Cell & Gene Therapy

MARKET SURVEY 2019

Insights from industry experts on:

- **Clinical Development**
- **Manufacturing**
- **Commercialisation**

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www.oxfordglobal.co.uk/cell-series

The cell and gene therapy industry has seen substantial growth in the past few years and shows little sign of slowing down. There is a staggering amount of activity in this space, with 800+ active companies worldwide and 1,028 clinical trials conducted by the end of 2018. While there remain few approved products, the sheer scale of innovation and investment in this area suggests there will be 20-25 cell based or gene therapy products approved per year between now and 2025.

Following another successful year for our Cell & Gene Therapy Congress, we decided to ask our attendees for their current and future perspectives on the regenerative medicine marketplace. The data presented in this survey includes insights from our pharmaceutical, biotech and academic institutions in order to identify critical challenges and priorities for advanced therapies.

Survey Contents

Our questions centre on the areas of strategic focus within the field, including the clinical developments necessary for therapeutic success, approaches to secure favourable access and reimbursement for products, and plans to reduce the technical and regulatory barriers to scale-up, industrialisation and manufacturing.

Opening Pandora's Box

Following the first-in-class market approval of Novartis' Kymriah and Gilead/Kite's Yescarta, autologous CAR T products have dominated the cell therapy landscape. It is unsurprising therefore that a large portion of our respondents signified they are currently working in CAR T products for oncology. However, given the expensive nature of autologous therapies, there are questions about the longevity of this approach. Accordingly, our contributors are excited by allogeneic approaches, as they have the potential to be produced at a lower cost. Beyond oncology, the focus is turning more towards previously untreatable and hard-to-reach therapeutic areas, with investment and interest in cell and gene therapies for rare and genetically inherited diseases growing rapidly.

Manufacturing Medical Miracles

Manufacturing is one of the key bottlenecks for Cell & Gene Therapy professionals, and analytical tools are required to develop and improve processes for this industry to succeed. The developers of complex advanced therapies

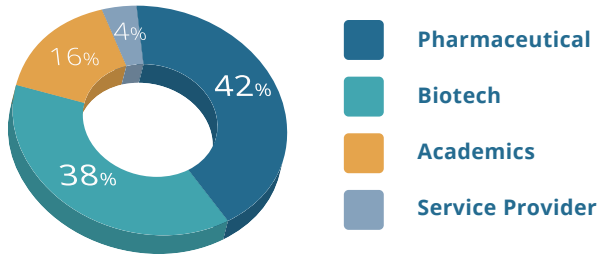
manufacture in house – but this is likely down to a need for the field to develop in experience and expertise. Perhaps the brand-new facilities that are appearing across the UK & Ireland (such as CGT Catapult's brand-new large-scale manufacturing centres) will meet the requirements for capacity, technical specificity and industry expertise necessary to properly produce new regenerative medicines. As indicated by our survey, cost of goods, trust and expertise are crucial requirements expected of CMOs working in this newly blossoming area.

Commercialising Cures

Commercial success for cell and gene therapy products is still an area fraught with the anxieties of new, complex products that treat difficult therapeutic areas. These medicines require better market and eventual patient access, and more efforts to collaborate and partner with other organisations in this space. As shown by this survey, the industry is making great strides towards designing and producing commercially viable products for patients with the most difficult to treat diseases.

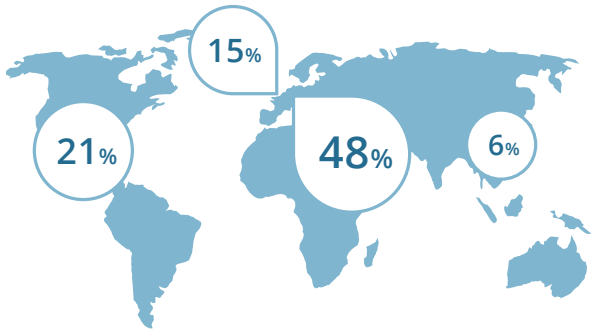
CONTRIBUTOR DEMOGRAPHIC

Organisation Category



Key Operating Area

Europe: 48%
North America: 21%
UK: 15%
Asia: 6%
Rest of World: 10%



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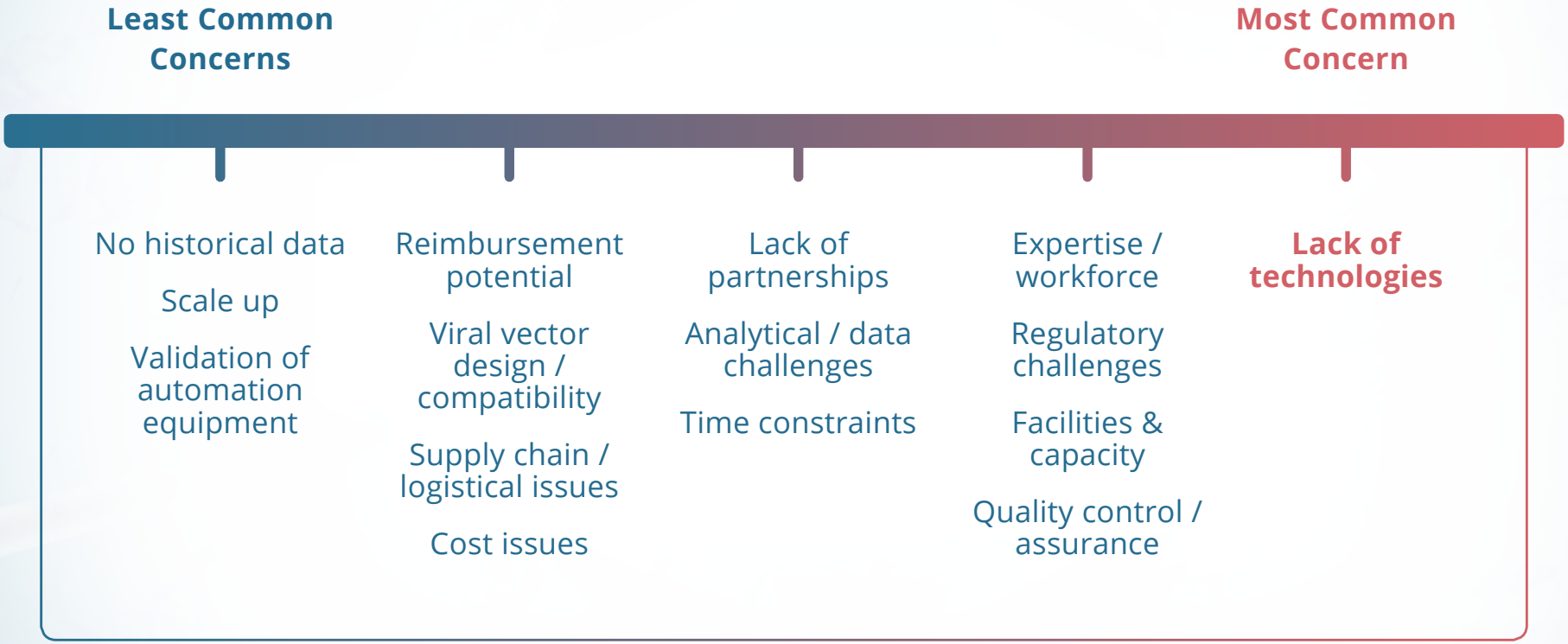
1 What were your key focus areas for 2018?

