ECONOMIST IMPACT

Post summary report

Cell and Gene Therapy Summit 2023

April 24th-25th 2023 | Brussels

Key takeaways

Cell and gene therapies must prove their worth with data to gain market access. For developers of new therapies, collecting the right trial and real-world data to satisfy regulators and health technology assessment agencies is essential to getting treatment to markets and patients.

Harmonising trial requirements is crucial. Being able to use synthetic controls and other methods for reducing the cost of clinical trials is essential to making advanced therapies more affordable. But disparate requirements between jurisdictions preclude those savings.

One-time treatments that offer lifetime cures will be a paradigm shift for many diseases. Particularly in the developing world, cell and gene therapies offering "one shot" cures for conditions like HIV and sickle-cell disease will dramatically boost the effectiveness of health-care spending.

Payment for performance could improve the quality of care. When contracts target the performance variables that matter most to patients, monitoring outcomes contributes directly to an improved experience.

Europe needs improved regulation to stay competitive. Clinical trials are expanding in the United States and China but stagnating in Europe. There is hope that revised EU pharmaceuticals legislation will keep up the pace of innovation.

Power dynamics matter in developing and delivering therapies. Optimising therapy takes the participation of both "David and Goliath"—from patients to big pharma companies and whole health-care systems.

Overview

Cell and gene therapies are redefining how many diseases are treated, giving hope to patients who previously had few options or none. These treatments will likely transform the outlook for people with cancer, genetic disorders and rare diseases. In Britain, the National Institute for Health and Care Excellence is to review 30 new cell and gene therapy products by 2023, and the United States Food and Drug Administration predicts 15-20 new products will be on the market by 2025.

To examine how health-care systems and policies must evolve before these therapies can become mainstream, and to hasten access to transformative products, Economist Impact's inaugural Cell and Gene Therapy Summit gathered leaders and policy decision-makers for two days of discussions at the Radisson Collection Hotel, Grand Place Brussels, on April 24th and 25th 2023.

Real-world data and reimbursement

Following his opening remarks to participants on April 24th, Victor Maertens, government affairs manager at the European Confederation of Pharmaceutical Entrepreneurs, led a panel discussion on using technology to collect real-world data for the development and use of cell and gene therapies, and on how health-care systems are meeting the data needs of the field. Participants included representatives of patient advocacy organisations, insurers and policymakers.

Panellists emphasised what became a prevalent theme of the summit: how advanced therapy medicinal products (ATMPs) must prove their worth with data to gain market access. Karen Facey, a senior adviser on health technology assessment (HTA) at RWE4Decisions, looked to real-world data to fill gaps in traditional clinical development, and to demonstrate the value of gene therapies, which tend to be administered earlier in life and have a long-term benefit. Collecting data to support that proposition could enable staged reimbursement based on success over time, said Matthew Brown, group medical director at Bupa.

> **"The value a lot of these therapies offer is as a one-time treatment, lifetime cure. So we need to find a way to measure whether or not that's reality."**
>
> **Matthew Brown**, group medical director, **Bupa**

Data gathered with patients' participation can focus attention on the outcomes that matter most to them. Len Valentino, president of the National Hemophilia Foundation in the United States, raised how patient data can shift attention from points like clotting-factor levels, which are most meaningful to health-care professionals, to outcomes like bleeding, infusions and pain, which patients most want to minimise. Jordan Abdi, life sciences partnership lead at PicnicHealth, emphasised that in treating rare diseases, where data can be scarce, patient data often offers the only way to see the whole treatment journey.

Governmental bodies like the European Commission are especially concerned about big-picture issues including the use of data in modernising health systems and addressing workforce shortages. Data must be verifiable, fair and exploitable for research, and health-care systems need to be supported in collecting and using it, said Carmen Laplaza Santos, head of the commission's health innovations and ecosystems unit.

On day two of the summit, a session on "The potential of transformative therapies in rare disease treatment"—eg, for inherited bleeding, neurological and pulmonary disorders—also saw panellists reflect on how assessments must capture real-world impacts so payers can trust new treatments whose efficacy is not yet proven in the long term. Lutz Bonacker, senior vice-president and general manager for Europe at CSL Behring, raised the importance of Europe getting regulation and reimbursement right, given the significant influence that pharmaceutical exports have on the region's trade balance.

"Getting regulatory approval almost seems easy nowadays in comparison to market access. … There's limitations around the data that some assessment bodies want to use. How could that potentially be assessed to make sure real-world evidence is allowed to flow in?"

