CELL & GENE THERAPY GUIDE

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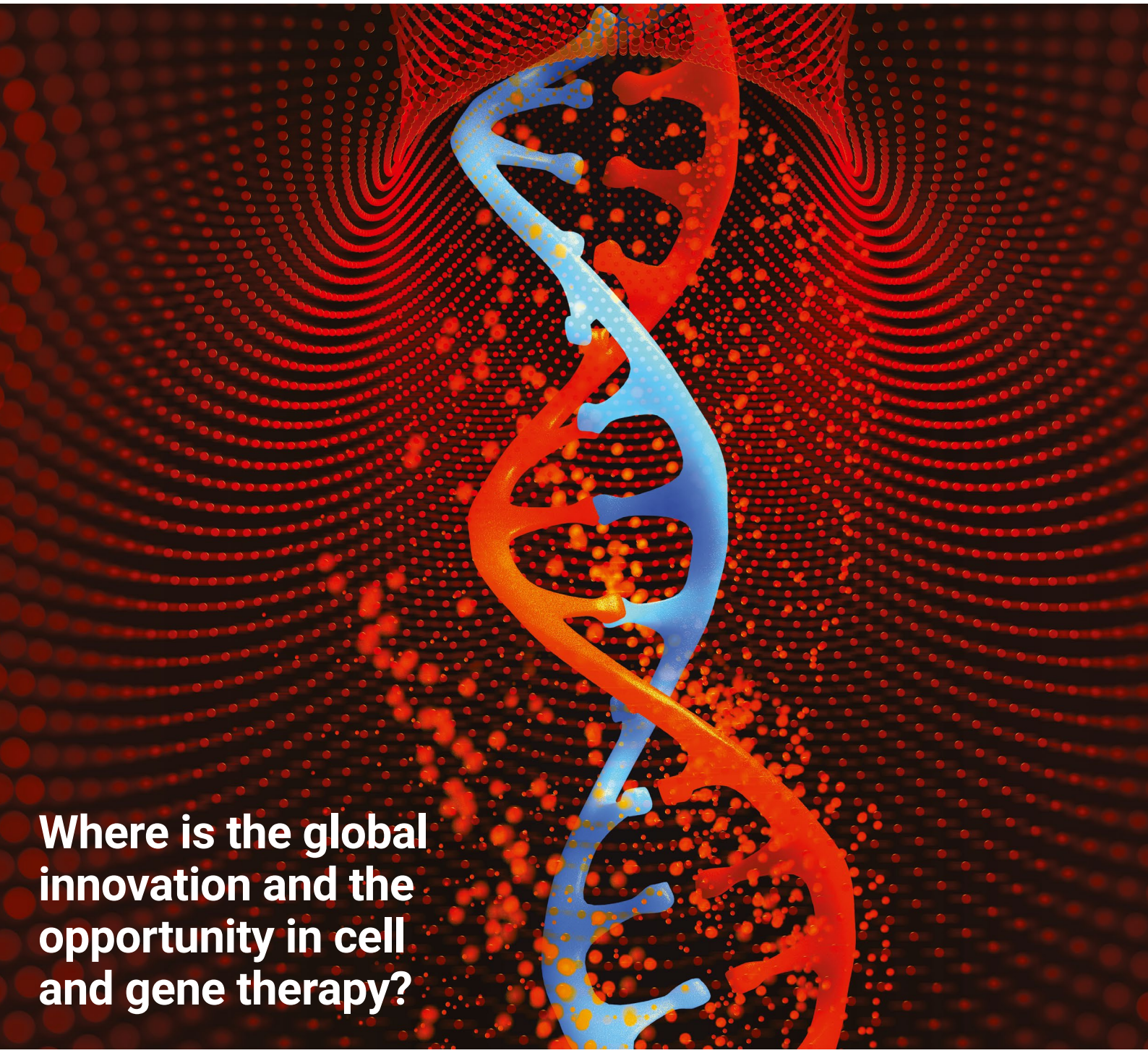

