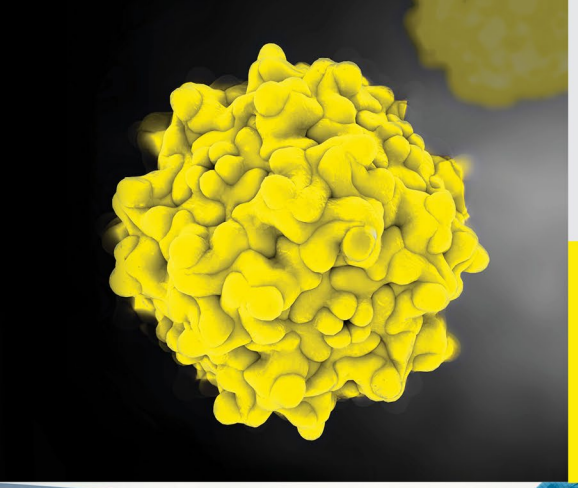
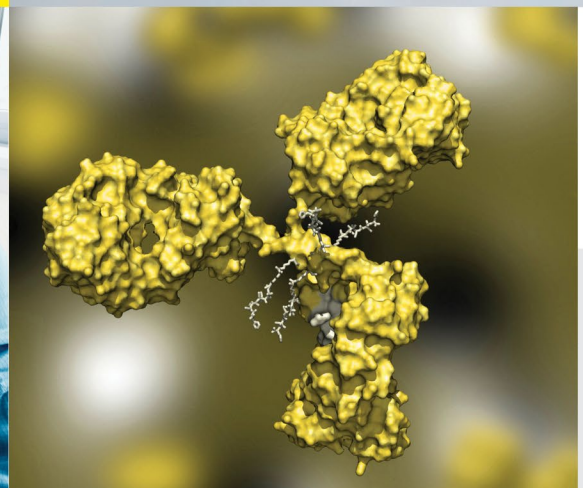




Simplifying Progress

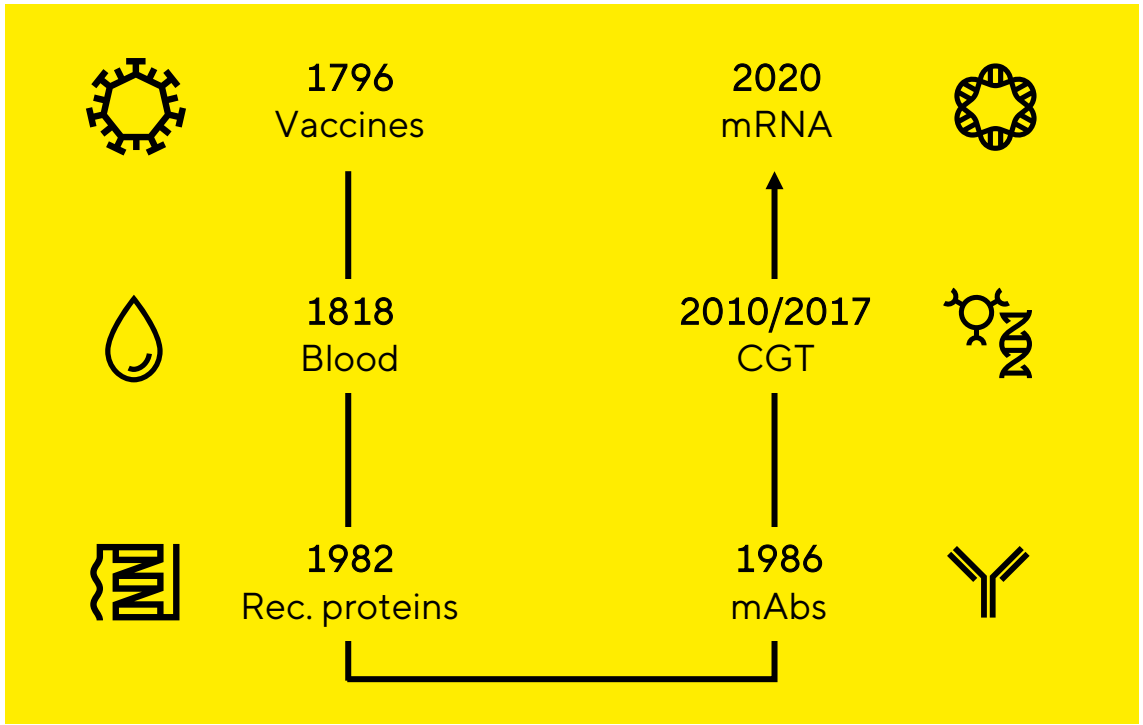
# Capital Markets Tutorial
# Sartorius offering for cell and gene therapies

