



# Viral Vector Manufacturing: Meeting Escalating GMP Demand for Gene Therapies

Ryan Scanlon, Global Head Lonza Viral Gene Therapy

# Lonza

# Additional Information and Disclaimer

Lonza Group Ltd has its headquarters in Basel, Switzerland, and is listed on the SIX Swiss Exchange. It has a secondary listing on the Singapore Exchange Securities Trading Limited (“SGX-ST”). Lonza Group Ltd is not subject to the SGX-ST’s continuing listing requirements but remains subject to Rules 217 and 751 of the SGX-ST Listing Manual.

Certain matters discussed in this presentation may constitute forward-looking statements. These statements are based on current expectations and estimates of Lonza Group Ltd, although Lonza Group Ltd can give no assurance that these expectations and estimates will be achieved. Investors are cautioned that all forward-looking statements involve risks and uncertainty and are qualified in their entirety.

The actual results may differ materially in the future from the forward-looking statements included in this presentation due to various factors. Furthermore, except as otherwise required by law, Lonza Group Ltd disclaims any intention or obligation to update the statements contained in this presentation.

# Agenda

---

---

**01** The Challenge – Fast Growth Across Different Vectors

---

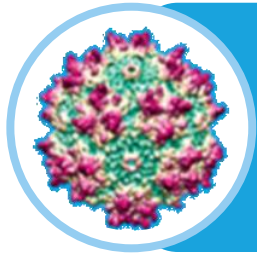
02 Flexible Facilities – Efficient Asset Utilization

---

03 Robust & Scalable Platforms – Speedy & Reliable Delivery

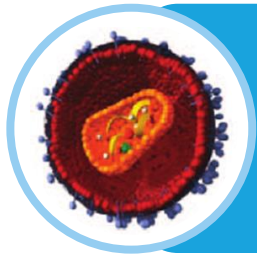
---

# Demand for Viral Gene Therapy Manufacturing Growing Across Multiple Types of Vectors



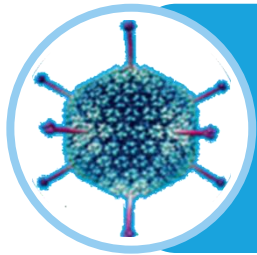
AAV

- Numerous promising and diverse clinical results from Hemophilia to genetic blindness to spinal muscular atrophy - driving increased demand
- Added demand from gene editing applications



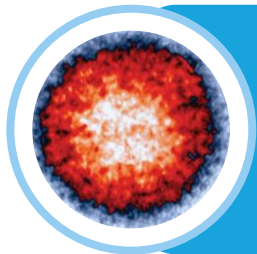
Lenti

- Large demand driven by CAR-T and other ex vivo HSC applications in rare diseases
- In vivo candidates hold additional promise



Adeno

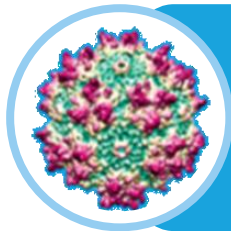
- Encouraging therapies advancing towards market (oncology, infectious disease)
- Several later phase candidates progressing in pivotal trials



Oncolytic

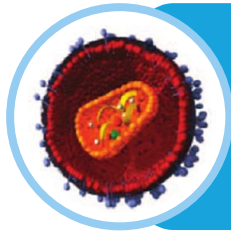
- Amgen's TVEC approval was a major catalyst. Several astonishing early clinical trials results
- Attracting substantial new investments (VC, BMS, Pfizer, Celgene, etc.)

