Advances in Regenerative Medicine (RM) And Gene Therapy:

The Key To Unlocking A More Personalized Healthcare Journey

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Executive Summary

In December 1967, when Dr Christiaan Barnard and a team of surgeons performed the first human heart-to-heart transplant, the medical landmark made front-page news around the world. Today, advances in regenerative medicine (RM) and gene therapy (see box 1) are so routine that they are typically only covered by specialist scientific and industry journals.

However, governments, healthcare providers (HCPs), and medical professionals cannot afford to ignore the latest developments in RM and gene therapy because they are one of the most promising pathways to ensuring better lifelong health and quality of life for all citizens. Treatments for genetic disorders, tissue regeneration techniques, and organ replacements are just some of the areas where RM and gene therapy are already helping to shift healthcare in developed countries from symptomatic to curative treatments that are less costly in the long term. Prevention is the ultimate objective, aiming to minimize healthcare costs, and precision medicine – especially with RM and gene therapy playing an increasingly important role – can be leveraged in combating rare diseases and non-communicable diseases (NCDs) such as cancers.

Two accelerating trends mean that governments must act now to maximize the potential benefits of RM and gene therapy before it is too late. First, healthcare budgets worldwide are spiraling out of control in both developed and developing countries. The reasons are complex and interconnected. They include increased life expectancy, the rise of non-communicable and rare diseases as a global health burden, and the impact of COVID-19. RM and gene therapy are relevant to all these challenges, providing an increasing number of treatments that are more cost-effective and deliver better long-term outcomes for patients than conventional medicine.

Second, the remarkable advances in RM and gene therapy are connected to the emergence of digital scientific and medical technologies and processes such as gene editing and AI-enabled surgical devices. These technologies are evolving at such speed that governments cannot afford to sit on the sidelines while other countries secure a head start in investing in RM and gene therapy, from the laboratory to the hospital bedside. The policy-making and budgetary dilemma, which we examine in this report, is how to balance the high initial R&D costs of RM and gene therapy against the considerable long-term cost benefits.

We make the following key recommendations for governments, drawing on academic and clinical RM and gene therapy policy research and examples of best practice from around the world:

01 **Make targeted, well-researched investments in RM and gene therapy research to maximize future healthcare savings.**

02 **Promote collaboration between universities, pharma and life sciences (PLS) companies, healthcare providers, and regulators to maximize opportunities for RM and gene therapy.**

03 **Ensure healthcare providers have sufficient digital technology and expertise to deliver RM and gene therapy.**

04 **Recruit clinical and PLS industry specialists to advise and inform health ministries on the latest advances in RM and gene therapy.**

Box 1: Regenerative Medicine And Gene Therapy – Related, Innovative Healthcare Approaches

Regenerative Medicine: Replaces or regenerates cells, tissues, or organs to restore or establish their normal function.[1]

Gene Therapy: Therapeutic application of cells or genetic material to modify a patient's cells.[2]

DNA: Deoxyribonucleic acid, the hereditary material in humans and almost all other organisms.[3]

Genome: Entire set of DNA instructions found in a cell.

Whole genome sequencing (WGS): Laboratory procedure that determines the order of bases in the genome of an organism in one process.[4]

Gene editing: Method for making specific changes to the DNA of a cell or organism.[5]

Stem cells: Unspecialized human body cells that can differentiate into any cell of an organism and have the ability to self-renew.[6]

CAR-T cell therapy: Chimeric antigen receptor (CAR)-T cell therapy that uses the body's immune system to help fight cancer.[7]

CRISPR/Cas9: A tool to induce precise modifications in the genome of an organism.[8]

Chapter 1

The Rise Of RM And Gene Therapy In A Deepening Global Healthcare Crisis

In recent decades, the world has confronted a series of interconnected health challenges that now impose an unsustainable strain on healthcare budgets in both developed and developing countries. All these challenges have been compounded by the financial, economic, and public health impact of COVID-19.

The Cost Of Healthcare Is Spiraling

In 2017, total global spending on health reached $7.8 trillion, according to data compiled by the World Health Organization (WHO).[9] By 2020, the figure had leapt to $9 trillion as governments raced to contain the first COVID-19 wave, spending vast sums on a host of emergency items, from personal protective equipment (PPE) for medical staff to funding for vaccine R&D.[10] In total, the WHO estimates that worldwide spending on health in 2020 amounted to 10.8% of gross global GDP.