René Fáber, Mario Philips
November 6, 2023

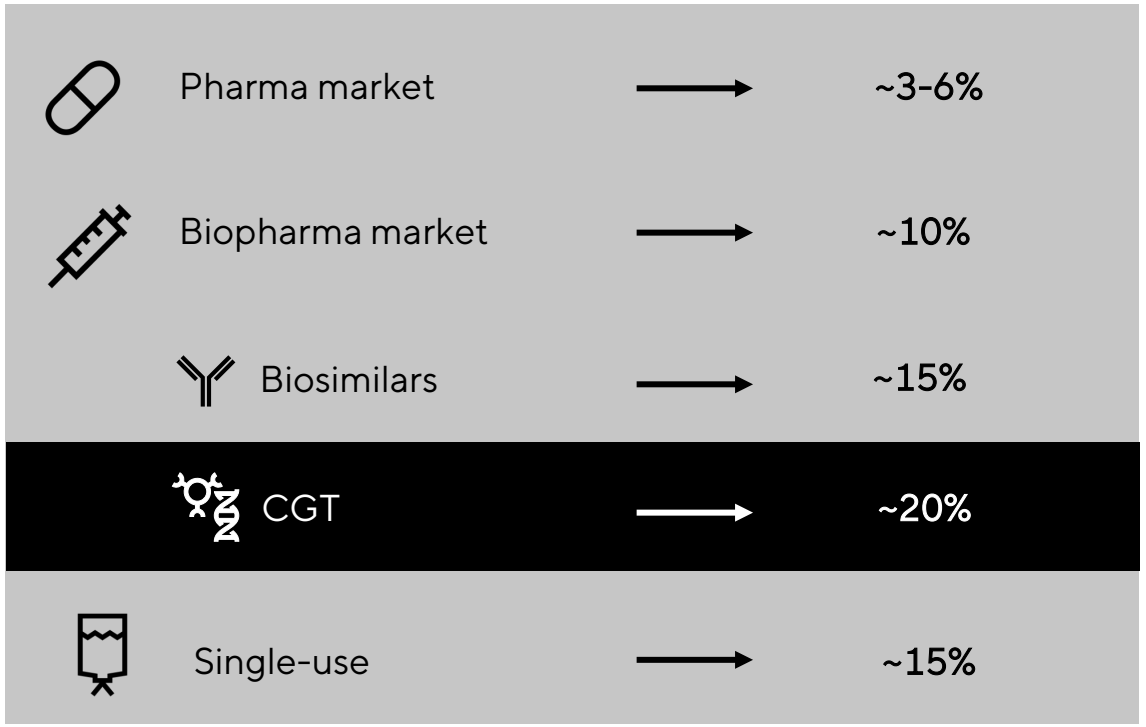
SARTORIUS

# Cell and gene therapies (CGT): an upcoming catalyst for growth

## The evolution of biologics




## Pillars of growth CAGRs 2022–2027[1]




1 Source: Company estimates based on EvaluatePharma 2023, IQVIA 2023, Global Data 2023, Roots Analysis 2021, Markets & Markets 2023