Lutz Bonacker, general manager, Europe, **CSL Behring**

Gene therapy: approaches and costs

The two following sessions focused on approaches to and the costs of gene therapy, particularly in treating rare diseases.

Rafael Sierra, executive director for global medical affairs and global gene therapy lead at PTC Therapeutics, led the way with a case study of his firm's drug Upstaza. Recently approved by the European Medicines Agency (EMA) and the British Medicines and Healthcare Products Regulatory Agency, Upstaza delivers the minimum amount of a gene therapy agent to the local area where it is needed, in this case the brain. This avoids immunogenicity while targeting long-lived cells to lengthen the effect, and minimises the amount of agent needed, reducing the burden on manufacturing.

A child treated with Upstaza at age two can go from having little voluntary movement to standing, walking or even running a few years later. With these long-term benefits accruing from a single injection, PTC is committed to bringing new therapies of this type to market, mostly for children who have no existing treatment options.

Reducing the cost of such therapies is a key part of improving access, which policymakers and scientists explored in a panel discussion, "Reducing gene therapy costs 50x and beyond".

The relatively low numbers of people suffering from rare diseases in most individual countries is a major hurdle for cell and gene therapies. By enabling access to international datasets, a therapy might go from targeting one patient to targeting 50, which goes a long way towards reducing costs.

International action can also improve equity. Joint procurement of covid-19 vaccines prevented prices differing between countries. But some families still have to move within the European Union (EU) to get affordable gene therapy for their children.

> **"Covid-19 underscored the need for local manufacturing…it's a natural step to tack on cell and gene therapy to those same manufacturing efforts."**
>
> **Jen Adair**, associate professor, **Fred Hutchinson Cancer Center**

Harmonisation of trial requirements is also essential. The ability to use synthetic controls, for instance, can greatly reduce costs, but some regulators, such as the EMA, do not allow it. Local manufacturing of treatments offers further hope for cost reduction.

Cell and gene therapies: a paradigm shift?

Day two of the summit opened with a panel on the social and economic impact of therapies that unlock innovation for largely untreated diseases.

Natasha Loder, health policy editor at The Economist, noted how cell and gene technologies are bringing curative therapies for diseases like spinal muscular atrophy, haemophilia, sickle-cell disease and Duchenne muscular dystrophy. Though they now come at a high cost, and with a risk of adverse effects, resolving such issues could change the face of health care.

Panellists looked forward to numerous therapies now in the pipeline, including treatments for severe combined immune deficiencies, CRISPR treatments, cell therapies for solid tumours and treatment of neurodegenerative diseases like Parkinson's. Joseph "Mike" McCune of the Bill and Melinda Gates Foundation is hopeful of seeing simple, cost-effective cures for HIV and sickle-cell disease, particularly in the developing world. This possibility is attracting the interest of both philanthropic organisations like the foundation, and pharma companies like Novartis.

> **"There's a lot of great things coming. We have over 2,000 chemicals in the pipeline right now for the gene and cell therapy sector, and 200 in the late stage."**
>
> **Mark Battaglini**, chief strategy officer, **Alliance for Regenerative Medicine**

One-time treatments that offer a lifetime benefit could cause a "paradigm shift" away from health-care systems that offer incremental benefits at a high cost while struggling to pay for ambulance services and nurses' salaries, said Steve Pearson of the Institute for Clinical and Economic Review. Cures would slash lifetime costs for haemophilia treatment (around $21m) and sickle-cell disease ($4m-6m), as highlighted by Mark Battaglini, chief strategy officer at the Alliance for Regenerative Medicine.

Developers of therapies should engage with health authorities such as the EMA early in clinical development, to get scientific advice on the data needed to smooth the pathway to market and reduce costs, said Hugues Malone of the Belgian Federal Agency for Medicines and Health Products.

But cost is not all that matters. Mr Battaglini noted how patient families and caregivers can now hope for previously impossible cures, while Mr McCune pointed out that single-shot gene therapies for HIV and sickle-cell disease would supercharge the effectiveness of money spent in sub-Saharan Africa. Claire Booth, a professor of gene therapy and paediatric immunology at the University College London Great Ormond Street Institute of Child Health, also looked to business models involving groups of families funding development of treatments for rare diseases, or "venture philanthropy", to help serve patients with an unmet clinical need.