Alarmingly, successful COVID-19 vaccine rollouts and the emergence of less lethal strains of the virus are unlikely to ease the world's increasingly severe health burden. On the contrary, the worst global inflationary crisis since the 1970s has highlighted how all the major health challenges that existed before COVID-19 have become even more daunting in the pandemic's aftermath. For example, annual global spending on cancer will almost double between 2022 and 2027 to $375 billion, according to IQVIA, a UK-based health data research company.[11]

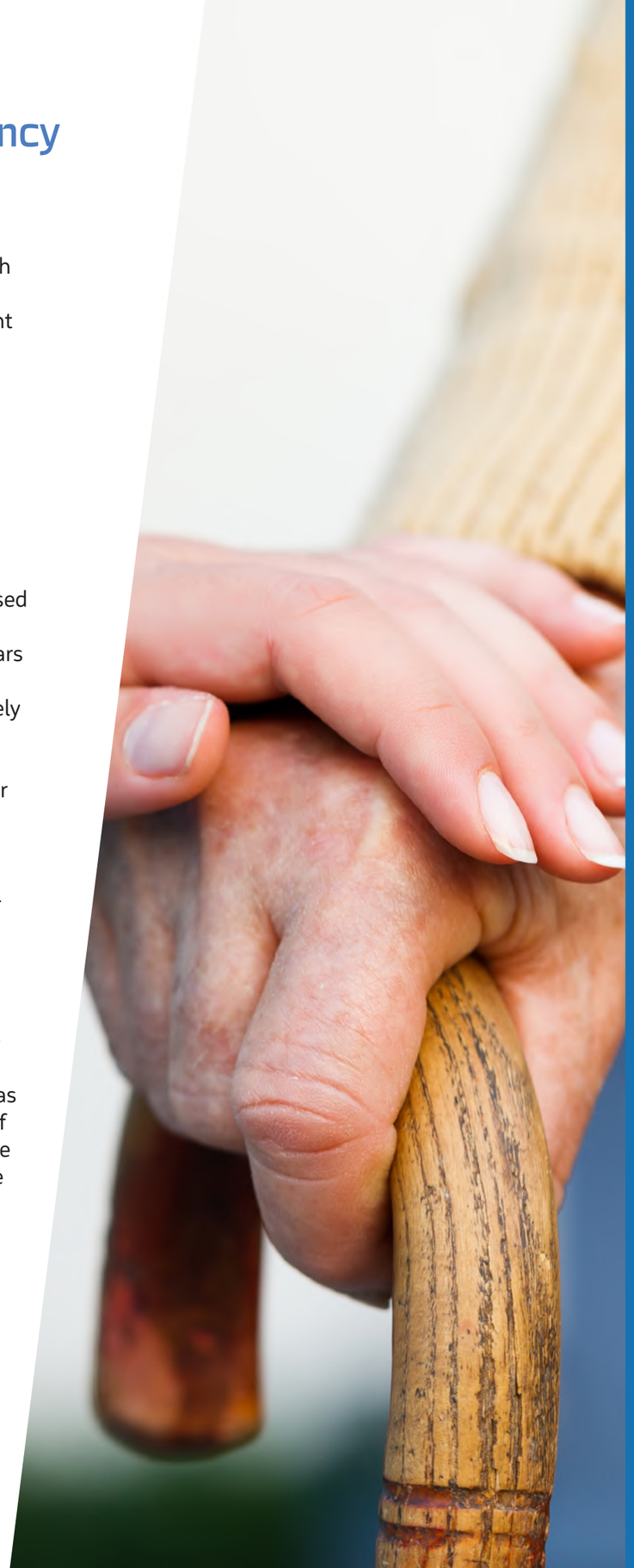
Chapter 1

Increasing Life Expectancy

The fact that people are living longer than at any time in human history ought to be cause for celebration. Between 2000 and 2019, average global life expectancy at birth increased from 66.8 years to 73.4 years, based on mortality rates remaining constant through an individual's lifetime.[12]

The full picture is more ambivalent once a person's quality of life is considered. The key metric is healthy life expectancy (HALE), meaning the average number of years through a lifetime that someone can expect to remain in 'full health,' without experiencing a debilitating disability or serious illness. Average global HALE increased by 5.4 years to 63 years between 2000 and 2019, compared with an increase of 6.6 years in average life expectancy.[13] Based on the trend of the past two decades, it seems likely that the next triennial WHO mortality and global health estimates in 2025 will show that the HALE gap has widened even further due to COVID-19.

