Workshop 3: Opportunities and Challenges to Bring Clinical and Commercial Cell and Gene Therapies to More Patients

Chairs: Andy Snowden (Janssen), Mercedes Segura (ElevateBio), Sean Palecek (University of Wisconsin)
Student Assistants: XXX

Please select a table by discussion topic (no more than 10 participants per table)

1. Opportunities for enhancing upstream yields of viral vectors: Repurposing existing vs. new technologies
2. New technologies and knowledge to enable nonviral gene and cell therapy products
3. Bridging comparability upon process scale-up with ATMPs (early phase vs. late phase)
4. New technologies needed for enabling manufacturing of effective allogeneic cell therapies
5. Process analytical technologies : Repurposing existing vs. new technologies
6. What kinds of partnerships are needed to develop solutions for gene and cell therapies (e.g. cost, scale)?
7. Navigating gaps in critical quality attributes and mechanism of action of ATMPs
8. Building resilient supply chains
9. Enabling manufacturing of effective autologous cell therapies (cost and scale-out)
10. Regulatory considerations in developing and manufacturing ATMPs

The Process

- Brief introductions
- Select a scribe to take notes and organize discussion
- Identify challenges and opportunities related to the table topic
  - What are key challenges and opportunities we face?
  - Can these be delivered in short (0-3 years), medium (3-5 years), or long term (5+ years)?
  - What is needed? Classify by: New Technologies, Process Improvements, Fundamental Understanding of Process and Product
  - Who can do it? Industry, Academia, Government, Vendors, Consortia, Others?
  - Uncertainty is ok and expected – better to include speculative ideas than strive for complete consensus
- 30 minutes for discussion.
- A presenter from each group will have up to 4 min to report back
- A summary of discussions will be distributed to all workshop attendees after the conference

Suggested Report Template

- Table Number and Topic

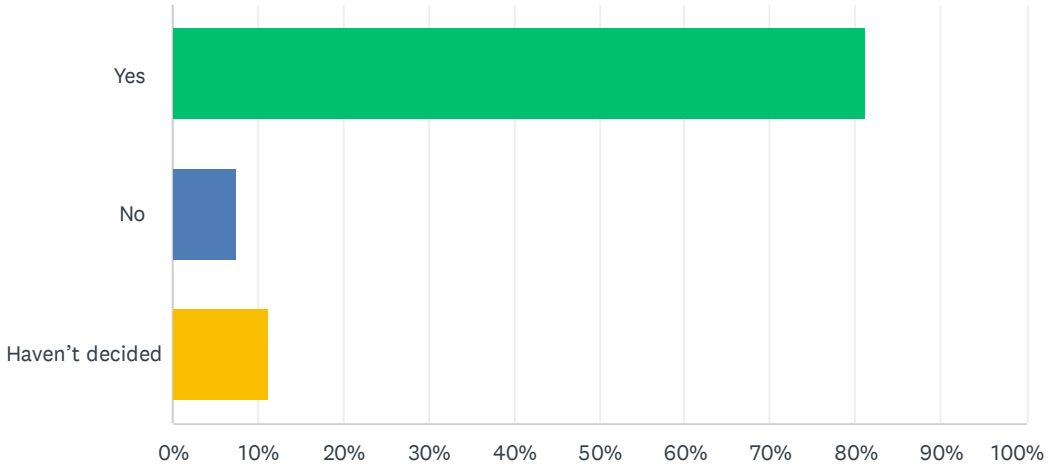
Challenge or Opportunity 1 (Short, Medium, or Long Term)
- Potential solution 1 (Technology, Process, Knowledge); who can solve
- Potential solution 2 (Technology, Process, Knowledge); who can solve
- Potential solution 3 (Technology, Process, Knowledge); who can solve
Challenge or Opportunity 2 (Short, Medium, or Long Term)
- Potential solution 1 (Technology, Process, Knowledge); who can solve
- Potential solution 2 (Technology, Process, Knowledge); who can solve

List as many challenges, opportunities, and solutions as your table feels appropriate

Please organize your ideas to assist in synthesizing discussion and conclusions

Q18 Are you planning on attending the Cell and Gene Therapy (current title) workshop at the conference this year?