Innovating in pricing and market access

A trio of sessions then dug deeper into innovations in pricing and market access, starting with the panel discussion, "The ends justify the means: how can products advance without compromising their commercial potential?"

Even if treatments may have a high lifetime benefit, therapy developers' interest in pricing to recoup costs may be in tension with the need for payers, patients and their families to keep within the current year's budget and minimise debt.

> **"A one-time price for this value assessment on cost-effectiveness will often come up very high. And that can be in real tension with the health system of today, where they are not worrying about paying for things 30 years from now."**
>
> **Steve Pearson**, founder and president, **Institute for Clinical and Economic Review**

Some HTA organisations gauge cost-effectiveness by extrapolating short-term results, while others acouldre more focused on comparing new treatments to existing ones. HTA bodies align their evaluation of cell and gene therapies—which can reduce the need for ongoing treatment—with a prevention or wellness paradigm. "How do we link the upfront, one-off costs of things such as gene therapies to the broader sustainability of the health-care system?" asked Jennifer Lee, therapy market access leader for Europe, the Middle East and Africa at Janssen. Manufacturing, infrastructure and logistics, clinical development and approval can all be streamlined to lower costs, which would help to make the case for reimbursement. Regulators such as the EMA and FDA have been supportive of new pathways for approving products more efficiently.

In a presentation on harmonising price points with payment strategies, Simon Boselli, public affairs director at the European Rare Diseases Network (Eurordis), expressed frustration with persistent differences in assessment processes for therapies. European integration affords scope to establish more uniform prices and to move patients within the region for cost-effective treatment in centres of excellence.

A fireside chat between Alexander Natz, secretary-general of the European Confederation of Pharmaceutical Entrepreneurs, and Declan Noone, president of the European Haemophilia Consortium, delved further into the matter of equity. Mr Natz noted the progress already made in having the EMA find ways to bring products to the marketing authorisation stage. Yet while advanced therapies have made it to patients, others have not, dampening optimism.

Investment in evidence can enable "payment for performance models" that look at results over three to 20 years. When contracts target performance variables that matter to patients—such as transfusion independence or confidence to engage in physical activity—they ensure that monitoring improves the quality of care. This is happening already with CAR T-cell treatments, and could happen with gene therapies too.

Bringing policy up to speed with science

Mid-afternoon sessions on day two focused on regulatory pathways and how policy can be brought up to speed with science to facilitate access to ATMPs. This conversation took place within the context of expectations for the European Commission to propose revisions to EU pharmaceuticals legislation later in the week. "How can we keep Europe competitive?" Mr Natz asked.

Over the past ten years, the focus of conversations has gone from putting treatments into the market to putting them into patients. Approved treatments have in some cases been withdrawn because reimbursement conditions made them unviable for the manufacturers or they were only used on a small number of patients a year.

A diversity of laws, including those on genetically modified organisms that apply to ATMPs, and of clinical trial regulation at the national level, make it more difficult to get products to markets and patients. Multiple participants had hope that new or revised regulation will alleviate this, and acknowledged that tremendous progress had already been made in frameworks and collaboration to enable access to ATMPs in the past decade.

"Clinical trials are expanding in the United States and China, and they're stagnating in Europe. We are seeing less innovation going into development, industry and patients."

Miguel Forte, president-elect, **International Society for Cell & Gene Therapies**

In the meantime, Europe is at risk of falling behind the United States and China, where clinical trial activity is expanding. Europe must reconcile processes that usefully centralise excellence, knowledge, decision-making and approval, with the needs of member states.

Delivery and patient experiences

The summit concluded with a panel discussion on engagement with patients and patient groups, whose trust is essential if cell and gene therapies are to be widely used, and whose data is crucial to support pricing decisions, HTA assessments and market access. What makes for good patient engagement and how can it be achieved? asked Jo Pisani, a trustee at rare-disease charity Beacon.

Patient engagement must be amplified to see the adoption of around 2,000 advanced therapies currently in the pipeline and 200 in late stage development. In educating patients about treatment pathways, "the group of patients involved are also educating you as to what it's like to live with that condition and what's important to them," said William Cole, life sciences partnership manager at Kidney Research UK.

Generating trust in therapies is part of this education. To do this, patients' involvement should not be confined to trials, but extend to real-world data collection and the whole ecosystem of care.

Panellists shared case studies of how patient engagement works in practice, in areas including collecting data on chronic kidney disease, improving access to cancer care, and tracking the patient experience of CAR T-cell treatment.