1. Process Development
2. Vector Design
3. Stem Cell Therapies
4. Enabling Technologies
5. Gene Therapy
6. Manufacturing
7. Cell Culture

2 What is your key priority for 2019?

1. Process Development
2. Clinical Development
3. Sample Management
4. Manufacturing
5. Automation
6. Cell Line Development
7. Gene Therapy

3 In order to succeed in this priority, what key challenges do you anticipate needing to overcome?



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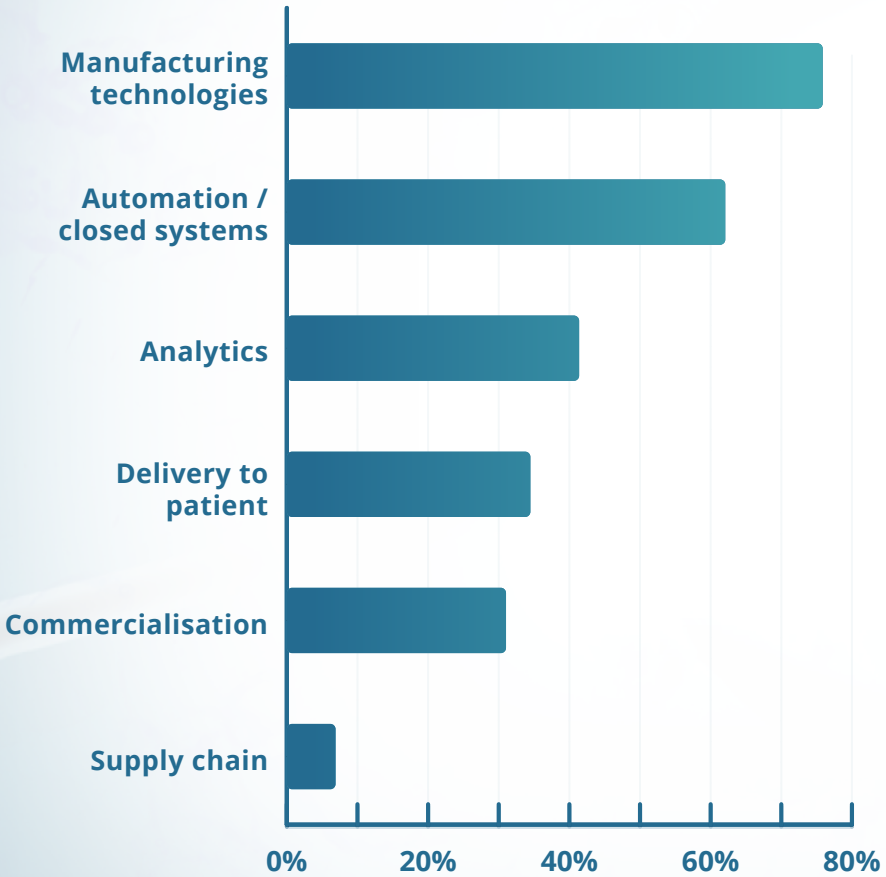
4 In which areas do you anticipate major advances impacting cell and gene therapy development?



5 What are the five most pressing issues in cell & gene therapy development?

1. Safety
2. Analytics
3. Cost of Goods
4. Pricing
5. Automation

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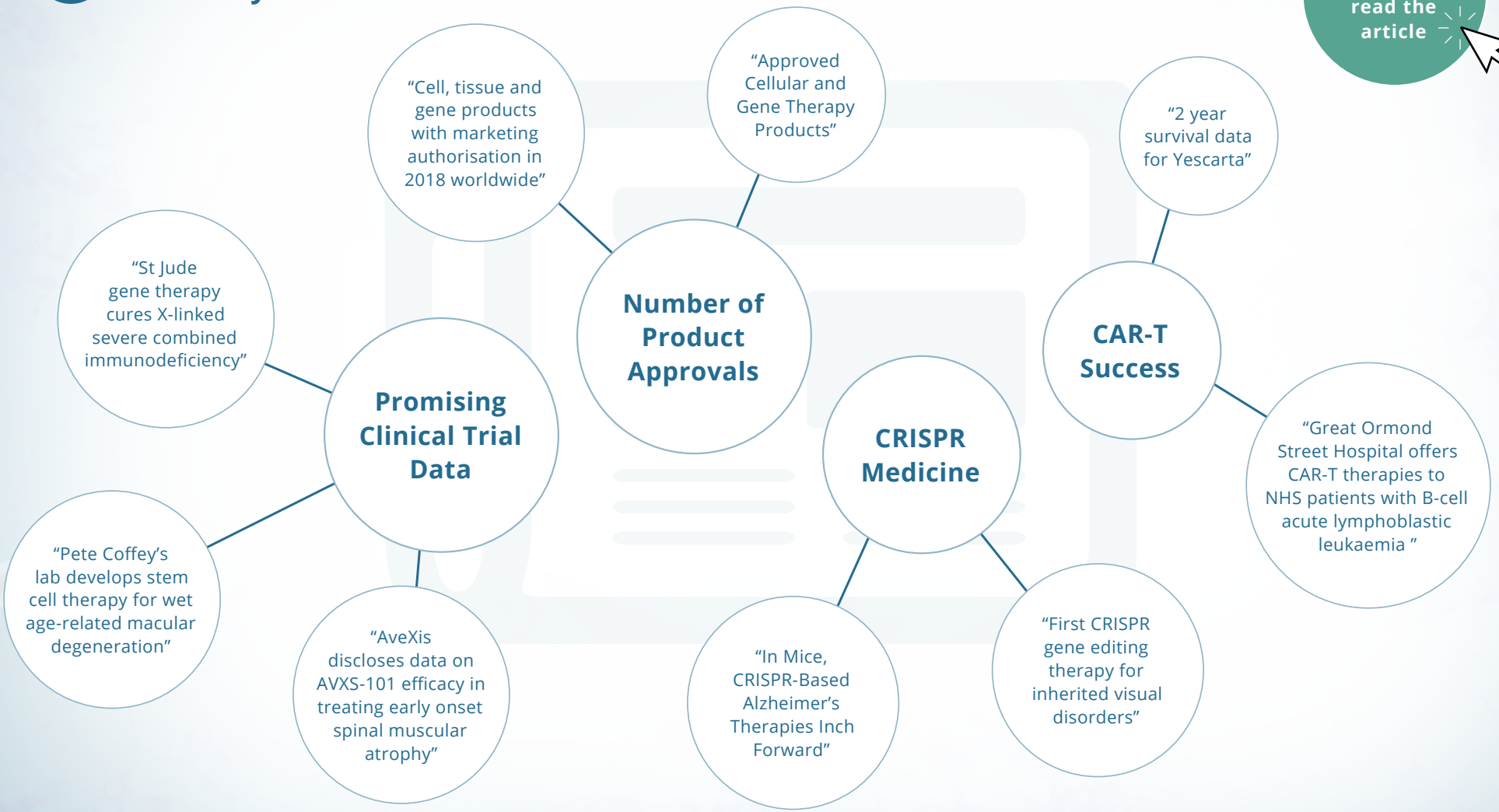
6 What is the most exciting single piece of news you have seen in this area recently?