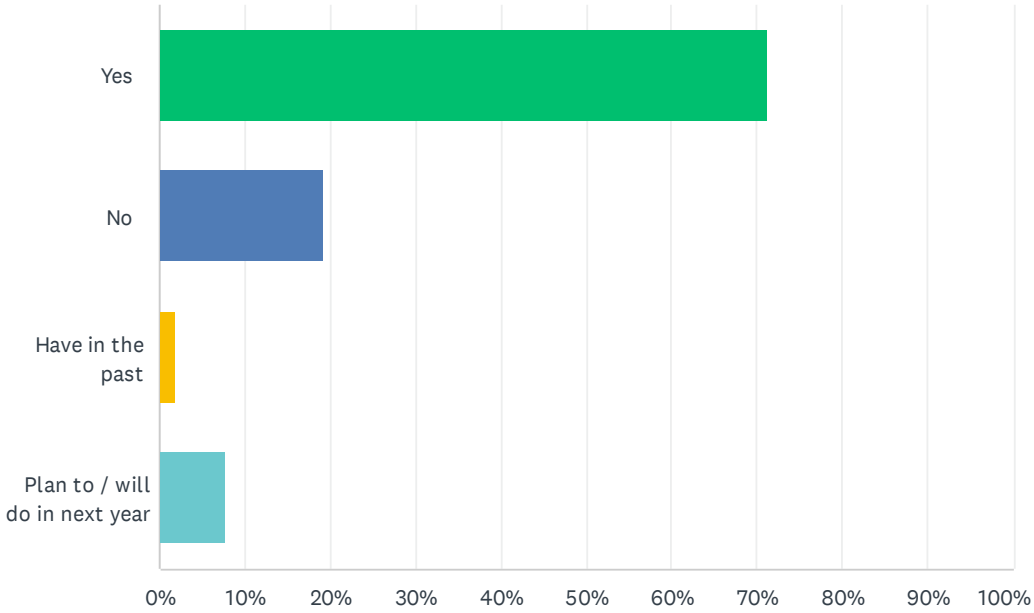
Answered: 53 Skipped: 276



ANSWER CHOICES	RESPONSES	
Yes	81.13%	43
No	7.55%	4
Haven't decided	11.32%	6
TOTAL		53

Q19 Are you currently working on cell or gene therapy related activities as part of your role?