# CGT is becoming an increasingly relevant market segment

## ~30% of biopharma pipeline is focusing on CGT[1]

~70%
Other biologics

~30%
CGT

**>6,000**
CGT candidates in development[1]

## Number of commercial processes is expanding rapidly

**~60**
approved CGT globally[1]

**11**
CGT approvals in 2022 and 2023 alone

**~10-20**
CGT approvals expected p.a. by 2025[2]

1 Source: GlobalData, October 2023 2 FDA



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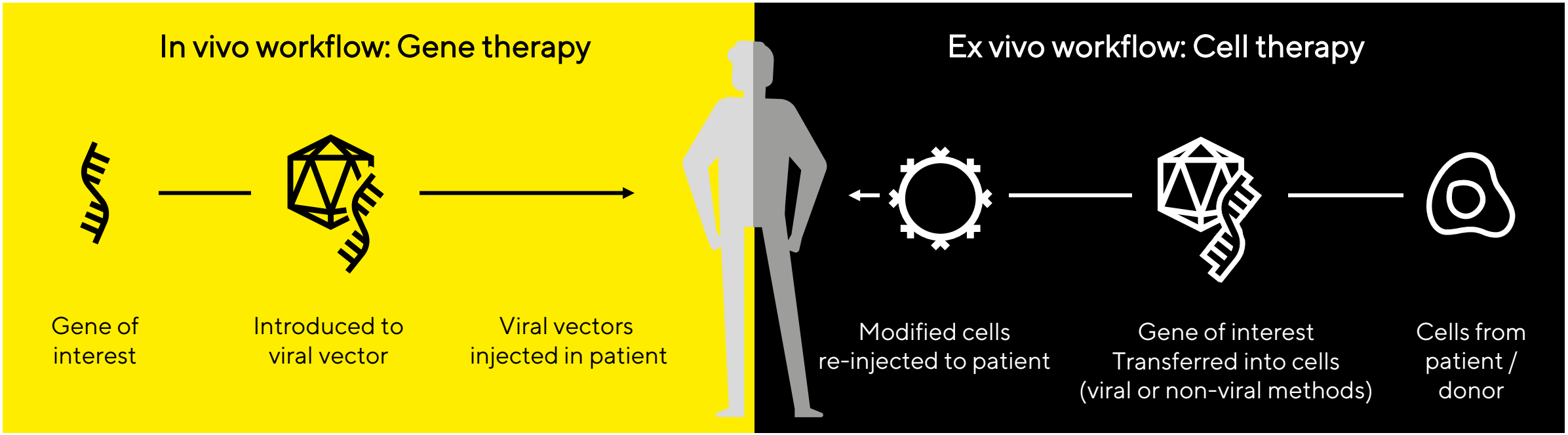
# How do gene and gene-modified cell therapies work?




**Viral vectors** are used to deliver genetic information into cells

# Need for innovation in CGT development and manufacturing

## High treatment costs

Gene therapy[1]:
Blood disorder ~€2.8 mn

CAR-T cell therapy[2]:
Blood cancer ~€280,000

Gene therapy[1]:
Hemophilia ~€3.5 mn

## Inefficient manufacturing processes

- High manufacturing costs
- Platforms not yet established
- Low yields
- Lack of process robustness

## Needs

- Higher transfection efficiency
- High-quality GMP raw materials
- Better purification technologies
- Automation and process analytics