Click on a headline to read the article



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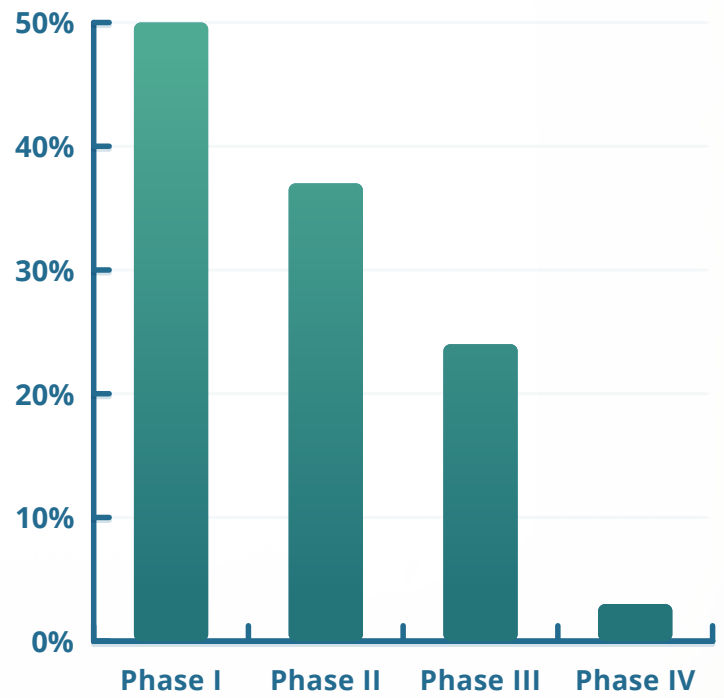
Clinical development of cell and gene therapies has seen significant growth in recent years, with successes in CAR T products and pivotal trials being undertaken. Companies active in this area have a variety of products in a range of different phases of clinical development, with the majority in the early stages and most of these products being in the oncology therapeutic area.

30% of our contributors had products in the rare disease space, and indicated that for rare disease therapeutics to succeed, safety, cost and patient access are critical aspects of future development. A variety of delivery methods are used for advanced therapeutics, with vector quality and patient safety being critical concerns going into clinical trials.

Looking to the future, cell & gene therapy developers will be looking to achieve more robust safety and efficacy for their drug, while reducing the cost of goods and technologies. Cell and gene therapy as a field will see incremental clinical trials and product approvals in the next few years, as it targets increasingly difficult therapeutic areas.

7 What phases are your organisation's cell & gene therapy clinical trials currently in (if applicable)?



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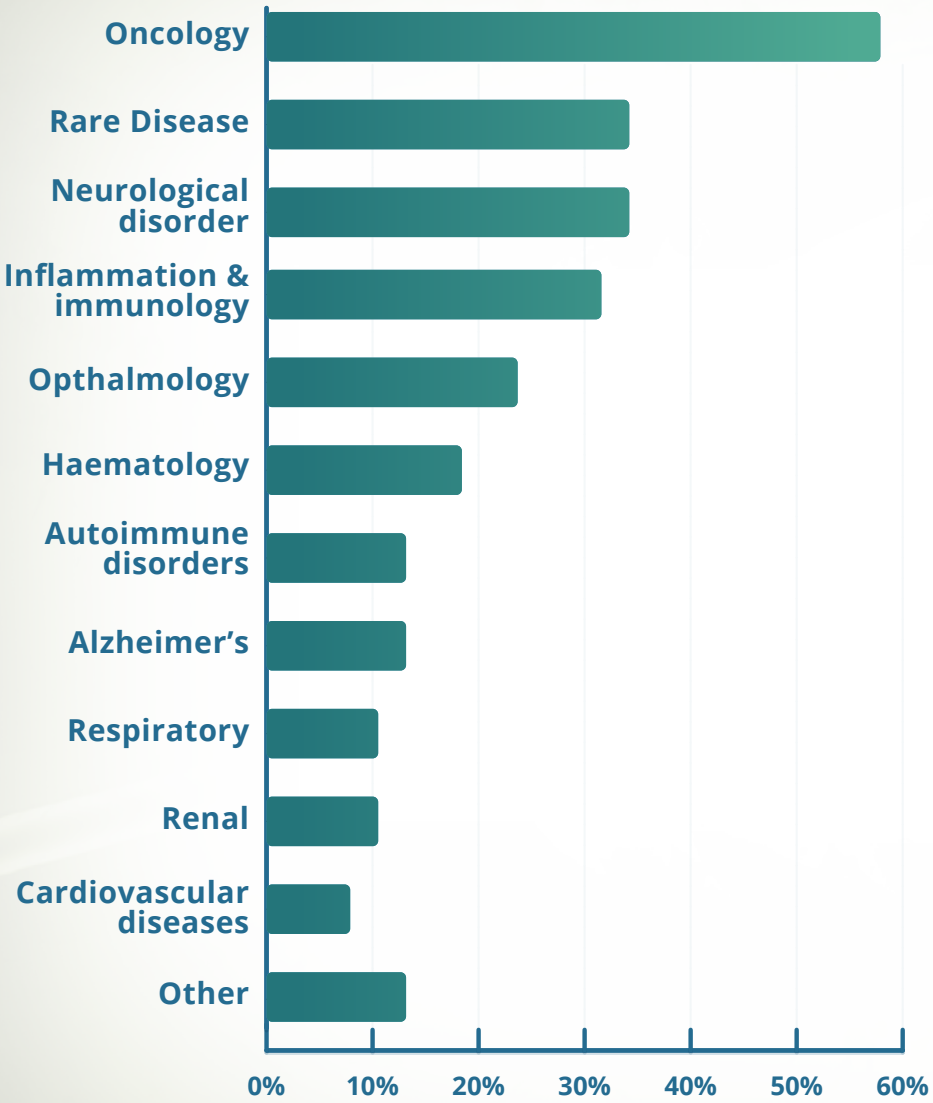

