

Timothy Hunt - CEO, Alliance for Regenerative Medicine



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Once centred on building awareness, the Alliance for Regenerative Medicine has grown into the world's foremost advocate for the cell and gene therapy sector, bringing together more than 400 members across biotech, pharma, academia, and patient organisations. In this conversation, CEO Timothy Hunt discusses how the field is moving from promise to real-world impact, tackling barriers to patient access, advancing value-based payment models, and preparing to scale beyond rare diseases.

How has the Alliance for Regenerative Medicine evolved over the years, and what are the main priorities driving its work today?

We've come a long way since those early days when ARM's primary goal was to build awareness around cell and gene therapies. Today, we're a nonprofit association of more than 400 members headquartered in Washington, DC, spanning the full spectrum of the sector, from small and public biotech companies to global pharmaceutical groups, leading academic centres, strategic suppliers such as CDMOs and CROs, and several dozen patient organisations, most of which focus on rare diseases. What unites this diverse membership is an unwavering focus on cell therapy, gene therapy, gene editing, and, to a lesser extent, tissue repair. We often say that if someone has developed an extraordinary monoclonal antibody, that's excellent, but it's not our focus. This precision in scope allows us to maintain depth, credibility, and meaningful engagement with key stakeholders across the healthcare ecosystem.

Our priorities today centre on convening the field and providing robust data to guide its growth. A flagship example is our annual *Meeting on the Mesa* in Phoenix, Arizona, which has become the sector's defining gathering, attracting over 1,500 participants, including a significant share of C-suite executives. Beyond that, we devote considerable energy to helping healthcare systems, both in the United States and Europe, adapt to these transformative modalities. Many were built around the small-molecule drugs of previous generations, and our role is, as we sometimes say half-jokingly, to drag them – kicking and screaming if necessary – into the modern era so that payers and regulators can evolve in step with the science.

What do you see as the main barriers to patient access for advanced therapies, and how is the field working to overcome them?

Access continues to be one of the most pressing challenges, even as these therapies demonstrate extraordinary potential to transform lives. No one turns to a cell or gene therapy because they are healthy; patients take this path when every other option has failed. In gene therapy or gene editing, it is often parents of children with rare, life-limiting conditions who make that decision, while in cell therapies such as CAR-T, it is patients facing aggressive, difficult-to-treat blood cancers. It is also important to recognise that not every therapy is perfect. This first generation of products brings genuine complexity, from dosing regimens and questions around durability or safety to the still-high cost of manufacturing. Yet for thousands of patients, these therapies have been nothing short of transformative.

CAR-T therapies illustrate this balance well. For a long time, manufacturing capacity was the main obstacle, but even as production improved, a significant proportion of eligible patients – in some cases as many as 80 percent – remained unable to access treatment. Much of this stemmed from outdated FDA labels based on early trial data from nearly a decade ago. These required patients not to drive for 60 days and to stay within two hours of a major treatment centre for 28 days, limitations that simply didn't reflect current evidence or patient realities.

This summer, the FDA revisited the real-world data and made pragmatic updates, reducing both restrictions to 14 days. It was a thoughtful, data-driven move that will make a tangible difference for patients and their families. We worked closely with the FDA, alongside several CAR-T developers, to draw attention to this issue, and we were very pleased with the outcome. It's a strong example of what can be achieved when regulators and industry act on evidence to deliver more patient-centred decisions.

With one-time treatments often carrying high upfront costs, how do you see payment and reimbursement models evolving to ensure broader access and sustainability?

I'd say we've made tremendous progress over the past three years. Not too long ago, there was real concern in the field about how payers would respond to therapies costing two million dollars or more. But as more products have reached the market and as treating physicians, insurers, and Medicaid programmes have gained experience with them, the discussion has matured considerably. People are beginning to appreciate that these are not only life-threatening but also extremely costly diseases to manage over a lifetime. Severe sickle cell disease, for instance, can cost between four and six million dollars per patient in lifetime healthcare expenses. Many of these conditions are also fatal, with patients rarely living beyond 40 years of age. So when you compare that to a one-time therapy that has the potential to be durable, the economics start to make much more sense.

Importantly, CMS leadership recognises this shift. Administrator Dr Mehmet Oz has spoken about the reality that, while these therapies may seem expensive upfront, leaving such diseases untreated can cost multiples more. Independent organisations such as the Institute for Clinical and Economic Review (ICER) have also demonstrated that, even at current price levels, many of these therapies deliver exceptional value when measured against the severity and cost of the diseases they target and the life-changing impact they can have on patients.

