ECONOMIST IMPACT

Roundtable discussion

Creating a sustainable business model for cell and gene therapies

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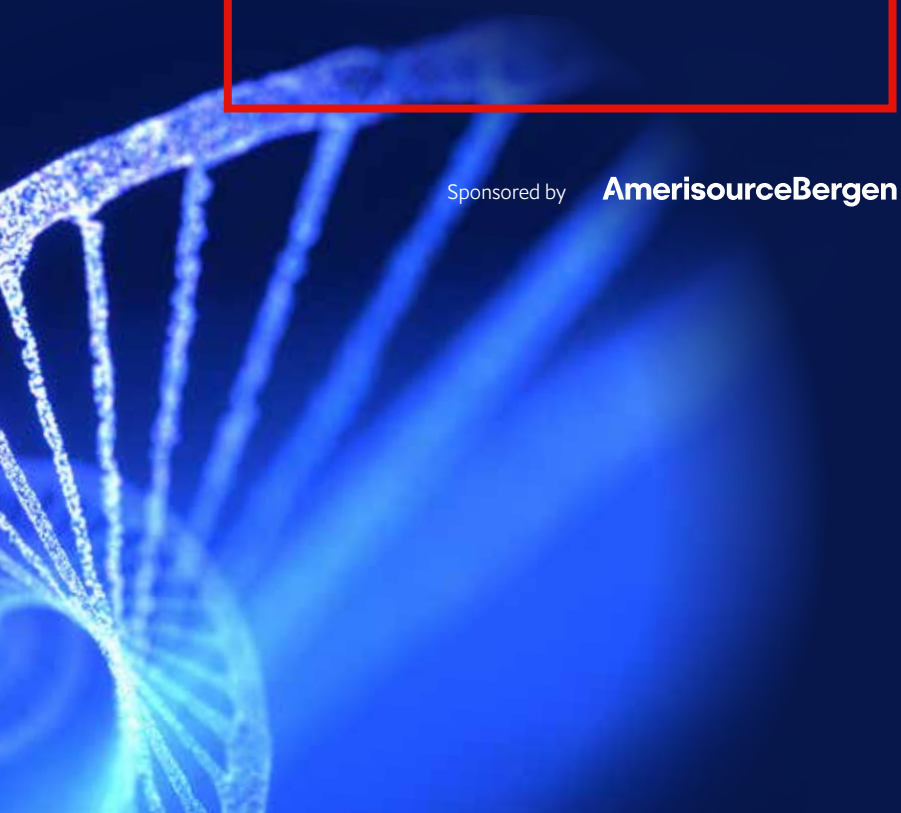

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Roundtable discussion

Creating a sustainable business model for cell and gene therapies

Moderated by: Lung-I Cheng, Vice President, Cell and Gene Therapy, AmerisourceBergen

Stock-market weakness during 2022 and early 2023, as central banks have raised rates to combat inflation, has put pressure on start-ups to deliver results. In the biomedical field, innovators need to think more carefully about their strategies for developing and commercialising new therapies. The path to success for businesses involved in cell and gene therapy is not without challenges. Unlike other areas of pharmaceuticals, in this field 90% of companies are still trying to launch their first product.

To examine how the development of cell and gene therapies can be made a sustainable business, Economist Impact facilitated a roundtable discussion, supported by AmerisourceBergen, as part of the Cell and Gene Therapy Summit 2023. Participants included senior staff in the biopharmaceutical industry, clinicians, academics and researchers, and members of patient-advocacy organisations.

Overcoming regulatory challenges

Lung-I Cheng, vice-president for cell and gene therapy commercial solutions at AmerisourceBergen, which provides services to the pharmaceutical industry from pre-clinical to commercialization in areas including distribution, logistics, regulatory consulting, market access, and patient support, started the discussion with a focus on regulatory challenges. One participant said that "regulatory risk" in highly regulated jurisdictions such as the United States can discourage the development of therapies. Places like Japan, where regulators "don't necessarily dive into the nuances" as much as the United States Food and Drug Administration (FDA) does, can be more accommodating. Erica Whittaker, vice-president, head of corporate venture fund at UCB Pharma, also observed how regulation can influence the design of clinical trials and consequently the ease of securing funding.

"Securing approval for reimbursement of treatment costs means proving that a treatment not only works and is safe, but is cost-effective and better than alternatives."

