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Democratizing medicine:

How *in vivo* therapeutics are shifting drug development

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The paradigm of how we discover medicines is changing. From painstaking search to targeted code.

The ways we deliver them to patients are changing, too. Together, these developments have potentially seismic implications—for drug developers, clinicians, and patients. What's driving these changes? There are many factors, but it's possible to point to a few key enablers: innovations in drug development, new solutions for drug delivery, and easier access to the tools needed for both.

The earliest medicines were stumbled upon in nature. A classic example is willow bark, used as a pain reliever in ancient times. Later, the small molecule responsible for willow bark's therapeutic effects was isolated, and aspirin was born. For much of human history, this is how drug development proceeded:

"I found some new plant. Let's see what we can isolate from this plant and [whether] that has any therapeutic effect," says Tim Leaver, Senior Director of Nanomedicine, Lipids, and Services at Cytiva.

We've come a long way since then.

Computational modeling and AI tools accomplish screening tasks in a fraction of the time needed for traditional methods, while advances in gene sequencing and molecular analysis have blurred the lines between discovery and optimization. But what we're seeing now is a more dramatic directional shift: whereas in the past we've often known *that* a drug works before learning *how*, today we're increasingly able to reverse-engineer therapies based on how we want them to work. In other words, the route from discovery to mechanistic understanding is now a two-way street.

That street is how we arrived at CAR T cell therapy, for example, where a patient's immune cells are modified to express chimeric antigen receptor (CAR), a protein that directs the patient's immune cells to fight the cancer from within. The approach is not only much more targeted than traditional methods like chemotherapy and radiation but has also provided durable remission for many patients (1).

But producing this therapy is resource-intensive (2), both because of the viral vectors used to deliver it and because the cells are modified outside the patient's body.

Tim Leaver
Senior Director of Nanomedicine, Lipids, and Services at Cytiva

In CAR T [immune cell] therapies, you're taking cells out of the person, you're modifying them *ex vivo*—so outside of the living being—and putting them back.

Doing this safely requires dedicated equipment, space, and time—it can take several weeks to grow enough cells to transfer back to the patient, who will have been in the hospital receiving preparative treatment in the meantime. What if there were a way to administer a treatment like this *in vivo*—using the patient's body to produce biologics or cell therapies without removing the patient's cells?

Growth in *in vivo* cell therapy programs

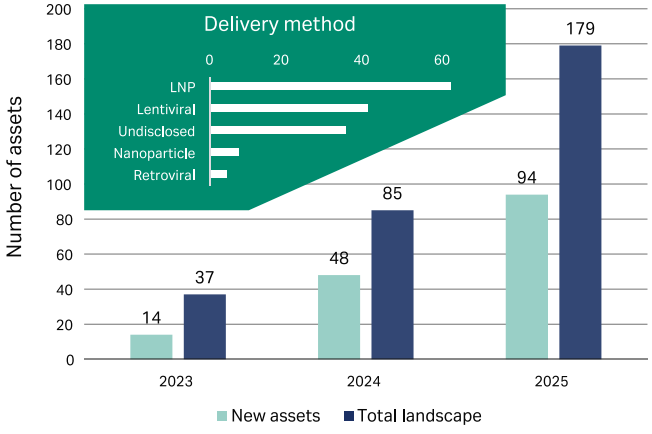

Fig 1. *In vivo* cell therapies are gaining traction, with a year-over-year doubling of assets in 2025. Source: Hanson Wade. Cell Therapy Beacon. Webinar: Cell therapy in 2026: where the science, strategy, and capital are moving. Published 19 February 2026. Accessed 13 April 2026.

This is where the second key enabler comes in: emergent drug delivery systems that can be used to deliver therapeutic molecules, such as mRNA or CRISPR gene editing systems, to cells. These molecules are most often delivered by viral vector or by lipid nanoparticle (LNP), tiny fat particles that can protect payloads from degradation in the cell. This technology has already delivered on a grand scale—without LNPs we would not have the Pfizer-BioNTech or Moderna Covid vaccines. Moreover, the number of *in vivo* cell therapy assets doubled in 2025 (Fig 1), while recent pre-clinical and early clinical data demonstrate in vivo generation of therapeutic functions (like CAR expression) from targeted LNPs.

