WHITE PAPER

Equitable and timely access to appropriate innovative treatments and technologies in healthcare

Steering Committee for Human Rights
in the fields of Biomedicine
and Health (CDBIO)

COUNCIL OF EUROPE
CONSEIL DE L'EUROPE

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Livre blanc – Accès équitable et en temps opportun à des traitements et des technologies innovants et appropriés dans les soins de santé

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Executive summary

Innovative treatments and technologies are transforming healthcare by offering new possibilities for diagnosing, managing and treating complex diseases. These approaches depart significantly from conventional medical practices in terms of purpose, method, design or delivery. Examples include precision medicine, gene editing, immunotherapy, neurotechnology, artificial intelligence (AI), telehealth, digital therapeutics and robotics. While these advancements have the potential to significantly improve patient care and outcomes, their introduction also raises important ethical and policy challenges – particularly the need to ensure that they are accessible to everyone in accordance with their needs. This White Paper, developed by the Steering Committee for Human Rights in the fields of Biomedicine and Health (CDBIO), explores the ethical and policy challenges related to equitable and timely access to appropriate innovative treatments and technologies in healthcare and offers guidance.

To ensure that innovative treatments and technologies contribute to health equity rather than widening existing disparities, this White Paper identifies five priority areas for action.

First, access must be grounded in human rights and guided by equity, as affirmed by Article 3 of the Convention for the Protection of Human Rights and Dignity of the Human Being with regard to the Application of Biology and Medicine: Convention on Human Rights and Biomedicine (ETS No. 164). This requires proactive efforts to prohibit discrimination, remove structural barriers and support disadvantaged individuals and groups throughout the entire process of innovation – from research and development to implementation and reimbursement.

Second, the safety, effectiveness and superiority of innovative interventions should be rigorously assessed, with flexible frameworks that can adapt to evolving evidence and maintain public trust in medical innovation. Evaluation processes should incorporate representative data, monitor real-world effectiveness, be accountable, resist undue influence such as lobbying or hype, and support independent validation by expert bodies.

Third, decision-making processes around access should be inclusive, transparent and accountable to the public. Access policies should be clearly defined, grounded in the best available evidence, and justified by reasons that are understandable and acceptable to a broad public. They should allow for meaningful stakeholder involvement, remain open to revision as new evidence or perspectives emerge, and be subject to ongoing review and oversight by independent, accountable authorities.

Fourth, affordability should be addressed through sustainable pricing and reimbursement strategies, guided by clinical benefit, medical need, social justice and the principle of reciprocity. Mechanisms such as reference pricing, managed entry agreements, post-marketing evidence requirements and regular reimbursement reviews can help control costs and support long-term sustainability, while international collaboration on health technology assessments can reduce duplication of efforts and public-private partnerships can share development risks more fairly.

Fifth, health systems should strengthen their ability to deliver innovative treatments and technologies by investing in specialised infrastructure and personnel, expanding regional capacity and developing new healthcare delivery models. As digital tools and expert knowledge become increasingly central to care, bridging the digital divide and promoting health and digital literacy – through digital inclusion and clear, accessible health communication – are essential to ensure that everyone can benefit.

By integrating these principles into policy and practice, policy makers can uphold equitable and timely access to appropriate innovative treatments and technologies, foster public trust and ensure that the benefits of medical progress are shared with everyone according to their needs.

1. Introduction

1.1. Importance of equitable and timely access to appropriate innovative treatments and technologies

Article 3 of the Convention on Human Rights and Biomedicine requires parties, taking into account health needs and available resources, to take appropriate measures with a view to providing, within their jurisdiction, equitable access to healthcare of appropriate quality.

Equitable access must be interpreted in accordance with the meaning provided in Article 3 and clarified in paragraph 25 of its explanatory report. In this context, equitable access means first and foremost the absence of discrimination on any ground. Equitable access also implies that, depending on their medical needs and the available resources, individuals must be guaranteed access allowing them to effectively obtain a satisfactory degree of care. This involves removing barriers that may prevent access and providing appropriate support to individuals or groups who may be disadvantaged or exposed to a higher risk of harm to their health. In accordance with the right to the protection of health enshrined in Article 11 of the European Social Charter (revised) (ETS No. 163), the ultimate goal is health equity – namely the absence of avoidable, unfair or remediable differences among groups of people – where ideally everyone should have a fair opportunity to attain their full health potential and no one should be disadvantaged from achieving this potential.

Equitable and timely access to innovative treatments and technologies is crucial for addressing health inequities and ensuring that all individuals, regardless of socioeconomic status or other social determinants of health, can benefit from advancements in healthcare. The importance of equitable access to innovative treatments and technologies is highlighted by three critical considerations.

First, ensuring that advances in healthcare truly benefit everyone is a moral imperative and a human rights concern, which can be alternatively grounded in the right to the highest attainable standard of health (International Covenant on Economic, Social and Cultural Rights), the right to the protection of health (European Social Charter, ETS No. 35) and the right to equitable access to healthcare (Convention on Human Rights and Biomedicine). This means that everyone should have the opportunity to obtain a satisfactory standard of care, taking into account health needs and available resources. Within the context of achieving universal health coverage (United Nations Sustainable Development Goal, Target 3.8), access to the full range of essential health services, medicinal products and health technologies should be provided without causing financial hardship. As innovative treatments and technologies have the potential to transform, if not revolutionise, healthcare and drastically improve health outcomes, it becomes crucial to ensure that no one is excluded from their benefits. Innovative treatments and technologies may be particularly important in addressing complex, chronic and life-threatening conditions, leading to earlier and more accurate diagnoses and new interventions that can stop the progression of disease, offer long-term survival benefits and, in some cases, provide cures for previously untreatable diseases. However, without access to these innovations in healthcare, individuals may continue to suffer from preventable diseases, delayed diagnoses and deteriorating health conditions that could have been managed or treated more effectively.

Second, innovative treatments and technologies may hold significant potential to reduce health inequities by enabling more proactive, preventive and tailored approaches to healthcare compared to more traditional methods. Since disadvantaged populations often face higher rates of preventable diseases, delayed diagnoses, untreated medical conditions and interruptions in ongoing treatments, these new pathways in prevention, diagnosis and integrated care management could bring the greatest benefits to them. Moreover, when properly implemented and supported by efforts to bridge the digital divide, innovative technologies can create new care delivery models that may further reduce health disparities. For instance, telemedicine can enable remote consultations, diagnoses and treatments for individuals in rural, hard-to-reach or underserved areas where healthcare services are often scarce. This may eliminate geographical barriers to medical care that may have otherwise been unavailable. Additionally, wearable and implantable devices and health-tracking systems may allow continuous monitoring and personalised interventions for chronic conditions such as diabetes and heart disease. These technologies may provide more timely care for patients with limited access to healthcare services, helping to mitigate some of the most pressing healthcare inequities.

Third, several social determinants that directly influence the ability of individuals to obtain timely and appropriate healthcare, such as income, education, geographical location and social support networks, play an even more critical role in determining access to innovative treatments and technologies. Innovative treatments and technologies are often expensive, depend on advanced infrastructure and highly specialised healthcare professionals, and require a high level of health literacy. Consequently, individuals who are already struggling financially, those living in rural or underserved areas with limited access to specialised healthcare services, and those with lower education levels and limited social support networks – which are essential

to understanding the value of new treatments and technologies and to navigating complex healthcare systems – are at risk of being excluded from the advantages of medical innovations. Without comprehensive policies aimed at improving the availability, affordability and accessibility of innovations in healthcare, there is a real risk of exacerbating health inequities. This concern is particularly relevant in light of the principles outlined in Recommendation CM/Rec(2023)1 of the Committee of Ministers to member States on equitable access to medicinal products and medical equipment in a situation of shortage. Failing to address these barriers can lead to discrimination based on factors such as socio-economic status, geographical location, and educational and literacy levels. Disadvantaged populations, who often already face barriers to healthcare, could see their exclusion worsen, deepening existing inequities. Importantly, the recommendation also highlights the need for prioritisation based on medical criteria and the removal of barriers for systematically disadvantaged individuals in situations where innovative treatments and technologies are in short supply. Without targeted interventions, it is most likely that only those who are more privileged, with the necessary financial resources, knowledge and social support, will benefit from these medical innovations. As a result, rather than mitigating health disparities, innovative treatments and technologies risk perpetuating and exacerbating them.

1.2. Potential benefits and risks of innovative treatments and technologies in healthcare

Innovative treatments and technologies in healthcare present a broad range of benefits, as they may improve diagnostic and treatment capabilities, patient care and outcomes, and efficiency and productivity in healthcare processes.

Innovative technologies offer the potential for more precise, timely and individualised care through tools such as precision medicine, genomic profiling, pharmacogenomics and AI-based diagnostic platforms. These technologies allow treatments to be better tailored to a patient's unique health profile, offering more targeted and effective interventions. Additionally, advancements in predictive analytics, genetic screening and real-time health monitoring allow earlier detection of risks and diseases, and a more preventive and proactive approach to healthcare. AI-powered diagnostic tools may enhance both the speed and accuracy of disease detection and enable healthcare professionals to make informed decisions faster. Moreover, advancements in minimally invasive techniques, including robotic surgery and neurotechnology, may provide new ways to treat complex conditions with greater precision, reducing recovery times and complications. Furthermore, AI-assisted systems may help reduce medical errors in diagnosis, prognosis and treatment planning, enhancing overall safety and improving patient outcomes.

Innovative treatments and technologies may also improve patient care and outcomes in different ways. For instance, telemedicine platforms, along with wearable devices and remote monitoring systems, may facilitate continuous care and rapid responses to health changes even outside traditional clinical settings. Telemedicine platforms may allow patients in rural or underserved areas to access specialist consultations, diagnoses and follow-up care without the need for in-person visits. In addition,

mHealth and bioelectronic implantable devices may empower patients to have more control over their health by giving them access to real-time information and by improving chronic disease management and adherence to treatment plans. Advances in certain directions, for instance in the fields of gene editing, immunotherapy, antiviral therapy and regenerative medicine, are pushing the boundaries of what is possible in healthcare, leading to cures for previously untreatable conditions and groundbreaking treatments that can stop the progression of disease or offer long-term survival benefits.

Innovative treatments and technologies also hold the potential to significantly enhance efficiency and productivity in healthcare by streamlining diagnostic and treatment processes. Tools such as AI-assisted medical imaging analysis, predictive analytics for assessing disease risks and remote patient monitoring systems can lead to faster and more accurate diagnoses and interventions, which can save valuable time and resources in patient management. Additionally, innovative treatments and technologies can lead to more targeted and timely interventions, reducing the need for frequent consultations and extended hospital stays, while also minimising complications and recovery times. This can in turn decrease the burden on healthcare facilities, reduce healthcare costs for both patients and providers, and allow healthcare professionals to treat more patients efficiently and with higher precision, increasing the overall capacity of healthcare systems to meet growing demands. Moreover, big data and AI may contribute to accelerating medical research and innovation by analysing vast and complex datasets, enabling the identification of new therapeutic targets, a deeper understanding of disease mechanisms, and the development of more advanced treatments and technologies, which could result in more effective and timely healthcare solutions for patients and more efficient and responsive healthcare delivery.

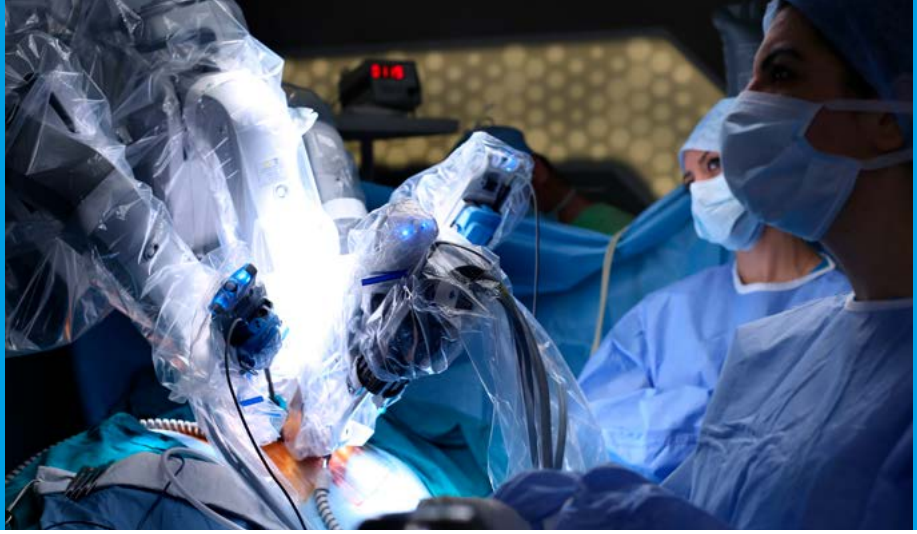
While innovative treatments and technologies in healthcare offer significant benefits in enhancing diagnostic and therapeutic capabilities, improving patient care and outcomes, and increasing efficiency, it is essential to remain aware of the limitations and risks they present. As these innovations move from the experimental stage to clinical application, challenges such as limited clinical evidence, lack of large-scale trials and concerns about generalisability across diverse populations complicate the assessment of their long-term safety, efficacy and potential superiority over conventional treatments, requiring ongoing evaluation and monitoring. In addition, issues such as disparities in access, data privacy and security concerns, scalability of solutions, potential over-reliance on technology at the expense of human judgment, the tendency to prioritise innovative solutions over traditional treatments and technologies that may be equally or more effective, and the costs associated with implementing such advancements should be addressed to fully realise the potential of these innovations. This White Paper will explore these limitations and risks in greater depth, with a particular focus on ensuring equitable access.