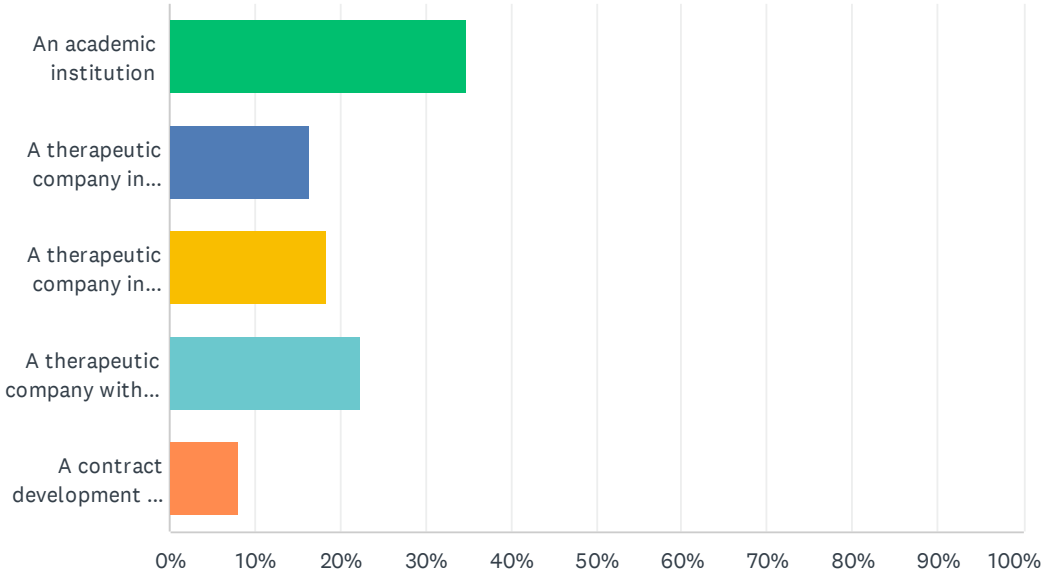
Answered: 52 Skipped: 277



ANSWER CHOICES	RESPONSES	
Yes	71.15%	37
No	19.23%	10
Have in the past	1.92%	1
Plan to / will do in next year	7.69%	4
TOTAL		52

Q20 Are you currently employed by (please specify)

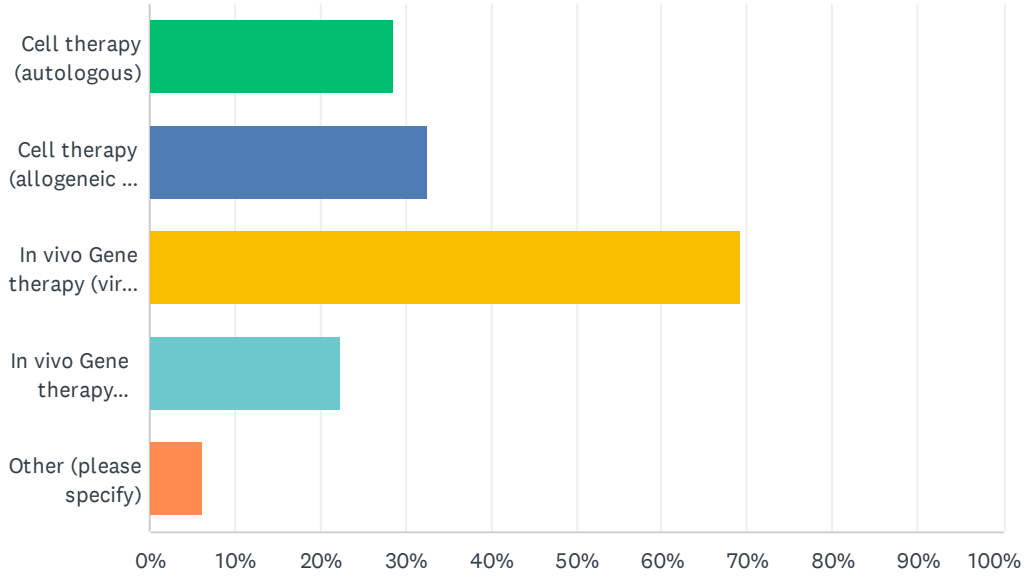
Answered: 49 Skipped: 280



ANSWER CHOICES	RESPONSES	
An academic institution	34.69%	17
A therapeutic company in preclinical phases of development	16.33%	8
A therapeutic company in clinical phases of development	18.37%	9
A therapeutic company with commercial therapies	22.45%	11
A contract development and manufacturing organization	8.16%	4
TOTAL		49

Q21 Are you primarily interested or engaged in (select all that apply)