This gap is to some extent a natural consequence of aging societies, since older people are more prone to becoming ill. For example, per capita healthcare costs for US adults over the age of 65 rose from $2,206 in 1978 to $12,411 in 2018, well ahead of inflation during the same 40-year period.[14] However, rising healthcare costs for older people globally are driven by other factors as well – notably, the increasing importance of noncommunicable diseases (NCDs) and rare diseases as health burdens affecting all age groups.



Chapter 1

Case Study 1: Japan
How A Faster Regulatory Pathway For RM And Gene Therapy Could Improve Healthcare For Older People

The UN forecasts that by 2050, the proportion of the world's population older than 65 will have increased from less than 10% today to around 17%.[15] New gene therapies and RM treatments could potentially provide more effective methods for treating the symptoms of aging and age-related diseases, thereby alleviating the increasing health burden represented by an older population. However, as a 2022 study of gene therapies for aging patients noted, the R&D pathway is so tortuous and risky that there is currently a dearth of effective RM and gene therapy treatments for age-induced conditions.[16,17]

Over the past decade, Japan has overhauled regulations governing RM treatments and gene therapies to facilitate faster, more effective R&D. Japan is arguably the world's oldest society, with almost 30% of the population aged 65 or older. It is also a global leader in areas such as stem cell research and CRISPR gene editing, techniques critical to developing more effective and less costly treatments for conditions and illnesses that disproportionately affect older people such as dementia, heart failure, and chronic kidney disease.

Japan's drive to streamline its RM and gene therapy regulations began in 2012 when Shinya Yamanaka, based at Kyoto University, shared the Nobel Prize in Physiology or Medicine for establishing that mature cells can be reprogrammed to become 'pluripotent,' with the ability to self-renew and form all three of the basic body layers – ectoderm, endoderm and mesoderm.[18] This breakthrough was seen by the government as an opportunity to strengthen Japan's position as an international RM and gene therapy hub.

In 2014, Japan updated its legislation to create a fast-track route for companies to gain regulatory approval for new RM treatments and cell and gene therapies. The Pharmaceutical and Medical Devices Act[19] allows developers to sell new products as soon as clinical trials have proved that they are safe and have the potential to benefit patients. The company then has up to seven years to conduct more trials to confirm the claimed benefits and gain full regulatory approval.

As with all pharma and medical regulations, there is still plenty of small print to navigate. Yet the act could help accelerate Japan's ability to provide older people with life-enhancing RM treatments and gene therapies, which can also reduce the growing financial burden of geriatric healthcare.

Noncommunicable Diseases (NCDs)

From 2000 to 2019, the proportion of deaths worldwide caused by NCDs such as cardiovascular illnesses, cancers, diabetes, and Alzheimer's rose from 61% (31 million people) to 74% (41 million).[20] While this upward trend was steepened by a reduction in some other causes of death such as infant mortality, nonetheless it still represents a dramatic increase in the contribution of NCDs to the global health burden. A key measure of this impact is the growing proportion of global disability-adjusted life years (DALYs) – the number of years lost to disability or ill health – due to NCDs, which increased from 47% to 63%.

This trend is difficult to reverse because the factors driving the spread of NCDS are so diverse, ranging from unhealthy lifestyles to rapid urbanization. Globally, NCDs are now estimated to kill around 15 million people prematurely (before the age of 70).[21] Furthermore, the damage wreaked on healthcare budgets by NCDs is most acute in poorer countries, not only due to limited access to healthcare services and financial barriers, but also frequently diagnostic complexities. For example, a study in 2019 of NCDs in sub-Saharan Africa found that these were major barriers to the region achieving the first three UN Sustainable Development Goals: ending poverty, ending hunger, and ensuring healthy lives.[22]

Rare Diseases

Rare diseases that affect a limited number of individuals have always existed. The difference today is the ability of researchers to identify and classify a rapidly growing number of these diseases, many of which have a genetic cause, and of doctors to treat such conditions. Currently there are around 5,500 known rare diseases affecting around 400 million people globally, based on data compiled by the WHO (other estimates go as high as 8,000).[23] Many are complex genetic disorders such as Fabry Disease, where the body lacks the enzymes to break down fats, and Krabbe Disease, where nerves lose their protective myelin sheath.