8 What therapeutic areas do you currently target?

9 What therapeutic areas are you hoping to move into in the future?

1.	Oncology
2.	Neurological disorder Inflammation and immunology
3.	Autoimmune disorders
4.	Opthalmology Rare disease CVD
5.	Haematology
6.	Renal Diabetes

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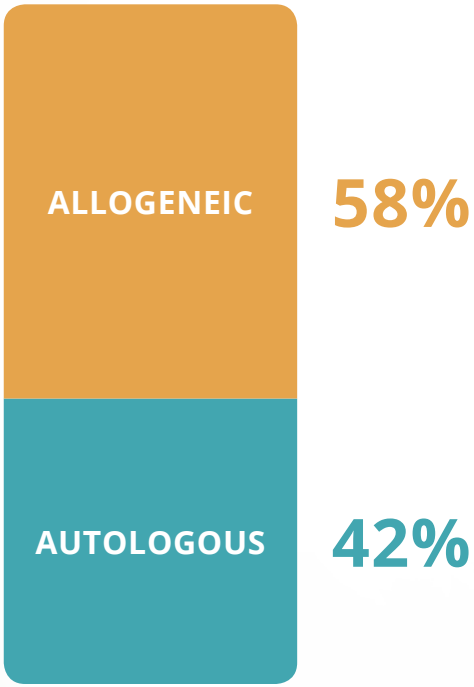
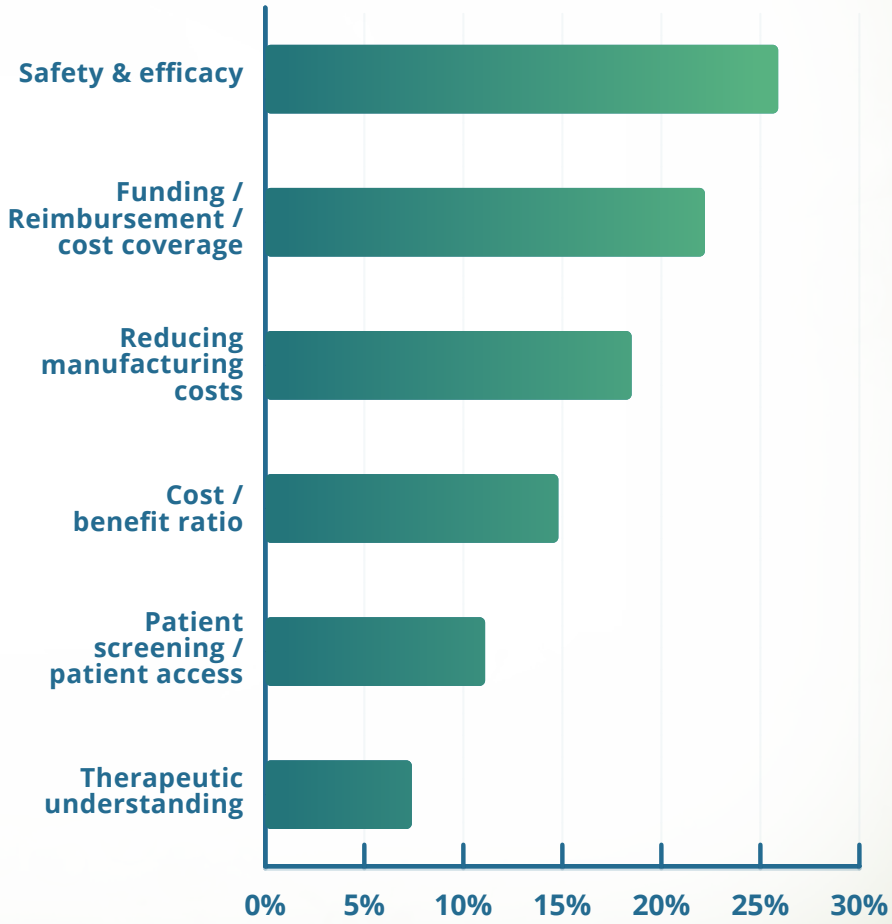
10 Which therapies have the biggest potential for success in the future?



11 What are the major requirements for the successful development of cell & gene therapies for rare disease?



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12 What delivery methods do you currently or intend to utilise?

1. IV administration
2. Viral vector
3. Lentivirus
   AAV
   Electroporation
   Local targeted administration
4. Ex-vivo
   Autoinjector
5. IM administration
   Parenteral
   Oral

13 What are the biggest challenges facing cell & gene therapy therapeutic development?

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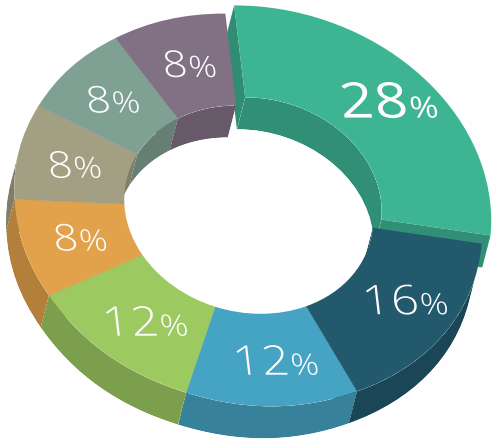
14 What are the biggest barriers to preclinical and clinical funding in cell & gene therapy?

15 What critical strategies will reduce risk and increase benefits to patients?



- Understanding safety and efficacy profile
- Process development and validation
- Raw Material Variabilities
- Personalised Medicines
- Quality Control
- Viral Safety
- Control of Immune Response
- Assay Profile

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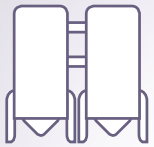
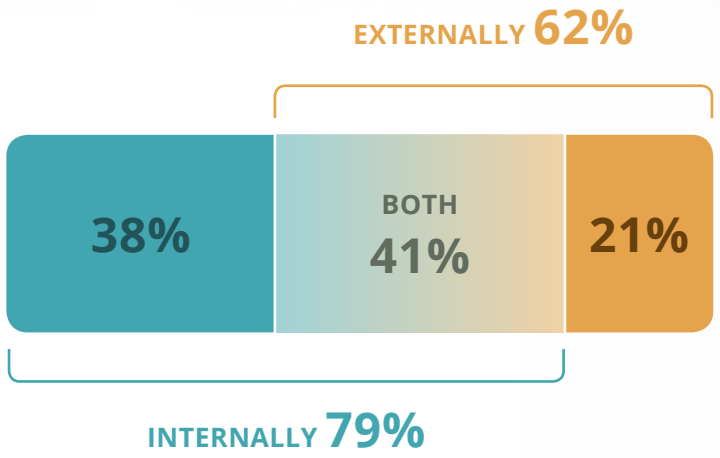


Manufacturing has been highlighted as one of the biggest focus areas and bottlenecks by our expert contributors. This section explores the challenges and priorities for cell & gene therapy Manufacturing, Science & Technology professionals.