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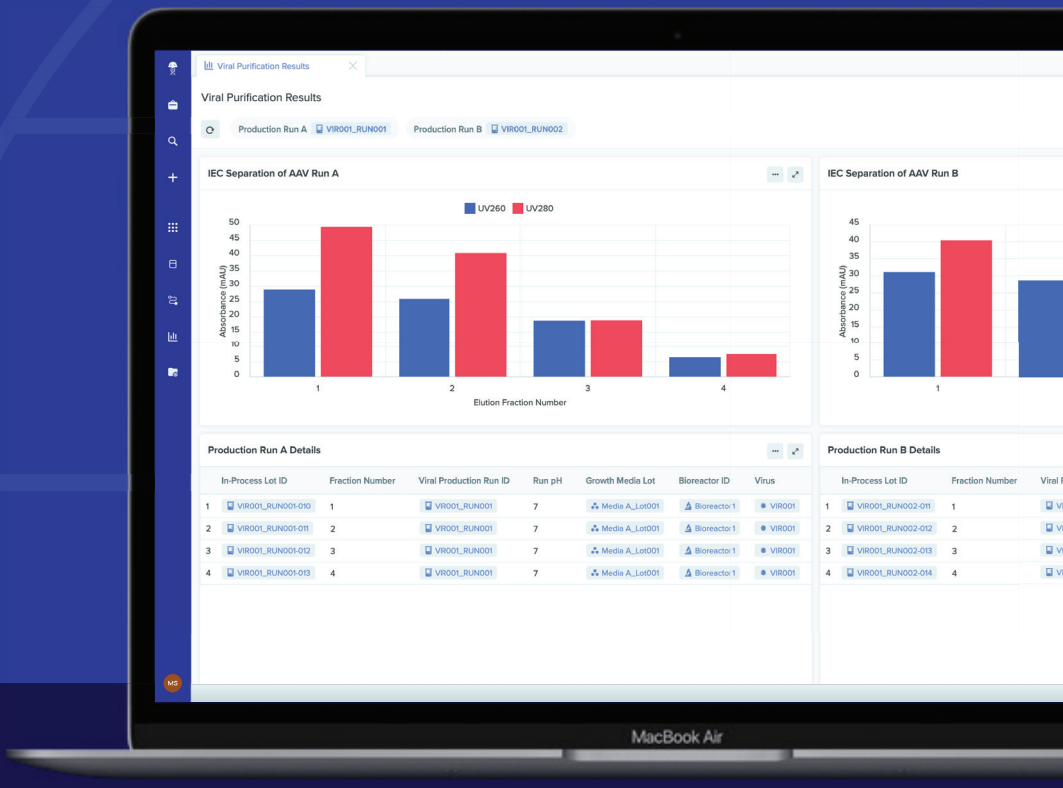

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The promise and growth of cell and gene therapies

By Megan Thomas

Putting this guide on cell and gene therapy (CGT) together has not only been an exercise in realising the seemingly boundless opportunity within the sector, where words like 'cure' and 'cancer' can be used optimistically in the same sentence, but it has also shone a light on just how many people – scientists, researchers, business leaders, lab technicians, managers – play a role in its development. This is a community working towards a common goal.

Collaboration can come in many shapes and sizes. This can come in the form of events and forums such as the ISCT Commercialization Signature Series. ISCT also offers a joint biomanufacturing training programme through a partnership with Engineering Research Center for Cell Manufacturing Technologies (CMaT), which is funded by the US National Science Foundation. Later in this guide, George White shares insight into Cytiva's industry courses and we hear from Phacilitate's Ryan Leahy ahead of Advanced Therapies Week 2023.

This is not an industry that can evolve in isolation. Thankfully, this is well-known, evidenced through the collaborations taking place across the sector. GenScript and Avectas, for instance, have joined biotech forces for a research project to streamline manufacturing of non-viral CRISPR gene editing components, with the goal of improving the editing efficiency and cell viability of non-viral based cell therapies. Meanwhile, CGT Catapult partnered with the UK Dementia Research Institute (UK DRI) to help advance the development of adeno-associated virus (AAV) based gene therapies for dementia. This only just scratches the surface.

Despite how innovative this work can feel, the reality is that the first therapy was only approved in 2017 and the number of approved treatments remains small. Of the 50-plus drugs approved by the FDA in 2021, only two were cell or gene therapies[1]. That said, there are plenty of developments which encourage optimism. With 'why' and hopefully 'how' covered, the next questions are: What is underway? What does the future hold? Where are the developments? When will we see deliverables? These are the sorts of questions the experts across this guide try to answer.

To name a few, Emulate has launched new AAV transduction application for the liver-chip that enables gene therapy researchers to test the delivery efficiency and safety of AAV vectors in a validated, human-relevant model of the liver and get results in weeks, not months, as with animal models. SiSaf, an RNA delivery and therapeutics company, is initiating the US FDA Regulatory process to obtain an Orphan Drug Designation for a therapeutic for patients with a rare genetic skeletal disorder. In late November, uniQure received FDA approval from for HEMGENIX (etranacogene dezaparvovec-drlb), a one-time gene therapy for the treatment of adults 18 years of age and older living with haemophilia B – the first of its kind. At Lonza, two CGTs have reached commercial approval: ZYNTEGLO, for the treatment of transfusion-dependent beta-thalassemia; and SKYSONA, for the treatment of early, active cerebral adrenoleukodystrophy.

According to McKinsey, in 2024 alone, up to 21 cell therapy launches and as many as 31 gene therapy launches—including more than 29 AAV therapies—are expected[2]. Currently, there are more than 1,000 ongoing clinical trials for CGTs (registered with ClinicalTrials.gov); the majority of them remain in Phase I and II and are thus not within immediate reach of commercialisation[1]. Regardless, the proportion of early-stage CGT trials still represents 10% of all clinical activity, suggesting the only way is up.

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1 https://www.cellandgene.com/

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Disruptive nanofiber technology enabling the future of cell and gene therapy bioprocessing

Therapeutic developers face significant challenges in purifying cell and gene therapy (CGT) modalities. Current processes rely on solutions developed for monoclonal antibodies that are not fit for purpose for viral vector purification, resulting in low yields, long processing times, and high costs (Figure 1).

Developers require significant improvements over current methods in order to meet patients' needs for affordable, novel therapies. A novel adsorbent that provides the difficult-to-purify large modalities used in CGTs with access to considerably more binding surface area than legacy generation technologies has been developed. AstreAdept is a composite electrospun nanofiber material, providing increased binding surface area that is highly accessible for larger modalities, with significantly faster flow rates compared to traditional bead- or membrane-based adsorbents. AstreAdept technology has been incorporated into the easy-to-use Nereus LentiHERO spin-column format to bring the advantages of functionalised nanofibers to viral vector purification at laboratory scale.

