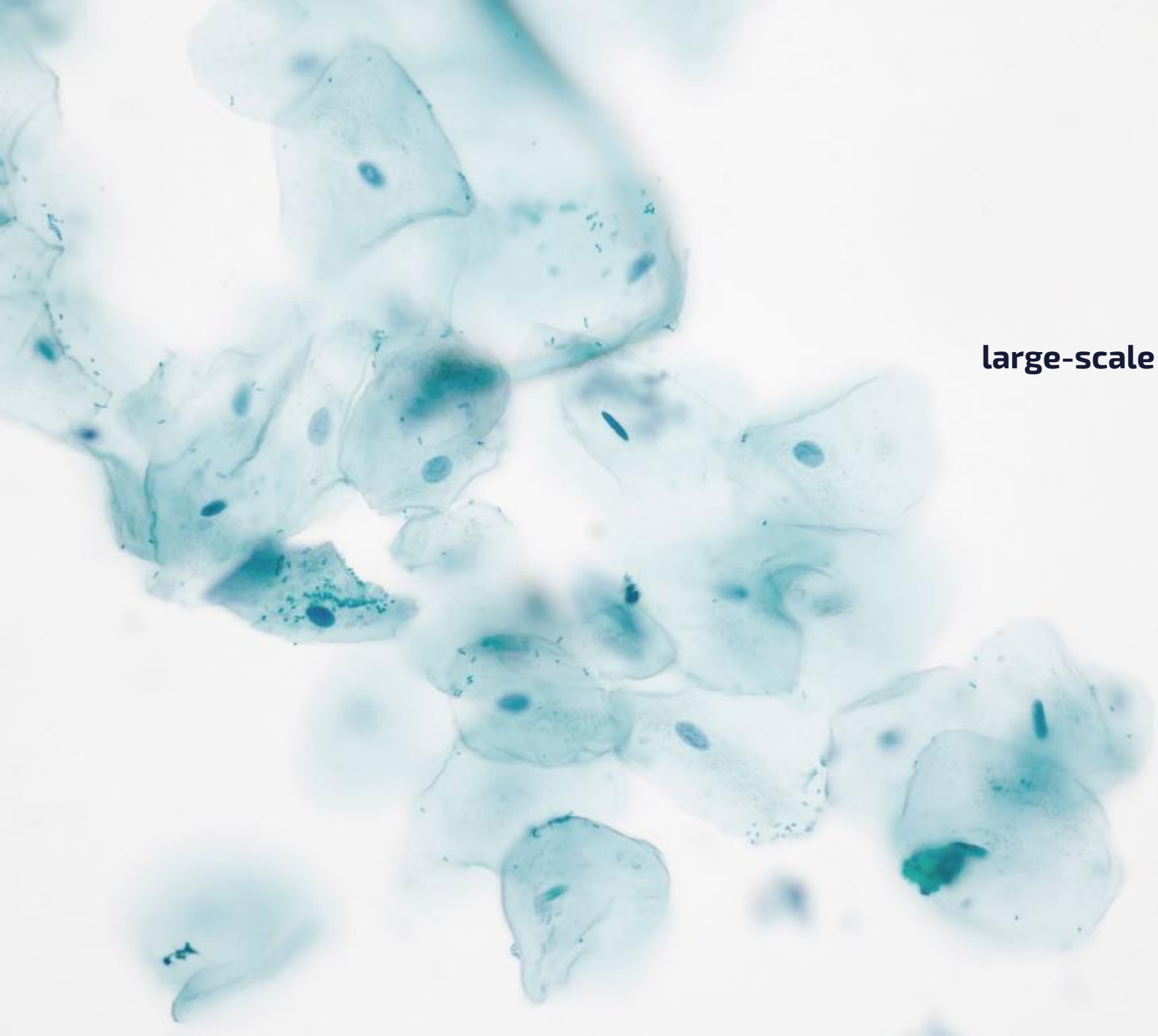
YOUR NEW HORIZON IN cGMP GENE THERAPY MANUFACTURING

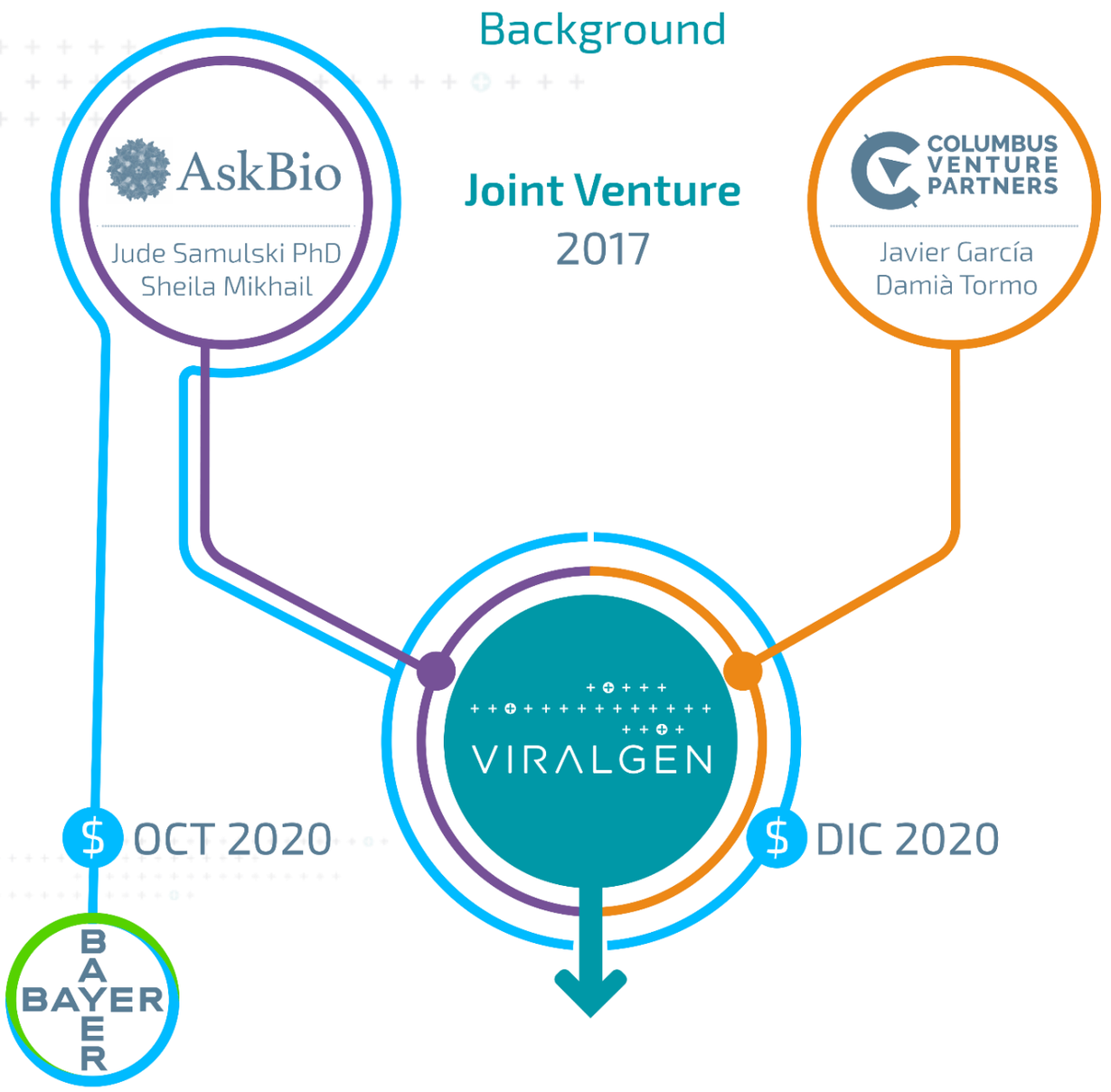
VIRALGEN



VIRALGEN

Addressing the challenge for
large-scale cGMP manufacturing of rAAV vectors
with an efficient triple transfection in
suspension **Pro10 ™ cells.**

About us



Viralgen is a CDMO born as a joint venture between AskBio and Columbus Venture Partners, combining decades of technology and drug development experience in multiple platforms to support best-in-class service offerings to the gene therapy market.

Viralgen was created in 2017 to respond to the unmet need for manufacturing of gene therapies, with the goal to help broaden access to these life-saving therapeutics and to contribute to the advancement of health and human welfare around the world.

We specialize in the production of rAAV viral vectors, and have built an optimized facility in San Sebastian, Spain that maximizes throughput and efficiency of our proprietary Pro10™ suspension manufacturing platform, enabling industry-leading scalability, reproducibility, and speed to market.

Through our superior technology platform, we deliver industry- leading titers and cGMP-certified quality for all AAV serotypes to our client partners, optimize the cost-of-goods and accelerate clinical development and commercialization of life-saving genetic medicines.

In October 2020, Bayer acquired Askbio, giving the project a definitive boost. We will continue to operate with the same brand, corporate culture and current management, adding the financial and technical capacity of the multinational. This will enable us to fulfill more effectively our founding purpose of providing healing through gene therapy to patients who, until now, did not have it.

