Type 1 diabetes, heart failure, HIV - all examples of diseases that have transitioned from fatal conditions to chronic diseases, thanks to the progress in modern medicine. Indeed, for the last several decades, this has been the primary model for breakthrough innovation; medications that enable the long-term management of previously life-shortening diseases, which are affordable to healthcare systems because the costs are spread out over a lifetime.

But we now have medical breakthroughs that completely buck that trend. Cell and gene therapies in particular are heralding remarkable curative medicines, which are given only once, or over the short term. These include Kymriah, the pioneering CAR-T cell therapy from Novartis, which demonstrated a remarkable 83% remission rate in children and young adults with B-cell precursor acute lymphoblastic leukaemia. Hepatitis C virus treatments like Maviret from AbbVie, which cures people with hard-to-treat genotype 3 after an 8 to 12-week course. And Spinraza from Biogen, the first ever disease-modifying treatment for spinal muscular atrophy (SMA), which increases the body’s ability to produce SMN protein critical to the health of motor neurons.

The advent of these ‘regenerative medicines’ - often highly tailored to the patient’s specific genetic make-up - is a genuine revolution in healthcare. And it is a revolution that is happening right now; there are many such therapies in company pipelines, several of which are due for marketing authorisation decisions soon.

So, the urgent question is this: how do we price, pay for and otherwise incentivise, groundbreaking treatments that are given once, or over a short-term period, and completely cure previously incurable diseases?

Cost-effective but not affordable
It is a question that the healthcare industry is trying to find an answer to. In August 2017, Novartis announced that Kymriah would be $475,000 per treatment, which caused a lot of headlines, but was actually lower than many analysts were expecting; a cost-effectiveness assessment found a price of $600,000-750,000 for Kymriah would have been warranted.

Indeed, the cost-effectiveness evidence for many innovative medicines is compelling. But this is the paradox facing many innovative curative therapies - they may be cost-effective, but that doesn’t mean they’re affordable.

And the lack of affordability - as well as the bad press surrounding these high-ticket items, which inevitably appeared for the likes of Kymriah and Spinraza, and tends to confl ate with general negativity around pharma pricing - could mean a backward step for innovation. Healthcare systems say they cannot afford these medicines and therefore either do not pay for them, or do so only for subpopulations, or they implement tenders to force significant price reductions very early in the life cycle. Subsequently there will be no incentive for the industry to discover and develop new curative therapies.

Current healthcare systems raise several barriers
The situation is made more challenging by the range of different healthcare systems and reimbursement schemes across Europe, from social insurance systems in countries such as Germany and France, to so-called ‘single payer’ systems in the UK and Scandinavia.

Each of the different models are variations of a fee-for-service reimbursement model, which have evolved to meet the needs of chronic illnesses. Healthcare systems are
and hospitalisation. In theory, it should be desirable to get eligible patients on curative treatments very early in order to realise the full public health benefit. However, there is the question of risk. We still do not really know the long-term effects of most of these medicines. We won’t know for many years if the clinical and economic impact has been as significant as hoped. Then there is a disconnect between paying a large sum for a one-off treatment, and the savings to the healthcare system that may result over decades. Systems are simply not set up in this way.

Finding a system that accurately accounts for the clinical impact, quality of life impact, healthcare system cost savings and societal benefits of innovative curative medicines will be key.

However, even if we do manage to fairly establish the value (and therefore the cost) of these innovations, healthcare systems can still say they are unaffordable.

So, where do we go from here?

Incremental or systemic solutions?

All stakeholders need to engage with this topic and actively find solutions. The stakes are too high for this not to happen. The risk we face from antimicrobial resistance and the current lack of new antibiotics tells us all we need to know about the consequences when there is no incentive for the biopharmaceutical industry.

We know that initial steps made to address these challenges have often come up against established reimbursement pathways, structural barriers and misaligned incentives as well as lack of data and follow-up data-tracking mechanisms. What can we do that is different?

Broadly speaking, the industry has two choices; either incremental solutions, implemented in the confines of the existing health system and reimbursement environment, or sweeping systemic solutions, which reimagines the systems to fit the scope of science’s ambition.

The question is, are incremental improvements sustainable in the long-term, given the ever-increasing costs and demands on the system from chronic diseases?

Incremental changes may work in the short-term

Incremental changes include solutions such as outcome-based/ value-based reimbursement, annuity models and risk-sharing agreements between healthcare systems and biopharmaceutical companies.

This is the route the NHS in the UK has taken with innovative hepatitis C treatments; it will only pay for the medication if a patient is successfully cured, otherwise the manufacturer foots the cost.

‘The risk we face from antimicrobial resistance and the current lack of new antibiotics tells us all we need to know about the consequences’

However, the level of uptake of these types of agreements remains low, and in any case, payers will still question the affordability, the definition of cure, and whether the proposed prices reflect the true ‘value’ of these innovative curative therapies.

Potential systemic solutions

To match the innovation in healthcare, we urgently need to innovate our approaches, systems, incentives and financing in order to embrace the curative treatment paradigm.

What could this look like?

Potential solutions could include innovative payment models, such as evolving funding pathways, innovative pricing that shifts from the product to the solution, and splitting the burden of healthcare costs across several stakeholders (eg public payers, corporations and individual patients). Are there lessons to be learnt from other industries, such as from the financing/mortgage industry, where high upfront costs are spread over a longer period of time when benefits are proven and from the music industry, where the ‘Spotify approach’ of licensing rather than purchasing could be applied to drugs. Another option is to split the management of chronic diseases that could be managed by private stakeholders and establish dedicated budgets and units with independent funding for diseases that have curative treatments.

There is also the potential to think more creatively beyond just changing the pricing models. Greater incentives could be provided through things like market exclusivity, guaranteed sales volumes or even a bounty or prize to the company that solves and cures particular diseases.

The debate continues

This is an exciting time in terms of healthcare innovation. How we ensure that life-saving medicines are embraced by healthcare systems, and therefore ensure that companies are incentivised to continue to develop them, is one of the most pressing questions facing the industry today. The stakes are high and the window of opportunity may be relatively small.

The issues are complex, the barriers are many and the potential solutions are fascinating. We believe we need to have a ‘blue sky’ approach to this; that the innovation of thought needs to match the innovation in the labs. Where could this take us? The possibilities are many, but we hope the end result is the same: a system that rewards and encourages life-saving, curative innovations that could reshape healthcare.

What is your opinion? Should we take an incremental change or systemic solution approach? What is realistic? What would incentivise pharma?

Get involved in the debate on www.pmlive.com or in the PME LinkedIn group.