Can Europe become a major player in cell and gene therapy?

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With European life sciences R&D investment at a 20-year low, biopharmaceutical associations and MEPs are calling on the EU to modernise its policies. DDW's Diana Spencer asks what will be needed for Europe to compete in the growing global market for advanced therapies.

A new report by Charles River Associates for the European Federation of Pharmaceutical Industries and Associations (EFPIA has revealed that Europe is falling behind the US and Asia in its pharmaceutical R&D investment, particularly when it comes to Advanced Therapies Medicinal Products (ATMPs) like tissue, gene and cell therapies.

Despite the importance of the pharmaceutical industry to national economies and population health, the report paints a picture of falling competitiveness, with the global share of R&D investment, clinical trials and manufacturing output all decreasing.

This concerning trend will also be raised by the Alliance for Regenerative Medicine (ARM) at EU parliament event 'The Future of ATMPs in Europe' on the 29 November, which will discuss key policy initiatives that will determine the role of cell and gene therapies in Europe's healthcare landscape for years to come.

So, what can European countries do to encourage growth in drug discovery, attract investment and ensure long-term stability? Fortunately, the EFPIA report also offers a detailed list of recommendations and the hope that with a proactive approach to nurturing life sciences, this trend can be reversed.

The current situation

The EFPIA report demonstrates a significant drop in European investment over the last 20 years. It reveals that in 2002 the US spent just $2billion more than Europe on R&D, but by 2021 that figure increased to $20billion. Of the total R&D investment made in the US, Europe, China and Japan, only 31% occurs in Europe, which has declined steadily from 41% in 2001. China has meanwhile grown its share from 1% to 8%.

In terms of ATMPs, other countries are ramping up support for these innovative therapies. ATMP clinical trial activity is twice as high in the US and almost three times as high in China than in Europe. The number of ATMP trials conducted in the US and Asia-Pacific region grew by 70% and 67% respectively from 2014-21, while Europe remained stagnant.

Overall clinical trial activity in the region is also on the decline. Europe accounted for a 19.3% share of global clinical trials activity in 2020, a decrease of 6.3%, compared with a 25.6% average over the last ten years.

In its 2022 *The Pharmaceutical Industry in Figures* publication, the EFPIA attributes this downturn to increased regulatory hurdles and escalating R&D costs, as well as fiscal austerity measures introduced by governments across much of Europe since 2010.

The report makes various policy recommendations, which it suggests could be implemented at national government level or EU level through the revision of the pharmaceutical legislation. However, it also warns that the options being discussed by policy makers in the context of the current review of the European Pharma Strategy, which is expected in December 2022, fall well short of addressing concerns.

The EFPIA's Director General Nathalie Moll comments: "Today's report should provide a wake-up call to all of us. It could not be more important to patients – and to the future of medicines development in Europe – that the Commission and national governments work with industry to retain – and grow – the sector here in Europe.

"While the Commission's ambition of balancing affordability for member states and future innovation is the right one, the current thinking is set to have a negative impact on patient care and further erode Europe's competitiveness."

What will improve Europe's attractiveness for life sciences?

Developing world class innovation hubs

The EFPIA report suggests that Europe is falling behind by not prioritising life science hubs for investment. While world-leading hubs like Boston, San Francisco and the UK's Golden Triangle receive significant policy focus and strategic funding, European research funding is more uniform. Indeed, the countries with the highest EU research spend per population are not the centres of innovation. The EFPIA recommends a review of existing life science and industrial policies across Member States to identify success factors and opportunities for replication.

Matthieu de Kalbermatten, CEO of French cell and gene company Cellprothera, agrees with this recommendation, having experienced delays due to EU funding being spread too thinly. "The willingness of EU administration to distribute funds equally among all partner states bears the risk of delaying the process unnecessarily and spreading out the resources across too many parties," he explained. "This is what we have experienced recently with a collaborative project whose submission was delayed by at least one year."

Funding disruptive innovation

Europe's share of emerging biopharma companies has been in decline for ten years. The report highlights Denmark as a good example, in supporting the growth of around 200 new life sciences companies in the Eastern Denmark cluster between 2017 and 2022. The government-led 'Innovation Centre Denmark' located in Boston, aims to accelerate cooperation between the clusters. Identifying and adopting successful initiatives such as this should be prioritised in the European Pharma strategy, the report recommends.