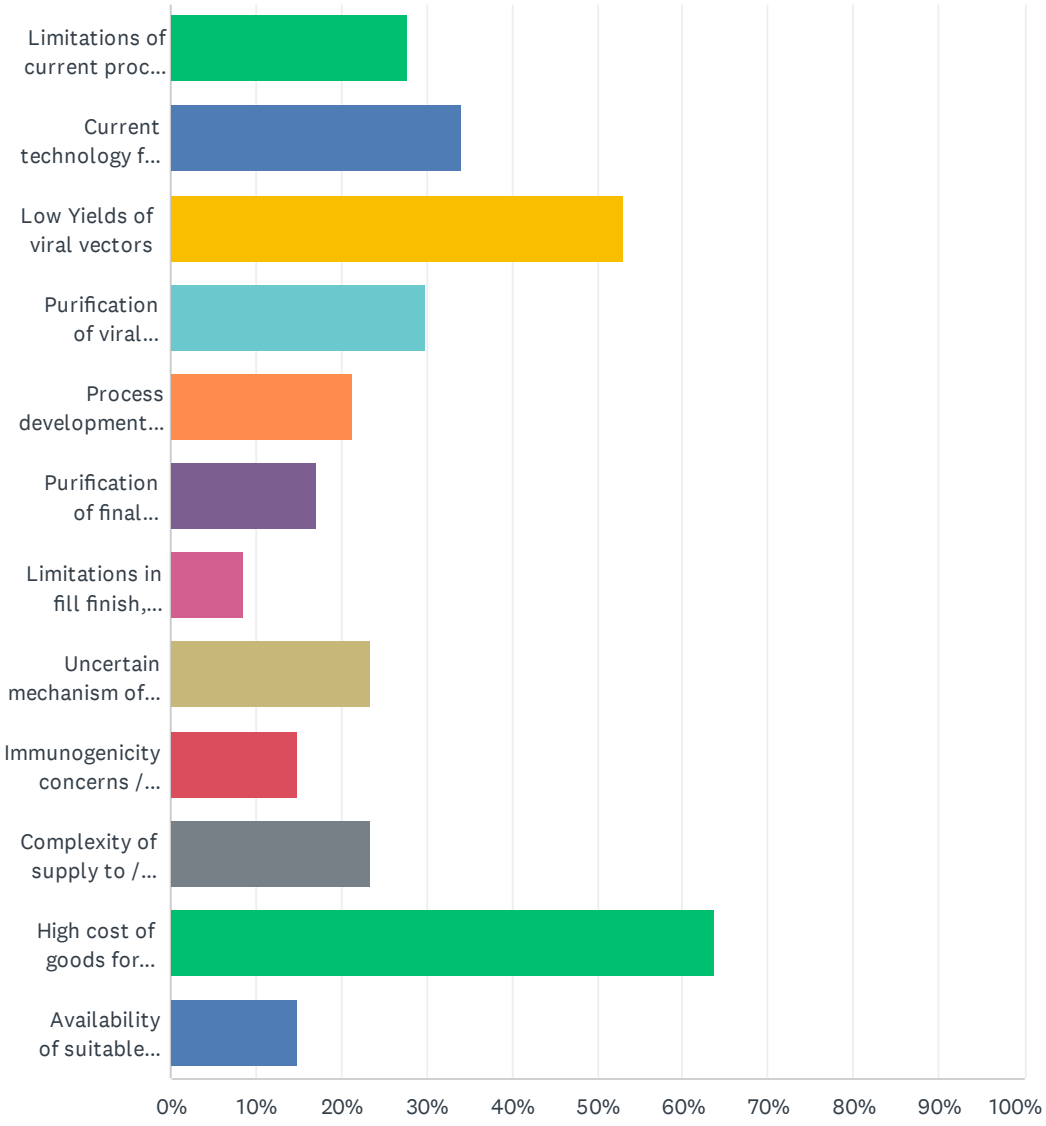
Answered: 49 Skipped: 280



ANSWER CHOICES	RESPONSES	
Cell therapy (autologous)	28.57%	14
Cell therapy (allogeneic – iPSC or HD derived)	32.65%	16
In vivo Gene therapy (viral vector)	69.39%	34
In vivo Gene therapy (non-viral)	22.45%	11
Other (please specify)	6.12%	3
Total Respondents: 49		

Q22 What do you see as the most critical current challenges you are experiencing in cell therapy or gene therapy processes ? (select all that apply)