Increased lentiviral vector (LVV) capture is essential to maximizing LVV production for viral vector development. The functionalised nanofibers provide dynamic binding capacities of 1.6E+11 lentiviral (LV) particles per millilitre of adsorbent, enabling users to increase the amount of recovered materials from their current feedstock volume and, in turn, reducing pressure to expand upstream production.

In addition to the superior capture characteristics of the underlying nanofiber technology, the Nereus LentiHERO solution enables gentle processing, including low centrifugal speed, reduced processing times, and mild elution conditions for high LVV recovery. The Nereus LentiHERO technology achieves yields of more than 60% of loaded LV particles which is a significant improvement over the 15–30% recoveries observed with other methods. The structure and infectivity of purified LV particles are uncompromised when processed with the Nereus LentiHERO spin-column format, thereby retaining both functionality and morphology.

Minimising host cell protein (HCP) contamination is important for limiting immunogenicity, even at laboratory scale. However, low process recovery has driven the practice of limiting the number of process steps, so purity often is sacrificed. The success rates of vector development can be affected by unwanted immunological effects on the outcomes of preclinical studies. The Nereus LentiHERO solution reduces HCP contamination of LVV feedstocks by 95% without compromising recovery, time, or throughput. Reducing both contaminant levels and sample volumes facilitates subsequent feedstock concentration.

Incorporating Nereus LentiHERO into LVV feedstock preparation workflows allows multiple samples to be processed simultaneously, by utilising a regular benchtop centrifuge, increasing sample throughput easily by maximising the centrifuge capacity.

Nereus LentiHERO is designed to increase sample-processing throughput and improve upon LV particle recoveries for LVV development and production laboratories. The nanofiber-based technology reduces bottlenecks in the development pipeline, enabling the CGT industry to bring therapies to patients faster than ever before.

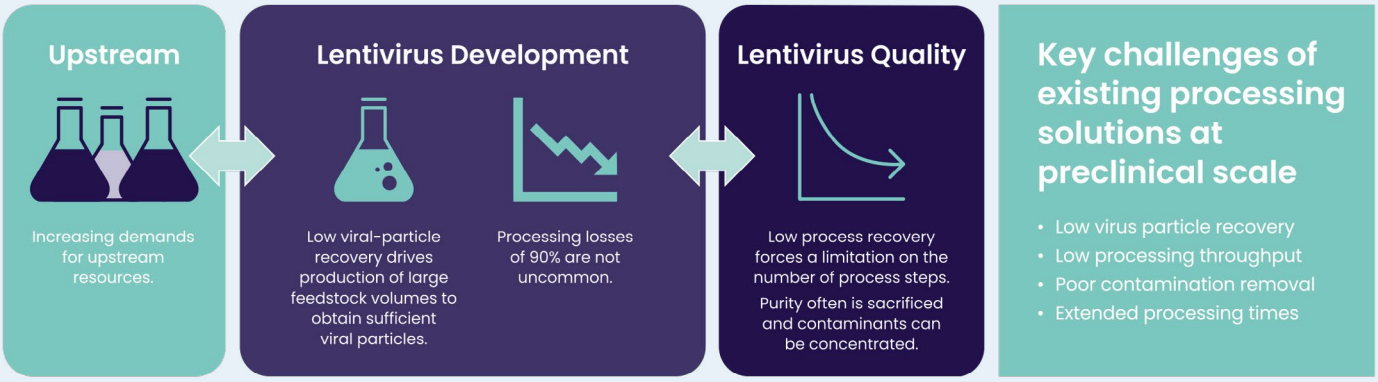


Figure 1: *Legacy processing solutions place pressures on upstream production and lentviral quality.*

Daniella Steel is senior manager, product strategy, at Astrea Bioseparations

https://www.astreabioseparations.com/

No sign of stopping: The rise of cell and gene therapy

Ryan Leahy, a science communication expert in cell and gene therapy, at Phacilitate, says there is a lot to celebrate but also much to assess, question and challenge in this sector.

While the global economic landscape faces a major crisis and biotech is not recession-proof, cell and gene therapy (CGT) has continued to progress with acquisitions, breaking ground on new facilities and approvals across the year – not limited to the breakout of the world's most expensive drug.

There's an element of stability that CGTs must achieve to encourage developers, investors, and manufacturers to take the jump to back them. Large pharma involvement in advanced therapies has seen ebbs and flows, with AstraZeneca taking a larger stake in the market and Takeda developing its automation and digital capabilities, but also with GSK letting go of its CGT projects, along with most of the staff involved in its R&D.