Implementing early access mechanisms

The report predicts that company research investment will increasingly be influenced by regulatory agility and flexibility and a supportive clinical trial environment, which ensures patients can benefit from new therapies. It points out that the related European Commission strategy does not link early patient access to new medicines and the attractiveness of Europe as a location for companies to locate their research, clinical trials and manufacturing.

Francois Rieger, CEO of Belgian company BioSenic, agrees that clinical trial support is a key weakness in the current European system: "The most important opportunity for support for the European system is the financing of clinical trials, and specifically the confirmatory phases. There is currently not enough access to finance to deliver clinical success for a number of therapies to patients that could benefit from them."

Boosting digital capabilities

The report highlights that the US is far ahead of Europe in digital infrastructure, and Europe's leading hubs for R&D and manufacturing – Germany, Belgium and Ireland – rank poorly in digital competitiveness. It suggests an EU-led effort to increase the interconnectedness of hubs, upskill the scientific workforce in digital technologies and accelerate the digitalisation of health systems to help boost innovation.

Fostering sustainable procurement and pricing

The EFPIA says that Europe's increased focus on manufacturing older, generic medicines will damage future innovation unless re-thought in the proposed revision of the EU pharmaceutical legislation.

Creating long-term stability

Given the long-term focus on investment decisions, the report states that regions that offer long-term stable environments coupled with growing markets will benefit from decreased perceived risk. A long-term outlook requires the EU Pharma Strategy to be implemented through a forward-looking partnership with industry, with tangible key performance indicators to ensure the legislation enables benchmarking of Europe's long-term competitiveness.

Establishing an ecosystem for cell and gene therapies

In a global pipeline of over 8,000 new medicines, 804 are next generation biotherapeutics (including cell and gene therapies and mRNA technology), indicating that it is in ATMPs that we will see the greatest innovation in drug discovery.

Europe is particularly strong when it comes to academic research for ATMPs. European institutions authored 48,000 more publications than the US between 2017 and 2019, and 20,000 more than China, but the research is not being realised into potential new therapies.

The US currently has 50% of the world's ATMP manufacturing facilities. Asia is fast becoming the most competitive region for attracting ATMP clinical trials (255 in 2021), but Europe is unfortunately in decline (89 in 2021).

ARM's H1 2022 Sector Report 'Regenerative Medicine: The Pipeline Momentum Builds' revealed that seven of the 23 ATMPs that have been approved in the EU have been withdrawn from the market.

The EFPIA says European regulation must recognise the complexity of these new technologies and build the interconnected ecosystem required to develop them, adding that the region's competitiveness is hindered by a siloed approach to policymaking and missed opportunities. A more proactive role is needed in fostering the growth of emerging ATMP clusters, providing support across the entire eco-system of R&D, manufacturing and clinical trials.

Matthieu de Kalbermatten feels that things are moving in the right generation, but there is a long way to go: "The EU has recognised the need to put a more aggressive strategy towards financing the development of biotherapies and the manufacturing capacity that comes with it. This need was emphasised by the recent pandemic and the lack of local production. Indeed, several investment programs have been initiated at the EU and national level which is a very positive sign.

"However, the translation of these EU funds into competitive projects with short to mid-term results is threatened by the overly complex administrative hurdles that characterise those centrally managed programs."

The future of ATMPs in Europe

At an EU parliament event on 29 November, ARM will make similar calls for the EU to modernise its policy and regulatory framework to reflect the distinct promise of cell and gene therapies as the future of medicine.

"Cell and gene therapies represent tremendous hope for patients with serious diseases in the EU – but if we don't act to ensure patients have access, hope is all we will have," says Dr Stelios Kympouropoulos, Member of the European Parliament of the European People's Party, who lives with spinal muscular atrophy. Kympouropoulos and 25 other MEPs have signed a letter asking the European Commission not to undermine the European pharmaceutical and biotechnology sectors with its upcoming proposal to revise the EU pharmaceuticals legislation.

"If Europe modernises its policy and regulatory framework to truly embrace ATMPs as the future of medicine, European patients living with cancer, rare diseases, and other serious and often life-threatening disorders will benefit tremendously," says Timothy D Hunt, Chief Executive Officer of ARM. "The same policies and approaches that brought us yesterday's biomedical innovation simply will not work for the cell and gene therapies of today and tomorrow. The EU has led before – and can lead once again – but the time to act is now."

https://www.ddw-online.com/can-europe-become-a-major-player-in-cell-and-gene-therapy-20633-202211/