1.3.Scope and objectives of the White Paper

This White Paper focuses on the challenges related to equitable and timely access to appropriate innovative treatments and technologies in healthcare.

The scope of the White Paper encompasses both innovative treatments and technologies that may or may not be intended for severe or life-threatening health patient conditions. The White Paper is restricted to innovative treatments and technologies of which the safety, efficacy and quality are supported by scientific evidence, and which have been approved or certified through an appropriate regulatory process provided for by law. It does not consider experimental treatments and technologies.

The primary objective of the White Paper is to explore the key barriers to equitable and timely access to appropriate innovative treatments and technologies in healthcare and to identify potential solutions based on the guidance of the Council of Europe and other international organisations. Importantly, the White Paper also examines the possible tensions between individual rights and public health needs when adopting innovative treatments and technologies.



2. Definition and characteristics of innovative treatments and technologies in healthcare

2.1. Definition

Innovative treatments and technologies refer to treatments and technologies that adopt approaches which depart significantly from conventional medical practices. They incorporate cutting-edge scientific discoveries and breakthroughs in engineering, with the aim of providing new therapeutic possibilities and better quality of healthcare. Innovative treatments and technologies have advanced through the various stages of research and development and have obtained regulatory approval for at least some specific indications or applications. In some cases, these treatments and technologies may already be available as standard treatment options.

Experimental treatments and technologies also use innovative approaches, but they are still in the phase of research, development or clinical evaluation. These treatments and technologies might involve unknown risks and their effectiveness is often uncertain. Patients typically receive experimental treatments and technologies as participants in clinical trials when existing treatments and technologies are either unavailable or ineffective. Access may be determined by different actors and subject to other criteria than those that apply to innovative treatments and technologies.

Differentiating between experimental and innovative treatments and technologies may be complex. For instance, with respect to certain treatments and technologies (for example immunotherapy, regenerative medicine and neurotechnology), the boundaries between experimental and innovative may be dynamic, as these treatments and technologies may have received approval for certain uses while at the same time being evaluated for expanded indications and applications or for additional patient populations in the future. It should also be noted that, whereas some innovative treatments and technologies may be available in some countries, they may still be considered experimental or may not be available in other countries. This may be related to differences in regulatory approval processes, healthcare infrastructure and resources, national health priorities and sociocultural factors.

Box I. Illustrative overview of innovative treatments and technologies

This overview highlights the wide range of innovative treatments and technologies that are currently applied in healthcare. These technologies may either be integrated directly into therapeutic applications or function as standalone interventions.

Precision medicine, or personalised medicine, tailors healthcare to individual patients on the basis of their genotype, the molecular characteristics of their disease, lifestyle and environment, in order to maximise therapeutic benefits and minimise adverse reactions. Precision medicine includes targeted cancer therapies, BRCA1 and BRCA2 gene testing, genomic profiling of tumours and pharmacogenomics (PGx), which explores how the patient's genetic profile influences their response to medications and helps in selecting medication, determining the optimal dosage or considering alternative treatment.

Regenerative medicine refers to innovative approaches to regenerate, repair or replace damaged or diseased organs, tissues and cells with a view to functional recovery and healing. Most regenerative medicine that is currently available is not innovative but part of the established field of transplantation. In some fields established, and in others innovative, are bioscaffolds (an artificial structure implanted in the body to support tissue growth), used in orthopaedics, dentistry, cardiology, wound healing and plastic and reconstructive surgery.

Gene editing is a medical approach to treating monogenic or polygenic disorders that involves replacing a defective gene with a healthy copy of that gene, modifying or inactivating a defective gene, or introducing a missing gene. CRISPR-Cas9 is a modern technique that employs molecular tools to make precise molecular changes ("edits") to the genetic material within a cell's genome. Gene editing has clinical applications in, for instance, inherited retinal dystrophy, spinal muscular atrophy in infants, and certain types of blood cancers such as acute lymphoblastic leukaemia and non-Hodgkin lymphoma.

Immunotherapy is a treatment that is used to boost the immune system to target and destroy cancer cells or to suppress the immune response to manage autoimmune diseases. Checkpoint inhibitors are used in the treatment of melanoma, lung cancer and bladder cancer. In addition, monoclonal antibodies

are used to treat autoimmune diseases such as Crohn's disease and rheumatoid arthritis, and CAR-T cell therapy is used to treat acute lymphoblastic leukaemia and non-Hodgkin lymphoma.

Nanomedicine focuses on using nanotechnology (involving the engineering of ultrafine particles between 1 and 100 nanometres in diameter) to diagnose and treat diseases at the molecular and cellular level. Nanoparticles can be functionalised with drugs to allow delivery of medications to specific cells or tissues, overcoming biological barriers and release of medications in a controlled way. Nanomedicines have been approved for use in progressed ovarian cancer and metastatic pancreatic cancer. Nanoparticles are also used as contrast agents in medical imaging techniques to enhance visualisation of tissues, and used in biosensors to identify biomarkers for cancer detection.

Artificial intelligence refers to systems that display "intelligent" behaviour by analysing their environment and taking actions to achieve specific goals. AI is often based on machine learning, which allows predictive algorithms to learn from experience and automatically improve their performance, and which increasingly takes the form of "deep learning" models, composed of multiple layers of processing that allow the AI system to learn representations of data with multiple levels of abstraction so as to progressively refine predictions for accuracy. AI is used for automation of hospital processes, for remote patient monitoring, in machine vision embedded in robot-assisted surgery, for acceleration of drug design, in chatbot-based patient interactions, in personalisation of treatment plans, and in predictive algorithms based on self-learning to assist in interpreting medical imaging and predicting clinical outcomes.

Neurotechnology in healthcare refers to the application of neuroscience and technology to monitor, understand, diagnose or modulate neural activity with the aim of improving neurological and mental health conditions. Neuroimaging technologies are used to visualise brain activity to diagnose neurological disorders. Neuromodulation technologies can be non-invasive or invasive. They involve the targeted electrical or magnetic stimulation of specific brain regions to modulate neural activity and are used to treat Parkinson's disease, dystonia, essential tremor, depression, migraine and chronic pain. Additionally, brain-computer interfaces (computer-based systems that translate brainwave signals into commands that are communicated to an external device) are being developed to allow individuals with neurological and neuromuscular conditions to control prosthetic limbs, computers or assistive technologies.

Digital therapeutics (DTx) refer to interventions that use digital technology to prevent, treat or manage health conditions in conjunction with more conventional treatment. They are software-based, delivered through wearables (mHealth), smartphone apps or web-based tools, typically collect real-time data, personalise interventions on the basis of user data and preferences, may be interactive, and can be integrated with healthcare systems and remote monitoring models that allow continuous management of patient status.

Point-of-care (POC) diagnostics refer to diagnostic tests conducted at the site of patient care rather than in a traditional laboratory setting. These diagnostics

are designed to provide rapid results to assist in immediate clinical decision making. These include biosensors (devices that integrate a biological component with a physicochemical detector to detect and measure specific biological markers such as glucose biosensors to manage insulin doses in diabetes) and biomarker tests such as cardiac biomarker tests to diagnose heart attacks and rapid infectious disease tests.

Telehealth refers to the use of telecommunication and digital platforms to provide healthcare services remotely. Telemedicine is a subset of telehealth, involving remote clinical consultations, diagnoses, counselling and follow-up care using real-time videoconferencing. In addition, telehealth also encompasses services such as telesurgery, remote patient monitoring, "store and forward" (a method allowing healthcare providers to forward medical data or images for specialist review and timely diagnosis), and the use of electronic health records and digital health platforms to securely store, access and exchange patient information.

Robotics are used in healthcare in a variety of applications. Robot-assisted surgery allows surgeons to perform complex surgeries with minimal invasiveness and increased accuracy and control, resulting in reduced complications and faster recovery. Robotic exoskeletons and devices can help in rehabilitation by improving movement of patients with neurological conditions or mobility impairments and assist healthcare providers in moving patients and minimise physical strain. In addition, robots can be used in telehealth to allow remote medical consultations, they can enable patients to maintain their independence at home longer, and they can be used to provide companionship and emotional support to patients.

3D printing technology is used in the healthcare context mainly to create customised medical implants, prosthetic limbs, orthotic devices and braces tailored to the anatomy and the medical needs of the patient. 3D printing can also be used to produce models of patient anatomy to organise preoperative planning and the simulation of surgery, and to print specialised surgical instruments for complex surgeries.

2.2. Characteristics

2.2.1. Common characteristics

Compared to conventional treatments and technologies, innovative treatments and technologies (see Box I for an illustrative overview) have certain characteristics that are different or more pronounced.

a. Complexity

Innovative treatments and technologies in healthcare are inherently complex, often involving new mechanisms of action and advanced systems. Many innovative treatments, such as gene editing, immunotherapies and regenerative medicine, operate through novel biological mechanisms. For instance, gene editing techniques such

as CRISPR-Cas9 allow for precise modification of the genome by targeting specific genetic defects, which is radically different from conventional treatments that aim to alleviate symptoms rather than modify genetic material. Immunotherapies leverage the body's own immune system to recognise, target and destroy cancer cells, which represents a fundamental departure from conventional therapies such as chemotherapy and radiation, which indiscriminately attack both healthy and cancerous cells. Regenerative medicine focuses on repairing or replacing damaged tissues by using the body's own healing capacity or using advanced biological materials, instead of managing symptoms or slowing disease progression. Furthermore, technologies such as robot-assisted surgeries, AI-driven diagnostic tools and nanomedicine involve the integration of complex software and hardware systems that require highly specialised technical and clinical knowledge.

b. Multidisciplinarity

Innovative treatments and technologies rely on the convergence of knowledge and expertise from diverse fields such as medicine, biology, (bio)engineering and information technology. This multidisciplinary collaboration is essential for the successful development and application of innovative treatments and technologies, as it allows for the integration of different perspectives that advance breakthroughs in patient care and enables the incorporation of innovative treatment methods, advanced diagnostic tools and sophisticated data analysis techniques. This multidisciplinary approach can accelerate the pace of innovation and enhance the potential to address complex healthcare challenges more effectively.

c. Limitations in establishing effectiveness

As innovative treatments and technologies move from the experimental stage to clinical application, several challenges arise in establishing their effectiveness. One significant limitation is the lack of robust clinical evidence, as many innovative treatments and technologies are still in the early phases of use, with limited long-term data on their safety and efficacy. The absence of comprehensive studies and large-scale clinical trials makes it difficult to determine the durability of therapeutic benefits, identify potential side effects and assess whether these innovations are superior to conventional approaches. This limitation complicates the validation process for regulatory authorities and healthcare professionals, posing obstacles to fully endorsing innovative treatments and technologies as viable alternatives. Furthermore, early trials often target narrow patient groups, raising concerns about the generalisability of results across diverse populations. The complexity and novelty of innovative treatments and technologies add to the difficulty in assessing their long-term impact, making continuous monitoring of evidence essential to ensure their safe and effective integration into healthcare.

d. Fragmented responsibility

The development, implementation and management of innovative healthcare treatments and technologies typically involve multiple stakeholders, leading to fragmented responsibility. These stakeholders may include researchers who drive

scientific discoveries, biotech firms that translate these discoveries into market-ready solutions, hardware manufacturers and software developers who create the necessary technological infrastructure, data scientists who process and interpret large datasets, and healthcare professionals applying these innovations in clinical settings. Additionally, regulatory bodies are responsible for overseeing these innovations, ensuring they meet rigorous standards of safety, quality and efficacy. This division of roles, while necessary for advancing complex innovations, can make it difficult to assign clear accountability when issues arise, such as serious adverse events or reactions.

e. Shift towards personalised healthcare

Innovative treatments and technologies are driving a shift towards personalised healthcare, where therapies are tailored to the genotype, molecular profile, lifestyle and environmental factors of each patient. This approach, enabled by advances in genomics, artificial intelligence and novel therapeutic techniques, aims to improve treatment accuracy and reduce adverse effects. In some fields, such as oncology, personalised approaches are already being successfully implemented, particularly through therapies targeting specific receptors, while in other areas they remain aspirational. Consequently, the timeframe for adopting personalised healthcare varies widely between treatments, with some still in early clinical stages and others advancing rapidly. For instance, precision medicine employs whole genome sequencing and pharmacogenomics to identify genetic predispositions to diseases and optimise medication choices and dosages for individual patients. AI also plays a vital role in personalising treatments by analysing vast datasets to provide more accurate diagnoses and tailored treatment plans. While personalised healthcare holds significant promise, it also presents challenges, including high costs and the need for sophisticated healthcare infrastructures.