Columbus Children's partnership with Viralgen Vector Core provides partner programs

- Access through our dedicated foundation slot availability.
- Flexible payment terms such that nonprofits do not have huge pay back requirements or penalties typical in private sector market.
- Waives typical up-front payments to secure GMP manufacturing slots required for nonprofit customers.
- Lower costs through "buying down" market rates.

Columbus Children and Viralgen have partnered with the following organizations to provide access to vector production for the following programs

- **September** | Cure Rare Disease, DMD Exon 1 deletion (initial vector)
- **September** | Cure SPG50, SPG50 (initial vector)
- **October** | Phoenix Nest, MPSIIIC (San Filippo)
- **November** | CCF, AADC new vector
- **January** | CCF, NPA (AAV9 new vector)
- **February** | CCF, NPA (AAV2 new vector)


The New York Times

HEALTH

Gene Therapy Hits a Peculiar Roadblock: A Virus Shortage

Pfizer chooses Sanford, North Carolina site for $100m gene therapy plant

PFIZER AIMS TO BECOME INDUSTRY LEADER IN GENE THERAPY WITH AQUISITION OF BAMBOO THERAPEUTICS, INC.

Strategic location| San Sebastián | Basque Country

- 2 Facilities, ~ 1 km apart
- Key location for Spanish, French and international talent
- Strong local government support for training and growth

Viralgen Clinical: opened August 2018

- 4000 m^2, 1250 m^2 GMP
- 4 independent suites 250/500L
- In house QC and Fill-Finish