The cost

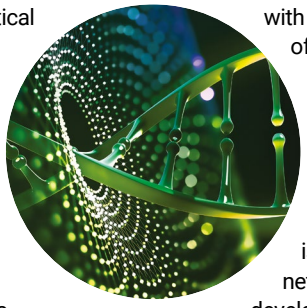
2022 has not been shy of approvals – Uniqure and CSL's Haemophilia B drug even nabbed the title of "Most Expensive Drug" at $3.5 million per dose. But are these price tags a barrier to access? Simply, yes, as we saw when the European Commission rejected the price point for bluebird bio's Zynteglo. Its subsequent success with approval in the US shows us that some drugs will be unavailable to some markets and patients for now. The fact remains that the science behind the drugs is sound, there is direct patient need, and the medical benefits are pertinent. In the case of Hemgenix, the assurance behind the price point is that the drug would reduce long-term healthcare costs as patients would have fewer healthcare incidents and need fewer clotting treatments. For the first gene therapies, prices are high but these reflect costs and are in line with the benefits – and when the three most common haemophilia B treatments have a lifetime cost of upwards of $20 million per patient, it's easy to see this price/benefit relationship.

Biotech and pharma are actively working with payers and insurers to deliver on equitable pricing models that ensure access. It is imperative that these payment processes are supported to expand access. From private plans to government programmes, there are workable systems that support CGTs, proven by MediCare's support of CAR-T and the UK's National Health Service coverage.

Patient access

A study on the socioeconomic backgrounds of CAR-T patients showed that children from across the spectrum of treatment for acute lymphoblastic leukaemia received similar results. You could thus infer that in some cancers, CAR-T treatment may be the great equaliser, which is why access should be more widespread and costs of goods, and eventually cost of drugs, must be brought down.

CGTs are classified as 'living drugs' and are much more complex entities than other biologics. The reliance on variable and unstable patient materials is a lynchpin of cell therapy drugs, so maintenance of critical quality attributes becomes paramount, and the variability of a product must be vetted throughout the entire lifecycle. Closed and automated systems are an oft-cited solution to the problems of process variability and contamination, and even under the particularly delicate therapeutic modalities of CGT, technical innovation has come along leaps and bounds over the past 10 years.



Looking forward

Automation might be the solution to the lack of skilled personnel. Certain CDMOs that are giants outside of the CGT space regard it tepidly, but creative solutions are never far behind in our industry. For instance, apprenticeships are on the rise as technical areas such as bioprocessing become more reliant on unconventional candidates, including training of personnel without a biologics or life science background.

There are questions regarding whether enough doctors are able to recommend advanced therapies, as well as where to send patients to receive treatment. This tracks with a similar lack of public, medical and government knowledge about the availability, progress, and effectiveness of CGTs. This is something the network of CGT developers will have to consider going forward.

Advanced Therapies Week highlights the dynamic landscape of CGTs. The scope of advanced therapies for the next five to 10 years is a bright but relatively unforgiving road. Public awareness of these therapies will accompany a surge of new approvals, while manufacturers and developers look to bring down costs – to a contrary extent – to the level of advancement and innovation for new medicinal products.

About the author:

Ryan Leahy is a science communication professional and Vice President of Research and Head of Content for Phacilitate. He is engaged in the increase of effective scientific communication for a variety of stakeholders and populations to advance the development of and access to cell and gene therapies.

Expert view: what to expect from cell and gene therapy in 2023

What are the global innovations in cell and gene therapy? What opportunities and challenges are emerging and can therapies get to market faster? DDW's **Megan Thomas** finds out.

Skills gap

Bruce Levine, International Society for Cell & Gene Therapy (ISCT) Immediate Past President and Chair, Strategic Advisory Council, says a critical challenge that has become apparent for the CGT sector is the need for workforce development and training resources. He says: "In recent years, the field has seen exponential growth, which has outpaced the rate at which new professionals enter. This has created significant skill gaps which urgently need to be addressed to support the continued and sustained growth of the field. This issue has become a key focus for the ISCT, and we have been working to identify and develop initiatives to help alleviate these shortages."

He continues: "ISCT has also embraced the opportunity to foster collaboration among the CGT community to enable successful process

development and regulatory compliance. Many developers encounter multi-year delays in programme development because they fail to invest in the development of later stage processes critical to scale-up while collecting early clinical data. The regulatory burden with respect to both comparability and the expectations of process and analytical robustness is ever-increasing during development. The deeper the understanding of product and process from a characterisation standpoint, the smoother the regulatory conversations and development pathway will be."

> **We have clinically proven cures for cancer and rare diseases that are, unfortunately, all but inaccessible for patients**

Talent retention

According to Stuart Curbishley, Head of Business and Project Development at the University of Birmingham's Advanced Therapies Facility, the academic CDMO considers one of the most pressing challenges year-on-year to be the attraction, and retention, of the best talent in the CGT arena. He adds: "Allied to talent acquisition, the need for adaptable and sustainable training to equip the growing workforce in CGT will be central to our, and the wider sector's, success."

Curbishley explains that as the wave of new therapies in development continues to increase, global pressures on manufacturing availability will become more acute. Whilst this is a challenge for the sector, it presents opportunities for small centres like the university's to attract new business and diversify its portfolio. Moreover, he says that global supply chain pressures will continue into 2023, combined with instability in the financial markets that will make cost of goods high on his watchlist of risks to manage. He says: "Whilst 2023 brings new and additional challenges, I remain positive that the outlook is exciting and anticipate a successful year for our centre and the CGT community."

