

EUCOPE Position

Gene & Cell Therapy – pioneering access for ground-breaking treatments

September 2018

Introduction

Gene & Cell therapies have arrived. They bring with them the promise not simply of treatment to manage the symptoms of a diverse group of severe, disabling or life-limiting conditions but the promise of one-time disease modifying treatments that can transform and save lives. What was once the realm of science fiction is now reality for a growing number of patients and clinicians. The value of these innovative therapies lies not only in their promise to transform the lives of patients with severe and disabling diseases but also their potential to transform the way in which healthcare is delivered.

However, many healthcare systems are not yet ready for such transformative innovations and one time treatments. As Commissioner Andriukaitis has said*, obstacles to the development of successful therapies must be removed, and Europe must not miss out on innovation and progress in this area.

In the past, payment and reimbursement considerations have proven barriers to timely patient access to novel therapies, a risk that may only be exacerbated for gene and cell therapies. Without pioneering approaches to **Health Technology Assessments**, reimbursement and **sustainable funding models**, cross-border healthcare and the readiness of healthcare systems to adopt breakthrough innovations, **patients** are at risk of missing out on access to game-changing gene & cell therapies.

1. HTA and evidential options

Gene and cell therapies are novel approaches to tackling the underlying causes of many severe and disabling diseases. Gene therapies involve the transfer of a therapeutic or working gene copy into specific cells of an individual in order to repair a faulty gene copy. Cell therapy is the administration of living whole cells to a patient for the treatment of a disease¹. The innovative nature of these therapies means there might be greater uncertainty in the evidence of long term outcomes when Health Technology Assessment (HTA) and reimbursement decisions are being made.

HTA processes may need to adapt in order to assess new treatments that promise long-term benefits but typically start with highly positive short-term data on clinical benefit from relatively small trials. EUCOPE is keen to work with HTA agencies to look at how current HTA processes can offer greater flexibility to address evidential challenges and uncertainty in the HTA of cell and gene therapies to improve uptake and access in line with core HTA principles that only effective treatments should be funded.

EUCOPE recognises the important role of registries and ongoing data collection to demonstrate ongoing safety and efficacy and improve the evidence base for gene and cell therapies. These requirements can vary from country to country and it can be challenging to meet these different requirements. EUCOPE would welcome the opportunity to align registry requirements.

¹ [Commissioner Andriukaitis' speech at the STOA workshop: 'Therapies for the future exploring solutions for innovative treatments in Europe'. EP, Brussels 11 October](#)

² [ERN RITA fact sheet](#)



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2. Affordability and funding solutions

Gene and cell therapies typically involve costly research, development, and manufacturing costs and are associated with greater logistical demands for patients (e.g. some therapies require patients or cells to be transported, often to different countries). There are regulatory requirements, and companies recognise the importance of these as measures of quality control and are committed to demonstrating the long term safety and effectiveness of these new technologies through the establishment of long-term registries. While individual hospitals are sometimes able to develop or deliver treatments “in-house”, it is critical to note that these efforts are exempt from some of the same regulatory and HTA requirements companies must meet to demonstrate that a therapy is safe and efficacious. In addition, hospitals are normally unable to deliver treatments on a large scale. Given these significant differences, these products should not be used in reimbursement processes as a benchmark - or comparator - for those gene and cell therapies that have gone through the rigors of obtaining a Market Authorisation. The additional requirements provide reassurances about the safety and efficacy of treatments and EUCOPE would like to see the costs associated with these requirements recognised in funding decisions.

In awarding Orphan Drug Designation to new products the European Commission recognises this distinction, requiring products to demonstrate significant benefit over existing authorised therapies and explicitly excluding products with a hospital exemption as benchmarks³. EUCOPE would hope to see this reflected in HTA and reimbursement processes.

Gene and cell therapies offer the potential to disrupt historic patterns of delivering healthcare often focussed on managing symptoms rather than effecting a cure. Reimbursement processes and uptake of innovative medicines are based on medicines that are more traditional rather than on one-time treatments. Recent developments in European healthcare decision-making have focussed on a common need to manage constrained budgets. Thus, there is a concern that one-time administered therapies coming to market over the next several years will represent a significant challenge for health care systems and payers, and that widespread uptake is uncertain... The European Commission has acknowledged that there is no single approach to ensuring access to these treatments in different countries but a common understanding of the promise of the science of gene & cell therapies and a shared recognition of the disruption to historic patterns of funding, payment mechanisms and healthcare delivery will help to create a more receptive environment for these therapies. Working together to identify approaches to funding, reimbursement and payment mechanisms will ensure appropriate prices for cell and gene therapies are achieved that are both affordable to healthcare systems and can incentivise continued research and innovation in this area. Innovative funding models such as outcome based or pay-per-performance agreements may play a crucial role in ensuring payers have the confidence to invest in one-time treatments and enabling patients to benefit from effective gene and cell therapies.