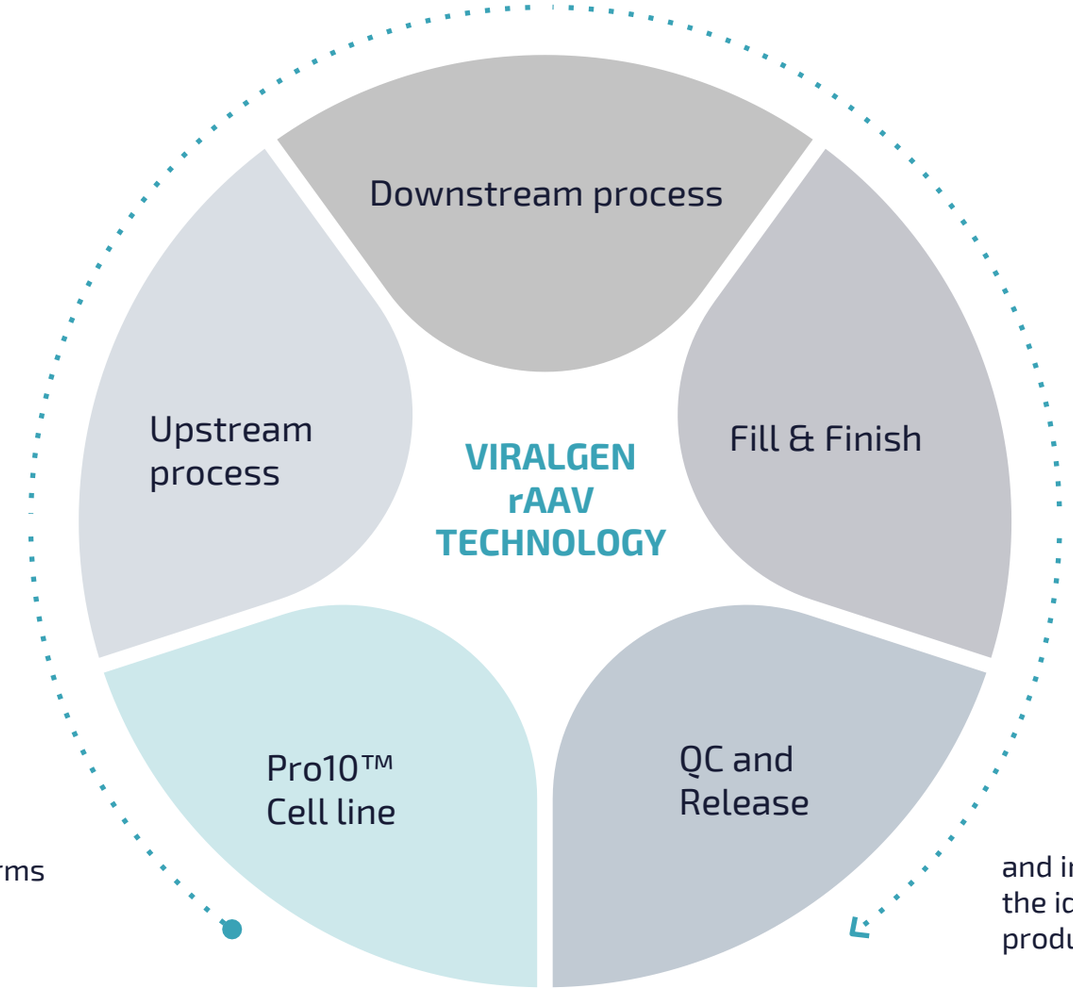
Viralgen Commercial: opened June 2021

- 9 suites, 3 independent modules
- ~18000 m^2 of manufacturing, QC and warehousing
- 2,000L scale

Manufacturing platform

VIRALGEN has the capability of continuously processing 50L to 2000L batches with depth filtration, affinity and Ion exchange chromatography & tangential flow filtration.

Plasmid-based triple transfection. 50 to 2000L single-use stirred-tank bioreactors.



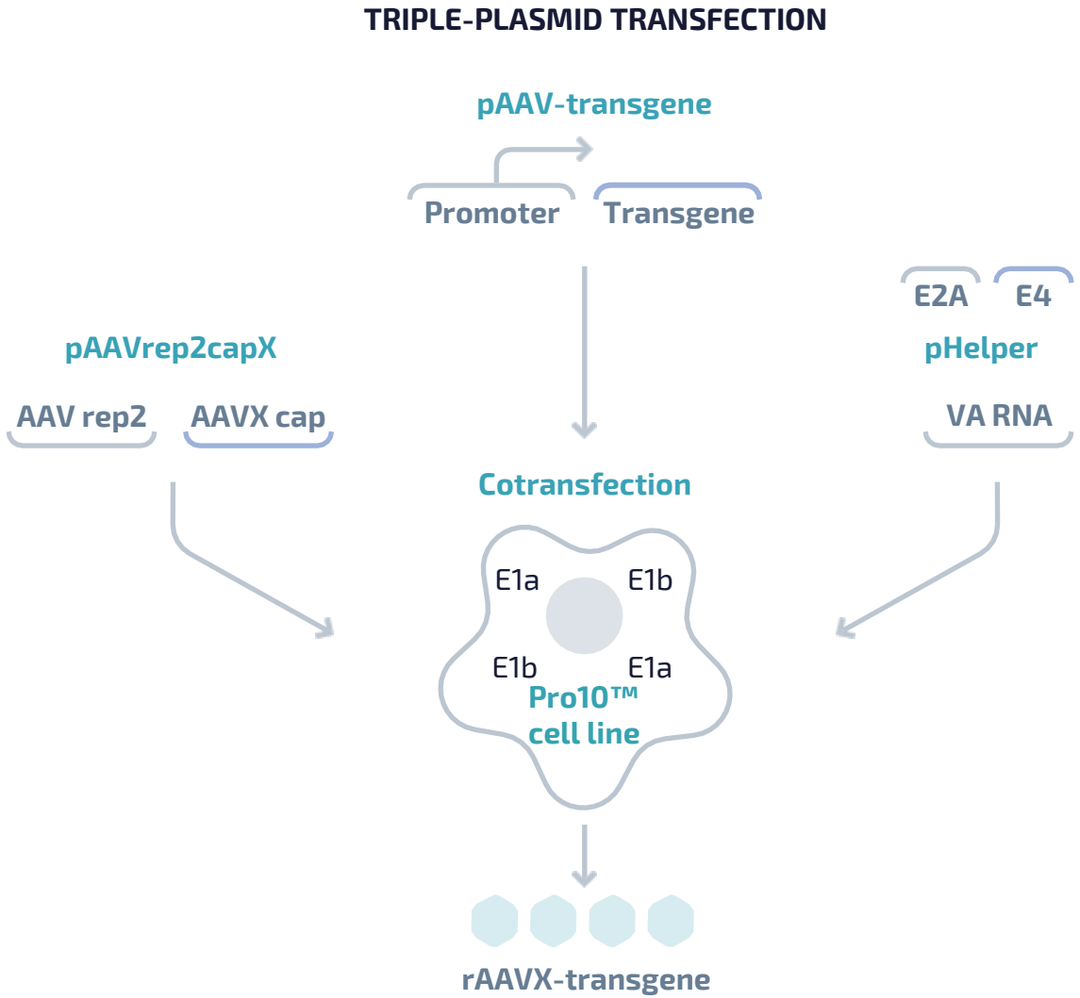
Inhouse aseptic fill & finish process.