1 Cost per treatment in the USA  2 Cost per treatment in Europe



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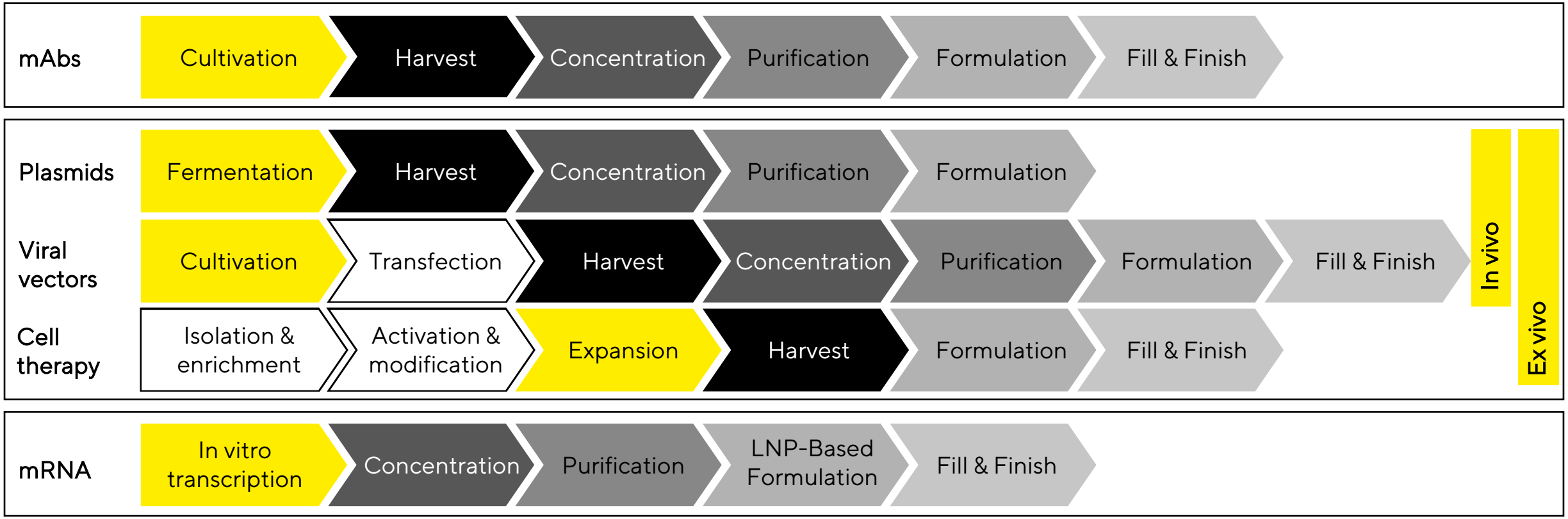
# Manufacturing of new modalities has similarities with antibody production



# Sartorius offers scalable up- and downstream solutions for all CGT...

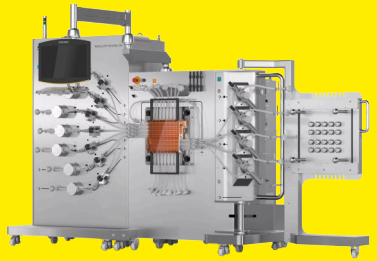
## Analytics

## Fluid management

## Expansion

## Harvest / Filtration / Concentration

## Purification



Source: Sartorius Group: The illustration shows only a small part of the relevant portfolio



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# ...with differentiated separation technologies and analytics...

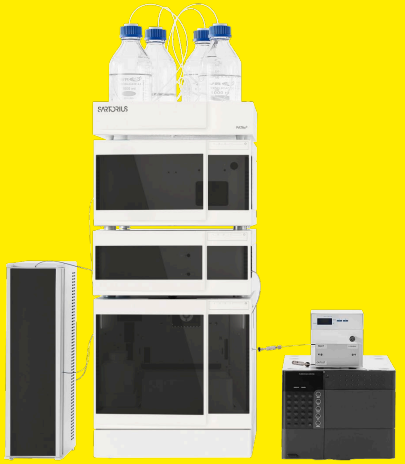
## Purification of large molecules with CIMmultus monoliths

Higher drug quality,
higher chance of clinical sucess

## Automated centrifugation system Ksep

Single-use gentle cell washing
for allogeneic cell therapies

## At-line process analytics with PATfix



Fast analytics to optimize and
control transfection process

# … and a complementary offering of media and critical raw material

| Biological Industries | CellGenix | Xell | Albumedix | Polyplus |
|---|---|---|---|---|