Investment landscape

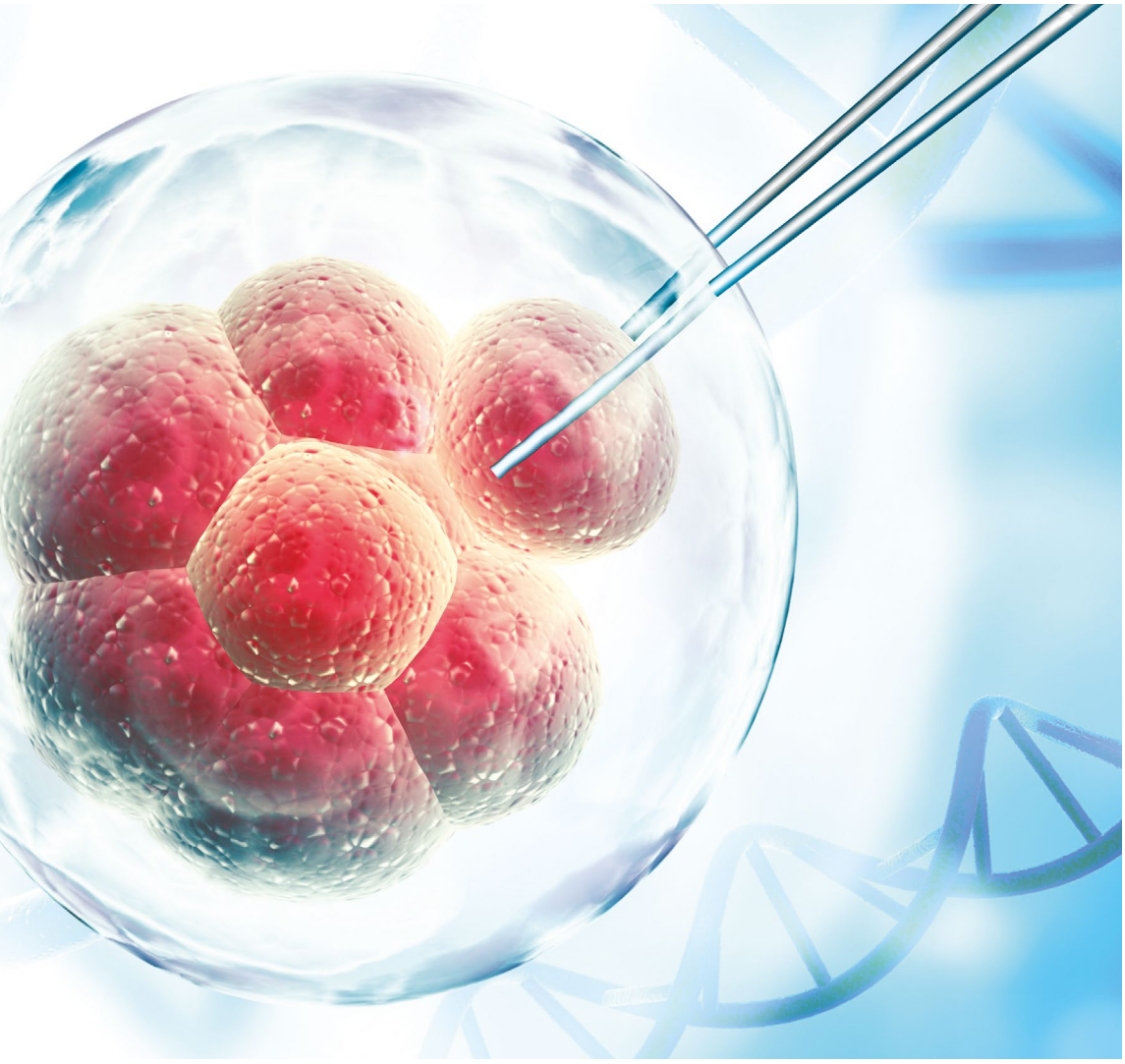
In 2023, Matthieu de Kalbermatten, CEO of CellProthera, looks forward to presenting the final safety and efficacy results of the company's Phase II clinical trial, and for moving into Phase III. He says: "Significantly, there is a new scheme set up in France whereby innovative biotherapies that address unmet medical needs can enter the market following a successful Phase II clinical, while running a Phase III in parallel. This will give us the opportunity to open dialogues with the regulatory body and gain invaluable experience in the marketing of our CGT product."

For the industry, especially in Europe, initiatives and government regulatory reforms represent a great opportunity for companies such as CellProthera to bring their therapies to patients more easily. De Kalbermatten says the accelerated regulatory pathways will be key to getting more CGTs to market, however, in order to make it to approval pathways, many start-ups and smaller biotechs need to secure investments. He says: "The investment landscape has been tough this year and that looks to be carrying over into 2023. However, there is solid hope that the situation should come back to normal by the end of 2023 according to the usual 'up and down' cycle in the industry."

Industry at crossroads

According to Jason Foster, Chief Executive Officer and Executive Director of Ori BioTech, the CGT industry is at an important crossroads: "We have clinically proven cures for cancer and rare diseases that are unfortunately, all but inaccessible for patients. The first-generation CGT companies have failed to achieve market access and therefore have not generated the necessary return on the investment made in their programmes. Without commercial success soon, we fear that CGT might be relegated to the dustbin of history as an interesting science project that never really made it."