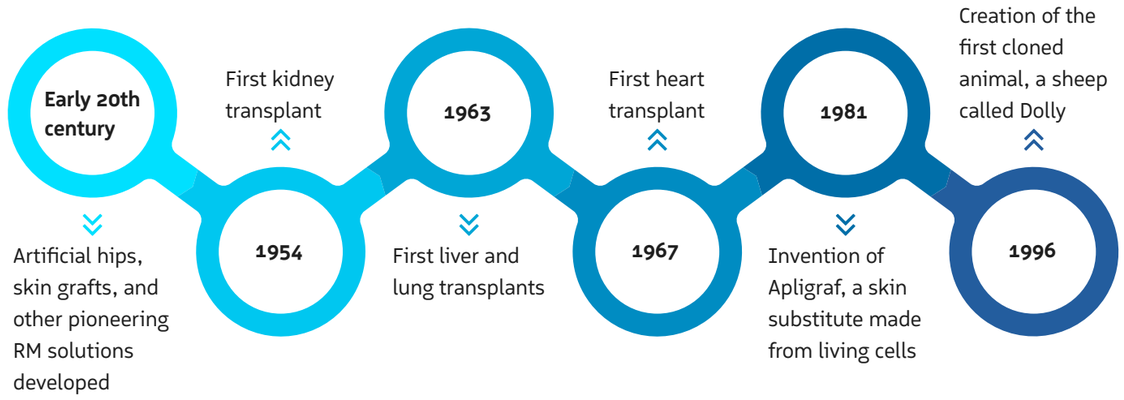
By their nature, rare diseases such as neuromuscular and blood disorders are difficult and expensive to identify, research, and treat. Worldwide, they impose an economic burden that is 10 times greater per patient per year than mass diseases, according to a study in 2022 for the Italy-based Chiesi Global Rare Diseases Pharma Company.[24] The financial impact of rare diseases on healthcare budgets is, of course, offset by their rarity. Even so, the same study estimated that in the US alone, the cost of treating 8.4 million patients whose conditions covered 373 rare diseases was around $2.2 trillion per year.

RM And Gene Therapy: Essential Tools For Addressing The Global Health Burden

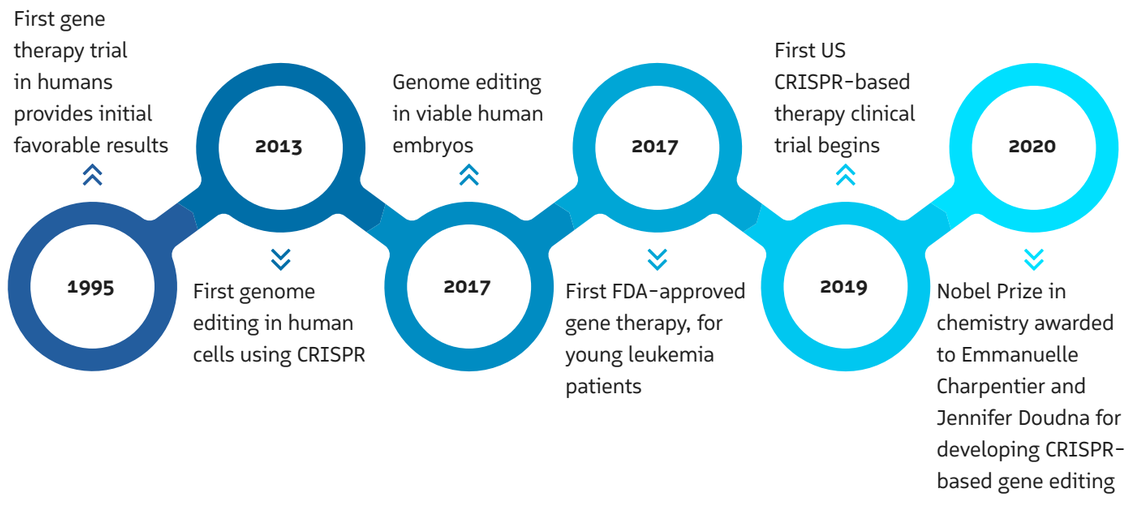
To be clear, RM – a term first coined in 1999 by Harvard Medical School professor William Haseltine – and gene therapy cannot solve all these interlocking healthcare challenges on their own. Yet as we show in the next section, RM and gene therapy are increasingly important in developing innovative, effective approaches to the treatment of NCDs and rare diseases. At the same time, they are fast emerging as indispensable tools in other healthcare fields that need to transition toward more personalized, cost-effective solutions.