LNPs are less expensive to make than the viral vectors used most often for cell and gene therapies, but designing and manufacturing them requires highly specialized expertise across a range of fields including fluid dynamics and medicinal chemistry. And this is where we find the third key enabler: active efforts to democratize the technology so that it's more accessible to more drug developers.

Kamalinder K Singh
Professor of Pharmaceutical Technology and Drug Delivery and Research Lead in Nanomedicine and Innovative Technologies at the University of Lancashire

This is a special time in the history of genomic medicines. This could create a plausible path to systemic change: from bespoke, inpatient, weeks-long processes to more standardized, outpatient-friendly, rapidly iterated genetic medicines.

1. The drug discovery crisis

The business model of drug development means new medicines need to be sold and bought at a very large scale to make development worthwhile. That, along with a steady increase in the average cost of R&D per approved drug (Fig 2), has concentrated discovery into the hands of relatively few biopharma companies, and production into even fewer sites, creating the potential for bottlenecks and systemic fragility. It also means that rare conditions often fall by the wayside: without a large potential customer base, there is little financial incentive to develop drugs for rare diseases, a deeply painful situation for patients and their families.

Many conditions, meanwhile, resist the traditional discovery process. These include most cancers, especially solid tumor cancers; genetic disorders; and many degenerative conditions such as those associated with aging. These complex diseases frequently lack clearly druggable targets or involve biological mechanisms that are not easily addressed through conventional methods.

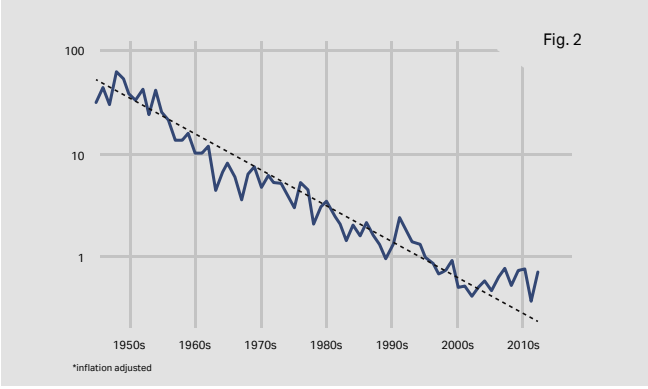

Fig 2. The number of new drugs approved by the US FDA per billion USD (inflation-adjusted) spent on R&D. Source: Scannell JW, Blanckley A, Boldon H, Warrington B. Diagnosing the decline in pharmaceutical R&D efficiency. Nat Rev Drug Discov. 2012;11(3):191-200. 2012 Mar 1. doi:10.1038/nrd3681

What if, instead of hunting for a solution, we were to shift to designing one?

Gene editing is one way to rewrite the body's "codes", and it isn't brand new. Indeed, a lot has been promised in the realm of cell and gene therapy over the last decades, and the barriers to large-scale implementation remain stubbornly high. Impressive advances have been counterbalanced by punitive costs, and progress has moved more slowly than hoped.

But that could be about to change.

Ongoing need is one reason. Another is the particular combination of technologies available now, converging in new ways. And a third is the vision driving efforts to open opportunities to a wider base of scientists.

This report is concerned with the potential these combined changes have to redistribute the power of innovation—opening new scientific pathways and new production pathways at once. This shift, which we'll characterize as democratization, is especially exciting in the context of *in vivo* medicine.

2. From discovery to engineered design

So what does this shift look like in practice? What's driving it? And, crucially, what's different this time around? Why now? One key step is the ability to design drugs that can change how the body's cells function at the molecular level.