# Manufacturing Process Diversity and Immaturity Makes the Challenge Complicated



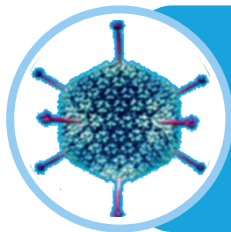
AAV

- Triple Transfection HEK293 (adherent or suspension)
- Baculovirus / sf9 (suspension)
- HeLa producer cell line (suspension)
- HSV (adherent or suspension)



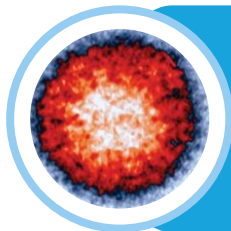
Lenti

- HEK293T (adherent or suspension)



Adeno

- HEK293 (suspension or adherent)
- PER.C6 (suspension)
- Others (generally adherent)



Oncolytic

- HEK293 (adherent or suspension)
- Vero (adherent)
- Others (adherent & suspension)

# Agenda

---

01

The Challenge – Fast Growth Across Different Vectors

---

02

**Flexible Facilities – Efficient Asset Utilization**

---

03

Robust & Scalable Platforms – Speedy & Reliable Delivery

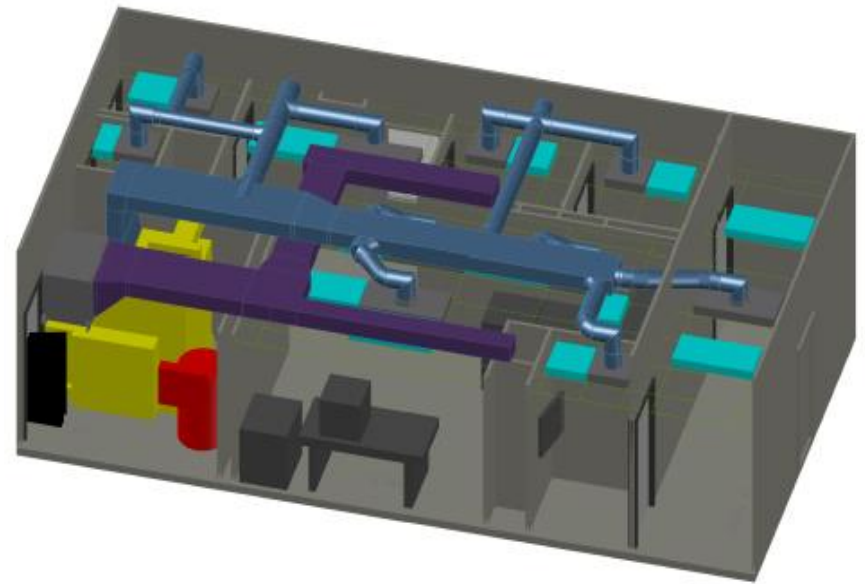
---

# Adoption of Modular Cleanroom Construction and Single-Use Technologies Are Critical

Capital efficient

Fast construction / installation

Flexible to accommodate variety of unit operations (cell factories, wave, roller bottle, SUBs, etc)



# Lonza Is Building World's Largest (Anticipated) Viral Vector Facility in a Suburb of Houston, TX\*

Significant up-front investment in infrastructure / support areas

Oversized “ballroom” manufacturing areas allow for quick modular clean room expansion