Cell culture media

Growth factors and cytokines

Media and supplements

Recombinant albumin

Transfection reagents and plasmid DNA



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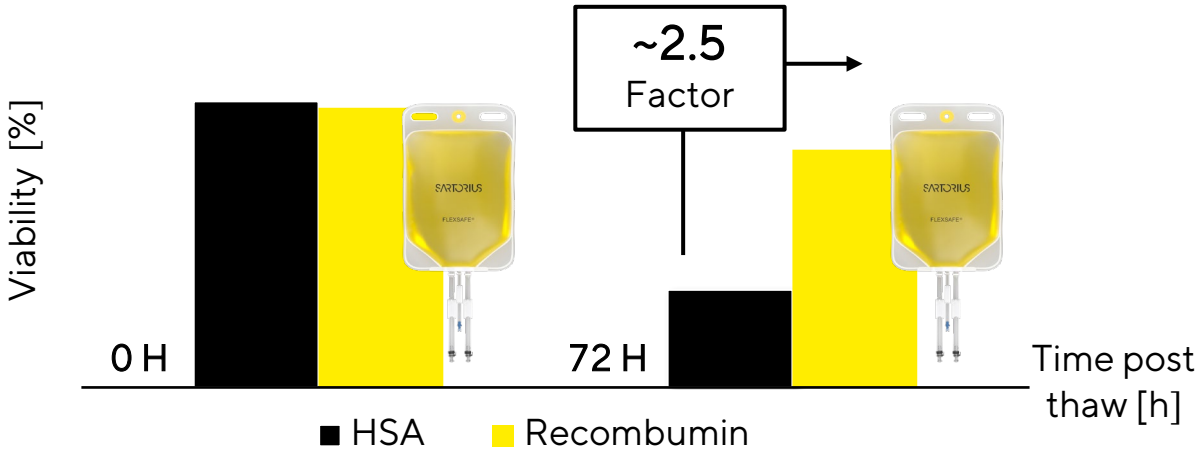
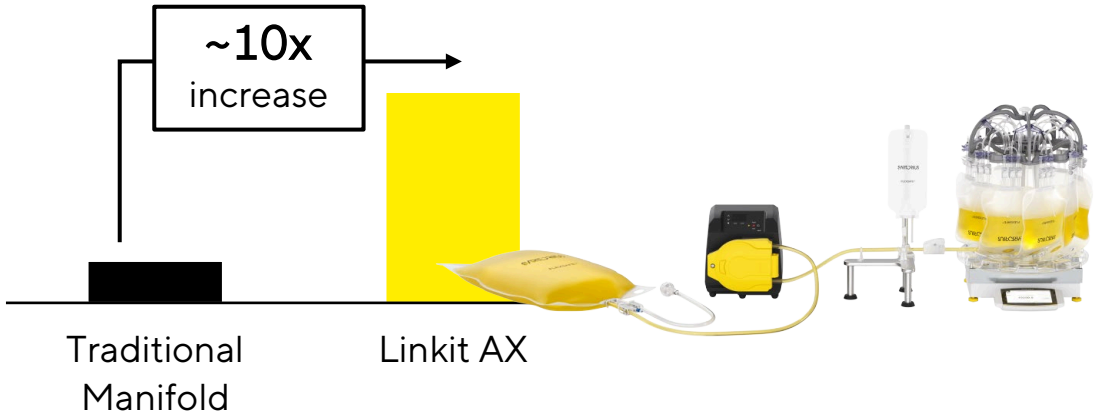
# Strong core capabilities paired with media and raw materials create enhanced customer value

## Combining Recombumin® with media




- Higher and more consistent quality profile
- Can be used in multiple process steps to improve cell growth and protect cells from physical stress, e.g. during separation or freezing