2.2.2. Notable characteristics of selected innovative treatments and technologies

In addition to the general characteristics that distinguish innovative treatments and technologies from conventional ones, some also possess distinctive features that may complicate the assessment of their quality, safety and efficacy, or give rise to new or heightened ethical concerns.

a. Irreversibility of effects

Some innovative treatments and technologies introduce irreversible effects that are broader and more profound than those of conventional treatments, which are often reversible or more localised in their impact. Once administered or applied, these innovative treatments and technologies lead to permanent, systemic changes at a fundamental biological level, intentionally altering the patient's condition in ways that are irreversible – the full consequences of which may not yet be fully understood at the outset of the procedure. For instance, gene editing techniques like CRISPR-Cas9 and immunotherapies such as CAR-T cell therapy operate at the genetic and molecular levels, aiming to fundamentally alter biological processes. These interventions can

result in permanent changes to how genes express themselves or how the immune system functions. Similarly, neurotechnologies such as deep brain stimulation, which involves implanting electrodes in the brain to modulate neural activity, may have the potential to induce long-term or even permanent alterations in brain function.

b. Opacity

Due to their novelty, complexity or lack of transparency, some innovative treatments and technologies introduce a significant level of opacity. This makes it challenging for healthcare professionals and regulatory bodies to fully understand their mechanisms and potential implications, potentially leaving patients uncertain about their treatments. For instance, AI-driven diagnostic systems often operate as "black boxes" because their decision-making processes, based on complex self-learning algorithms, are not easily interpretable or validated by clinicians, which can result in hesitancy in fully trusting AI-generated findings. Similarly, treatments such as gene editing and immunotherapy introduce novel mechanisms of action that may not be fully understood, particularly with regard to unintended effects and long-term outcomes. Often, healthcare professionals may need to rely on external experts or cutting-edge research to interpret complex data or struggle to stay up to date with rapid advancements in the field, which can create knowledge gaps and erode confidence in the safety, efficacy and quality of these innovations.

c. Commercial stakeholder involvement

The introduction of some innovative treatments and technologies has resulted in a significant expansion of commercial stakeholder involvement in healthcare, including tech firms, data companies and social media platforms. This increased involvement raises concerns about potential conflicts of interest, commercialisation of health data and external influence on treatment choices. For instance, data companies may have a commercial interest in the health data they store and analyse. Large tech firms and social media platforms entering the domain of healthcare may drive healthcare professionals and patients towards technology-heavy solutions, potentially sidelining more traditional treatments and technologies that may be equally or more effective. This shift risks creating a technology-driven bias in healthcare delivery. In addition, these stakeholders can contribute to media hype and the marketing of unproven therapies and technologies, fuelling unrealistic patient expectations and creating societal pressure on regulators to fast-track approvals.

d. Big data

The rise of innovative treatments and technologies has made big data a central component of modern healthcare. The integration of big data involves the collection, storage and linking of various types of datasets, such as electronic health records, genetic data, lifestyle data and real-time patient monitoring, across diverse platforms and devices. The applications of big data are expanding rapidly, with the goal of enabling more efficient, proactive and personalised approaches to patient care. Big data allow a transition towards precision medicine, where treatments are tailored to each individual based on their unique genetic and lifestyle profiles, leading to earlier

detection of diseases, more accurate treatment plans and better forecasting of health outcomes. A key advantage of big data is its ability to support real-time monitoring through wearable devices and sensors, providing continuous oversight of patient health outside of the clinical setting, allowing for quicker interventions and better management of chronic conditions. Additionally, big data facilitate predictive analytics, which can identify high-risk individuals before serious health issues arise, further advancing the shift from reactive to proactive healthcare. However, the extensive use of big data may also introduce ethical concerns around privacy, transparency and the potential for discrimination. The involvement of third parties, such as pharmaceutical companies, tech firms and insurance providers, can raise the risk of misuse of sensitive health information, highlighting the need for robust data protection measures.

e. Digital integration

Many innovative treatments and technologies depend heavily on the digital integration and connectivity of healthcare systems, representing a paradigm shift in healthcare delivery and management, called Health 4.0. Digital integration in healthcare refers to the increasing use of interconnected digital tools, platforms and technologies, such as electronic health records, telemedicine, wearable devices, remote patient monitoring, cloud-based platforms and AI-powered diagnostic tools. These tools enable faster data processing, better communication and improved collaboration between healthcare professionals, contributing to more efficient and co-ordinated healthcare management. This digital transformation also promotes a more integrated, personalised and real-time approach to patient care, allowing for the identification of new treatment pathways and enabling more proactive, preventive and customised interventions. This marks a departure from traditional, reactive models of care that often rely on slower diagnostic processes and generalised treatments, shifting towards a data-driven model of care that empowers healthcare professionals and patients to make better-informed and timely treatment decisions based on comprehensive patient data.

f. Innovative care delivery models

In addition to the broader trend toward more proactive, preventive and personalised care, innovative treatments and technologies related to big data and digital integration are driving the emergence of new care delivery models. A key example is telemedicine, which enables healthcare professionals to provide remote consultations, diagnoses and treatment recommendations via digital platforms. This reduces the need for in-person visits, improving access to healthcare, particularly in rural or underserved areas. The Covid-19 pandemic further accelerated this shift, as telemedicine became essential when face-to-face consultations were limited. Another component of these innovative care models is continuous health monitoring through wearable devices and remote patient monitoring systems. Devices like fitness trackers, heart rhythm monitors and blood glucose sensors can be integrated into healthcare systems, enabling real-time tracking of vital signs and health metrics. These data can be shared with healthcare professionals, allowing for more personalised and timely interventions, which is especially beneficial in managing chronic conditions like diabetes and heart disease.

g. Technological integration with the human body

Some innovative treatments and technologies are reshaping the relationship between technology and the human body, as devices and therapies not only interact with, but increasingly integrate into, biological systems. For instance, neurotechnologies are being developed to interface with the brain, modulating neural activity to improve cognitive and motor functions, with more experimental applications including brain-computer interfaces enabling individuals to control external devices such as prosthetics or computers using their brainwave signals. In addition, bioelectronic medicine merges biology and electronics by developing implantable devices that use electrical impulses to modulate nerve signals, aimed at restoring or adjusting normal physiological functions in chronic conditions such as diabetes and arthritis. In the field of regenerative medicine, innovations such as bioscaffolds, which are artificial structures that provide a framework for tissue growth and are gradually absorbed by the body, are being used in wound healing, surgeries and orthopaedics.

h. Access to mental states

Some innovative technologies are starting to offer unprecedented insights into cognitive functions and emotional states that were previously inaccessible or difficult to measure or treat. For example, neuroimaging technologies allow healthcare professionals to visualise brain activity and gain a deeper understanding of how emotions and cognitive processes, such as decision making or memory, are influenced by specific brain regions or changes in neural connectivity. Other neurotechnologies, such as deep brain stimulation, are being used to alter brain function, potentially offering treatment for conditions like Parkinson's disease, severe depression and obsessive-compulsive disorder. Additionally, virtual reality is being used to access and treat conditions such as phobias, anxiety and post-traumatic stress disorder by immersing patients in controlled environments where therapists can tailor treatment in real time based on their emotional and cognitive feedback.

3. Challenges related to assessing quality

Innovative treatments and technologies introduce significant complexities into healthcare decision making, particularly due to their evolving nature and the uncertainties surrounding their long-term safety and efficacy. These challenges are crucial, as Article 3 of the Convention on Human Rights and Biomedicine emphasises the need for equitable access to healthcare of "appropriate" quality, meaning care that meets "a fitting standard in the light of scientific progress".[1] Difficulties in assessing safety and efficacy and whether these innovations offer improvements over the existing standard of care make the validation process more complex for regulatory authorities, reimbursement agencies (in countries where they are separate from regulatory authorities) and healthcare professionals. This section will examine these challenges, focusing on the potential barriers they present to ensuring equitable access to innovative treatments and technologies.

3.1. Challenges in evaluating safety, efficacy and superiority of innovative treatments and technologies

Evaluating the safety, efficacy and potential advantages of innovative treatments and technologies is challenging due to a range of factors.

1. Explanatory report to the Convention on Human Rights and Biomedicine, paragraph 24.

3.1.1. Limited availability of robust clinical evidence

A major difficulty is the limited availability of robust clinical evidence. Many innovative treatments rely on cutting-edge technologies that are still in the early stages of clinical application, meaning that long-term data on their safety and efficacy are often limited. Comprehensive studies are necessary to evaluate their effectiveness, monitor their long-term effects and assess how they perform in broader clinical applications. Additionally, many of these treatments lack large-scale clinical trials and reliable longitudinal data, which are crucial for identifying the durability of therapeutic benefits and the potential for side effects, and for comparing these innovative therapies to established diagnostic or treatment standards. This lack of robust data may make it difficult to determine whether these innovative treatments are superior or even equivalent to more conventional treatment options. This may complicate efforts by regulatory agencies, reimbursement authorities and healthcare professionals to validate these treatments and fully endorse them as viable alternatives to conventional treatments.

3.1.2. Concerns about generalisability of findings

Innovative treatments often encounter challenges in generalising their findings across diverse patient populations, as early clinical trials are typically conducted in controlled settings and focus on highly specific patient groups. These trials frequently lack sufficient representation of key demographics such as women, older persons, ethnic minorities or patients with rare diseases or pre-existing conditions, resulting in unequal treatment outcomes when applied in real-world clinical settings. This issue is especially relevant for innovative therapies, where treatment responses may be influenced by both intrinsic factors (for example genetic polymorphism, age, gender, height, weight, lean body mass, body composition and organ dysfunction) and extrinsic factors (for example factors associated with the environment and the cultural background of the person). Since, for instance, differences in drug metabolism are well-documented across ethnicity, gender, age and genetic variations, treatments that are introduced in the absence of large-scale, inclusive trials may be less effective or even harmful for underrepresented populations. To promote equity in healthcare, it is essential to expand the diversity of clinical trial participants so that they more accurately reflect the diversity of real-world populations. This inclusive approach ensures that innovative treatments are safe and effective for all patient groups, avoiding the risk that certain groups disproportionately benefit from these advancements, thereby perpetuating or even exacerbating health inequities.

3.1.3. Algorithmic bias

AI systems used in healthcare have the potential to significantly improve clinical decision making, but they also come with the risk of algorithmic bias, particularly when these systems rely on imbalanced or incomplete datasets. Clinical trials and medical datasets have historically lacked adequate representation of certain populations such as women, older adults, ethnic minorities or people with rare diseases. When AI systems are trained, validated or tested on such skewed data, the resulting algorithms may deliver biased outcomes, in that underrepresented groups risk

being misdiagnosed or presented with less effective or even harmful treatment recommendations, thus reinforcing health inequities. Mitigating algorithmic bias requires ongoing efforts to ensure that the datasets used for training AI systems are inclusive and representative of diverse populations, and that AI systems are evaluated and monitored for biases in their outcomes through bias benchmarking frameworks and by diverse teams assessing data quality[2]. Additionally, improving the transparency and explainability of AI models is essential for enabling healthcare professionals to understand how AI-derived recommendations are generated, assess the appropriateness of suggested treatments and detect potential risks of bias and discrimination. These measures are crucial to ensure that the benefits of AI-based medical technologies are accessible to all patient populations and do not unintentionally reinforce or exacerbate existing health inequities.

3.1.4. The role of external stakeholders regarding quality assessment

During the initial phases of introducing innovative treatments or technologies, assessing their effectiveness and comparing them to existing alternatives can be complicated by external factors such as marketing hype, media interest and patient advocacy. Companies developing these innovations often promote their products as revolutionary breakthroughs, even when limited clinical data are available to support these claims. This may create a perception among patients and healthcare professionals that newer technologies are automatically superior to traditional treatments, despite a lack of robust data. Media reporting can further amplify these expectations by highlighting promising early-stage clinical trials or experimental therapies without always conveying the complexity or limitations of the medical evidence. This narrative can foster unrealistic expectations, presenting these innovations as definitive solutions to complex medical issues, even when substantial gaps in data still exist. Patient advocacy groups play a vital role in representing patient interests and pushing for timely access to promising treatments. However, when based on anecdotal success stories or preliminary trial results, their efforts may inadvertently contribute to pressure on regulatory bodies to accelerate approvals or reimbursement decisions. Regulatory bodies should therefore develop models for stakeholder involvement that foster transparent engagement with commercial stakeholders and patient representatives, acknowledging the essential perspectives they bring to the evaluation process without compromising scientific rigour or public trust.

3.1.5. Difficulties in determining professional standards

As outlined in Article 4 of the Convention on Human Rights and Biomedicine, interventions in the health field must always be carried out in accordance with relevant professional obligations and standards. In this regard, the explanatory report clarifies that the professional standard is determined by the current state of the art and, "[i]n following the progress of medicine, it changes with new developments and

2. Steering Committee for Human Rights in the fields of Biomedicine and Health (CDBIO) (2024), "The application of artificial intelligence in healthcare and its impact on the 'patient-doctor' relationship", Council of Europe.

eliminates methods which do not reflect the state of the art."[3] Due to the rapid pace of advancements and the evolving nature of medical practice, healthcare professionals increasingly face challenges in determining whether an established treatment or technology has been superseded by an innovative one, prompting a shift in the standard of care. This challenge may be compounded by the limited availability of robust clinical evidence, as well as concerns regarding the generalisability of clinical findings across diverse patient populations, leaving healthcare professionals struggling to assess their actual benefits for individual patients. Deciding when to integrate new treatments and technologies into healthcare practice is made even more complex by the potential implications for professional liability and regulatory compliance, as healthcare professionals should carefully navigate shifting standards of care while balancing the risks and benefits for their individual patients.