The biggest challenges facing the manufacturing of complex regenerative medicines include scaling up of processes and achieving this without sacrificing quality or product safety. Scaling up products has various repercussions for technology and facility selection. There is an extremely high cost and long turnaround time for CGT manufacturing, with a defined lack of enclosed automation options, and little in the way of analytical technologies for processes. Experts would like to see a reduction in the cost of goods, more closed-system technologies and facilities, and improvements in high-yield production.

Selecting manufacturing partners is a key issue for cell and gene therapy products, with our contributors looking for companies with tried and tested experience in the regenerative medicine space. Having a qualified and experienced workforce of experts is a necessity, and cost, timing and logistics management is critical to the decision of which manufacturing partners to choose.

16 Does your institution manufacture internally, externally or both?



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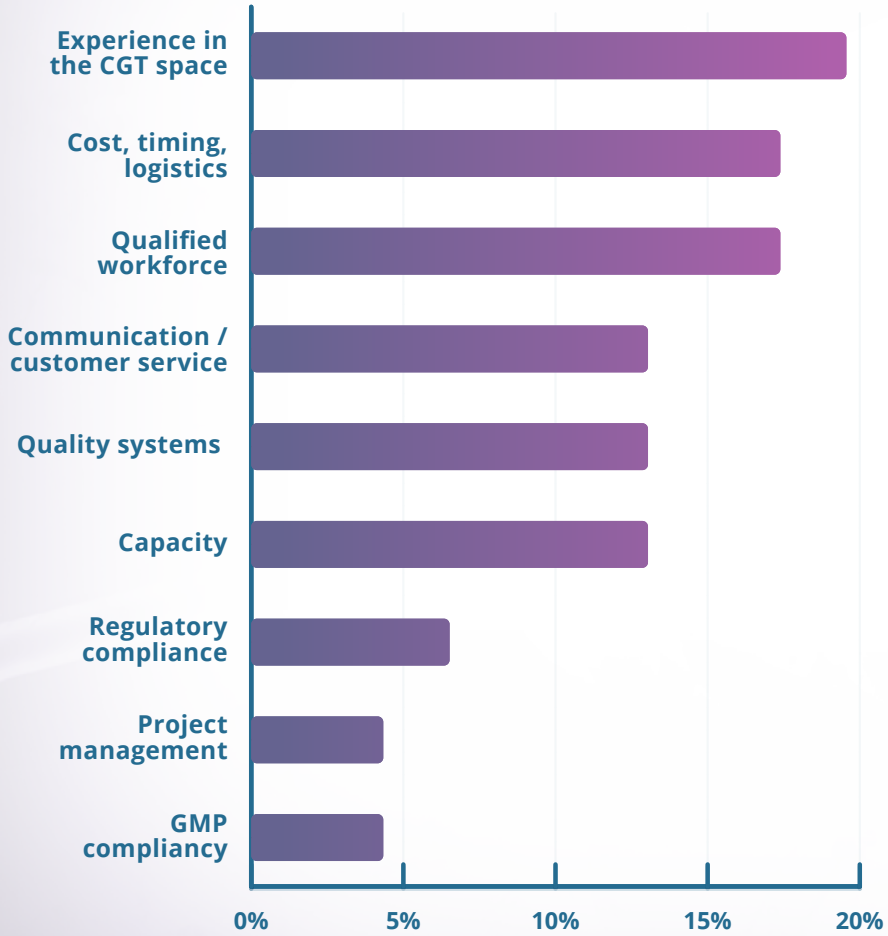
17 What are the three most important considerations when selecting manufacturing partners?



18 How would you describe your ideal manufacturing partner in one word?

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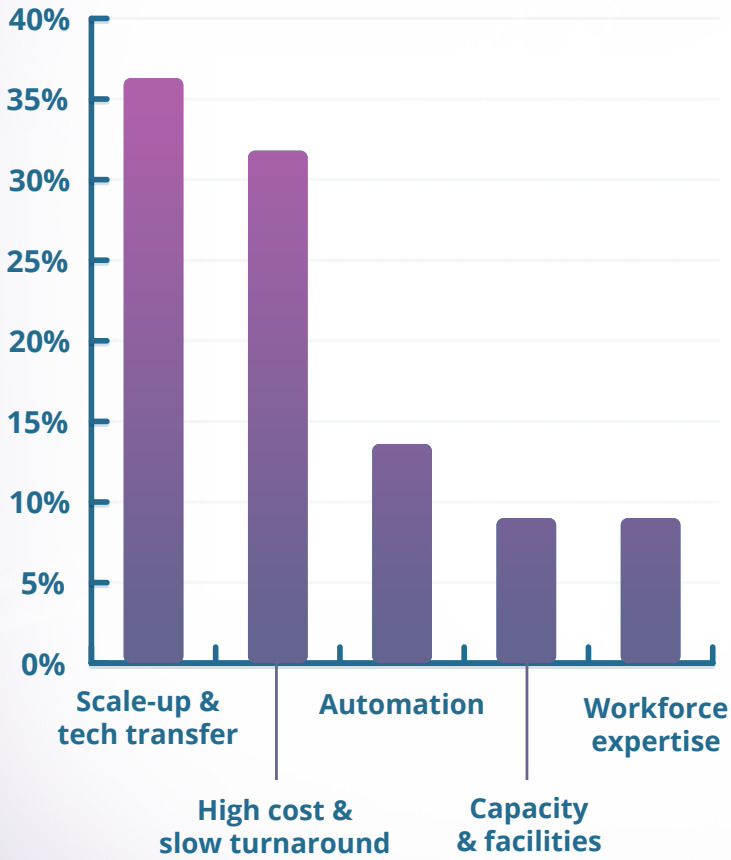
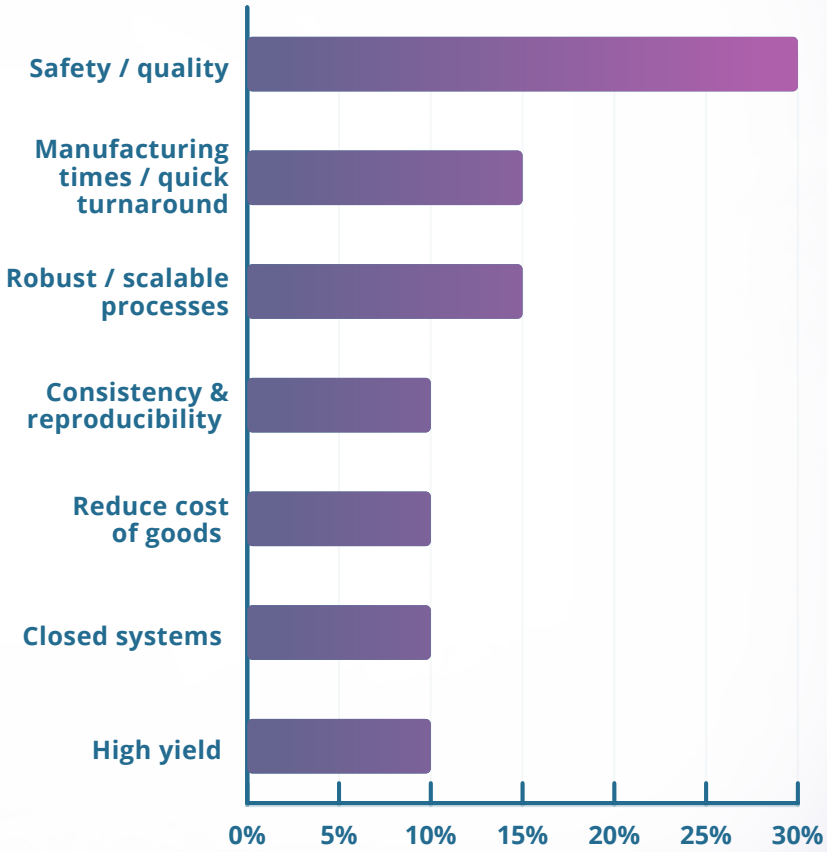
19 What are the biggest challenges facing cell & gene therapy manufacturing?