EUCOPE recognises the importance of developing outcome based funding agreements that will provide HTA agencies and payers the certainty and confidence in these new technologies that they need in order to ensure that patient are able to access to these new therapies.

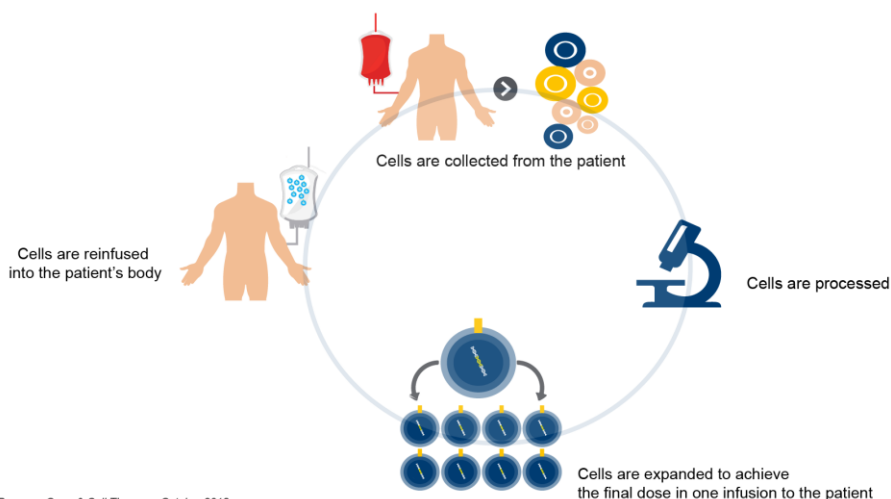
3. Access for patients across Europe

Gene and cell therapies are highly personalised treatments with complex manufacturing and distribution processes. Many of these therapies will be available in only a small number of treatment centres in Europe with the critical expertise in manufacturing and the administration of therapy including essential safety monitoring after infusion of the cell and gene therapies. Even patients in larger countries may have to travel to a different region or country to receive these treatments.

Even when patients don't need to travel, cells may need to be transported to a limited number of laboratories. For example, *ex vivo* gene and cell therapies require that a patient's own cells - which are the starting material for the drug product - be transported to expert manufacturing facilities specifically suited for treatment manufacturing and quality control and assurance testing, and then shipped back to a treatment centre.

³ Commission notice on the application of Articles 3, 5 and 7 of Regulation (EC) N° 141/2000 on orphan medicinal products

Ex-Vivo Gene Therapy: How does it work?



EUCOPE Position Paper on Gene & Cell Therapy - October 2018

Whilst Directive 2011/24/EU on patient s' rights in cross-border healthcare goes some way to making the movement of patients simpler, the processes at national level for ensuring the movement and funding of patients to another EU country vary widely and could well delay or prevent patients from accessing these therapies even when they are approved in the patient's home country.

EUCOPE welcomes the upcoming publication of a new guidance document clarifying the position of the European Reference Networks' governing bodies on the role that the industry will be able to play in the ERNs. We note with high interest that several ERNs are already focusing in their mission statements on reducing inequalities faced by patients seeking to access diagnostic testing and innovative treatments such as biologic therapies, immunoglobulin replacement, stem cell transplantation and gene therapy¹, and EUCOPE is ready to engage with all partners active in the ERNs to define new solutions or approaches pursuant to that objective. We look forward to working with all stakeholders to ensure the removal of any practical barriers to simplifying the movement of patients to treatment centres for gene and cell therapies.

Conclusions

Gene & cell therapies are reflecting major scientific breakthroughs but there remain barriers for their success in Europe in terms of uptake and adoption. Specific national HTA processes and new funding models are still to be developed in order to guarantee better patients access throughout Europe. The innovation of gene and cell therapies must not stop with the science. 'To provide patient access, we need innovation in the process as much as in the therapy'². It is the responsibility of all stakeholders to come together to make the adoption of gene and cell therapy work because it is the future of medicine. There is a need for thoughtfulness, but also urgency, as the next 3-5 years are likely to see a significant increase in gene and cell therapies coming to market.

¹ [ERN RITA fact sheet](#)

² Yann Le Cam, CEO Eurordis Rare Diseases Europe:
<http://www.europarl.europa.eu/cmsdata/148954/Yann%20Le%20Cam.pdf>



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About EUCOPE

The European Confederation of Pharmaceutical Entrepreneurs (EUCOPE) is Europe's principal trade body for small-to-medium sized innovative companies working in the field of pharmaceuticals and medical devices. EUCOPE represents 900+ mid-sized innovative pharma and biotech companies, directly and national trade associations. It provides a platform for owners and CEOs of pharmaceutical companies to discuss solutions to improve the quality of life for patients and to strengthen the competitiveness of the European pharmaceutical industry.

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