As outlined in the report on the application of artificial intelligence in healthcare and its impact on the 'patient-doctor' relationship, when it comes to aligning with professional standards based on the best interests of patients, the implementation of AI in healthcare introduces unique challenges, primarily due to the lack of transparency, explainability and reproducibility. AI models that function as "black boxes" may deliver highly accurate results but make it nearly impossible for healthcare providers to understand the rationale behind their decisions, impairing the ability to assess the appropriateness of AI-generated treatment recommendations and detect potential errors and biases. As a result, healthcare professionals might either rely too heavily on AI or dismiss it altogether, both of which can negatively impact patient care. While the adoption of any new technology results in a loss of skills (deskilling) and simultaneously requires the acquisition of new skills (upskilling), the rapid adoption of AI technology in healthcare poses a particularly significant risk. Healthcare professionals might become over-reliant on AI recommendations without fully understanding the underlying medical reasoning, which could potentially lead to a decline in critical thinking and diagnostic skills over time. To mitigate these risks, it is crucial to carefully manage the transition to AI-enabled healthcare. Clear guidelines and regulations should be established for the development and use of AI systems, ensuring that these systems are rigorously validated through clinical trials and subject to regulatory standards to guarantee their safety and reliability. It is also essential to support and empower healthcare professionals during this transition, promote minimum standards for information and explainability in AI systems, and ensure that AI in healthcare is governed by meaningful human control. Furthermore, healthcare professionals should retain the discretion to distance themselves from or challenge AI system outputs when uncertainty arises, thereby preserving their core clinical skills while acquiring the necessary technological expertise.

3.2. Complexity in decision making

The difficulties related to evaluating the safety, efficacy and potential superiority of innovative treatments and technologies make decisions about their availability and prioritisation especially challenging. This process is made even more challenging

3. Explanatory report to the Convention on Human Rights and Biomedicine, paragraph 32.

as additional factors related to the distinctive nature and broader impact of these innovations should also be carefully considered.

3.2.1. Important implications for patient outcomes, healthcare system sustainability and stakeholder interests

The availability and prioritisation of innovative treatments and technologies can significantly impact both individual patient outcomes and broader public health. Advanced therapies, such as CAR-T cell therapy, gene editing and immunotherapies, have the potential to greatly enhance or even save the lives of patients with serious conditions, especially when conventional treatments have failed. For these patients, access to groundbreaking therapies can mean the difference between life and death, making decisions about their availability and prioritisation highly impactful. However, these decisions come with challenges. Some treatments, such as gene therapies or nanomedicine, may have far-reaching, unforeseen public health impacts, even when their benefits for individual patients are clear. Additionally, decision making is further compounded by the high cost of many innovative treatments and technologies. Healthcare systems should balance the immediate needs of patients with serious or life-threatening health conditions against long-term financial sustainability. This introduces an ethical dilemma: how to ensure access for those in need without overburdening the system or limiting future access to innovations.

In situations where innovative treatments are in limited supply, Article 6 of Recommendation CM/Rec(2023)1 of the Committee of Ministers to member States emphasises that prioritisation should be guided by individual medical need, based on a thorough assessment of the severity of the condition, the expected benefit of the treatment, the availability of alternative options and the potential consequences if the treatment is not provided. In addition, decisions about introducing innovative treatments often rely on an assessment of the opportunity cost, estimating the potential health benefits that other patients might lose if resources are allocated to a particular intervention, with cost-effectiveness thresholds applied to help ensure that limited healthcare resources achieve the greatest possible overall health benefit for the population.

Moreover, given the complexity and technicality of these treatments, decision making should involve a diverse group of experts from various disciplines. While interdisciplinary collaboration is essential, it also introduces additional layers of complexity. Stakeholders such as healthcare professionals, patient advocacy groups, regulatory bodies and pharmaceutical companies often have conflicting priorities, which can complicate the decision-making process. For instance, while healthcare professionals focus on clinical outcomes and patient safety, patient advocacy groups may prioritise rapid access to potentially life-saving treatments. Regulators, on the other hand, are responsible for ensuring that these therapies meet safety and efficacy standards before they are made widely available.

3.2.2. Difficulties in defining what is "reasonable"

Defining what is "reasonable" in granting access is particularly difficult in the context of innovative treatments and technologies, due to evolving evidence, scientific uncertainty, diverse stakeholder views and potential for future improvements. According

to Article 10 of Recommendation CM/Rec(2023)1 of the Committee of Ministers to member States, access to medicinal products and medical equipment, and their prioritisation, should be guided by the best available evidence. This evidence should rely on parameters that are relevant, measurable, clear, objective and consistent. However, compared to more established therapies, this poses distinct challenges for innovative treatments and technologies, as they often lack a robust body of evidence regarding their safety and effectiveness. This shortage of data, further complicated by the ongoing nature of clinical trials and the uncertainty surrounding long-term outcomes, creates difficulties in constructing sound arguments for their integration into healthcare systems and in determining the prioritisation of patients, although regulators are increasingly responding by combining clinical trial results with real-world evidence to strengthen decision making.

Additionally, innovative treatments and technologies can be highly complex and novel in their mechanisms of action, making it difficult for decision makers to fully understand their benefits, risks and long-term implications. This is particularly true for digital and AI-based technologies, where traditional evaluation methods, such as clinical trials, may be insufficient, as these technologies are frequently updated and influenced by user interactions. Issues such as algorithmic transparency, potential bias across different population groups and real-world performance require new evaluation approaches that are still in development. Moreover, confronted with the rapid pace of innovation, decision makers should frequently revise their arguments about what is reasonable in order to incorporate new evidence and newly emerging treatments, technologies and applications.

In addition, what is "reasonable" may be different depending on the perspectives of different stakeholders. Patient advocacy groups and the public, influenced by media coverage and heightened expectations, may have different views on what is relevant and fair in terms of access, particularly when the treatments offer life-saving or life-changing potential where no alternative exists. This divergence in views can complicate the decision-making process, making it difficult to maintain a balanced, evidence-based approach. Furthermore, when assessing the reasonableness of an innovative treatment, especially one that initially offers limited benefits, it may be essential to consider its future potential. Some therapies that initially offer modest benefits, such as a few weeks of life extension in oncology, may later evolve to provide significantly improved outcomes as clinical experience grows. This dynamic nature of innovation requires a flexible and forward-looking approach in policy making to accommodate the potential future value of treatments.

3.2.3. Challenges in maintaining consistency in policies of access

The principle, outlined in Article 12 of Recommendation CM/Rec(2023)1 of the Committee of Ministers to member States, that policies that define and implement priority-setting standards should be applied in a consistent way is also essential in the context of innovative treatments and technologies. The consistent application of policies based on predetermined criteria helps to prevent discrimination, promotes transparency in decision making and, in this way, enhances trust in the healthcare system and decision makers, enables the effective allocation of scarce healthcare resources, and provides predictability for patients and healthcare providers. However,

the principle of consistency may be more challenging in the context of innovative treatments and technologies as compared to the context of more conventional treatment and technology, considering that policies may need frequent and ongoing adaptation in the light of rapid technological advancements and a changing evidence base. The policies that define and implement priority-setting standards should be designed to prevent corruption, arbitrary exceptions, access on the basis of financial means, activities such as lobbying and political interference. This becomes particularly relevant in the context of innovative treatments and technologies, where rapid access can be a matter of life and death. Patients, their families and patient advocacy groups may intensify lobbying efforts to secure approvals, reimbursements or exceptional access to innovative treatments.

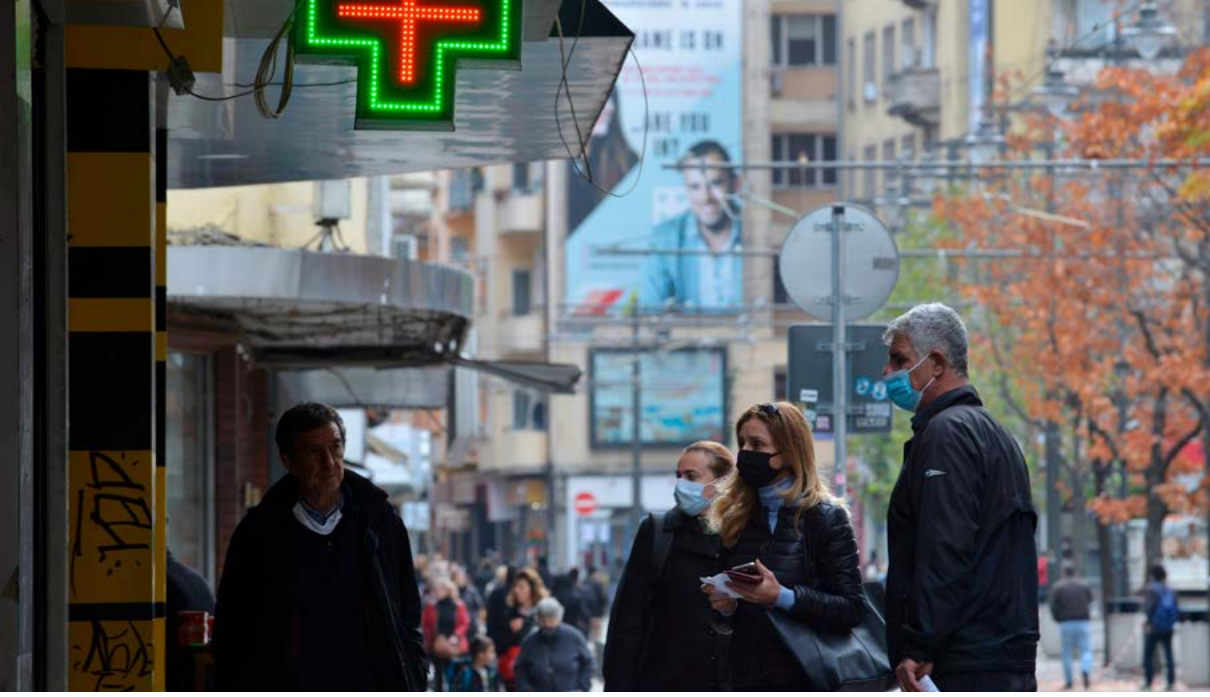
3.2.4. Need for additional stakeholder involvement

According to Article 11 of Recommendation CM/Rec(2023)1 of the Committee of Ministers to member States, the process of determining access to medicinal products and medical equipment, as well as their prioritisation, should be inclusive to ensure that the views of all parties affected by these decisions are taken into account. While meaningful engagement is important for medicinal products and equipment, it becomes even more critical in the context of innovative treatments and technologies, as their introduction may have an impact on a broader and more diverse range of stakeholders. Unlike conventional treatments, these innovations often involve complex scientific advancements, incorporating cutting-edge scientific discoveries, breakthroughs in engineering, and digital tools that require collaboration across multiple disciplines, such as medicine, engineering and data science. Input from a wide range of experts, clinicians, patients and ethicists is necessary to evaluate the benefits, risks and implications of these treatments, particularly in light of the uncertainties, evolving evidence and rapid advancements in this field.

Moreover, innovative treatments and technologies can have far-reaching societal implications beyond their clinical applications. These therapies often raise more complex ethical, legal and societal questions than conventional treatments. For example, discussions about the prioritisation of access to life-saving innovations, such as gene therapies or nanomedicine, can be more contentious, particularly when they involve potential impacts on future generations. Additionally, advanced treatments may raise healthcare budget concerns that are either non-existent or less prominent in conventional treatments. Public engagement is also crucial, not only to address these concerns but because biomedical advancements might more fundamentally impact public trust in biomedicine than conventional treatments and technologies. This may require gathering data on how different groups are affected by priority-setting decisions and identifying disparities that necessitate adjustment of policies.

However, fostering inclusiveness in the decision-making process also presents challenges. The diversity of stakeholders complicates efforts to ensure that all relevant perspectives are adequately represented. Additionally, ensuring that non-experts, including patient groups, have a meaningful say in the decision-making process will be resource-intensive and demanding. Significant efforts are required to educate these groups, considering the complexity, rapid evolution, and potentially contentious nature of innovative treatments and technologies. Substantial efforts are also

needed to inform these stakeholders about the available evidence and manage public expectations, while educating them on the nuances of the decision-making process. This inclusiveness requires careful co-ordination and transparent communication between diverse groups. Despite these challenges, providing all stakeholders, including patients, with a meaningful voice in the decision-making process is crucial for the development of balanced and fair policies. This inclusiveness also helps ensure that decisions are part of a broader deliberative democratic process, involving all groups who may bear the consequences. Public dialogue, as recommended in the "Guide to public debate on human rights and biomedicine", offers a useful model for fostering this engagement and ensuring that the scientific, societal and ethical dimensions of innovative treatments and technologies are appropriately addressed, making decision making more transparent and equitable.



4. Challenges related to equitable and timely access to appropriate innovative treatments and technologies

4.1. Impact of social determinants of health

The ability of individuals to access innovative treatments and technologies is shaped by the social determinants of health. These refer to a wide range of socio-economic conditions – such as income, education, employment, housing and social support networks – which significantly influence health outcomes and access to care. These factors interact in complex and often reinforcing ways, contributing to persisting patterns of health inequality across populations. In the context of medical innovation, the impact of the social determinants of health becomes even more pronounced: individuals who are socially or economically disadvantaged are more likely to encounter multiple, overlapping barriers that limit their ability to benefit from medical advances.