New start-ups could benefit from taking a more holistic approach to meeting regulatory requirements. Eugena Stamuli, a health economist who consults for pharmaceutical companies with HES Choices, pointed out that not only do new therapies need approval from authorities such as the FDA, but elsewhere, gaining market access means securing approval for reimbursement of treatment costs by health providers and insurers. That means proving that a treatment not only works and is safe, but is cost-effective and better than alternatives. "Alignment between regulatory experts and market-access experts internally is so important," Mr Cheng confirmed.

Adopting a forward-thinking approach

It may not be realistic for each small company to put its first product into the market or engage with every health technology assessment (HTA) organisation and regulator worldwide, said Rick Vreman, a patient access manager at Roche. Stakeholders such as patient organisations could think about coming together early to consider and articulate what outcomes and products they expect from firms developing therapies.

> **"A good plan can start from the end of the process, with a vision of where you want the product to end up."**

For businesses, too, a good plan can start from the end of the process, with a vision of where in the world you want the product to end up. "Hire the right market access person," said Ms Whittaker. In the biggest European countries and the United States, it is very possible to map the pathway a drug will take to reach patients from early in its development.

What makes a therapy commercially viable?

Academics, industry and patient groups need to work together in identifying which innovations are most likely to be commercially viable. "If the product reaches the market and it's over €2m for one patient, there's a high chance it will fail," said Delphi Coppens, a science liaison for advanced therapy medicinal products with the Dutch Cancer Society. Mr Cheng suggested that more industry involvement with academia could help reduce the risk for businesses, for example by producing more clinical evidence to validate, or invalidate, the business case for a possible new therapy.

Juan Carlos Lopez of the RTW Charitable Foundation looks at the treatment of rare diseases from both a venture-capital and philanthropic lens. "At what point do you decide that something is commercially viable?" he asked. In the United States, he now thinks 10,000 patients is the minimum. But the foundation looks at development of therapeutics for conditions affecting 300 people or fewer – so commercialisation cannot be the only model. In some cases, funding people to travel to an institution for a customised treatment is cheaper than turning it into a product.

"For very rare conditions, commercialisation cannot be the only model. Funding people to travel for customised treatment can be cheaper than turning it into a product."

Comparing different jurisdictions for developing treatments, Mr Lopez found the more permissive regulatory landscape for clinical trials in Europe a plus, while manufacturing capabilities are superior in the United States. In Britain, Cell and Gene Therapy Catapult, an organisation that collaborates with academia, industry and health-care providers, is helping to bring innovations to market faster.

Quality-adjusted life years (QALYs) can be a metric for HTA organisations to use in benchmarking the cost-effectiveness of treatments—with £30,000 per year gained being a magic number, according to Ms Stamuli. For cell and gene therapies, though, costs can reach up to £100,000 per QALY. But unmet need also makes the case for a therapy. "How desperate is society for a new treatment? Are people dying? All these things are examined together," Ms Stamuli said.

Making supply chains work for advanced therapies

In the final stages of the discussion, Mr Cheng put the spotlight on manufacturing and supply-chain challenges. Some cell and gene therapies have short shelf lives, or must be stored and shipped at ultra-low temperatures. One participant noted particular challenges with allogeneic and autologous cell therapies, where regulatory arrangements and business models that facilitate decentralised manufacturing can ease shipping and storage, cut production times and reduce costs. Relative to each company behind one of these therapies having its own manufacturing facility, outsourced manufacturing could enable the sharing of facilities for greater economies of scale.

"Decentralised manufacturing can ease shipping and storage, cut production times and reduce costs."

In the United States, the Bespoke Gene Therapy Consortium (BGTC) has been formed by the National Institutes of Health (NIH), FDA, pharma companies and non-profits to establish a protocol book that the research community can use to develop therapies for rare conditions more efficiently. "If your company's not part of the bespoke consortium, you want to join it," said Mr Lopez of the RTW Charitable Foundation. "It's not cheap but it's worth it." The NIH also has its Platform Vector Gene Therapy (PaVe-GT) pilot project, which aims to reduce costs by testing use of the same gene delivery system for multiple gene-therapy clinical trials. RTW itself has founded a company called Rocket Pharmaceuticals, which is optimising manufacturing to reduce costs when bringing new projects to market.

To conclude, Mr Cheng reiterated the importance of beginning with the end in mind, which is ultimately to get therapies to patients. Multi-stakeholder engagement is essential to this goal—between pharmaceutical companies, regulators and payers, and also patient groups. And for manufacturers, there are new business models yet to be explored. "As we progress this field," Mr Cheng asked, "how do we make sure that we create a more sustainable future?"

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