## Filling media for autologous cell therapies using Linkit® AX

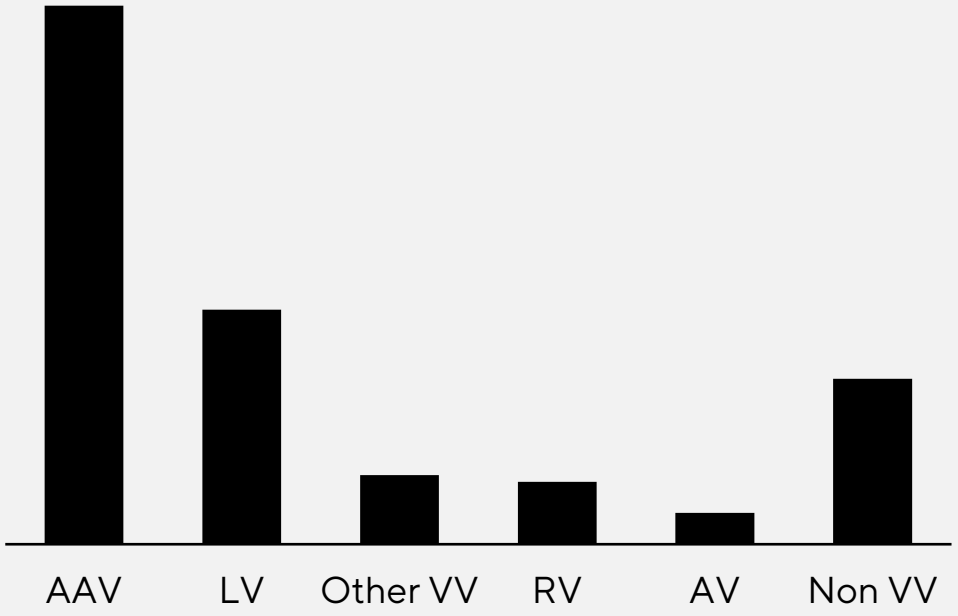



- Very accurate and quick in filling media for cell therapies
- Workflow simplification leads to efficiency gains and lower labor costs