20 What is your key consideration when manufacturing cell & gene therapies?



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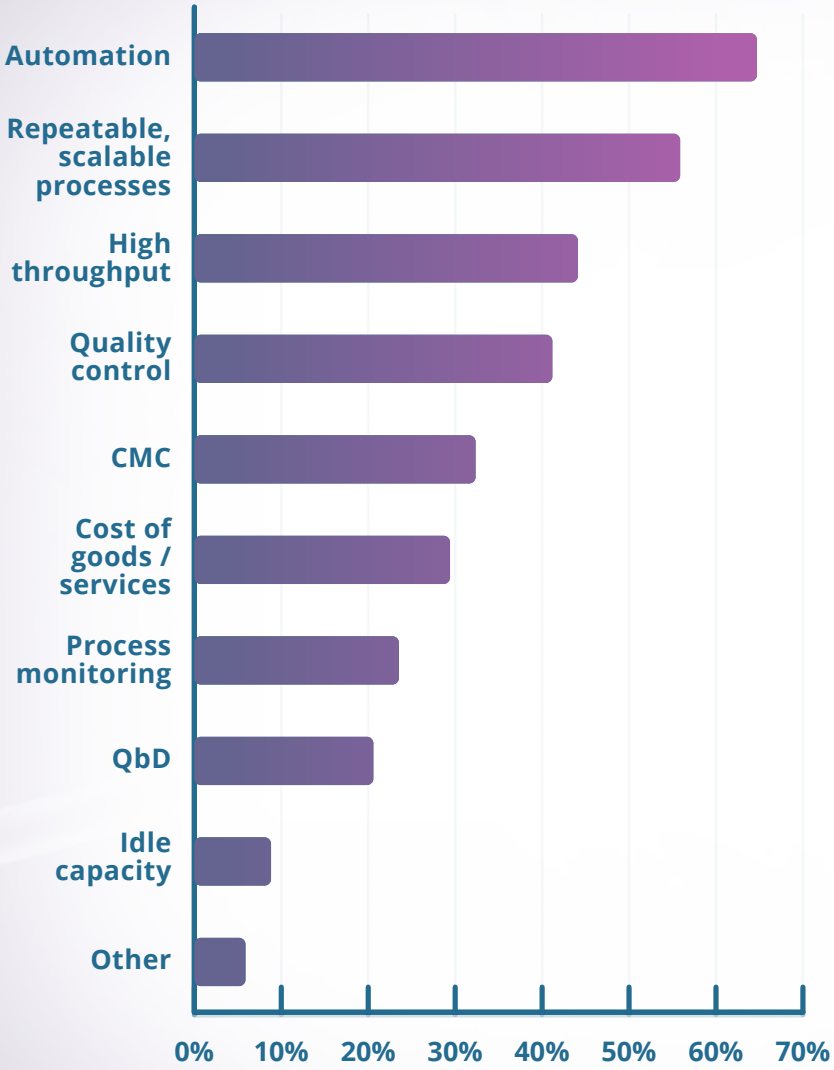
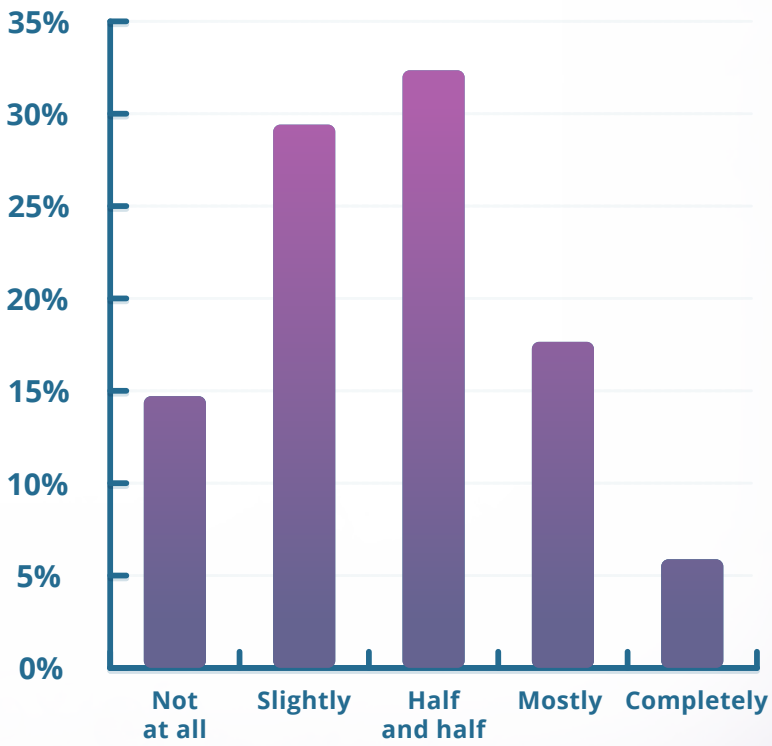