Proprietary Pro10™ is an animal-derived component-free suspension-adapted cell line, derived from HEK293 cells.
Pro10™ is a unique high yield universal system that can produce all serotypes and chimeric forms of rAAV.

Manufacturing includes drug product and in-process testing services to ensure the identity, quality and purity of the product.

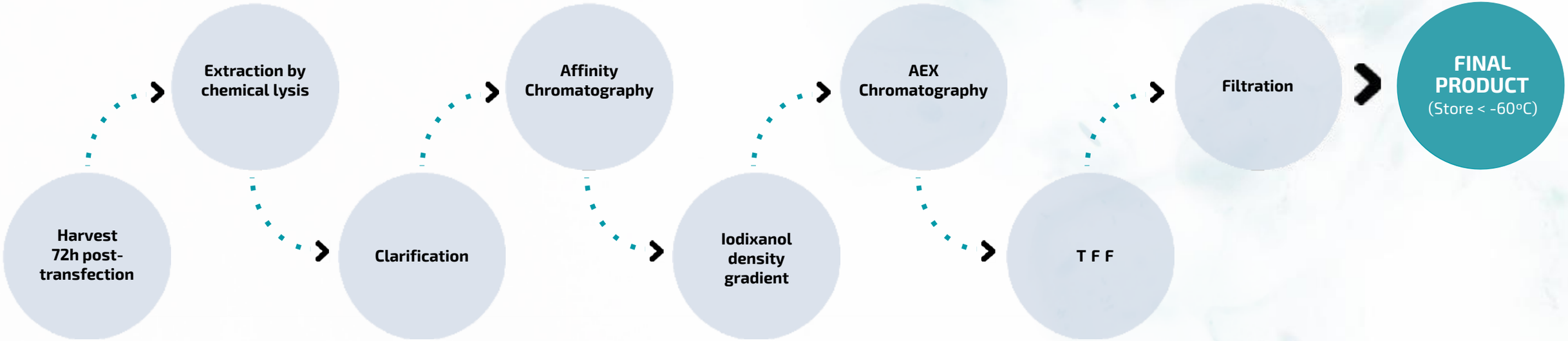
Viralgen proprietary technology for rAAV production

Pro 10™



Powerful and scalable production platform

Pro 10™ Cell Line



Main Strengths | Viralgen cGMP manufacturing

Proprietary Mammalian cell-based production platform

- Viralgen has licensed **Pro10 ™** cell line and the manufacturing process from AskBio.
- **Pro10 ™** cell line has been derived from HEK293 cells and shows high yield.
- SV40 sequences are not presented in **Pro10 ™** cell line.
- Industrial **scale serum-free** suspension culture system: manufacturing process based on triple transfection of **Pro10 ™** cell line.

Large-Scale cGMP Capacity

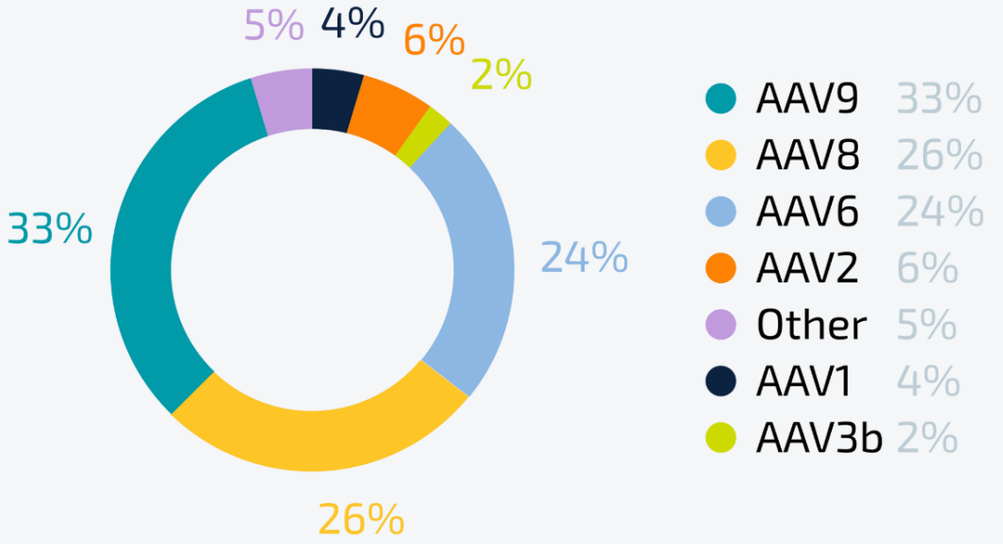
- **4 independent state-of-the-art cGMP** production suites (>1800m2), providing the capability to continuously manufacture 4 different products simultaneously.
- **50L** scale to supply Toxicology/biodistribution studies.
- Viralgen uses **250L** and **500L** single-use stirred-tank bioreactors for culture of suspension cells in clinical manufacturing and **2000L** bioreactors in its commercial facility
- In-house QC lab for critical release assays.

Continuous Improvement and Development partnership with AskBio

- Upstream and Downstream Process Improvements.
- QC Development and Validation.