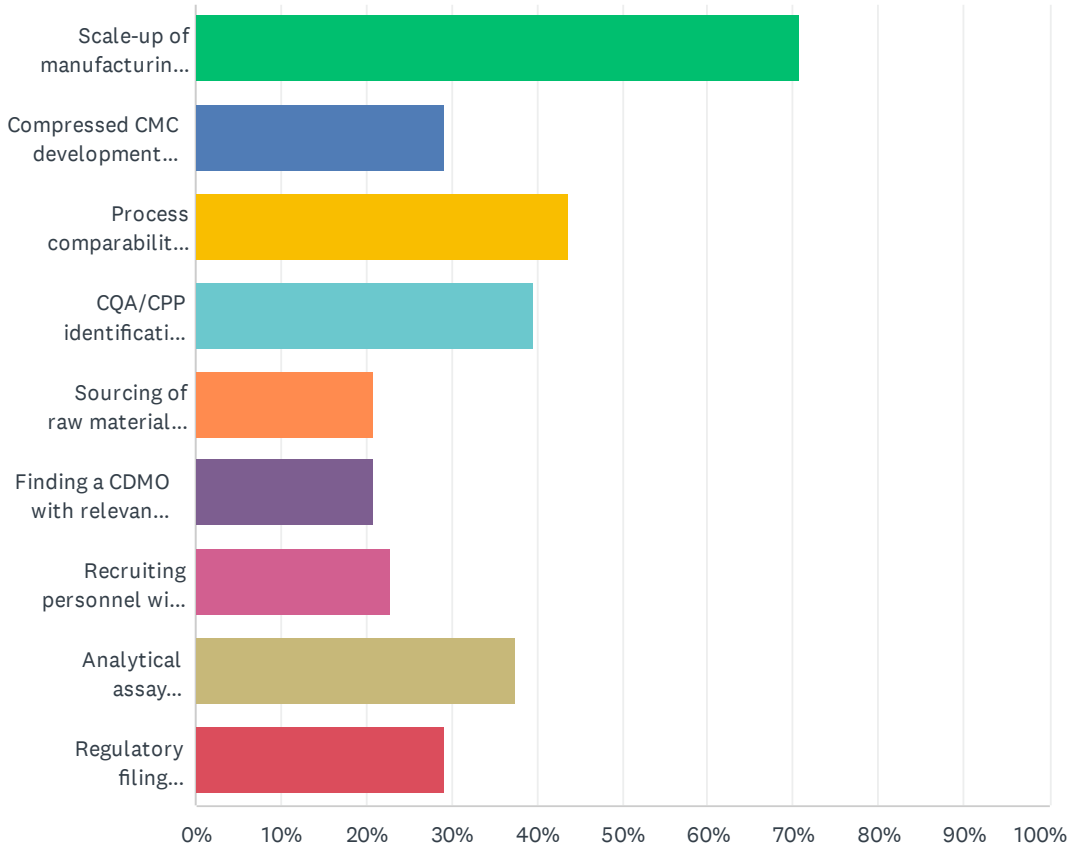
Answered: 47 Skipped: 282



ANSWER CHOICES	RESPONSES	
Limitations of current process equipment	27.66%	13
Current technology for gene delivery (e.g. AAV, LV, non-viral, other)	34.04%	16
Low Yields of viral vectors	53.19%	25
Purification of viral vectors / impurities	29.79%	14
Process development with healthy donors vs patient material	21.28%	10
Purification of final product	17.02%	8
Limitations in fill finish, formulation or final product stability	8.51%	4
Uncertain mechanism of action of gene or cell therapy products	23.40%	11
Immunogenicity concerns / patient delivery limitations	14.89%	7
Complexity of supply to / from patients	23.40%	11
High cost of goods for manufacturing	63.83%	30
Availability of suitable commercial reagents	14.89%	7
Total Respondents: 47		

Q23 What do you believe are the most critical limitations to cell or gene therapy manufacturing ? (select all that apply)

Answered: 48 Skipped: 281



ANSWER CHOICES	RESPONSES	
Scale-up of manufacturing process (facilities, equipment, personnel)	70.83%	34
Compressed CMC development timelines	29.17%	14
Process comparability vs. early phase process	43.75%	21
CQA/CPP identification and limited ability to perform in-process monitoring	39.58%	19
Sourcing of raw materials / components	20.83%	10
Finding a CDMO with relevant technical expertise, capabilities and available capacity	20.83%	10
Recruiting personnel with relevant expertise	22.92%	11
Analytical assay development / deficiencies limiting process understanding	37.50%	18
Regulatory filing challenges / lack of clear guidance	29.17%	14
Total Respondents: 48		