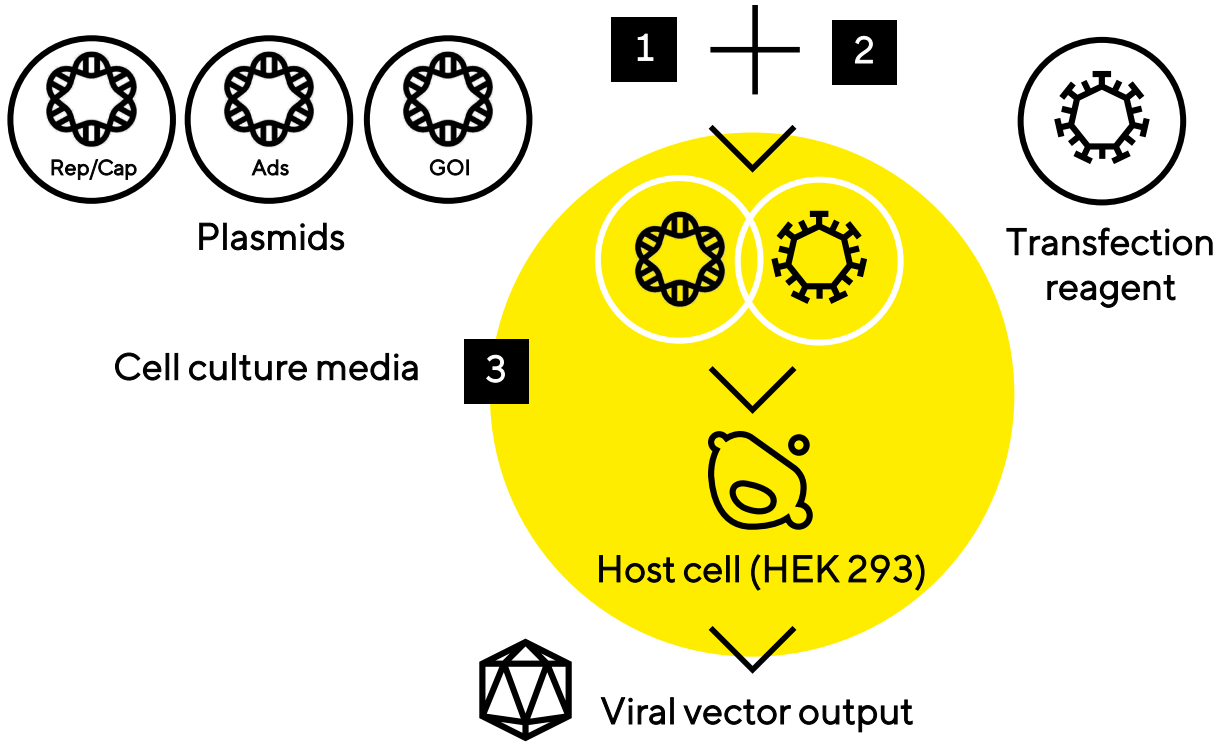
1 HSA = (standard) Human Serum Albumin

# Plasmids and viral vectors are vital for the functioning of most CGT

**Types of viral vectors**
CGT pipeline by vector type[1]




**Plasmids are critical raw materials for the production of viral vectors**




Acronym: AAV = Adeno Associated Virus, LV = Lentivirus, Other VV = Other viral vector, RV = Retrovirus, AV = Adenovirus, Non VV = Non viral vector; 1 GlobalData, 2023

# Polyplus: Mission-critical reagents for gene therapies

## Transfection

- First mover advantage with PEIpro®
- Leading innovation with next generation transfection technology
- Strong market penetration with FectoVIR-AAV in next-gen suspension processes

## Plasmid design and production

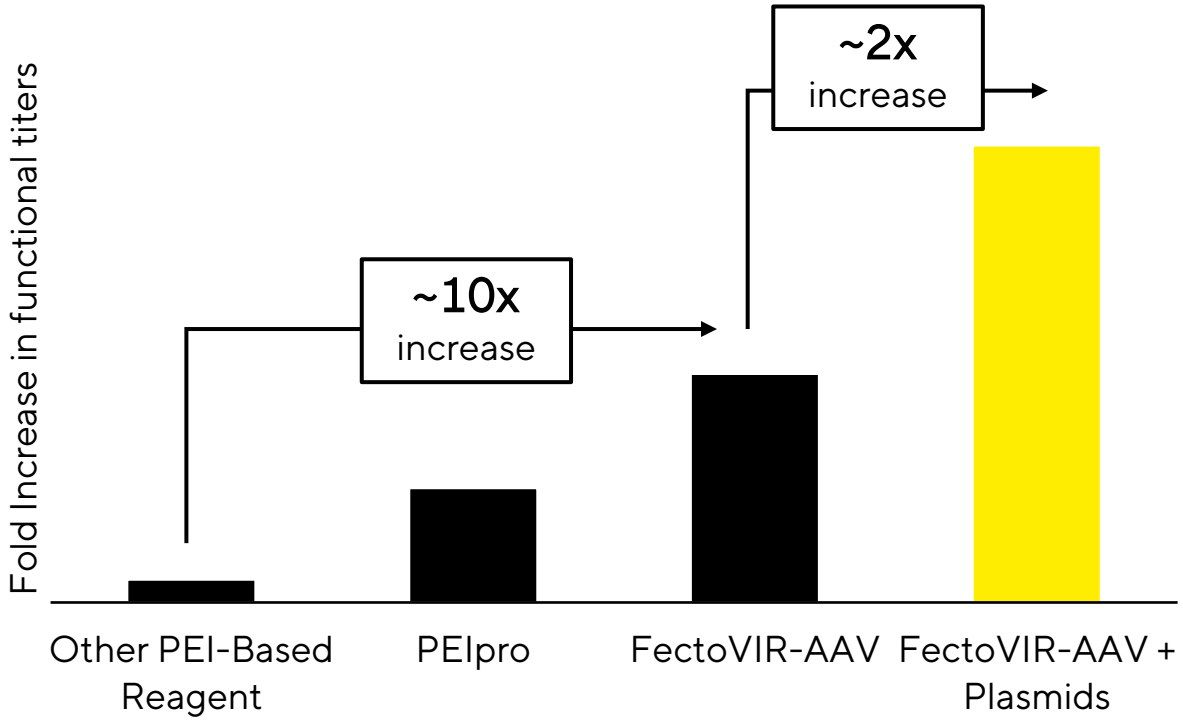
- Next-gen plasmid engineering technology
- High success rate
- GMP accreditation
- Fast turnaround time, proprietary manufacturing process, flexibility