BOX 2: Regenerative Medicine And Gene Therapy: The Story So Far

01 Regenerative Medicine (RM) – Key Early Landmarks



02 Gene Therapy – Key Recent Advances[25]



Chapter 2

How Regenerative Medicine And Gene Therapy Can Ease The World's Health Burden

The need for more innovative, personalized healthcare approaches is the starting point for any coherent strategy to address the challenges described above. Consider the example of genetic diseases. Conventional medicine cannot provide a cure, precisely because these diseases are tied to the patient's unique genetic profile. Instead, as with other types of disease that cannot be cured with medication or surgery, the only imperfect solution has been to offer long-term, expensive treatments that can improve the patient's quality of life. By contrast, 'autologous' gene therapies use the patient's cells to develop a bespoke product that in the best scenario can cure the disease. They are "arguably one of the most personalized forms of medicine," an academic review noted in 2016.[26]

Innovation and personalization feature in almost every branch of healthcare where RM and gene therapy play a role. For instance, a patient suffering from burn wounds can benefit from bioactive glass-based fibrous wound dressings that aid skin repair. The patient convalesces more quickly, freeing up their hospital bed and medical staff, which in turn reduces pressure on healthcare budgets.[27] In the long term, the same, now recovered patient will achieve a better Quality Adjusted Life Year (QALY) score, an international metric that measures how much time a patient can continue to enjoy life after a treatment or intervention.

This is just one illustration of how RM treatments and gene therapies can benefit both the patient and healthcare budgets in the long term. Yet this positive outcome is obscured by the fact that RM and gene therapy approaches are extremely expensive in the short term, because they are so innovative, and therefore have costly and lengthy R&D, regulatory and manufacturing pipelines. In addition, gene therapies and gene editing processes target rare diseases and are therefore difficult to commercialize, because of the relatively small number of patients suffering from a particular illness.

In the US, the world's leading RM and gene therapy market, the term 'financial toxicity' has been coined by policy-makers and the PLS industry to describe RM and gene therapy drugs that are so expensive to research, develop, test, and gain regulatory compliance that they may never reach the patient, even though their benefits are clinically proven.

Drugs that surmount these hurdles are astoundingly high-priced. In May 2023, for instance, Fierce Pharma – a PLS industry research company – reported that the most expensive drug on the US market was Hemgenix, a single-dose gene therapy for hemophilia B that cost $3.5 million at the time.[28] CSL Behring and uniQure, the PLS companies that produce Hemgenix, contend that the drug can deliver significant savings for healthcare systems because patients with hemophilia B currently receive conventional repeat blood infusions, which are cumulatively very costly over a lifetime. But that still leaves the short-term problem for patients, hospitals, insurers, and other healthcare payers of how to afford the $3.5 million.

RM And Gene Therapy Cost-Benefit Analysis – Harder Than Conventional Healthcare Budget Planning

The task for healthcare policy-makers is to make the right judgments about which areas of RM and gene and cell therapies are worth significant investment, bearing in mind their steep upfront costs and long path to market. One reason why these calculations are so difficult to make is because they are not big-picture decisions, given that the global RM and gene therapy industry is dominated by specialized SMEs and start-ups, which are typically focused on single diseases and conditions. In addition, a correct investment decision for one country may not be right for another with different healthcare priorities. It makes sense, for example, for African and Caribbean countries to invest in gene therapies for patients with sickle cell disease (with support from international aid agencies), because of its higher prevalence among their populations. The same level of investment per person in North American and European countries would probably not be money well spent.

These challenges can only be surmounted by cost-benefit analysis that is informed by the latest insights and information about advances in RM and gene therapy. This is why one of our key recommendations is that governments should recruit clinical and PLS industry specialists to advise and inform health ministries. In addition, governments should systematically collect the necessary data to develop disease registries that provide a detailed overview of where to target resources for RM and gene therapy investment.

This report is not a comprehensive guide to all the latest developments. Such a task is pointless, for the reasons stated above. Instead, we set out below examples of the types of decisions that governments will need to make when integrating RM and gene therapy into healthcare policies and programs.

Innovation

Many innovations in RM and gene therapy have roots that go back more than a decade. For example, twenty years ago, researchers were using mice to investigate whether the infusion of human umbilical-cord blood cells in stroke victims could aid recovery by increasing their blood-vessel production.[29] Today, research on stem cell-based therapies for stroke patients is at the pre-clinical and clinical trial phase, although as one study in 2019 noted, the evidence to support this approach is still insufficient.[30]

Without question, the most significant technical innovation in gene therapy in the past decade is the development of CRISPR-Cas9. The acronym stands for 'clustered regularly interspaced short palindromic repeats' and the associated CRISPR protein 9. In non-scientific language, CRISPR-Cas9 is a unique technology that has made the previously laborious process of editing parts of the human genome by altering the DNA sequence far swifter, simpler, and more precise. Since 2013, when CRISPR was first used to edit human cells, the technology has dramatically increased the potential to develop more effective, individualized gene therapies for an ever-widening range of diseases and disorders.