Socio-economic factors play a particularly important role in determining access to innovative treatments, with inequalities arising across different income levels. While individuals with lower incomes often face difficulties in affording advanced treatments or navigating the complexities of modern healthcare systems, financial barriers may also affect those with moderate or higher incomes, depending on the extent of health insurance coverage. These challenges vary across national healthcare systems, depending on the level of public health insurance coverage available for innovative treatments. In addition, the growing involvement of private healthcare providers and private health insurance may give rise to additional concerns, as public healthcare services come under increased pressure and access to innovative treatments may become increasingly dependent on individual financial means.

In addition to financial factors, education and health literacy also have a major effect. Innovative treatments often involve complex procedures, consent processes and responsibilities for self-management. Patients with lower levels of education may find it more difficult to understand medical information or participate meaningfully in decision-making processes. This can hinder their ability to access or fully benefit from innovation, perpetuating exclusion.

Geographic location remains another significant barrier. Rural and remote communities often lack the specialised infrastructure, qualified healthcare professionals and digital connectivity necessary for delivering complex treatments or enabling access to advanced technologies and digital tools. Since many innovative technologies are concentrated in large urban hospitals or academic centres, people living far from these hubs may experience significant delays or be excluded entirely from access. Social support networks can also be essential to successful treatment outcomes. Individuals undergoing complex or long-term therapies often depend on family, friends or community services for emotional encouragement, transport or practical assistance. Those who lack such support – particularly individuals who are socially isolated or marginalised – are at greater risk of discontinuing treatment or missing out on available care altogether.

Employment and working conditions further influence access. When treatment regimes demand frequent medical appointments, extended time off work or temporary relocation, individuals in insecure jobs or without paid leave may be unable to pursue them. Financial instability or fear of job loss can make seeking advanced care practically unfeasible. Cultural and linguistic factors also affect access to innovative treatments and technologies. Miscommunication, implicit bias and the lack of culturally appropriate care can discourage individuals from seeking care or completing treatment. This is particularly relevant for migrant populations and ethnic minorities, who may already experience structural disadvantages in healthcare.

The impact of social determinants is cumulative over an individual's lifetime. Disadvantages experienced in early life – such as growing up in poverty or with limited education or poor nutrition – are likely to persist into adulthood and older age, influencing employment opportunities, income, housing and long-term health outcomes. These accumulated disadvantages may increase not only the likelihood that individuals will develop health conditions requiring new treatments, but also significantly limit individuals' ability to access and benefit from those innovations.

The "inverse care concept" describes how those who need healthcare the most often have the least access to it. In the context of innovative treatments, this means that individuals with the greatest health burdens may be the least likely to benefit due to systemic barriers. Addressing this requires ensuring access to care for everyone, while allocating additional support and resources to those with the greatest needs. Without targeted strategies to account for the social determinants of health, innovative treatments and technologies risk reinforcing rather than reducing existing health inequalities.

Ensuring equitable access to innovative treatments and technologies requires addressing the wide range of barriers individuals may encounter, many of which are rooted in the social determinants of health. These determinants influence not only whether people can afford advanced therapies, but also whether such treatments are available in their area, accessible in practice and delivered in culturally appropriate and understandable ways. The following sections explore these dimensions in greater depth, examining how affordability, availability and timely access, accessibility and acceptability each play a critical role in determining who ultimately benefits from medical innovation and who continues to be left behind.

4.2. Affordability

One of the most significant barriers to accessing innovative treatments and technologies can be their high cost. While some innovations, such as mHealth applications and telemedicine, are relatively affordable and can even help reduce overall healthcare expenses, many advanced treatments remain prohibitively expensive for the majority of patients. Once these innovations receive market authorisation, access largely depends on the reimbursement policies in place in the healthcare system. For most patients, decisions about access to these innovations are therefore made when it is decided what treatment or technology will be reimbursed, as compared to conventional treatments, where access is often determined through priority-setting mechanisms. If a decision is made not to cover an innovative treatment or technology through public health insurance, access becomes virtually unattainable for the majority of patients. This creates a major disparity in access between patients with many financial means and those with limited financial means, reinforcing or exacerbating existing health inequities. Importantly, even when a treatment is reimbursed, individual patients may still face significant obstacles to access, such as co-payments, lack of information or limited health literacy. This highlights the need for policies at the level of the healthcare system to be supported by practical actions that help patients receive the treatments they need.

Examples of innovative treatments and health technologies that are very expensive include gene therapies, CAR-T cell therapies, immunotherapies, precision medicine and treatments for rare diseases. These therapies, used for conditions like spinal muscular atrophy, inherited retinal dystrophy, certain types of cancer and rare genetic disorders, can cost from several hundred thousand to over a million euros per treatment. Several factors contribute to these high prices, including high development and production costs, expensive clinical trials and regulatory approval procedures, intellectual property considerations, the level of competition, market demand and

profit margins that are informed by the expected long-term cost savings offered by these treatments and the "willingness to pay" of individuals and society as a whole. The primary factor contributing to the high price is the market analysis, which is often not transparently connected to the proposed price. Since for most patients availability will depend on the reimbursement policies that are in place in the healthcare system, removal of financial barriers may, in the context of innovative treatment and health technology, need to involve optimisation of reimbursement policies and control of costs.

4.2.1. Principles in regulating financial coverage

Given the potential of innovative treatments and technologies to substantially improve health outcomes while significantly impacting healthcare budgets, it is strongly recommended that national competent authorities conduct thorough evaluations of their safety, efficacy, added value over existing treatments and cost-effectiveness before integrating them into healthcare systems. Based on the available clinical evidence concerning these parameters, innovative treatments and technologies that have received regulatory approval should, in principle, be considered for reimbursement, while carefully balancing available resources and other healthcare priorities. This approach would help promote equitable access to advanced therapies, while maintaining high standards of care and the sustainability of the healthcare system.

In the process of decision making on reimbursement, several considerations are important, in line with the principles outlined in Recommendation CM/Rec(2023)1 of the Committee of Ministers to member States. First, the time gap between regulatory approval and public health coverage for innovative treatments can significantly impact patient outcomes. Delays of one or two years are not uncommon. These delays can result from a lack of comparative evidence, the absence of a reimbursement request or the introduction of procedural pauses by the manufacturer, but they are often caused by the time required to implement reimbursement policies following regulatory authorisation. Such delays may prevent patients from accessing these treatments, even when they have been proven effective and approved for use. Streamlining the process for reimbursement after regulatory approval is therefore essential to avoid bottlenecks in access and ensure that patients can benefit from new therapies in a timely manner.

Second, reimbursement decisions for authorised innovative treatments and technologies should be transparent. In accordance with Article 13 of Recommendation CM/Rec(2023)1 of the Committee of Ministers to member States, all stakeholders, including patients, healthcare professionals and policy makers, should understand the criteria and rationale behind reimbursement decisions. Clear communication about how evidence is evaluated is essential to maintaining public trust and accountability, particularly when high-cost therapies are involved. Article 9 of Recommendation CM/Rec(2023)1 of the Committee of Ministers to member States also highlights the importance of clearly defining responsibilities in the decision-making process, ensuring that healthcare professionals and the public are informed about the entities responsible for making reimbursement decisions and those which should be consulted to address concerns. This level of transparency is especially important for

innovative treatments, where delays in coverage can mean the difference between timely access to life-saving therapies and further deterioration of patient health.

Third, healthcare professionals need easily accessible and regularly updated guidelines on the reimbursement status of innovative treatments, enabling them to offer their patients the most effective treatments without uncertainty or delay. Additionally, they should be aware of any conditions tied to reimbursement, such as patient eligibility criteria or specific health outcomes that must be met.

Fourth, reimbursement decisions should be based on the best available evidence, relying on relevant, measurable, clear, objective and consistent criteria, as outlined in Article 10 of Recommendation CM/Rec(2023)1 of the Committee of Ministers to member States. National reimbursement agencies – which, in some countries, may be the same as the regulatory body responsible for approval – should establish clear thresholds for clinical benefit, such as the effect size required for coverage, to ensure that only therapies with proven value are reimbursed. In cases where clinical benefits are marginal, such as a minor extension of life expectancy, the costs should be carefully weighed against the outcomes to determine whether reimbursement is justified. This evidence-based approach helps maintain the sustainability of healthcare systems while ensuring that patients have access to treatments with real, demonstrable benefits.

Finally, the reimbursement status of innovative treatments and technologies should be subject to regular review, in accordance with Article 14 of Recommendation CM/Rec(2023)1 of the Committee of Ministers to member States. As more data become available through ongoing clinical trials or real-world use, healthcare systems should adapt policies based on new clinical evidence, changes in pricing or the availability of alternative therapies. Regular reviews ensure that patients receive the most up-to-date and effective care without unnecessary delays or gaps in access.

4.2.2. Controlling costs

a. Reciprocity for public investment

The development of innovative treatments and technologies is characterised by significant uncertainty, as many candidates fail due to safety or efficacy concerns, while the process involves substantial costs for clinical trials, regulatory compliance, licensing and manufacturing. To incentivise private-sector investment in this high-risk environment, intellectual property rights play a central role by granting market exclusivities that allow companies and manufacturers to recoup their investments. However, intellectual property rights may also create barriers to access, particularly when monopoly pricing leads to high costs that limit affordability and availability. This tension between innovation and access becomes especially pronounced when companies and manufacturers hold extensive exclusivity rights, enabling them to set prices that generate profits far beyond what could reasonably be considered a fair return on investment.

While recognising the significant contributions and risks taken on by these companies and manufacturers, there are growing calls for pharmaceutical companies and manufactures to take into account the substantial public investment that also

underpins much of their development when setting prices. The expertise and knowledge driving these innovations are often rooted in research that is conducted at publicly funded universities and by highly educated professionals whose education and training were in large part supported by public resources. Moreover, the development of these treatments and technologies frequently relies on data and information provided by the public, whether through participation in clinical trials or the use of healthcare services. Considering this significant public contribution, there is an increasing expectation of reciprocity, where pharmaceutical companies and manufacturers should acknowledge this support by adopting pricing strategies that are fair and socially responsive, for example by offering appropriate discounts, participating in public-private agreements with clauses about reasonable pricing or implementing other mechanisms that facilitate broader and more equitable access.

b. Greater transparency

In addition, there are also increasing calls for greater transparency in the pricing of innovative treatments and technologies, especially in light of their rising costs. Stakeholders, including patient advocacy groups, policy makers and healthcare professionals, argue that the lack of transparency in how prices are set makes it difficult to assess whether these innovations truly offer value for money. A key concern is the confidentiality of price negotiations and agreements between pharmaceutical companies and public or private healthcare insurers, which often leaves the public and even some decision makers unaware of the actual costs and the discounts being offered. While confidentiality is sometimes justified to protect sensitive business information or to allow for flexible pricing arrangements, critics argue that it can lead to inequitable access and create disparities across different regions or countries. To address these concerns, many advocate for a more transparent approach to price setting, where the methodologies and justifications for pricing are clearly disclosed. This increased transparency would help ensure that prices reflect the actual value of the treatment, enhance accountability and build public trust.

c. Mechanisms to control costs

Apart from these general considerations, several mechanisms have been proposed by the Organisation for Economic Co-operation and Development (OECD) for governments to control the costs of very expensive innovative treatments and technologies, thereby improving access.[4] These mechanisms include implementing a system of

4. Barrenho E. and Lopert R. (2022), "Exploring the consequences of greater price transparency on the dynamics of pharmaceutical markets", *OECD Health Working Papers*, No. 146, OECD Publishing, Paris; Lindner L. and Hayen A. (2023), "Value-based payment models in primary care: an assessment of the Menzis Shared Savings programme in the Netherlands", *OECD Health Working Papers*, No. 158, OECD Publishing, Paris; Lindner L. and Lorenzoni L. (2023), "Innovative providers' payment models for promoting value-based health systems: start small, prove value, and scale up", *OECD Health Working Papers*, No. 154, OECD Publishing, Paris; OECD (2017), *New health technologies: managing access, value and sustainability*, OECD Publishing, Paris; OECD (2018), "Pharmaceutical innovation and access to medicines", *OECD Health Policy Studies*, OECD Publishing, Paris; Wenzl M. and Chapman S. (2019), "Performance-based managed entry agreements for new medicines in OECD countries and EU member states: how they work and possible improvements going forward", *OECD Health Working Papers*, No. 115, OECD Publishing, Paris.

reference pricing, negotiating innovative payment models such as managed entry agreements, fostering international co-operation in health technology assessment to streamline evaluation processes, promoting public-private partnerships to distribute the high development costs between public institutions and private companies, and pooled procurement and joint negotiations (see Box II).

Box II. Mechanisms to control costs

1. Reference pricing

A tool proposed to enhance the affordability of innovative treatments is reference pricing, where healthcare payers (such as government agencies, public health bodies or insurers) base the price of a new treatment on the cost of comparable treatments that are already available. This approach aims to control the pricing of high-cost innovative treatments, especially when there is uncertainty about their long-term effectiveness or value for money. In a reference pricing model, the price of an innovative treatment is compared to a benchmark price set by existing treatments that offer similar therapeutic benefits. By using this framework, governments can ensure that the price of new treatments remains in line with the value they deliver, preventing companies from charging excessively high prices simply due to the innovative nature of their treatments. Reference pricing also helps promote price transparency and accountability, as it forces pharmaceutical companies to justify why their innovative treatment should be priced significantly higher than existing alternatives. The system of reference pricing also encourages manufacturers to improve the cost-effectiveness of their treatments, knowing that they will be benchmarked against similar products. Since reference pricing can limit the financial impact of introducing expensive treatments, healthcare systems will be more able to provide access to cutting-edge treatments without overwhelming their budgets. This is particularly useful for healthcare systems operating under tight budgetary constraints, as it prevents them from overpaying for new treatments that may not offer significantly better outcomes than alternatives. The model of reference pricing can be particularly effective when combined with other cost-containment measures.