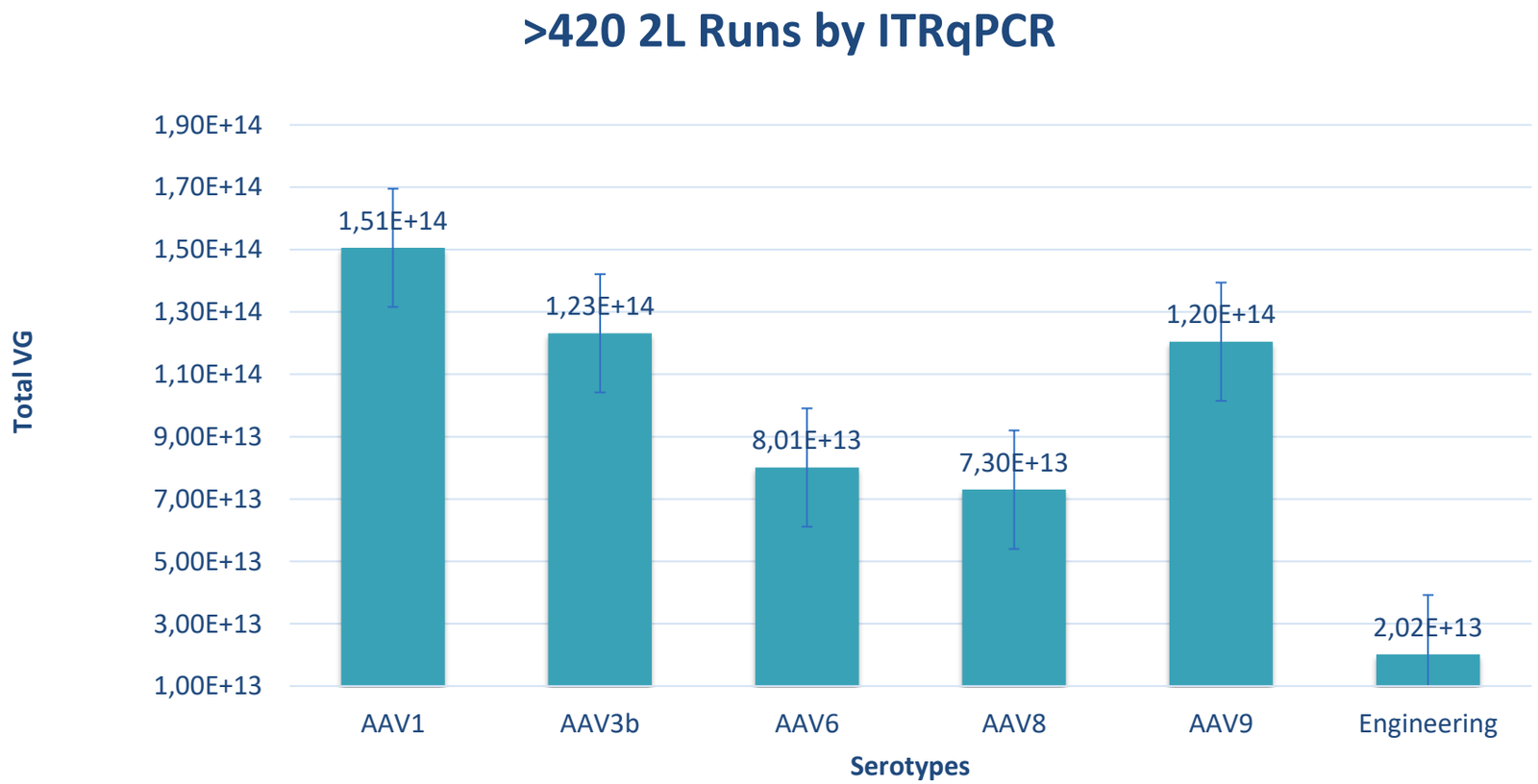
Viralgen platform has succesfully manufactured
numerous batches of diverse serotypes

	Batches Produced
Research Batches (2L)	**420**
Toxicology Batches (50L)	**70**
Clinical GMP Batches (250 L | 500L)	**45**
Engineering Runs in Commercial Facility (2000L)	**4**

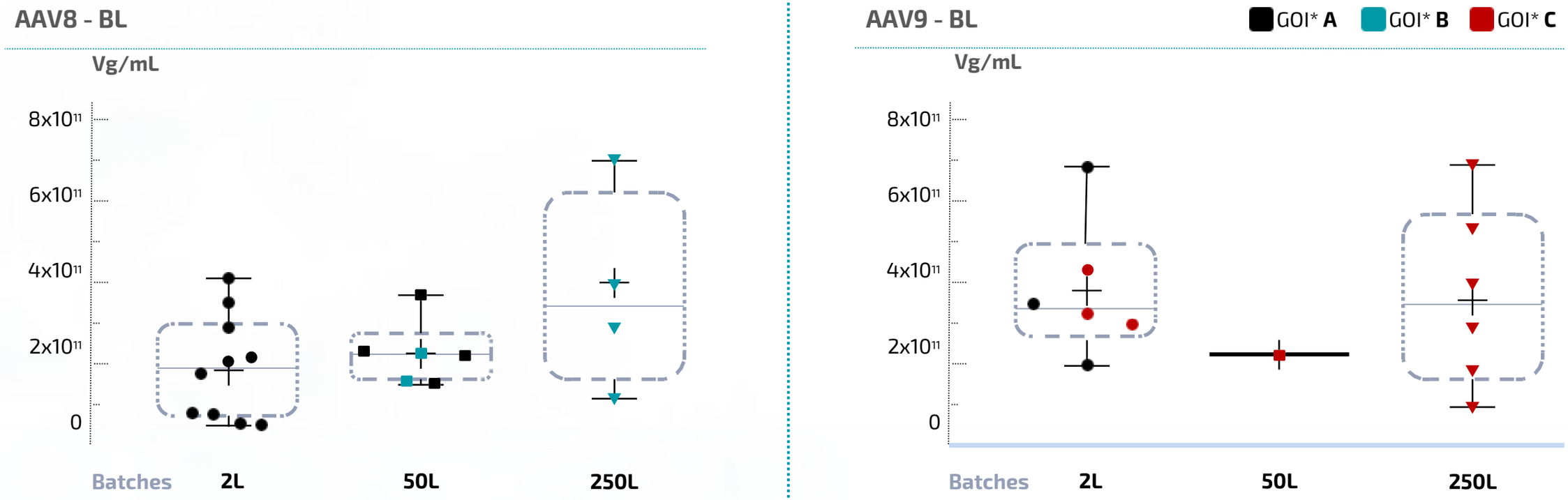


Powerful and scalable production platform

We've captured process data from every run and use that to provide powerful analysis to you on your vector's manufacturability and quality.