The solution? According to Foster, the industry needs to work together to address the problems from the earliest phases of pre-clinical development. "Entering pivot trials with a low-throughput, high-cost, highly variable, paper-based process is no longer acceptable. Clinical endpoints are no longer



the only things that matter. Manufacturability matters. Cost of goods (COGs) matter. Accessibility and affordability matter. Doing all the hard work to get a product approved that is too hard to make and too expensive to reach patients is no longer an acceptable outcome. The only endpoint that really matters is how many patients can be successfully treated (per year) with a therapy. If we use that as the measuring stick, we still have a long way to go."

Decentralised processing

Vered Caplan, CEO of Orgenesis, shares that the company has spent 2020-2022 setting up its global point-of-care (POCare) platform/network. He believes that 2023 will be a banner year for decentralised processing as Orgenesis and its partners move projects into clinical trials all over the world. He says: "In 2023, CGT will continue its rapid growth, and several more candidates will be approved. However current processing methods (and associated cost) will continue hampering the global availability and accessibility of these life changing therapies. In addition to processing complexities and associated cost, recruiting/training the workforce will remains a significant issue."

Caplan says that this is why Orgenesis has created an accredited Master's Degree programme in the EU – both wet lab training in person and virtual/in-person classwork. He says: "To solve processing issues, many technology companies will continue working on the tools/equipment of the future but those solutions are still a long way away from approval for commercial use, so the field will need to look at shorter term solutions to address the disparity between demand and supply of CGTs globally."

Lentiviral vectors demand

Naiara Tejados, Head of Marketing and Technology Development at VIVEbiotech & Natalia Elizalde, Chief Business Development Officer at VIVEbiotech, anticipate that 2023 will be a very good year for VIVEbiotech, due to the increasing demand for lentiviral vectors. They note that the gene therapy market in general, and the lentiviruses market by association, is growing enormously. However, ATMPs are highly personalised medicines whose productions often need to be tailored to each patient. While this often translates into longer delivery times, the initial dedication is well worth it.

Tejados and Elizalde believe that as with any other sector, in gene therapy there are also threats that must be closely watched. But given that viral vectors are today the most efficient vehicle for successfully delivering healthy genes to diseased cells, we believe 2023 will be a promising year for viral vector manufacturers.

CGT growth and interest

Head of Strategic Marketing & Commercial Development for CGT at Lonza, Fatma Senkesen has observed tremendous growth and interest in the CGT space in recent years. She says: "We anticipate this will continue because of the unmatched levels of efficacy demonstrated by many CGTs. The drive to get to the clinic and the market first will be even more pronounced, especially in areas such as rare diseases, where there are smaller patient populations."

Senkesen sees several opportunities: "First, we see more products reaching commercialisation and expanding into new indications and regions. In cell therapy, immune cell-based therapies dominate the market and viral vectors experience substantial growth as they can be therapeutics on their own or support cell therapies as

raw materials. Cost of goods management emerges as an important topic relating to productivity, scalability, and raw materials. Furthermore, providing the flexibility needed to ramp manufacturing up or down depending on demand cycles has become more critical."

Sector challenges

Steven Henck, Vice President of R&D at Integrated DNA Technologies, observes: "From the larger industry perspective, we see increased attention from regulatory agencies with newly released in vitro diagnostics (IVD) regulations in the EU as well as 'draft guidance for industry' provided for Human CGT, particularly CAR-T therapies, from the FDA. There is a challenge to balance the time required to provide an appropriate focus on safety with the opportunity to treat patients in critical need. Lending to the time-to-clinic discrepancy, we also see increasing demands on manufacturing capacity for CGT reagents."

Henck considers there to be a shared challenge to provide efficient, end-to-end manufacturing solutions for targeted CGTs by methods that are well-tolerated, enabling delivery to patients.

Continued growth

Aaron Dulgar-Tulloch, Head of R&D, Cell Therapy at Cytiva, observes continued growth in the CGT industry, despite the impact of Covid-19. In order to this to be sustained, he says: "There is a strong clinical trial pipeline that will need automated and integrated manufacturing technologies for advanced clinical trials and commercial production. We also recognise the growing pipeline of allogeneic cell therapies entering development and early clinical trials and have initiated

> **We fear that CGT might be relegated to the dustbin of history as an interesting science project that never really made it**

collaborations to ensure these therapies have strong manufacturing platforms as they progress."

Dulgar-Tulloch sees continued growth in autologous cell therapy, with a variety of new indications progressing through clinical development. He says: "This includes indication expansion for on-market therapies as well as potential opportunities beyond haematological cancer with new technical advances. Allogeneic cell therapy is also starting to accelerate in early development programs. However, the field is still working through Covid's impact on clinical trial recruitment, supply chains challenges, and decreased funding availability."

Covid after-effects

After many years, Clive Glover, General Manager of Gene Therapy at Pall, is seeing the therapeutic and scientific benefits of gene therapies being recognised. Despite this progress, he says the industry is continuing to feel the after-effects of Covid. Looking to 2023, he says Pall will continue working to develop the integrated solutions and tools needed to accelerate the development of these novel therapeutics.

He observes: "Like many industries, the gene therapy market is impacted by global financial markets. However, the change in financial markets can also be an opportunity for us. Previously, when investments were easier to access, companies focused a lot on speed to market without fully developing their manufacturing processes. Now, when companies must be more strategic with funding, it gives them an opportunity to step back and think longer term about their manufacturing processes and commercialising their pipeline."