2. Innovative payment models: managed entry agreements

Managed entry agreements between healthcare payers and pharmaceutical companies provide a flexible framework in which payment for a new treatment is tied to specific criteria, helping to manage the high upfront costs and actual expenditures that are often associated with these treatments. Financially based managed entry agreements focus purely on managing financial risk without considering clinical outcomes. These agreements help healthcare payers manage the budgetary impact through mechanisms such as price discounts, rebates, price-volume agreements or expenditure caps. For instance, manufacturers may offer a predetermined discount on the list price of a treatment, or a rebate may be triggered once a certain usage threshold is met. In some cases, the price may decrease as the volume of the drug administered increases, or an expenditure cap may be set, ensuring the manufacturer provides the remaining doses for free or refunds the difference if spending exceeds this cap. These financial safeguards

help prevent healthcare systems from bearing unsustainable financial burdens when adopting new, high-cost medical products. By contrast, outcome-based managed entry agreements are tied to the clinical performance of the therapy, with reimbursement linked directly to the health outcomes in patients. In this model, payments are contingent on the real-world effectiveness of the treatment, and if the expected outcomes are not met, the manufacturer may provide refunds or adjust pricing accordingly. This approach reduces the financial burden on healthcare systems by only fully reimbursing therapies that are effective. Outcome-based agreements are particularly useful when uncertainty exists about how a medicinal product will perform in real-world settings. Financially based and outcome-based elements can also be combined within a single managed entry agreement, providing flexibility to address both financial risks and clinical uncertainties and allowing for more efficient resource allocation.

Despite their advantages, managed entry agreements face significant challenges that limit their widespread adoption. Negotiating managed entry agreements can be complex and time consuming, often involving high administrative burdens, detailed financial forecasting and careful co-ordination between payers and manufacturers. A lack of consensus on how payments should be structured may further complicate these agreements. Outcome-based managed entry agreements also require the collection of real-world evidence to assess treatment effectiveness, which adds complexity, as defining and tracking clinical outcomes is a resource-intensive process. Additionally, the confidential nature of these agreements, particularly regarding pricing terms, can reduce transparency and hinder comparisons across diverse markets. To overcome these challenges, a more standardised and transparent approach to managed entry agreements should be adopted to improve their effectiveness.

3. International co-operation in health technology assessment

Fostering international co-operation in health technology assessment allows regulatory bodies to streamline processes, reduce duplication of efforts and lower the administrative costs for agencies and compliance costs for manufacturers. By pooling resources, sharing expertise and standardising regulatory and reimbursement requirements, countries can share the costs of health technology assessments, alleviating financial pressure on individual healthcare systems and facilitating the adoption of innovative treatments without compromising financial sustainability. An example of this collaboration can be seen in the European Union (EU)'s initiative for joint clinical assessments of health technologies, which harmonise the clinical evaluation of newly developed therapies.[5] This process reduces time to market and prevents duplication of efforts across member states. Smaller countries with limited health technology assessment capacity can rely on assessments conducted by larger healthcare systems, allowing them to access innovative treatments more quickly. Harmonisation also prevents delays caused by separate national reviews, improving timely access to life-saving therapies

5. Regulation (EU) 2021/2282 of the European Parliament and of the Council of 15 December 2021 on health technology assessment and amending Directive 2011/24/EU, *OJ L 458,* 22 December 2021, pp. 1-32.

for patients. Moreover, international co-operation in health technology assessments can play an important role in reducing barriers to market entry, which in turn increases competition and availability. In some cases, particularly for digital health products, it may influence not just the speed of market entry but whether a manufacturer is willing to enter a market at all.

Sharing health technology assessment results also improves the efficiency of decision making, as it reduces the time and financial resources needed to bring effective treatments to the market. Furthermore, co-operation strengthens the evidence base for evaluating new health technologies, especially those with uncertain long-term clinical outcomes or financial implications. By co-ordinating the collection of data and evaluations, countries can establish robust, internationally recognised standards for assessing the efficacy and safety of treatments. This allows healthcare systems to allocate resources to proven technologies while avoiding investments in those with insufficient value. Moreover, combining real-world data from diverse healthcare systems can further strengthen the evidence used to evaluate innovative treatments, as a broader assessment of performance is achieved, ensuring that only innovative treatments with significant clinical benefits are adopted. This reduces the risk of overspending on ineffective treatments, which is particularly important for therapies with limited or incomplete clinical trials. Additionally, countries can share best practices for designing and implementing managed entry agreements, linking payments to real-world performance and ensuring that reimbursement decisions are based on strong evidence.

4. Public-private partnerships

Public-private partnerships can help mitigate financial risks and challenges by combining resources from public institutions, such as universities and public healthcare systems, with the expertise and investment capital of private pharmaceutical companies. These partnerships can play an important role in speeding up the development of cost-effective treatments, particularly for rare diseases, where traditional market dynamics may not result in sufficient investment. Through these collaborations, the high costs associated with research, development and clinical trials are shared, reducing the financial burden on private companies and ensuring that public funds are used efficiently. For example, public institutions may provide access to scientific expertise, aggregate patient data and research infrastructure, while private companies may bring investment capital, technological advancements and strategies for commercialisation. This collective effort reduces inefficiencies, ultimately allowing treatments to reach the market at a lower price.

Public-private partnerships ideally include early agreements on pricing, intellectual property rights and access so as to align public health objectives with the innovation incentives for private companies. This ensures that affordability is prioritised from the outset without undermining the commercial viability of the therapies. Public health bodies can negotiate pricing structures that reflect both the public health value of the treatment and the commercial interests of private companies, for instance by incorporating outcome-based managed

entry agreements. By sharing both the risks and rewards of innovation, public-private partnerships help ensure that life-saving treatments are accessible to a broader patient population.

5. Pooled procurement and joint negotiations

International co-operation through pooled procurement and joint negotiations may also contribute to facilitating access to innovative treatments and technologies. These approaches may enhance the bargaining power of public authorities, enabling them to negotiate better prices and terms, particularly in the context of high-cost treatments. In addition, co-ordinated procurement may promote efficiency by reducing duplication of regulatory assessments and administrative processes, thereby supporting timely decision making. These forms of co-operation may be particularly beneficial for smaller or more resource-constrained healthcare systems, which may face challenges in negotiating independently, and for promoting equitable access to high-cost innovative treatments across countries.

4.3. Availability and timely access

Innovative treatments and technologies also present unique challenges in terms of availability and timely access. This access is often hindered by delays in regulatory approval and unequal reimbursement policies, which can make such treatments unaffordable or inaccessible for many patients. Even when therapies are technically available, access may still be limited by geographic disparities in specialised infrastructure, shortages of qualified healthcare professionals, insufficient professional training and limited interdisciplinary collaboration. Moreover, to fully address these barriers, availability should be understood not only at the healthcare system level, including regulatory approval, reimbursement and investments in infrastructure, but also at the patient level, ensuring that individuals can actually receive the treatments for which they are eligible.

4.3.1. Delays in regulatory approval and unequal reimbursement policies

Approval and reimbursement policies should strike a delicate balance between making groundbreaking treatments widely available, maintaining high standards of care and ensuring the financial sustainability of the healthcare system. Delays in the regulatory approval process can significantly impact the timely availability of advanced therapies, leaving many patients waiting for care that could save or greatly improve their lives. Even after advanced therapies, such as CAR-T cell therapy and immunotherapies, have been approved and introduced on the market, they may remain prohibitively expensive for large segments of the population if reimbursement policies are not implemented swiftly and comprehensively. In regions where public healthcare systems lack sufficient funding or prioritise other areas of care, or where insurance policies do not cover these high-cost treatments, financial barriers may significantly delay or entirely block access. This creates a major disparity in access,

disproportionately affecting lower-income and uninsured or underinsured populations. As indicated, these challenges can be mitigated by adopting more transparent pricing strategies, fostering public-private partnerships and implementing innovative approaches such as value-based pricing, where payment is linked to patient outcomes, and tiered pricing systems based on income or geographic factors. These efforts, combined with policies that promote timely approval and reimbursement, can help ensure that breakthrough therapies with proven effectiveness become more widely accessible, without compromising the sustainability of the healthcare system. Here, it is important to distinguish between availability within the healthcare system (namely whether a treatment is approved and reimbursed) and actual individual access, where patients can realistically receive the treatment when needed. Taking into account health needs and available resources, policies should not only aim to include innovative treatments in the system, but also ensure that patients receive the necessary support to access them in practice.

4.3.2. Geographic disparities in specialised infrastructure

When innovative treatments and technologies are technically available, they may raise specific challenges due to the need for specialised infrastructure. Many advanced therapies require cutting-edge equipment and expert knowledge, which are typically concentrated in urban centres or well-resourced hospitals, making them difficult to access for individuals in rural, remote and underserved areas. Patients living in these regions may need to travel long distances to reach these healthcare facilities, leading to delays in access and worsening health conditions. This geographic divide exacerbates existing healthcare inequities, as those living in underserved areas may be deprived of innovative treatments that could drastically improve their outcomes. Addressing these barriers requires significant investment in infrastructure. Expanding and equipping healthcare facilities in underserved areas would help reduce the need for long-distance travel and improve equitable and timely access. In cases where the healthcare system cannot afford to expand specialised infrastructure beyond well-resourced urban hospitals, patients should be fully informed about these limitations and provided with guidance, through established protocols, on where and how to access care at those centres in a timely manner.

4.3.3. Shortage of qualified healthcare professionals

Even when physical infrastructure is available, access to innovative treatments may still be restricted by a shortage of healthcare professionals with the specialised skills needed to administer these therapies. This creates a bottleneck in the healthcare system, where innovative treatments and technologies are technically available but cannot be delivered in a timely manner due to a lack of qualified personnel. Addressing these barriers requires sustained and targeted investment in the healthcare workforce. Offering incentives such as competitive salaries and improved working conditions can help attract specialised professionals to rural, remote or underserved areas, thereby reducing disparities in timely access to innovative healthcare. In some cases, innovative treatments such as immunotherapy could be delivered in remote hospitals under the supervision of specialists based in larger centres. However, it should be noted that all investments in infrastructure and human resources for innovative

treatments and technologies should be carefully balanced against other urgent healthcare priorities to ensure that resources are allocated efficiently and equitably.

4.3.4. Gaps in professional education and collaboration

One of the main challenges to ensuring the availability of, and timely access to, innovative treatments is the possible lack of sufficient training of healthcare professionals needed to administer these therapies safely and effectively. As innovative treatments and technologies become increasingly central to medical practice, it is also essential that medical education evolves accordingly. This does not only require the inclusion of specialised modules on new treatment approaches and emerging technologies, but also the promotion of interdisciplinary collaboration with experts in fields such as informatics, data science and neuroscience. In addition, healthcare professionals should have access to reliable and regularly updated information on recent medical developments through continuing professional education, clinical guidelines and knowledge-sharing platforms, to ensure that decisions are based on the most recent evidence and standards of care.

4.4. Accessibility

The issue of accessibility is particularly relevant in innovative treatments and technologies due to their complexity and the resources required to implement them. Many innovative treatments depend on access to reliable internet, digital devices and specialised healthcare professionals, creating barriers for individuals in low-resource, rural or remote areas. Additionally, limited health literacy and digital literacy may prevent certain populations from understanding and utilising these technologies, further exacerbating disparities in healthcare access.

4.4.1. Digital divide

With the rise of digital and data-driven healthcare technologies, a "digital divide" may emerge between individuals who have access to affordable digital devices and reliable high-speed internet services and those who do not. This digital divide can limit the ability of individuals in low-resource communities, as well as those living in rural or remote areas, to access technologies such as digital therapeutics (including mHealth apps), telehealth consultations, point-of-care diagnostics and neurotechnology. This lack of access may delay diagnoses, reduce treatment options and exacerbate pre-existing healthcare inequities. For instance, during the Covid-19 pandemic, telemedicine proved invaluable in providing care remotely, yet patients without access to reliable internet or digital devices were unable to use these services, further deepening disparities in healthcare access. Moreover, digital therapeutics, such as apps designed for managing chronic conditions like diabetes, may require stable internet access connections and smart devices to track health metrics, monitor symptoms and adjust treatments in real time. Individuals without reliable internet or the necessary technology miss opportunities for early diagnosis, preventive care and better disease management. This may create a significant gap in healthcare outcomes between individuals living in low-resource communities, rural or remote areas compared to individuals with consistent digital access.

Addressing this digital divide requires targeted efforts to promote equitable access to digital healthcare innovations. Investments in broadband infrastructure, particularly in rural, remote and underserved areas, are crucial to enable telehealth consultations and other digital health services. Additionally, governments, private sectors and healthcare organisations can collaborate to provide low-cost or subsidised digital devices for individuals who cannot afford them, while public-private partnerships or government subsidies should be implemented to ensure that affordable, reliable internet plans are available to all. By implementing these strategies, it is possible to bridge the digital divide, allowing more people to benefit from innovative healthcare technologies and ultimately improving health equity.