21 What are the most important areas for cost efficiency?

22 To what extent do you automate processes?



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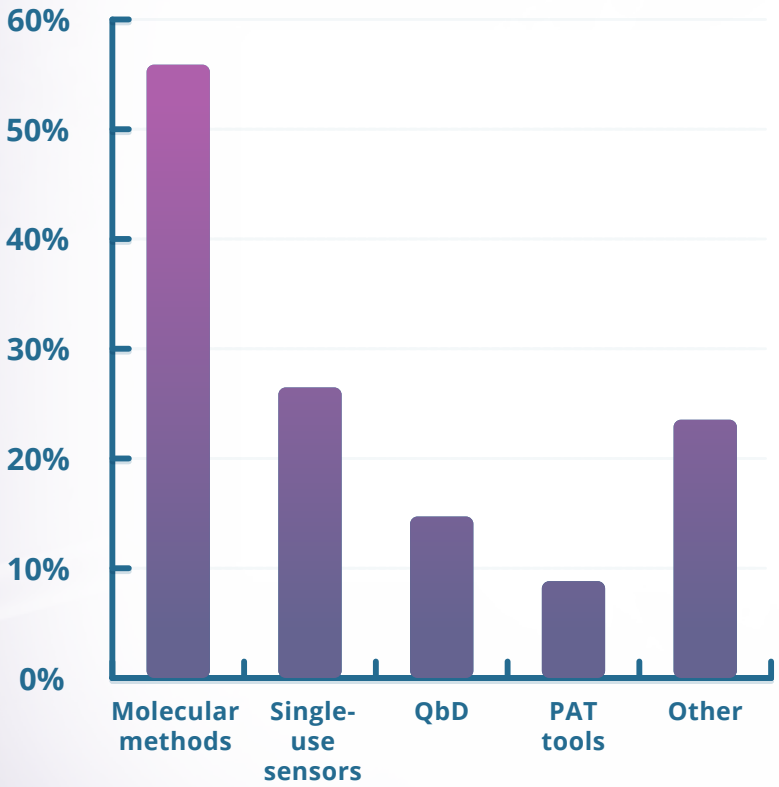
23 How are you currently monitoring processes?



24 How would you rate the importance of analytics to the manufacture of therapeutic products?

8.5 / 10 VERY IMPORTANT

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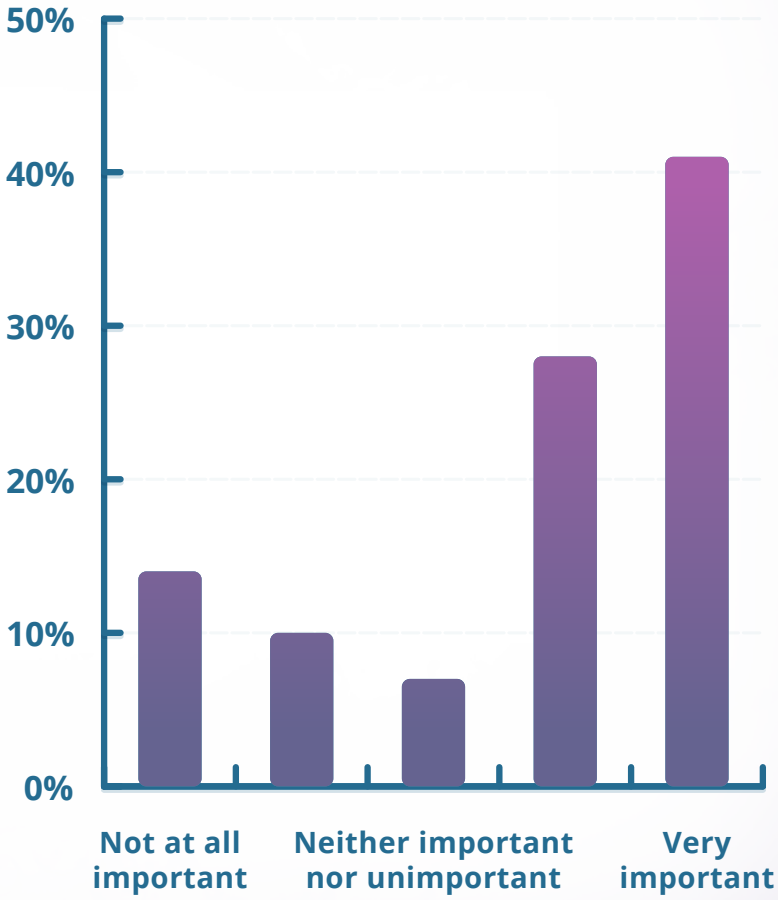
25 What are the biggest factors for success in viral vector production?

26 How important of a role do cell banks play in your work?



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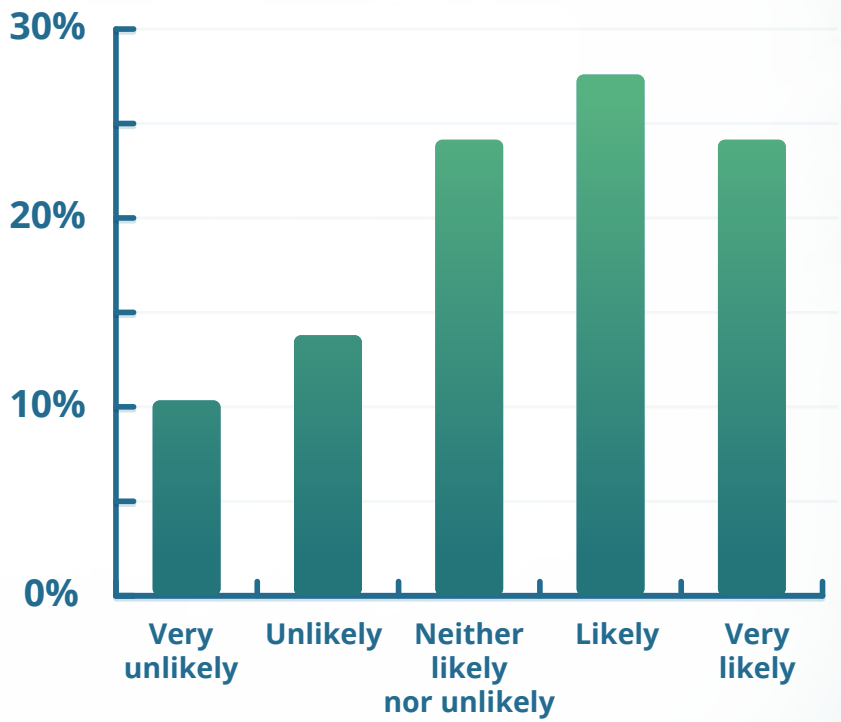
Commercialising cell and gene therapy products is particularly challenging: with little market history for advanced therapeutics, launch products are market pioneers, often defining the price of medicines - with substantial repercussions on patient access, partnering and market access.