Initial rooms on track to be **GMP-ready late 2017**



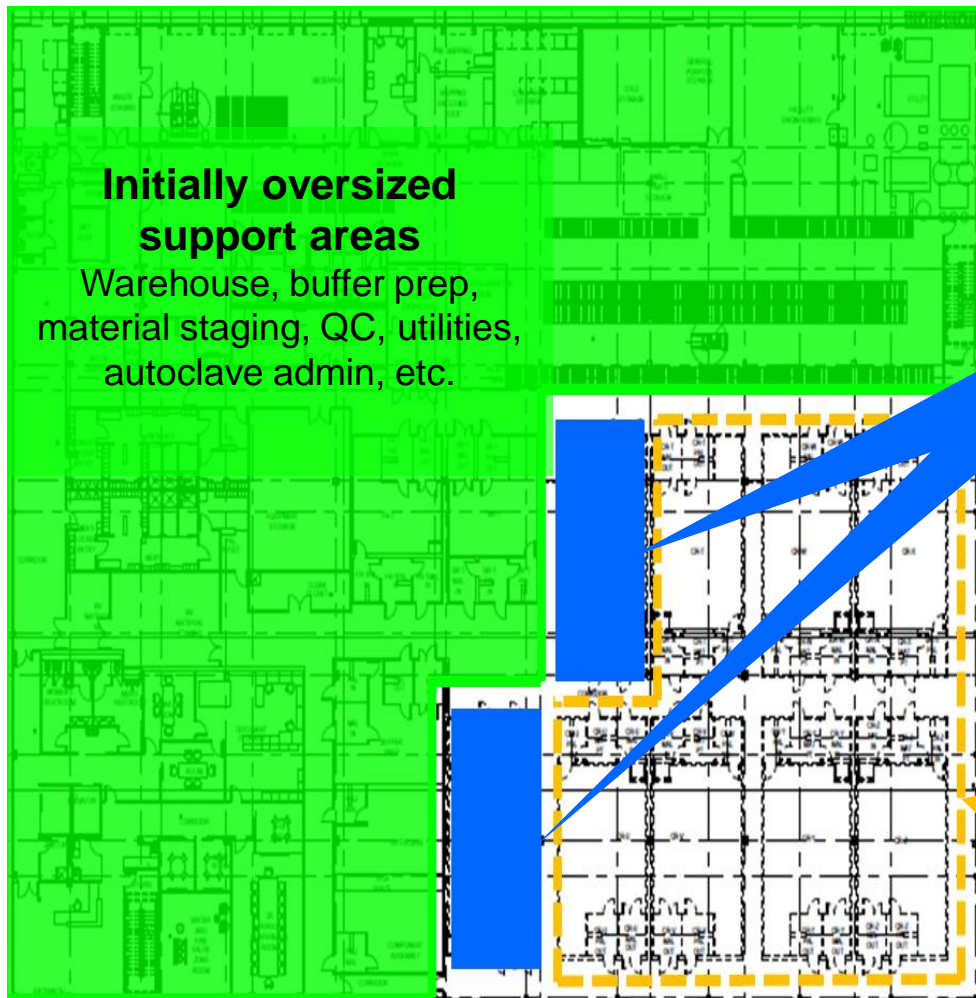
Clinical GMP

Commercial GMP

\*NOTE: Relocating from existing smaller Houston facility which will continue to operate as needed until new facility is operational



# Concept for Commercial Capacity Expansion



## Initially oversized support areas

Warehouse, buffer prep, material staging, QC, utilities, autoclave admin, etc.

Initial cleanrooms to supply initial demands

Expansion “ballroom” space  
Additional modules can be installed quickly with relatively little incremental CAPEX to meet growing demand