A strong example of this evolution is the new *Cell and Gene Therapy (CGT) Access Model* launched by the Centre for Medicare & Medicaid Innovation (CMMI) for sickle cell disease. So far, 33 states, along with the District of Columbia and Puerto Rico – including major states such as California and New York – have opted in. The programme represents a major step toward value-based payment frameworks that better account for long-term outcomes. CMS will oversee its implementation, and both companies with approved sickle cell treatments have voluntarily joined. It's still early, but it could well serve as a blueprint for how healthcare systems can modernise to accommodate these transformative one-time therapies.

As gene and cell therapies move beyond ultra-rare and oncology indications, what challenges and opportunities do you see in scaling to larger patient populations?

That's an important and timely question. While we're now seeing progress in more prevalent conditions, I'd emphasise that innovation in rare and ultra-rare diseases remains vital. These are patients with very few options, and the models being developed – from nonprofits to leading academic centres – continue to be remarkable. One striking example is *Baby KJ*, an *n-of-one* case where scientists at the University of Pennsylvania, the Children's Hospital of Philadelphia, and UC Berkeley's Innovative Genomics Institute, together with life sciences partners affiliated with DanaHER, created a bespoke CRISPR-based therapy for a severe metabolic disorder known as CPS1 deficiency. That collaboration allowed them to move quickly and stabilise the child's condition, which is extraordinary.

At the same time, we're seeing encouraging signs in larger rare conditions. Most recently, promising data from uniQure's AAV5-based gene therapy *AMT-130* for Huntington's disease showed a slowdown in disease progression of about 75 percent over three years in the high-dose group. Huntington's is a devastating inherited disorder that typically emerges in people in their thirties or forties and affects around 75,000 patients across the US and Europe, a larger rare indication that sits between the ultra-rare and more common diseases.

Beyond that, innovation is expanding into conditions like wet age-related macular degeneration, diabetes, and Parkinson's disease. However, scaling into broader populations comes with real challenges. Healthcare systems need to evolve to keep pace, not only structurally but also in terms of patient and physician understanding of what these therapies entail. People naturally ask, "If this is a one-time treatment and it doesn't work as expected, can I be treated again?" These are complex, legitimate questions that require clear scientific and clinical frameworks.

There are also significant considerations around reimbursement and manufacturing. While the field has made strong progress, costs of goods remain high, and as patient populations expand, ensuring both scalability and competitiveness becomes critical. This ties closely to broader adoption of outcomes-based, value-based payment models and continued progress in manufacturing and process innovation. These are the factors that will determine how effectively the field transitions from treating small, highly specialised patient populations to addressing much larger ones in a sustainable way.

Looking ahead, what milestones or areas of progress would you like to see over the next five years to signal a deeper global embrace of advanced therapies?

I think there are several key areas where progress will be essential. The most meaningful indicator will be the number of approved products, both in the US and across Europe and key markets in Asia. If you look at Wall Street consensus data – not ours, but what the market projects – it suggests we'll move from two blockbuster therapies at the start of this year to four by 2025, and around ten by the end of the decade, with another fifteen products expected to generate over 500 million dollars annually. Those are substantial figures that demonstrate the field's continued maturation and commercial validation.

At the same time, I'd like to see steady progress in expanding into more prevalent conditions such as wet age-related macular degeneration, autoimmune diseases, Parkinson's, and diabetes. Some of these are still a few years away, but the direction is clear. Reducing the cost of goods will also be critical, not only to make these therapies more accessible but to ensure the long-term sustainability of the sector as we scale to larger patient populations.

Finally, modernising regulatory frameworks remains a top priority. Greater use of mechanisms such as the accelerated approval pathway and broader adoption of platform approaches, particularly in areas like gene editing, could significantly improve development efficiency and lower costs. Encouragingly, we're already seeing signs that regulators such as the FDA, EMA, and MHRA are moving in that direction, and that shift could be transformative for the entire field.

In closing, is there a key message you'd like to emphasise as the field continues to evolve?

In January of last year, we launched an initiative called *Cell and Gene Therapy Ethics in Society*, which aims to bring a broader set of stakeholders – from patient organisations and bioethicists to policymakers and faith leaders – into the discussion about how these technologies are developed, understood, and responsibly integrated as they move beyond rare diseases.

As these therapies begin to reach more prevalent conditions, I believe it's vital to widen the conversation well beyond the traditional stakeholders. Regulators and payers remain key, of course, but so too are patient communities – including those representing larger populations – as well as ethical, religious, and policy leaders, whether on Capitol Hill or in the European Parliament. Building this broader understanding will help ensure that society not only recognises the transformative potential of these treatments but also trusts that they are being advanced in an ethically sound, transparent, and rigorously regulated way. That kind of engagement will be fundamental to the responsible and sustainable growth of this field.

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