## Innovative gene-delivery reagents

- Emerging portfolio for DNA/RNA therapeutics
- Cationic lipids and transfection polymers which are a critical component in the formulation of LNPs

# Industry-leading transfection reagents and plasmids deliver high performance and scalability

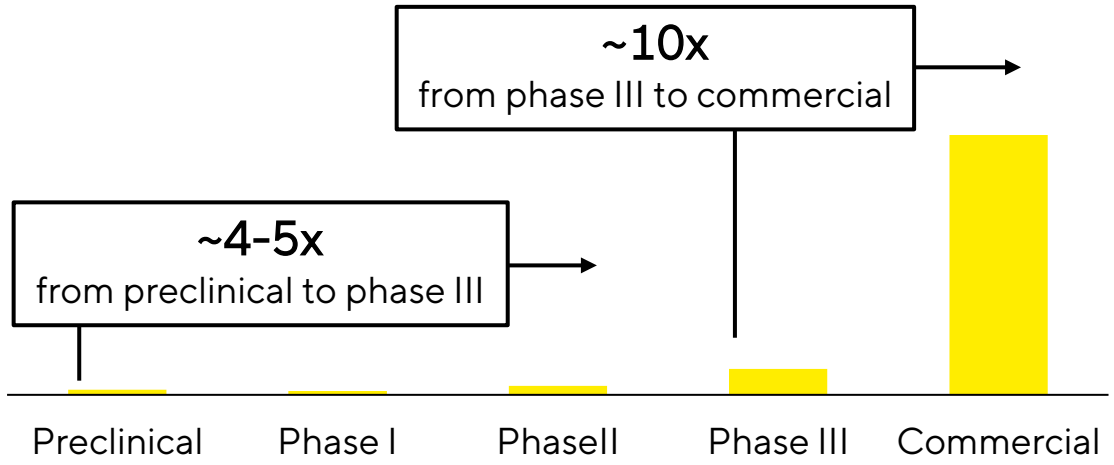
**Up to 20-fold increase in functional viral titers with FectoVIR-AAV + Polyplus' optimized plasmids**




- Highest viral vectors titers in suspension systems
- Improved full/empty capsids through optimization of plasmids, resulting in improved viral particle quality
- Adapted to large-scale production:
  - Long complex stability
  - Low complexation volume

# Strong market position and unique set of expertise

**Attractive business model - Upstream material spend by phase**
€ in millions




- Integrated workflow solutions to speed up time-to-market and improve process economics
- Spec`d into significant portion of approved & pipeline CGT
- Deep application know-how in areas such as polymer synthesis and formulation, advanced biologics processes, molecular and cell biology as well as scientific and regulatory support

# Excellently positioned for CGTs' uptake

- Large and growing pipeline of gene and gene modified cell therapies as well as DNA/RNA therapeutics in development
- Sartorius well positioned to benefit from above-average market growth
- Spec wins through best-in-class solutions, providing high productivity and low cost per dose
- Improve time-to-market through seamless scale-up support and best-in-class delivery and quality service levels
- Leveraging commercial, innovation and operations synergies

Critical raw materials

Up & downstream solutions

Cell line development, plasmid design & process development services

Thank you!

# Q&A

# Disclaimer

This presentation contains statements concerning the future performance of the Sartorius Group. These statements are based on assumptions and estimates. Although we are convinced that these forward-looking statements are realistic, we cannot guarantee that they will actually materialize.
This is because our assumptions harbor risks and uncertainties that could lead to actual results diverging substantially from the expected ones. It is not planned to update our forward-looking statements.

Throughout this presentation, differences may be apparent as a result of rounding during addition.