> **"One of the key things about patient involvement is power and power-sharing. … How much power do we give up and how much do we allow the patient to influence what we might be thinking?"**
>
> **Henny Braund**, chief executive, **Anthony Nolan**

As the discussion drew to a close, Rebecca Middleton, chief executive and founder of Hereditary Brain Aneurysm Support, painted an evocative picture of the need for both big and small organisations—from pharma companies and health-care systems to patient groups—to participate in optimising therapy. "You need David and Goliath," she said. "The true power is when you combine the two."

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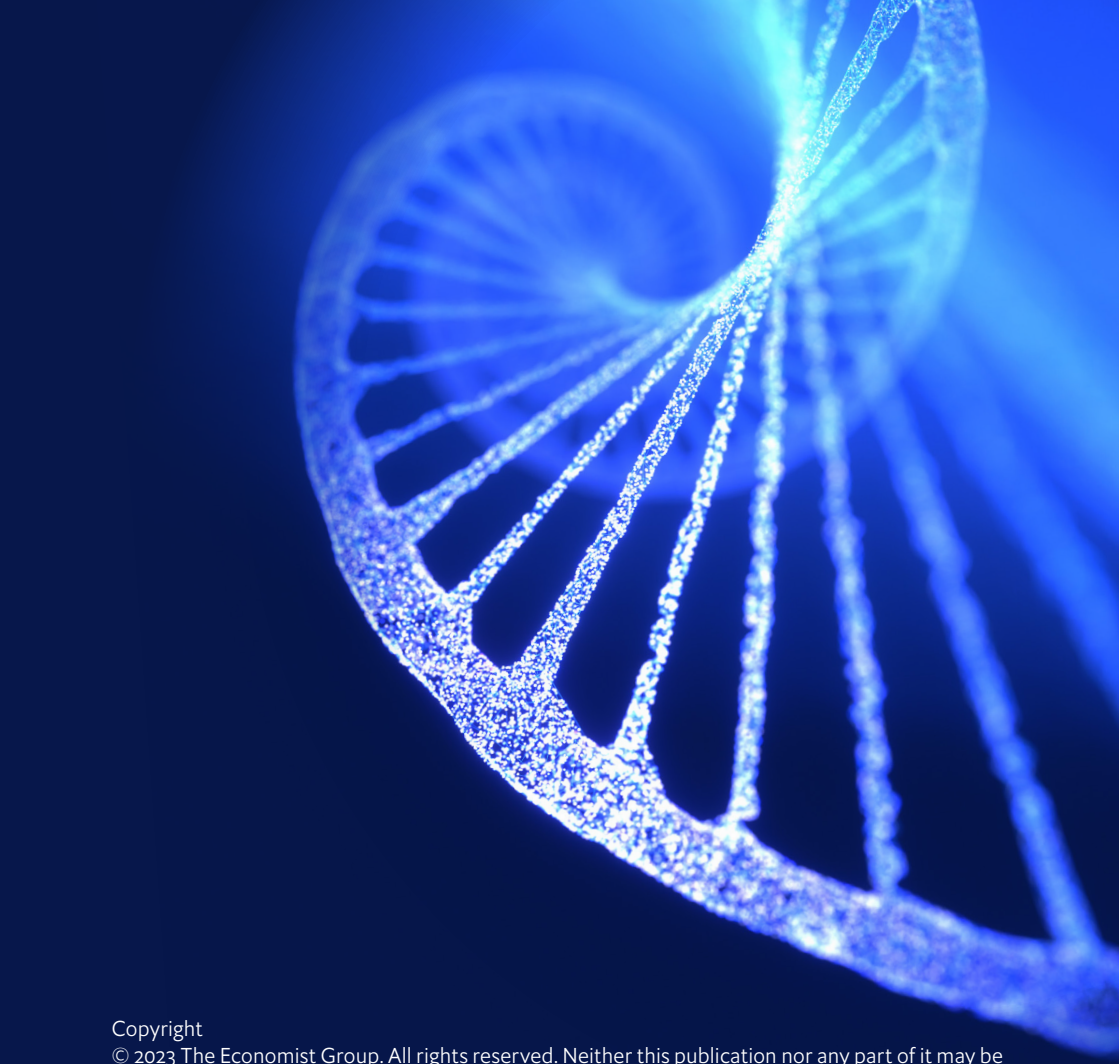

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