4.4.2. Limited health literacy

Access to innovative treatments and technologies can be significantly hindered by limited health literacy, which refers to an individual's ability to "access, understand, appraise, and apply information concerning healthcare, disease prevention and health promotion".[6] As highlighted in the "Guide to health literacy: contributing to trust building and equitable access to healthcare", a lack of awareness about available healthcare services, and how to find and use them, poses a major challenge, preventing individuals from obtaining the care they need. This challenge becomes even more pronounced with innovative treatments and technologies, which often require access to up-to-date information about the latest medical developments, as well as the ability to understand complex treatment protocols, potential side effects and instructions for follow-up care. Without these capabilities, patients might miss out on more proactive, preventive and personalised approaches to healthcare that could significantly improve their health outcomes. In this way, limited health literacy is becoming an increasingly important social determinant of health.

With the rise of innovative treatments and technologies, there is a growing responsibility to structure healthcare services in a way that ensures that individuals can access and understand essential information, regardless of literacy levels. Public health initiatives can play a crucial role in raising awareness about new therapies and technologies, ensuring that information is provided clearly and accurately. Healthcare systems and professionals should provide comprehensible information, free of medical jargon and difficult terminology, to make health information accessible in a user-friendly way. To achieve this, they should actively involve individuals with limited health literacy in designing accessible systems. Furthermore, collaboration with community advocates and mediators can facilitate the translation of complex medical concepts into language that is easily understandable for populations with limited health literacy. Abilities to access and understand health-related information can be developed from a young age through the school curriculum. In addition, health literacy should be developed as a professional skill among healthcare professionals. This can be achieved by incorporating health literacy training into the curricula of higher education and postgraduate programmes, focusing on equipping healthcare

6. Steering Committee for Human Rights in the fields of Biomedicine and Health (2023), Guide to health literacy: contributing to trust building and equitable access to healthcare, Council of Europe.

professionals with the necessary knowledge, skills and attitudes to effectively communicate with individuals with limited health literacy.

In the context of the digital transformation of healthcare, digital health literacy (or e-health literacy) has emerged as an essential component of overall health literacy, encompassing the skills needed to access, interpret and use digital health information and tools. However, disparities persist in the skills and resources required to engage effectively with these digital platforms. Overcoming these barriers involves not only ensuring that individuals have access to necessary equipment, such as reliable internet access and smart devices, but also ensuring that they possess the digital competencies to use them. This can be particularly challenging, as digital healthcare technologies, such as telemedicine and digital therapeutics, often involve new and unfamiliar interfaces. For example, patients may struggle to use apps designed to monitor chronic diseases or lack the confidence to participate in remote consultations via telemedicine platforms. For digital health services to be truly inclusive, they should be easy to use and tailored to individual needs. Otherwise, the result would be a growing gap in healthcare access, excluding individuals who are not digitally literate from the benefits of modern healthcare technologies, reinforcing existing inequities.

4.5. Acceptability

The provision of health services, including innovative treatments and technologies, should be acceptable to patients, culturally appropriate and sensitive to varying levels of educational attainment. Cultural beliefs and language barriers can profoundly shape how individuals perceive and accept innovative treatments and technologies. In some communities, traditional beliefs or deep-seated distrust of the healthcare system, rooted in historical injustices or previous negative experiences, can lead to hesitancy or even refusal of innovative treatments such as vaccines, gene therapy or advanced surgical procedures. This resistance not only limits access to promising healthcare innovations but also exacerbates existing health disparities, leading to poorer health outcomes in these populations.

Language barriers further compound these challenges, as they can impede effective communication between healthcare professionals and patients. Patients with limited proficiency in the dominant language may have difficulty understanding medical diagnoses, treatment options or the potential risks and benefits of innovative therapies. Consequently, they may struggle to follow medical instructions, fully comprehend the value of treatments or adhere to prescribed therapies. To address these cultural and language barriers, a multifaceted approach is essential. Healthcare professionals need to be trained in cultural competence, which involves understanding and respecting diverse cultural beliefs while delivering care. Offering translation services, multilingual resources and access to medical interpreters ensures that patients fully understand their treatment options and can make informed decisions. Additionally, culturally tailored healthcare campaigns, using the influence of community leaders or trusted cultural figures who share information in ways that align with patients' cultural values, have proven effective in increasing the acceptance of new treatments.[7] Public

7. Ibid.

health initiatives that adopt culturally relevant communication strategies can play a crucial role in closing the gap in access, reducing health inequities and ensuring that language and cultural differences do not prevent individuals from benefiting from medical innovations.

5. Balancing individual rights and public health needs

The introduction of innovative treatments offers significant potential to improve patient outcomes but also presents complex challenges for healthcare systems that must balance individual rights with broader public health needs. On the one hand, patients expect timely access to the most effective therapies; on the other hand, healthcare resources are finite and decision makers must ensure that limited budgets are allocated in ways that maximise overall population health. This balancing act requires careful consideration of both system-level resource allocation and patient-level prioritisation. Decisions must take into account not only the expected clinical benefits and severity of health conditions but also the costs, opportunity costs and ethical implications of introducing expensive new treatments. The following sections outline key principles that guide resource allocation at the health system level and prioritisation between individual patients, with the aim of ensuring that innovative treatments are introduced in a way that is equitable, timely and sustainable.

5.1. Principles for resource allocation at the health system level

When considering the introduction of innovative treatments and technologies, healthcare systems should rely on three principles to ensure that health outcomes are optimised while keeping costs manageable. The "benefit criterion" prioritises treatments in accordance with the expected benefit in extending the patient's life and/or enhancing the patient's quality of life. The "resource criterion" prioritises treatments that require fewer resources to achieve a benefit. The "severity criterion" prioritises treatments for more severe conditions in terms of risks of mortality and morbidity.

These criteria should be carefully weighed against each other. The more severe the condition or the more extensive the benefit of the treatment, the more acceptable it is to allocate more resources. Conversely, giving priority to conditions with low severity and treatments with limited benefit can only be justified if resource use is low.

The basis for decision making on introducing a new treatment is the estimated opportunity cost. This refers to the potential health benefits to other patients that could have been realised with the same resources. As an indicator for decision making in this area, the opportunity cost is typically measured in quality-adjusted life years, although this approach has recognised limitations, as it may favour younger over older individuals and does not always account for broader ethical considerations that impact on human dignity. In some cases, multicriteria decision analysis is preferred, as it allows for the inclusion of additional factors such as disease severity, unmet medical need and equity considerations. Many healthcare systems use the estimated opportunity cost to guide decisions about which treatments should be funded. This threshold represents the maximum amount a system is willing to spend to gain one additional quality-adjusted life year. Any new treatment should be assessed against this threshold to evaluate whether it delivers sufficient value. To that aim, the incremental cost-effective ratio of the treatment is determined, which calculates the incremental costs of the treatment in providing one additional quality-adjusted life year. This figure is then compared to the opportunity cost threshold to determine whether the treatment is cost effective. If the cost-effectiveness ratio of a new treatment is below this threshold, it is considered cost effective because the health benefits justify the additional expenditure. However, if the cost-effectiveness ratio exceeds the threshold, it is considered less cost effective, as the same resources could potentially generate more health benefits if spent on other treatments. This approach ensures that the introduction of innovative treatments does not lead to the displacement of more cost-effective therapies, helping to maximise the overall health benefits achieved with the available resources.

Importantly, the threshold for the incremental cost-effectiveness ratio, if applied, is often adjusted based on the severity of the condition being treated, reflecting society's willingness to prioritise patients with more urgent health needs and to accept a higher incremental cost per additional healthy life year gained. When a treatment targets a life-threatening or highly debilitating condition, such as advanced cancer or severe genetic disorders, healthcare systems are typically more willing to accept a higher incremental cost-effectiveness ratio, given the urgency and potential impact of these treatments. In contrast, treatments for moderately severe conditions are given moderate weight, and those for low-severity conditions are accepted only with a lower incremental cost-effectiveness ratio. This flexibility ensures that patients with the most severe conditions receive necessary care, even if the treatment has a higher cost-effectiveness ratio and might otherwise be declined.

This becomes particularly important when evaluating expensive new treatments. Treatments that require fewer resources are generally prioritised, but this principle should be balanced with considerations of benefit and severity. For instance, a highly effective but expensive new treatment may be justified through a health technology assessment if it addresses a severe, life-threatening condition. Conversely, to ensure

that treatments for less severe conditions are still introduced without straining the healthcare budget, innovative treatments targeting these conditions should generally be priced lower.

When introducing expensive new treatments, several concerns may arise. First, an expensive treatment that consumes a significant portion of the healthcare budget may limit the healthcare system's ability to treat other patients. In a system operating within a fixed budget, introducing such a new treatment will inevitably displace other services, potentially resulting in the loss of a certain number of quality-adjusted life years in other areas. If the budget impact is large enough, not only could less effective treatments be replaced, but more cost-effective and beneficial ones could also be displaced. If these treatments are introduced without considering their total effect on the healthcare budget and their practical challenges, such as retraining healthcare personnel or reallocating equipment, their benefit may be less than that of the displaced healthcare services. This may lead to a reduction in the overall number of healthy life years that the healthcare system can provide with its available resources and could undermine the principle of equitable access to healthcare for all patients. Therefore, the overall budget impact of an intervention should be factored into a broader discretionary assessment.

Second, when a treatment consumes a large share of the healthcare budget, it may be difficult to apply it across the entire relevant patient group. However, permanently limiting such a treatment to only a subset of patients may also be unreasonable, particularly if it is likely to offer more benefits, in terms of resource use and severity, than other available treatments. In such cases, a gradual introduction of the treatment may be necessary. This approach could involve offering the treatment initially to a specific subset of the patient group, such as those who are most critically ill or stand to benefit the most. As capacity increases, the treatment could then be extended to larger segments of the patient group. The conditions for such a phased implementation should align with the principles of priority setting, ensuring that treatments are first offered to patients with the most urgent medical needs and/or those who are likely to gain the greatest benefit.

Third, concerns over efficacy are particularly relevant when there is a limited evidence base, and these considerations should be carefully factored into prioritisation decisions. Provided that all other factors are equal, greater uncertainty regarding the efficacy or quality of a treatment should result in lower prioritisation. However, an important exception arises in the assessment of treatments aimed at small patient groups with extremely severe conditions. These groups are often too small to conduct large-scale clinical trials, making it difficult to perform health technology assessments comparable to those conducted for larger patient populations. In recognition of this challenge, a less stringent requirement for documented evidence may be appropriate. The legitimacy and sustainability of this approach depend on ongoing monitoring to document both the efficacy of the treatment and any associated risks, which could then serve as a basis for re-evaluating the continued funding of these treatments under the scheme after a certain period of time. Such a framework ensures that, while flexibility is offered in cases of limited evidence, the long-term effectiveness and safety of treatments remain a priority.

5.2. Principles for prioritisation at the patient level

As many innovative treatments and technologies are often in short supply, prioritisation between patients will frequently be necessary. In accordance with Recommendation CM/Rec(2023)1 of the Committee of Ministers to member States, this prioritisation should be based on medical need. When innovative treatments and technologies are required for the care of patients with serious or life-threatening health conditions in a context of shortage – defined as the insufficient availability of innovative treatment or technology relative to healthcare needs – the recommendation outlines several key principles.

The first principle is that, in accordance with the principle of non-discrimination defined in Article 4 of the recommendation, no person in need of innovative treatment or technology should be a priori excluded from access to them. Priority-setting standards for the allocation of these resources should be based on the principle that every individual has the right to have their health protected.

The second principle is that, in accordance with Article 6, prioritisation should be based on medical criteria. Before deciding whether an individual should receive access to an innovative treatment or technology in a situation of scarcity, a thorough individual medical assessment should be conducted. This assessment aims to determine whether the use of the treatment or technology is essential to meet the healthcare needs of the individual. This process should take into account four cumulative elements. The first element is the severity of the person's health condition, taking into account medical urgency and the specific care required to address it. The second element is the expected effectiveness of the innovative treatment or technology for the health of the individual concerned. A careful evaluation should be made of the clinical appropriateness and proportionality of the use of these resources in relation to the individual's healthcare needs. As outlined in this White Paper, a particular concern in this assessment is the uncertainty surrounding whether innovative treatments and technologies will genuinely deliver the expected benefits in terms of patient outcomes, safety and improvement over the existing standard of care. In many cases, the available clinical evidence is limited, for example because the clinical trials conducted to demonstrate safety and efficacy were small, follow-up data about long-term effectiveness and potential side effects are lacking or sufficient comparative data with standard treatments or technologies are unavailable. Furthermore, doubts may arise about how well the results can be applied to broader patient populations, especially when certain groups are underrepresented in clinical trials. These uncertainties should be carefully considered when weighing the expected benefit of innovative treatment or technology as part of the medical assessment. The third element is the availability of possible therapeutic alternatives. In a situation of shortage, the treatment or technology in question should only be used if no suitable therapeutic alternative is available. The fourth and final element concerns the likely consequences for the individual's health if they are denied access to the innovative treatment or technology.

When a patient is considered eligible for an innovative treatment or technology based on that individual medical assessment, but the healthcare system is facing scarcity and several patients are in urgent need, setting clear priorities becomes

even more essential. In line with the recommendation, priority setting should in such situations be guided by the principle of minimising the risk of mortality and, subsequently, the risk of morbidity. This implies that access should first be granted to those patients for whom the treatment is expected to be the most effective in preventing death or serious health deterioration. It should be noted, however, that the ability to carry out a full individual assessment will depend on the time available to evaluate the patient's condition. As such, decisions may sometimes need to be taken under conditions of uncertainty, particularly when immediate care is required.