Yet despite CRISPR-Cas9 and other innovations, it is still the norm for the most advanced RM and gene therapy treatments to require a lengthy R&D pipeline, especially when research avenues are superseded by more promising pathways opened up by further scientific breakthroughs. Nonetheless, an increasing flow of therapies and treatments with a proven clinical track record and regulatory approval become available every month. For example, by December 2023, the US Food & Drug Administration (FDA) had approved 32 cellular and gene therapy products for use in the world's most advanced RM and gene therapy market,[31] where treatments already range from biopharmaceuticals for diabetic ulcers to heart-valve substitutes and bioglass bone grafts.[32]

Artificial Intelligence (AI) And New Digital Medical Technologies

Gene editing (see glossary) is an outstanding illustration of how AI-enabled technologies are speeding up advances in RM and gene therapy. In recent years, the integration of AI into the CRISPR gene-editing tool (see glossary) has facilitated simpler, faster, cheaper editing than older methods.[33] In cancer immunotherapy, for example, a 2021 study of AI-enabled CRISPR gene editing found it was far quicker, more accurate, and less expensive than laboratory-based trial and error approaches.[34]

Other examples of AI-enabled RM and gene-therapy technologies that benefit patients include:

3D Printing For Surgery: Inkjet and microextrusion 3D bioprinting can be used respectively to engineer cartilage and fabricate aortic valve replacements.[35]

Drug Discovery: AI tools and platforms can be deployed to predict molecular properties, protein binding affinities, and the spatial arrangements of amino acids in protein sequences.[36]

Disease Modeling: Identification of biomarkers and genetic mutations by AI tools can be used to develop more accurate disease models and potential treatments.[37]

Medical Data Analytics: Machine learning-based algorithms can extract and analyze enormous quantities of data from academic journals, patient records, and pathology images to improve treatments and therapies.[38]

Advanced digitalized countries or those with ambitious digital transformation programs are self-evidently better placed to realize the potential of AI-enabled RM and gene therapy. Hence our recommendation that healthcare providers' countries must have the necessary digital technology and expertise to deliver RM and gene therapy effectively. This will not be easy, particularly in low-income countries with healthcare budgets stretched to the limit by COVID-19. In developing economies, aid agencies and charities will have a critical role to play in ensuring that a new RM and gene therapy digital health gap does not open up for the world's poorest people.

Case Study 2: UAE Leveraging Digital Transformation To Build A Significant RM And Gene Therapy Hub

Digital technology and expertise are critical for integrating RM and gene therapy into healthcare systems. The UAE's ambitious digital transformation agenda, like similar programs in other Gulf region states, shows how countries can leverage the tech-driven benefits of RM and gene therapy without being global leaders in these fields.

Starting in 2022, within 10 years the UAE hopes to double the contribution of digitalized products and services to 19.4% of national GDP. In AI-enabled technologies alone, the government expects total investment to be more than $27 billion by 2027. This expenditure on AI is already making an impact on the UAE's healthcare system, which is identified as a key emerging sector in the country's National Strategy for Artificial Intelligence.[39]

One example is the UAE's Emirati Genome Program, launched in March 2023, which aims to collect and analyze whole-genome sequencing data from up to 1 million citizens to identify individual genetic health risks. Meanwhile, patients in the UAE have begun to benefit from RM treatments and gene therapies. In 2022, researchers at Abu Dhabi's Khalifa University of Science and Technology conducted the UAE's first successful generation of an induced pluripotent stem cell line, using blood from a healthy 27-year-old donor.[40] And in September 2023, the newly opened Abu Dhabi Stem Cells Center successfully manufactured the UAE's first CAR-T cells (see glossary) to successfully treat an 11-year-old boy with leukemia.[41]

These are early steps toward achieving the UAE's goal of a more personalized, digitally advanced healthcare system capable of absorbing all the latest RM and gene therapy technologies. Progress so far has depended critically on the country's prescient investment in wholesale digital transformation.