Collaboration required

Kathie Scheider, Director, Global Commercial Lead, Cell Therapy Technologies at Terumo Blood and Cell Technologies, says that in order to keep pace with the rapidly changing CGT market, collaboration is needed.

Scheider highlights that scaling CGT manufacturing processes continues to be an industry challenge: "This is amplified when transitioning from development to commercial GMP manufacturing. There is a need for platforms and tools that provide flexibility in scale: smaller scale for use in process development and larger scale for production. The smaller scale would ease time and cost of testing various parameters of a process and larger size would provide efficiencies of scale."

> **The need for adaptable and sustainable training to equip the growing workforce in CGT will be central**

Eye on Asia

"The world of CGTs is incredibly exciting with so many differing therapies in development, with many currently looking very promising in clinical trials," says TrakCel CEO Fiona Withey. "This represents opportunity but also potential challenges. With each of these therapies comes different support partners and supply chain processes."

Withey notes that Asia is a hotbed for CGT development and predicts there will be opportunities and initiatives in the future. For her, this brings a new level of complexity to the global supply chain with different regulatory requirements - partners and languages, she gives as an example. She says: "We will all eventually be connected across a global ecosystem that needs to be carefully managed and controlled to ensure that patients get access to the best possible treatment options."

Investment issues

eXmoor, a technical and strategic consultancy, says that the biggest opportunity for the company in 2023 will be the launch of its CGT clinical GMP manufacturing facility in the UK. The company's founder and CEO Angela Osborne said: "We are developing infrastructure and building out a team to directly deliver the manufacturing capabilities that we know the sector needs. Adding capability to capacity for CGT manufacturing is another of the sector's biggest challenges."

Some of the challenges Osborne identifies include the current lack of investment opportunities in the current economic landscape following a boom during the Covid pandemic into 2021, as well as the ongoing post-pandemic and post-Brexit disruption to global manufacturing supply chains.

Understanding the future roadmaps of cell and gene therapy

DDW's **Megan Thomas** caught up with **George White**, General Manager, Product Management, Cell & Gene Therapy at Cytiva, to learn more about the company's insight into the CGT market.

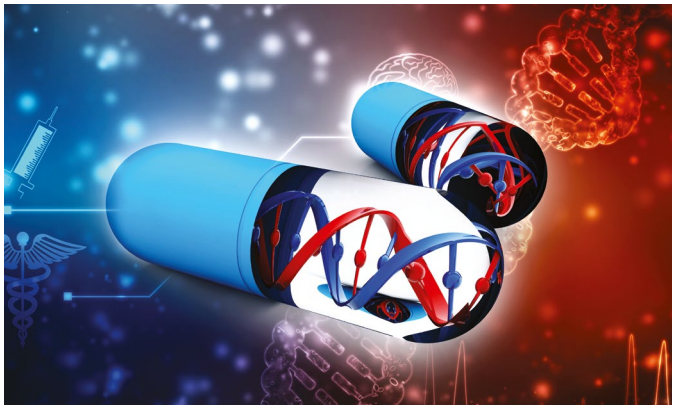
Key challenges for cell and gene therapies

In order for cell and gene (CGT) therapies to succeed, George White identifies several challenges to overcome. He says that despite the successes, particularly in certain blood cancers, major improvements are still required to reduce the cost of goods and improve the robustness of processes. He says: "One of the key areas we need to build upon is understanding the future roadmaps of where the therapies are heading. There's a lot of technology and innovation coming into this space."

White also highlights challenges in the workforce, skill sets, the cost of manufacture and the robustness of supply chains. He explains: "We want to enable greater access to patients. As treatments progress from third to second and then front-line treatment, this would be facilitated by changes in the healthcare system to increase access to therapies when they are prescribed, meaning that awareness of these important but oft-limited availability therapies must increase beyond the specialist treatment centres."

The opportunities

White sees opportunity: "Agility is needed. We're seeing a drive towards the allogeneic market with more progressing into clinical trials. Previously, there were a lot of the primary cell-based allogeneic therapies. Now, we are seeing through trials that



a large proportion, if not more than half, are induced pluripotent stem cells (IPSC) derived, which is a huge leap towards off-the-shelf products. Beyond 2023, I think we'll continue to see more innovations driven by improvements in gene editing and biological understanding."

Another opportunity White sees is shortened processes with autologous therapies. The goal is to provide flexible solutions that enable those types of developments. He says: "A key focus is to reduce the workload and resourcing required in the adoption of technology. The opportunity is there for developers willing to adopt these fit-for-purpose technologies and digital solutions, which are beginning to accelerate the progress of their clinical programmes rather than doing what should effectively be our job – to build the platforms."

Upskilling

Currently, skill sets are one of the general challenges in the life sciences. White says dependence on highly skilled operators is because historically, tools have not been fit-for-purpose, and there has not been enough automation. As a result, there are many manual handling steps plus the need for skilled interpretation. White says that's not going away soon, but will improve as we incorporate technologies to increase automation and controls.

He adds: "I don't think the need for high tech skills will ever go away in such an agile, innovative field - you're always going to have those earlier phases that are pushing the boundary. We've invested in training. CELLT1 is a course that we do in person and online and we've partnered with a world-renowned training organisation, NIBRT in Dublin, to ensure we can support customers in upskilling workforces. That's going to remain important as we build this industry and take some of that training burden off younger companies, who are trying to do everything. We need them focusing on creating therapies."