Pro 10TM Cell Line production for AAV8 and AAV9: scale-up study



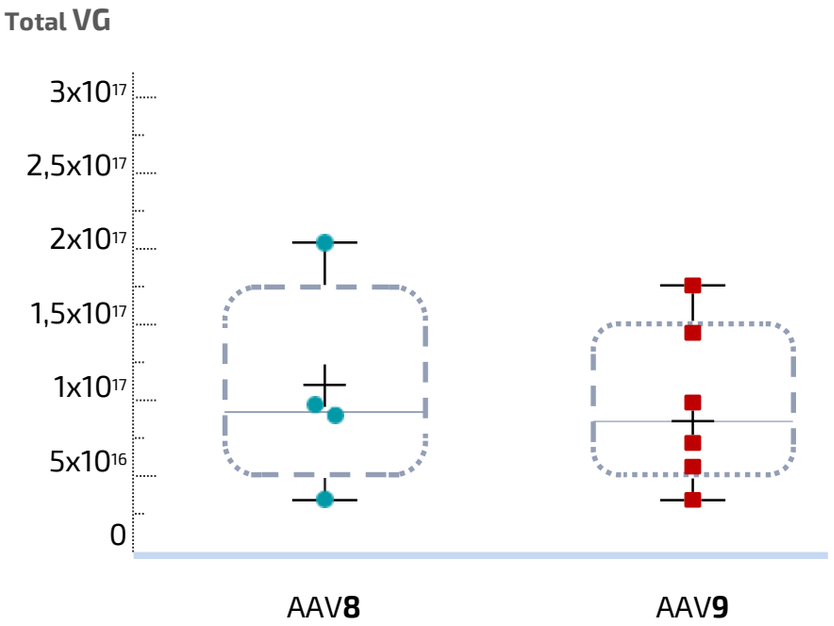
*GOI-Gene of Interest

Pro 10™ Cell Line production | Comparison between batches

AAV8 and AAV9 250L GMP Batches (Total VG at harvest)

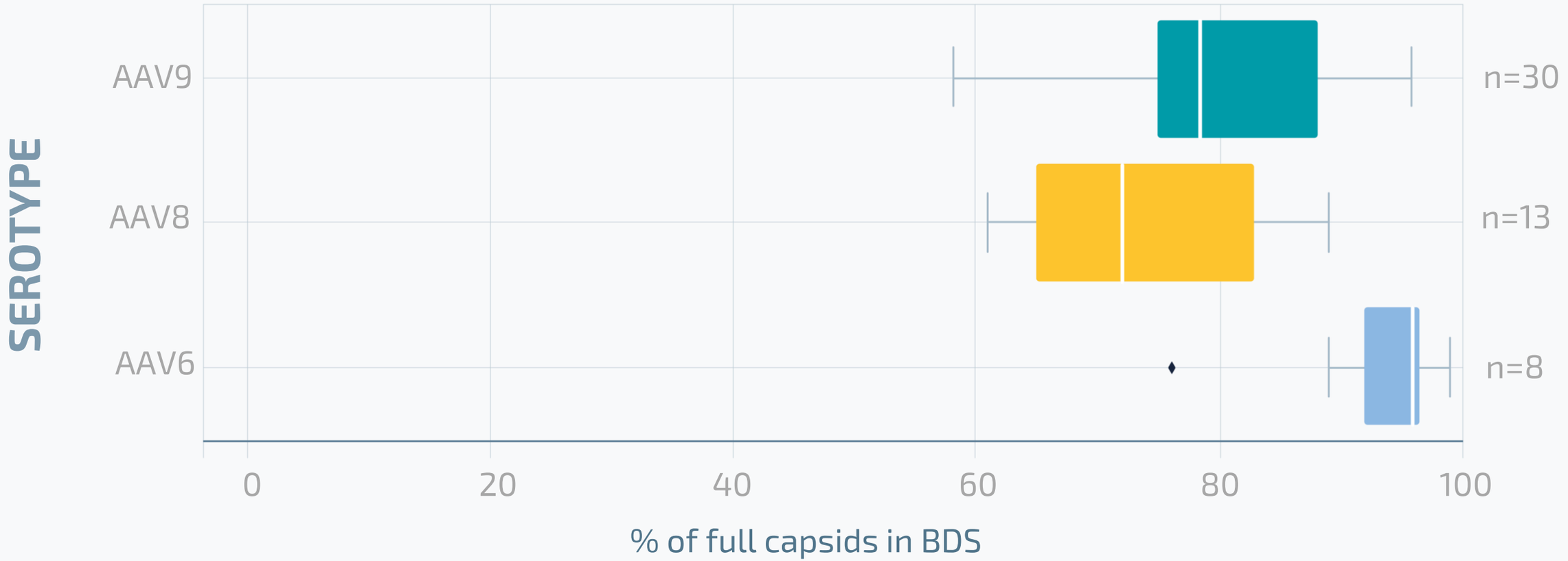
SUB 250L - BL

GOI* **A** GOI* **B**



*GOI-Gene of Interest

Full:Empty



- Production and purification protocol yields a high % of full particles.
- AAV6 higher % of full particles than AAV8 or AAV9.