Children, RM, And Gene Therapy: A Moral Imperative

The Children's Hospital of Pittsburgh in the US estimates that around two-thirds of all known rare diseases are found in children.[42] Many of these diseases are inherited genetic disorders that often cause lethal damage to the heart, liver, lungs, and other organs. Yet they are also increasingly treatable with advanced gene therapies and RM approaches.

The scale of identified and potentially treatable rare diseases in children demonstrates why pediatrics should be a major focus for governments and HCPs when integrating RM and gene therapy into healthcare policies and regimes.

Consider how various gene therapies and RM treatments are already increasing the survival and QALY prospects of children with rare diseases in the following areas:

Trachea and Lung Surgery: Lung stem cells from adult tissue or pluripotent stem cells are used to regenerate airways.

Stomach Surgery: Tissue engineering is deployed to regenerate the epithelial lineage and reinforce the gastric wall.

Enteric Nervous System (ENS): Stem-cell transplantation is a potentially curative method to regenerate genetically damaged ENS, which control motor and endocrine functions and local blood flows.[43]

Blood Disorders such as Sickle Cell Disease and Beta Thalassemia: Blood stem cells are taken from a child's bone marrow and treated with a retrovirus through gene editing, with the gene-corrected stem cells transferred back into the patient.[44]

Addressing Affordability: Governments, Industry, Healthcare Providers, And Regulators Must Work Together To Deliver Cost-Effective RM And Gene Therapy Healthcare

There is no escaping the fact that most RM and gene therapy treatments and therapies are extremely expensive and not obviously affordable for poorer countries where their beneficial impact may be greatest. A good example is a new one-dose CRISPR-Cas9 sickle cell disease therapy developed by the PLS companies Vertex Pharmaceuticals and CRISPR-Therapeutics, which was awaiting US regulatory approval in November 2023. The manufacturers estimate that the therapy will become cost-effective at a price of $1.9 million.[45]

Governments and the global PLS industry sector have a shared interest in collaborating to bring down the costs of developing RM and gene therapy drugs and treatments, starting with the difficulty of achieving economies of scale in such a fragmented sector. As a 2020 academic survey of RM noted: "The necessary technology to produce RM therapies at an industrial level does not exist yet. Scale-out and automated production methods for the manufacturing of regenerative therapies are needed."[46]

To sum up, think big when considering investments in RM and gene therapy technologies. But be highly targeted when deciding which approaches are most appropriate for your country's specific healthcare needs.

Case Study 3: Australia Targeted Government Investment In Specific RM And Gene Therapy Sectors

Australia's investment in stem cell research over the past two decades illustrates how focusing on a single, key sector can be a better RM and gene therapy funding strategy than spreading limited resources too thinly. At the same time, the government has learned from its own experience that being an early mover in such a complex, rapidly evolving field does not guarantee R&D and commercial success.

In 1987, Australia established its pioneering credentials in stem cell research and therapy when a team of scientists and doctors at the Royal Adelaide Hospital conducted one of the first successful blood stem cell transplants to treat a patient with leukemia.[47] Seeking to build on this early expertise, in 2003 the government established the Australian Stem Cell Centre (ASCC), based in Melbourne, which brought together specialists in stem cell research from nine universities and institutes to collaborate on projects.

A critical lesson learned during the ASCC's initial years was that converting scientific stem cell research into commercial treatments and therapies was not straightforward. In 2009, the ASCC launched a new business plan that moved its strategic focus toward pure research, implicitly accepting that the government would not get a direct, short-term market return on its original investment.[48]

In 2011, ASCC was succeeded by Stem Cells Australia (SCA), a new government-backed initiative in Melbourne. SCA was charged with spearheading stem cell R&D in the field while providing financial support to scientists to develop their careers and educating the public about the benefits of stem cell therapy. By 2019, when the original initiative expired, SCA had supported more than 300 early-career researchers at major Australian universities and medical research institutes.[49] Notable advances by Australian stem cell scientists during this period included testing new drugs for heart repair, growing replacement heart tissue, and using big data technologies to analyze cellular identity.[50]

Today, Australia's stem cell R&D is driven by the Stem Cell Therapies Mission (SCTM), the latest government-backed program. SCTM funds R&D in areas ranging from tissue regeneration and bio-engineered skin wound flaps to cell therapy for blindness.[51]

Australia's government is aware the country is still only a leading second-tier stem cell R&D player, behind the US, China, the UK, South Korea, and Japan.[52] Nonetheless, two decades of consistent government investment and support has ensured that Australia's world-class stem cell research and therapy sector continues to attract leading scientists in this field from around the world.