One way to think of it is like software. A software developer writes lines of code, telling a computer what to do. In much the same way, drug developers are now able to use "code" to give the body instructions. This is possible in a range of applications.

Tim Leaver
Senior Director of Nanomedicine, Lipids, and Services at Cytiva

With these types of medicines, you can switch one gene off, or modify another gene. So we're no longer screening and hoping. We're now actually just designing the effect we want. That's that switch.

Until recently, however, most of these approaches have involved engineering cells outside the body, then reinfusing them. The next step: *in vivo* medicine. As Tim Leaver says,

***In vivo* is when those genetic steps are taking place inside the body rather than outside.**

3. The path towards democratization

Once we have a way to produce that effect inside the body, we have the last leap to *in vivo* therapeutics.

Rewriting DNA, though, requires getting the molecular tools into the cell without harming the body. You need a carrier that is safe for patients and can protect vulnerable molecules when they're being delivered into the body. LNPs are one such carrier.

LNP solutions aren't simple to manufacture, however. There are many different kinds, with some better suited to the delivery of particular molecules than others. And there are various constraints and criteria, just as there are when creating the software for any complex system. These include:

- Reproducibility (How do we know it will work in the body the same way it works *ex vivo*?)
- Scalability (is it manufacturable outside a lab?)
- Stability for manufacturing these highly advanced delivery platforms
- Regulatory compliance, and the potential evolution of regulation
- User safety

Shell Ip has a lot of experience in these arenas as client learning and scientific content manager at Cytiva, where he runs training programs for customers using Cytiva products to formulate and test LNPs. Ip emphasizes the importance of making the technology—both the LNP solutions themselves, and the lab-based instruments that make them—available to other scientists.

Dr. Shell Ip
Client Learning & Scientific Content Manager at Cytiva

Training has always been essential to our clients' success: making sure that people understand the technologies and how they can be applied and best leveraged.

Often this includes visiting labs that have Cytiva machines systems (such as a NanoAssemblr™ nanoparticle assembly system) and demonstrating how to use them. Ip explains that Cytiva is democratizing the technology in four major ways:

1. Engineering systems that allow people to harness LNP mixing technology without advanced training in fluid dynamics.

2. Making the company's lipid library accessible via lipid kits featuring formulations pre-designed and tested for cell therapy and other applications, or via custom formulation services, and licensing for clinical use.

3. Offering formulation development, analytical development, process development, and/or manufacturing services.

4. Providing applications training and support to adopt these technologies.

Cytiva has also created and verified a library of different LNPs that drug developers can use to test different packaging systems for therapeutic molecules. Removing the need to design the delivery system means the end user doesn't have to understand the "niche bits" like fluid dynamics of how the mixing works, Ip says.

"Like when you use a computer…You don't need to know how it works, you just need to interact with it in a human way that makes sense to you. That's how we democratize that technology."

Singh says she used Cytiva's off-the-shelf lipids as a stepping stone to develop her own LNP solutions for work on drugs that cross the blood-brain barrier.

Singh is no stranger to LNP technology: she has been working with earlier-generation LNPs in her lab for years. Even so, she says, "when we started working on the ionizable LNPs [used for nucleic acid delivery], we started with Cytiva's GenVoy-ILM lipid mix."

With the mix, "it was easier to start and learn about microfluidic technology and process parameters before we moved to our own tailored LNPs for more specific applications and delivery across various biological barriers," Singh explains.

Tim Leaver
Senior Director of Nanomedicine, Lipids, and Services at Cytiva

The hope is that with tools like this, it could eventually be possible for "every scientist to be a genomic medicine developer."

Dan Peer, Director of the Laboratory of Precision Nanoemedicine at Tel Aviv University, is cautiously optimistic. Having started work in the fields of DNA vaccines and liposomes at least 20 years ago—and encountering plenty of resistance—he's seen both how far things have come, and how long they can take. What does he think about the possibility of a future where, for example, a clinician could take a biopsy of a cancer, sequence its DNA in situ, encode a therapy, and deliver it to a patient using an off-the-shelf LNP?