Our experts find that the current costs of cell and gene therapies need to reduce, payment structures need to be defined, and barriers to patient access need to be removed. The expensive nature of processes and high costs of goods are impacting the commercial potential of cell and gene therapies. The immature market is also a large factor, with a lack of vector suppliers and cell CMOs and expertise and technologies tailored specifically for the regenerative medicine area. Our experts anticipate better-defined processes and analytical technologies for CGTs will improve the cost discrepancy in this area and reduce the cost of production and translated cost to patients and healthcare providers.

27

In the next two years, how likely is your organisation to submit a marketing application for a current pipeline product?



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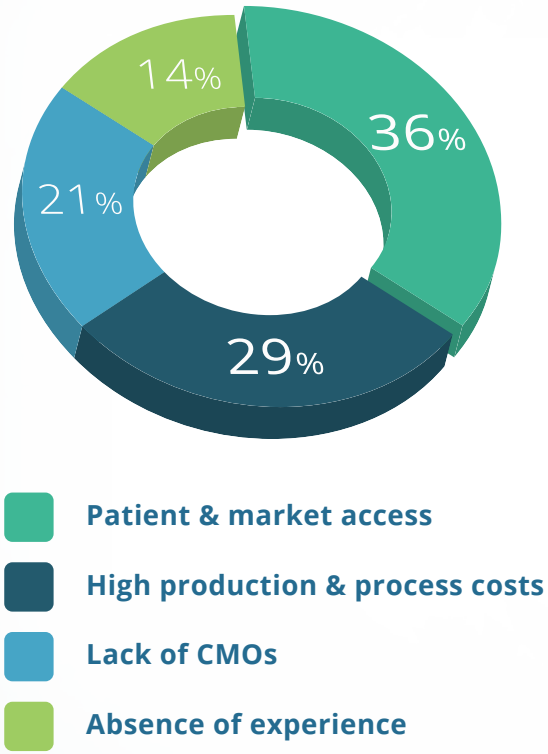
28 What are the major factors holding up commercialisation of cell & gene therapies?



29 What are the key logistical challenges that need to be overcome to enable commercialisation?

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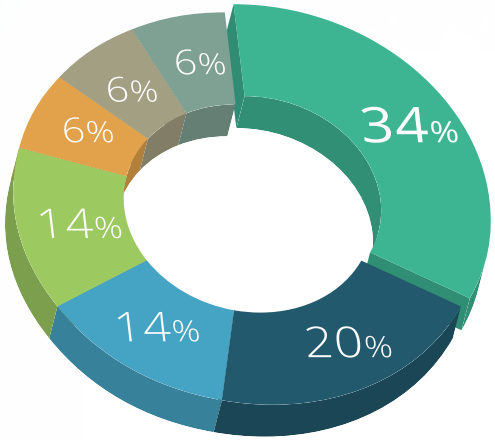
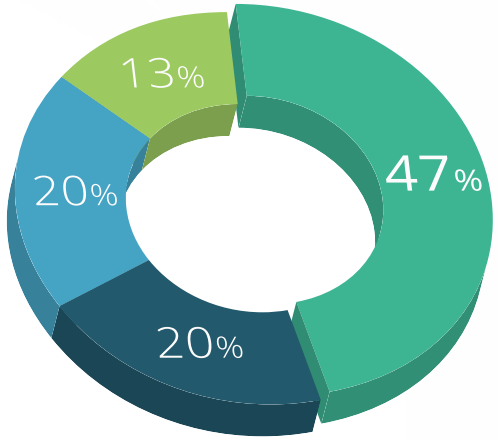
30 What are the major regulatory bottlenecks for cell & gene therapy?



- Knowledge base in agencies
- Regulatory compliance
- Data protection
- Regulations for Manufacturing
- Therapy Safety Requirements
- Clinical Trial Design
- Batch Release and Testing

31 What regulatory changes need to take place for widespread commercial success?



- Clear, standardised guidelines
- Increased funding
- International collaboration
- Faster approval

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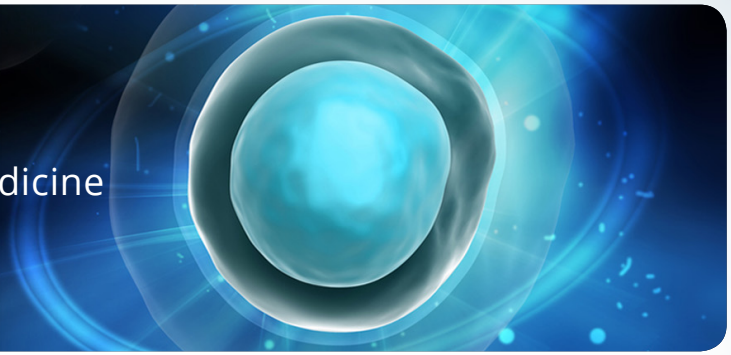
Cell Series UK 2019

Cell Culture & Bioprocessing | Stem Cell & Regenerative Medicine
Cell & Gene Therapy



29 - 30 October 2019, Novotel London West, London, UK

The Cell Series UK unites over 450 attendees from global pharmaceutical organisations, top biotech companies and internationally renowned academic institutions, alongside 40+ leading solution providers including MaxCyte, SCIEX, Horizon Discovery, Berkeley Lights, Sartorius Stedim and Eppendorf.

Join us in London this October to gain valuable insights into the novel technologies and therapeutic strategies propelling this marketplace forward: from tools to improve effectiveness in cell line development and bioprocessing to the latest clinical case studies in cell & gene therapies and regenerative medicine, as well as commercial considerations for cell banking and CAR-T & stem cell therapies.

> Download the Agenda

Cell & Gene Therapy Presentations Include

CAR-T Cells To Treat Solid Tumors – Challenges And Progress
Lothar Germeroth, Senior Vice President & Managing Director

GMP Quality For The Production Of Next-Generation Cell Therapies
Bethany Dudek, Executive Director, Quality Head Europe

Organizing For Commercial Success: Launching A Personalized Gene Therapy
Nicole Rickard, Senior Director, Gene Therapy Services