# On Track – Initial Late 2017 Capacity Already Sold; Taking Orders for H1 2018



# Agenda

---

01

The Challenge – Fast Growth Across Different Vectors

---

02

Flexible Facilities – Efficient Asset Utilization

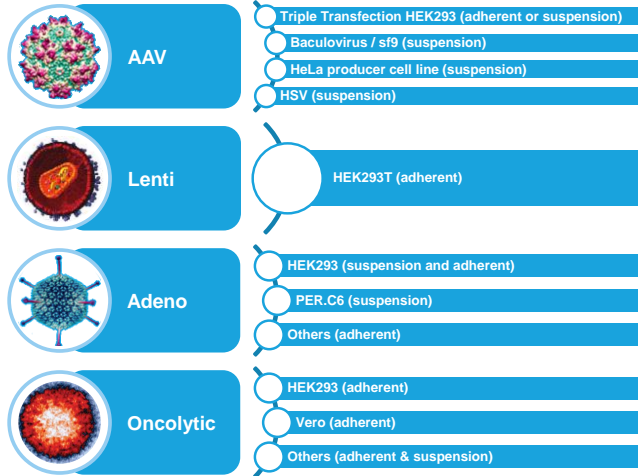
---

03

**Robust & Scalable Platforms – Speedy & Reliable Delivery**

---

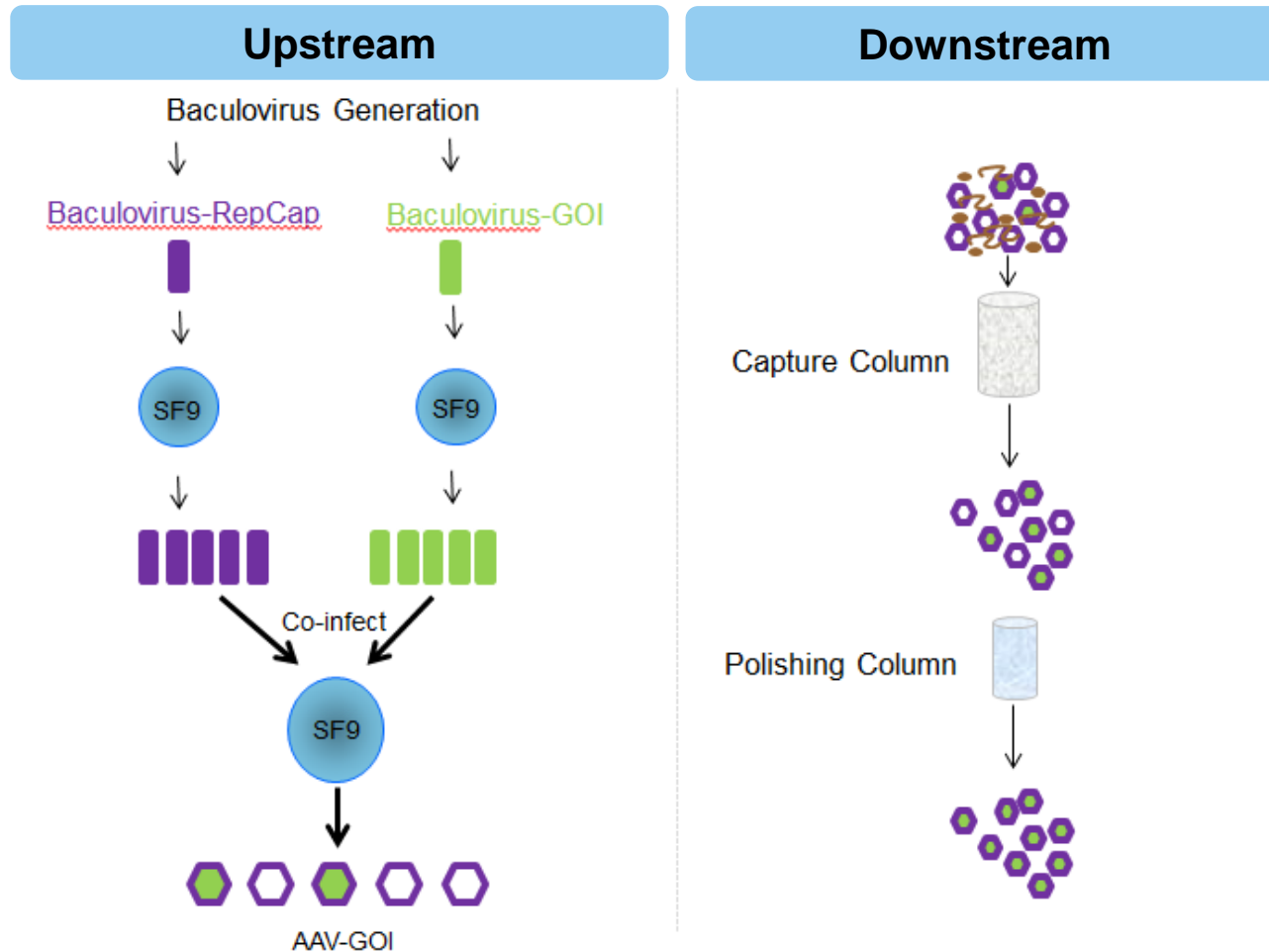
# Platforms Make Operations Simpler, More Reliable and Efficient



Investment in novel process technology & innovation



# Example: Baculovirus Platform Is a Commercially-Viable Platform for AAV Today



# Key Takeaways

---

**Demand for vector manufacturing** growing across several types

**Flexible facility designs** can quickly and efficiently add capacity in line with demand

**Manufacturing platforms** are key to operational excellence



# Q&A

---