Business Principles and Practices

VIRALGEN | CLIENT

Time to lot release

1 .5 months
2 L

Feasibility study and manufacturability assessment

4-5 months
50 L

Toxicology Batch production

5-6 months
250L & 500 L

cGMP Batch production

Overview Proposal

Feasibility Study Contract (simple T&Cs)

Master Service Agreement and Work Statement

Quality Agreement and Authorization Letter

Stage-appropriate and data driven process to fulfill customer requirements

Our model

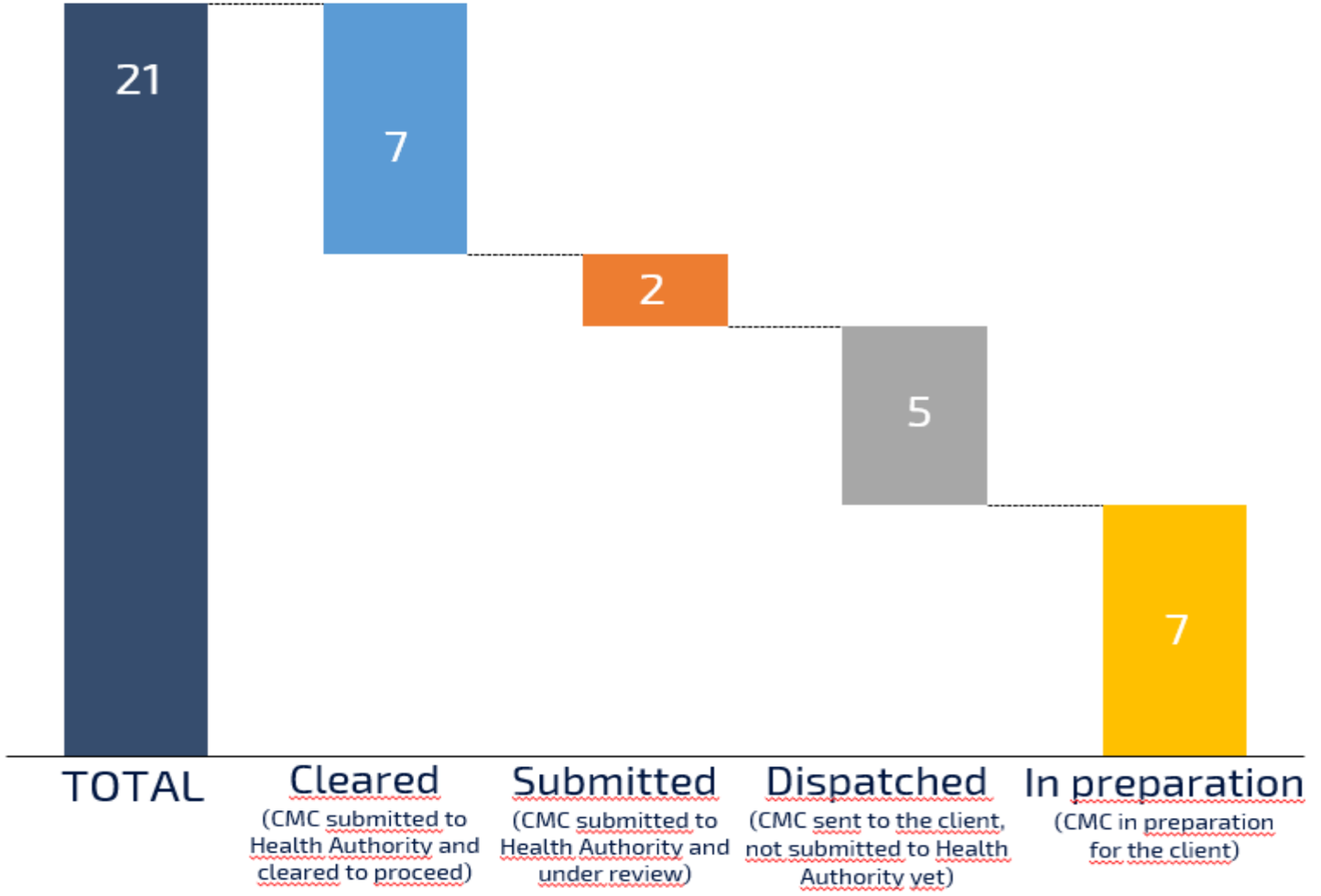
Fixed and inclusive price:

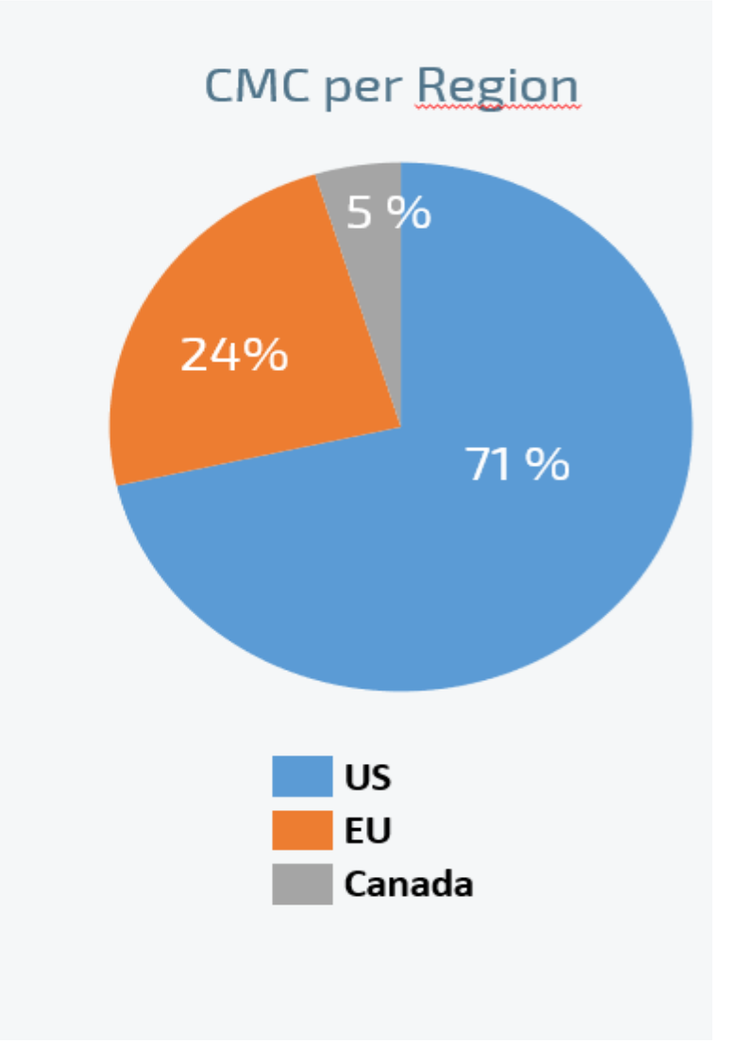
- QC, Fill and Finish, regulatory support all included
- Rep, cap and helper plasmids included
- No license fees or royalties