Looking forward

White emphasises the importance of looking at the transformative nature of existing patient outcomes. He says: "There's extreme potential within the emerging therapies and if we do our part to help expand capabilities and overcome challenges, then oncologists, clinicians and other types of physicians can harness the immune system and keep us all healthy."

He believes that if we solve these challenges, financing and innovation will follow. "It's working, and there is still so much to learn. With more understanding comes more potential."

Biography:

George White is the General Manager, Product Management, Cell & Gene Therapy at Cytiva. He studied genetics at the University of Newcastle (UK) and has held commercial roles for more than 15 years, supporting customers in research and the translation of therapies to clinical and commercial production.

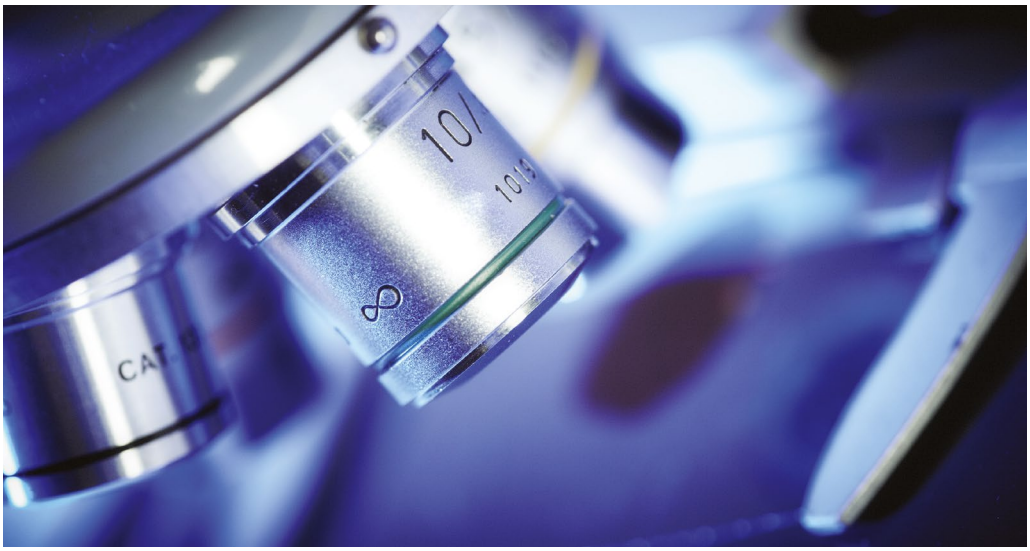
Under the microscope: Zoning in on the CGT sector

IB Communications' CEO **Michelle Boxall** explains why there are two sides to the cell and gene therapy coin.

Charles Dickens' quote, "It was the best of times, it was the worst of times", aptly describes the complexity surrounding CGT financing. With 2023's financial outlook and predictions for the CGT market, there are two sides to this coin.

The CGT sector received huge sums of investment off the back of the pandemic and through to mid 2021, as investors moved their cash into the next wave of therapeutic innovation. While this has increased scientific breakthroughs and therapies in development, it has also catalysed an influx of solutions providers entering the market. Despite the obvious benefits of industry growth, the increased competition is creating more pressure to diversify and demonstrate a company's unique selling points at a time when the industry calls for collaboration and standardisation. Balancing working with the competition on common industry goals without dampening innovation is going to be important in 2023 and will require good leadership.

On the other hand, the global economy has been impacted by the more recent downward trajectory and our clients are no exception. Budgets are being scrutinised. Deliverables, whether they be clinical progress or new products and services, must be on time, efficient and clear. There's less room for grey areas. That said,



the life science sector does run on differing investment cycles to other industries and success will still generate rewards in 2023. There remain companies gaining funds and investment.

Moreover, the routes to go public continue to broaden. Special purpose acquisition companies are slowing but remain a route to public markets, and now reverse mergers have offered additional opportunities for companies to list. The life science sector has come a long way from a simple binary choice of IPO or sale.

The fashion trends of CGTs

The CGT sector is seldom compared to the fashion industry, but there's a parallel in how variations in CGTs become flavours of the month. Attention has spiked in CAR-Ts, MSCs, SVFs, NK cells, as well as mRNA gene therapies with their Covid vaccine successes. Where the fashion parallel ends, however, is each period of attention for each CGT type brings more investment, research, development and clinical success, and pushes each respective field closer to the people that need them. This widening group of CGTs is bringing diversity in C-level leaders, scientists and organisations as scientific approaches broaden.

The potential and clinical successes of combination therapies with CGTs and therapies from other fields is also further democratising and diversifying the life science sector. Continuing to collaborate will be vital to deliver therapies to patients faster and cost effectively.

Patient-centric industry

We get closer to patients as the CGT industry matures. In the steps towards translating CGTs from the clinic into commercialised therapies, the industry is focusing on the manufacturing challenges of reducing costs and scaling up to serve more patients. This has facilitated conversations around patient numbers and waiting lists. Reimbursement and geographical constraints are also being considered, which edge us closer to health policy makers, the hospital community, health practitioners and ultimately the patient.

Engaging with the patient and health providers delivers opportunities for greater success in the market. This is a key factor in the high stakes of market authorisation, pricing, reimbursement, and sales.