Scaling up in a way that includes unique payloads is one issue, Peer says. Ensuring the purity of any product created for human use is another barrier, as is making the delicate molecules robust enough for effective use. Peer also cites regulatory hurdles (covered more extensively below).

At the same time, Peer (who has worked closely with Cytiva scientists in the past) is excited about the potential for real change in 2026:

Professor Dan Peer
Precision Nanomedicine Laboratory Director, Tel Aviv University

[It's] a very exciting year because we may start seeing more [regulatory] approval … but also more getting into the clinic in cell therapy *in vivo*.

4. Making way for change

Already, clinical results for LNPs in *in vivo* therapy are showing promise. In results published in the New England Journal of Medicine in October 2025, a team in China used LNPs and mRNA to effect in vivo generation of CAR T cells in five people with lupus (3). The authors noted "reduced disease activity, and no major toxic effects"—a promising initial result that underscores the real-world potential of LNP technology.

Peer predicts still more breakthroughs in the near future. "We're going to see more data on cancer," especially blood cancers, and other applications, including therapeutic proteins (such as antibodies or 'intrabodies') in different diseases, he says.

Professor Dan Peer
Precision Nanomedicine Laboratory
Director, Tel Aviv University

So the fantasy of making cells factories that produce therapeutic proteins will become, probably, less of a fantasy. And that's very exciting.

These innovations are also surfacing questions about how key players will adapt. Regulation is a concern shared by all the experts interviewed for this article. Right now, regulators license drugs to treat specific diseases. A treatment might be approved for use with pancreatic cancer, for example, and separately for bladder cancer.

It's not clear how regulators would handle cases where a generic delivery vehicle can be loaded interchangeably with different types of personalized therapeutic molecules.

But the FDA has signaled openness to such medicines. In late 2025, the agency said it would be open to the exploration of individualized pathways where randomized clinical trials aren't feasible (4), but specifics remain under development. The question can be framed as:

Tim Leaver
Senior Director of Nanomedicine, Lipids, and Services at Cytiva

Can we accelerate the development of new medicines by not starting from scratch every time? Let's see this as a platform rather than discrete independent therapies….a different code inside of the same physical thing.

If all the pieces are in place, Leaver continues, "the logical extreme of this is actually personalized medicine." It could even extend to breakthroughs like personalized cancer vaccines, or treatments for ultra-rare diseases, where "an entire clinical trial process just doesn't make sense."

Such a shift would be a radical one, requiring regulatory flexibility and a certain amount of rethinking, not just in how patients are treated, but in how drugs are manufactured and potentially in how insurers assess costs.

5. The price of progress

The status quo is expensive in many cases: ex vivo gene therapy is costly not only because of the labor and equipment needed to produce it, but because patients in some cases spend many weeks in the hospital, preparing for and then receiving the therapies. Traditional cancer treatments like chemotherapy and radiotherapy are more widely available, but they also have deep associated costs, financially and in their impacts on the health (and productivity, and happiness) of recipients and their loved ones.

Democratized medicine with more *in vivo* development wouldn't eliminate costs, but could it move them? For patients, the treatment process would be simplified, even if it takes longer for the price to come down. Moreover, there's reason to be optimistic about costs as well.

The modular nature of LNPs lends itself to a multidrug manufacturing model that could achieve economies of scope rather than scale. This would be analogous to the establishment of "foundries" in the computer chip manufacturing industry that precipitated rapid innovation and lower costs in that field (5). And in similar ways, an LNP foundry allows the capital costs to be shared among numerous smaller drug developers who all manufacture their LNP drugs in the same facility. Coupled with a platform-based regulatory framework, this could open up the bottlenecks drug developers face in translating their work to the clinic.

The last decade has shown, more clearly than ever, that when the imperative is strong enough and the key players are on board, the world of drug development can marshal its forces to radically change things for the better.

We don't have to wait for another pandemic. We can start now.

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