Robust Platform

- Minimal time in PD > rapid clinical acceleration
- Quick cycle times > high throughput, fewer capacity limitations

Global CMC Status | CMC managed up to date





Client-Centric Experience

PROGRAM MANAGEMENT

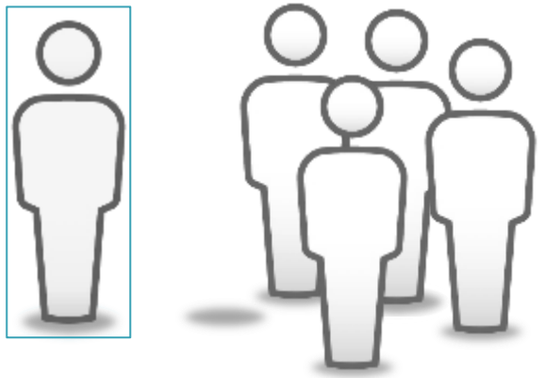
Your program will be fully supported by a dedicated program manger

PM owns from post-signature kickoff to completion of projects

Once contract signed PM serve as primary contact for the Program and communications with customer and internal Viralgen project team members.

Conduct project meetings with Customer on a regular basis.

Track Program progress against timeline and project plan and provide updates to Customer on an ongoing basis



Your success is our success and it is based on high quality, flexibility, fluent communication and a trusting relationship.

Further Viralgen Experience

>550
Batches Manufactured

>45
Clinical Batches Manufactured

>20
Clinical Supply Agreements

>50
Manufacturing Clients

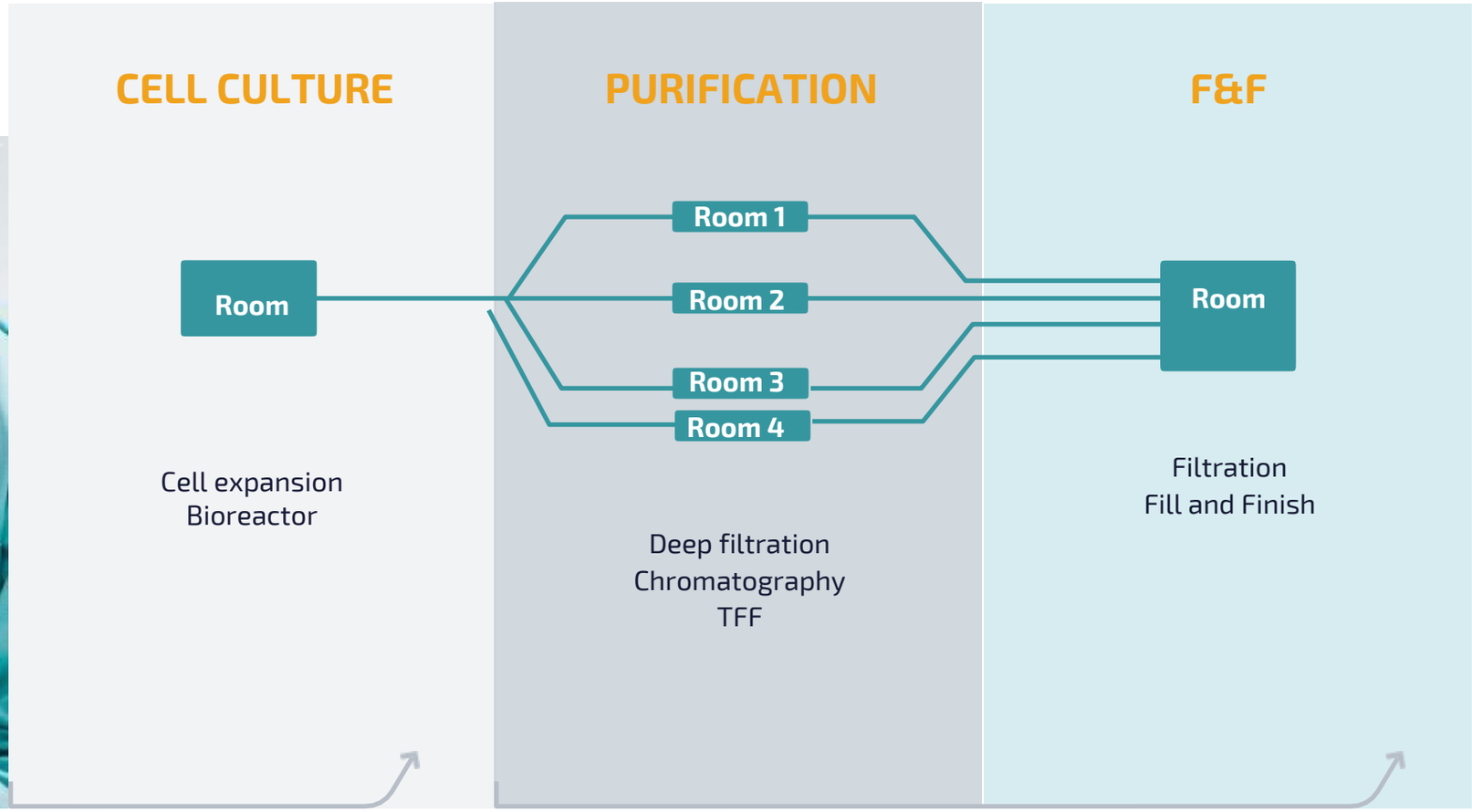
CombiGene

SIO GENE THERAPIES

AskBio MAKING HISTORY

CURE SPG50

Flowchart cGMP Production



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