Chapter 3

Regulators Need To Be RM And Gene Therapy Enablers As Well As Rule-Makers

When they commit public money to RM and gene therapy R&D, governments are investing in what is already a huge, rapidly growing global industry. Total RM and gene therapy market value estimates vary considerably, partly because of differences in how the sector is defined, but all forecasts project steep growth over the next decade. For instance, Grand View Research, an India and US-based company, calculated that in 2022 the worldwide RM market (including gene therapy) was worth $25.92 billion, measured by revenue, and forecast a compound annual growth rate of 16.8% for the rest of the decade.[53]

Any fast-growing global industry on this scale clearly demands robust regulation – and indeed, the rollout of regulatory frameworks for RM and gene therapy is itself a rapidly growing activity. A 2020 review of RM regulation in nine major markets found that specific RM legislation and frameworks already existed in the US, EU, Japan, South Korea, and Australia. All these jurisdictions had accelerated review or approval programs to expedite market approval of RM treatments and products.[54]

These fast-track compliance pathways point to the need for RM and gene therapy regulators to act as enablers as well as rule-makers (as seen in Case Study 1 – Japan). Best practice in advanced RM and gene therapy markets involves regulators working closely with PLS companies and healthcare providers to support the sector's rapid growth while managing the associated risks, from deficient clinical trial evidence for innovative products to the protection of patient data in gene therapy.

In the US, for example, the FDA's Center for Biologics Evaluation and Research (CBER) provides, in its own words, "proactive scientific and regulatory advice to medical researchers and manufacturers in the area of novel product development."[55] Similarly, the EU's European Medicines Authority provides specialized guidance and support for micro, small and medium-sized enterprises about certification procedures for advanced gene therapies that are under development.[56]

RM and gene therapy regulators are operating in a competitive global market. PLS companies developing the most advanced RM and gene therapy products will naturally gravitate toward the most supportive regulatory jurisdictions, which give them the best chance of gaining market approval as quickly as possible. Regulators therefore need to keep a close eye on their peers in other countries to ensure that their own frameworks are attractive to PLS companies and investors.

China's dual-track regulatory system for stem cell therapies provides a good illustration of how market rules can also act as growth drivers. In China, both pharma companies and medical institutions can conduct clinical stem cell research and gain market approval respectively from the National Medical Products Administration (NMPA) and the National Health Commission (NHC).[57] A 2022 study concluded that this dual-track system has played an important role in creating a "booming landscape" for gene and cell therapies in China, especially for the treatment of cancers.[58] Benefits include a greater number of qualified actors engaged in gene and cell research and more flexibility in R&D.

Of course, other factors are also relevant – notably, the massive investment by China's government in stem cell research in recent years. Yet without a favorable regulatory environment, China's stem cell R&D pipeline would not have expanded so rapidly.

Conclusion

Collaboration And Co-Operation: The Keys To Realizing The Full Potential Of RM And Gene Therapy

Imagine a future where RM and gene therapy were not central to healthcare systems. Their absence would increase the likelihood that a forecast in 2023 by the WHO turns out to be accurate. The WHO projects that on current trends, annual global deaths from chronic NCDs such as heart disease, cancers, and diabetes will increase to almost 77.5 million by 2050, a rise of around 90% in total numbers.[59]

This is just one illustration of why RM and gene therapy are so important when considering how to redesign healthcare systems to alleviate the world's current insupportable health burden. Yet the obstacles to implementing advanced RM treatments and gene therapies are immense – in particular, the high costs and risks involved in R&D and the resulting high prices of drugs and other products.

Conclusion

To clear these hurdles, governments should keep two goals in mind, which at first glance may seem at odds with each other.

Collaboration between governments, healthcare providers, the PLS industry, and academic institutions is crucial. The challenges we describe in this report are not solvable without all healthcare stakeholders working together. This includes patients, who should be active participants in personalized RM and gene therapy approaches.

Competition will also be a defining feature of cost-effective RM and gene therapy healthcare that delivers better clinical outcomes and quality of life for patients. Many advances in RM and gene therapy are driven by commercial rivalry. A key task for governments is to ensure a fair RM and gene therapy market that encourages innovation.

We do not believe these goals conflict. On the contrary, the evidence of the past two decades suggests that the rapid pace of advances in RM and gene therapy has depended on the right balance being struck by all stakeholders between commercial self-interest and the greater public good. This balance must be retained as the evolution of RM and gene therapy ecosystems continues to accelerate.

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Endnotes

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