The third principle, as outlined in Article 5, is that specific attention should be paid to individuals and groups who are systematically disadvantaged in relation to health, including as a result of economic or social conditions, legal status, disability, chronic disease or age.

The fourth and final principle, as set out in Article 8, emphasises that even when a patient cannot receive access to a life-saving innovative treatment or technology, for example due to prioritisation decisions based on medical need, resource limitations or uncertainty, they should not be left without care but offered alternative support, if such options are available and suitable for the patient's condition. This could include conventional treatments, symptom relief, psychological support or other forms of care that may not be curative but still provide benefit. If no other medical options are possible, the patient should receive compassionate and palliative care. As described in the "Guide on the decision-making process regarding medical treatment in end-of-life situations", the aim is to respect the dignity of the patient and provide comfort and the best possible quality of life during the final phase of life. The principle ensures that all patients – regardless of the outcome of the prioritisation process – are treated with respect and receive appropriate medical and emotional support.

6. Key considerations

Innovative treatments and technologies are transforming healthcare, offering new hope to patients. From gene therapies to AI-driven diagnostics, these advances have the potential to save lives, extend survival and improve quality of life. However, their introduction also raises important ethical and policy challenges, particularly the need to ensure that these benefits are accessible to everyone. As this White Paper has shown, significant inequalities persist, influenced by factors such as socio-economic status, geographic location and systemic limitations in health-care systems. Ensuring equitable and timely access to innovative treatments and technologies in healthcare represents both a moral imperative and a requirement for the effectiveness and sustainability of health systems. This concluding chapter brings together the most important insights of the White Paper and outlines key considerations to promote equitable and timely access to appropriate innovative treatments and technologies.

1. Access to innovative treatments and technologies should be grounded in human rights, with equity as the guiding principle

Ensuring equitable and timely access to innovative treatments and technologies is, above all, a matter of fundamental human rights. Article 3 of the Convention on Human Rights and Biomedicine and Article 11 of the European Social Charter affirm that timely access to appropriate healthcare – and by extension, access to innovative treatments and technologies – is not a privilege but a human rights concern. In this context, equitable access does not mean equal treatment in a strict sense, but treatment that is tailored to individual medical needs and addresses existing disadvantages, with the aim of ensuring that everyone can effectively

obtain an adequate standard of care. It involves eliminating discrimination based on factors such as economic status, ethnicity, gender, age, disability or other social determinants; removing structural barriers – whether financial, geographical, digital or informational –; and providing targeted support to individuals or groups with specific vulnerabilities. Taking into account available resources, access to innovative treatments and technologies, especially those that are life-saving or significantly improve quality of life, should therefore be guaranteed for all, not only for those who can afford them or are well-equipped to navigate complex healthcare systems.

The commitment to equitable access implies endorsement of the principle that healthcare services should be available to all, but additional attention and resources should be allocated to those in greater need. Disadvantaged groups, such as individuals with lower incomes, those living in rural or underserved areas, people with limited digital access or individuals with low health literacy, face a higher risk of exclusion from medical innovation. These disparities deepen existing health inequalities and should be addressed through comprehensive strategies, including investment in infrastructure, tailored education and outreach initiatives, and the development of healthcare services in underserved communities. In accordance with Recommendation CM/Rec(2023)1 of the Committee of Ministers to member States, prioritisation should be based on medical need and fairness, especially in contexts of scarcity. Access policies should be designed to identify and address structural inequities and prevent innovative treatments and technologies from reinforcing existing advantages. These policies should give particular attention to vulnerable individuals and groups and ensure that the benefits of innovation are not disproportionately concentrated among those who are already advantaged.

This commitment to equitable access should be present throughout the entire process of innovation, from research and development to implementation and reimbursement. For instance, there remains a notable gap in research and development focused on diseases that disproportionately affect specific population groups. To address this imbalance, it is essential that research and innovation frameworks incorporate safeguards that prevent the continued neglect of conditions primarily impacting disadvantaged communities. In addition, many early clinical trials exclude important segments of the population, such as older adults, ethnic minorities or individuals with comorbidities, thereby limiting the generalisability of findings and perpetuating health inequities. Clinical trial design should be more inclusive, setting explicit diversity targets to ensure that new treatments are both safe and effective across diverse populations. Similarly, health technology assessments may need to evolve beyond cost-effectiveness analyses to evaluate the broader social and ethical implications of innovations, including whether an innovation is likely to increase or reduce disparities. National frameworks should support inclusive research, require evaluations that consider equity impacts and ensure that patients and other stakeholders are meaningfully involved in decision making. Only by embedding equity at every stage of innovation can health systems guarantee that scientific progress truly benefits the entire population and upholds the fundamental right to the protection of health.

To operationalise equitable access to innovative treatments and technologies in healthcare, regulatory frameworks should clearly define the criteria for access to them, including how priorities are determined in situations of scarcity. These

frameworks should also provide mechanisms for oversight, transparency and public participation. Institutions responsible for healthcare policy – such as ministries of health, regulatory agencies and hospital boards – should be given clear mandates to promote equity and provided with sufficient financial and human resources to carry out this task. In this way, the right to equitable access becomes not just a principle on paper but a reality in practice.

To ensure that this commitment is effectively implemented, it is essential to generate and analyse high-quality data that reveal where gaps in access persist. Without reliable data, it becomes difficult to assess the impact of policies or to identify which populations are being left behind. Monitoring systems should therefore be established that are capable of tracking access across different population groups. Data should be disaggregated by income, gender, ethnicity, disability and other relevant characteristics. Key equity indicators – such as treatment uptake, time to access and financial burden – should be included in standard health system performance evaluations. Monitoring results should be made publicly available and used to guide regular policy reviews, enabling adjustments to ensure that access strategies continue to meet the needs of diverse populations.

2. Innovative treatments and technologies should be thoroughly evaluated to ensure their safety, effectiveness and superiority over existing standards of care

One of the most critical policy challenges in facilitating equitable and timely access to innovative treatments and technologies is ensuring that these interventions meet appropriate standards of quality, safety and therapeutic benefit. As stated in Article 3 of the Convention on Human Rights and Biomedicine, individuals have a right to healthcare of "appropriate quality" – that is, treatment that reflects a fitting standard in light of scientific progress. However, the rapid pace of innovation often leads to the introduction of new treatments based on early or limited evidence, with insufficient long-term data on safety and effectiveness. Many treatments receive authorisation through accelerated pathways or under conditional approval, with a reliance on surrogate endpoints or small-scale trials. This creates uncertainty for regulatory authorities, reimbursement bodies and healthcare professionals when attempting to validate claims of clinical benefit and superiority over existing standards of care.

Because early clinical trials often enrol narrowly defined patient groups and AI-driven tools may rely on unrepresentative datasets, there is a risk that the lack of generalisability and algorithmic bias may cause certain groups not only to miss out on the benefits of innovation but also to experience adverse effects. To avoid reinforcing existing health inequities, it is therefore essential to promote inclusiveness in clinical trial design, monitor real-world effectiveness across diverse populations and ensure responsible deployment of AI through bias auditing, explainability standards and independent clinical validation. Evaluation processes should prioritise transparency and accountability and resist external pressures such as commercial lobbying, media hypes or advocacy campaigns that may distort the public perception of a treatment's value and lead to premature or unjustified adoption. Finally, the evolving nature of clinical evidence necessitates a flexible and adaptive approach to the definition of professional standards, ensuring that healthcare providers are guided by up-to-date

scientific knowledge while being empowered to exercise their professional judgment in uncertain or complex situations.

3. Equitable access to innovative treatments and technologies requires inclusivity, transparency and accountability in decision making

Ensuring equitable and timely access to innovative treatments and technologies requires democratic legitimacy. Decisions regarding the approval, prioritisation, reimbursement and implementation of innovative treatments and technologies have far-reaching consequences for individual patients and society as a whole. Given the complexity, cost and ethical sensitivity of many of these innovations, such decisions should be guided by transparent procedures, grounded in ethical reasoning and open to meaningful participation by a wide range of stakeholders. As highlighted in Article 11 of Recommendation CM/Rec(2023)1 of the Committee of Ministers to member States, the process of determining access and setting priorities, particularly in situations of scarcity, should be inclusive, ensuring that the views and interests of all affected parties – including patients, healthcare professionals, civil society organisations and representatives of disadvantaged populations – are taken into account. Inclusive participation enables decision makers to understand access barriers and ensures that no group is systematically excluded from the benefits of innovation.

Trust and legitimacy in healthcare are further reinforced when people see that decisions about access are guided by transparent procedures and sound reasoning, and when there are mechanisms for review and accountability. In this regard, access policies for innovative treatments and technologies should be transparent, based on reasons that are considered acceptable by a wide audience, subject to revision in light of new evidence or arguments and overseen by accountable authorities. Transparency should be strengthened through clear and accessible information about reimbursement criteria, justifications for decision making and, where appropriate, pricing negotiations and reimbursement agreements. In accordance with the "Guide to public debate on human rights and biomedicine", public dialogue should be encouraged to ensure that policy decisions reflect human rights standards and societal values. This may include setting up multistakeholder advisory boards, independent ethics committees that evaluate the equity and distributive implications of new technologies and citizen panels to deliberate on access priorities. In addition, regular equity audits and evaluations of real-world distribution outcomes should be conducted to assess whether access policies truly benefit a diverse population. As algorithmic and digital tools increasingly influence access decisions, it is also essential to apply explainability and transparency standards to these technologies, enabling clinicians and patients to understand and, where necessary, question recommendations.

Together, these mechanisms reinforce the legitimacy and equity of strategies that determine access to innovative treatments and technologies, particularly in light of growing concerns about opaque pricing, unequal bargaining power between public and private actors, and the influence of commercial interests in shaping public health priorities. By embedding transparency, inclusiveness and accountability, health systems can remain both responsive to the needs of all members of society and accountable to the diverse public they are meant to serve.

4. Affordability and sustainable financing of innovative treatments and technologies should be ensured

Affordability remains one of the most pressing and complex barriers to equitable and timely access to innovative treatments and technologies. While some digital or preventive innovations may be cost-saving or reduce long-term healthcare burdens, many advanced therapies – such as gene therapies, CAR-T cell treatments, precision medicine and orphan drugs – are prohibitively expensive. Once these treatments receive market authorisation, access becomes largely dependent on reimbursement decisions. Without public coverage, such treatments are often beyond the financial reach of most patients, thereby reinforcing inequalities between those with sufficient financial means and those without. Equitable access will therefore also depend on removing financial barriers and ensuring that public funding decisions are informed by considerations of medical need and social justice.

To address these challenges, national authorities should adopt strong, evidence-based approaches to pricing and reimbursement. In accordance with Article 13 of Recommendation CM/Rec(2023)1 of the Committee of Ministers to member States, reimbursement decisions should be publicly justified and clearly communicated, especially for high-cost therapies. This includes setting clear thresholds for clinical benefit and ensuring that reimbursement frameworks are timely and based on transparent criteria. Regular reviews of reimbursement status are also necessary to respond to evolving evidence, pricing changes and alternative options. Importantly, pricing decisions should take into account the substantial public investment often involved in the development of these therapies – including research funding, data access and infrastructure – reflecting the principle of reciprocity. The value of an innovative treatment or technology should be assessed also in light of its broader contribution to public health and equitable outcomes. Governments should therefore develop national pricing strategies that align clinical benefit with public return on investment, while maintaining a predictable and fair regulatory environment for industry.

To manage high costs without undermining innovation, a range of cost-containment mechanisms should be implemented. These may include reference pricing systems that benchmark costs against comparable therapies and managed entry agreements – both financially based and outcome-based – which tie reimbursement to real-world performance and budgetary safeguards. Furthermore, international co-operation in health technology assessments can reduce duplication of effort, harmonise standards and lower costs, especially benefiting countries with more limited regulatory or economic capacity. Public-private partnerships can also play a critical role in distributing risk and development costs, provided they include clear commitments to affordability, intellectual property sharing and data transparency. Such collaborations should always be guided by explicit public health objectives, not solely commercial interest. Additionally, conditional approvals and risk-sharing models should be linked to strong post-marketing evidence requirements, ensuring that access decisions will be adjusted in response to new data.

Crucially, financial sustainability should not be pursued in isolation. Policies should be complemented by regular equity audits and assessments of how access is realised across different population groups, to ensure that reimbursement decisions do

not inadvertently exclude those in greatest need. Affordability strategies should be aligned with broader efforts to promote transparency, stakeholder participation and accountability in procurement and coverage decisions. Reimbursement systems should also take into account income disparities between countries and regions, providing sufficient flexibility for low- and middle-income settings. Only through co-ordinated, value-based and socially responsive financial governance can health systems ensure that the benefits of innovation are distributed equitably and sustainably. By balancing innovation with affordability and distributive justice, public trust will be maintained while safeguarding the long-term viability of universal health coverage.

5. Strengthen availability of innovative treatments and technologies

Equitable and timely access to innovative treatments and technologies not only depends on affordability or regulatory approval but also on the healthcare system's ability to deliver these treatments in practice. Many advanced therapies – such as gene or cell therapies – require specialised facilities, equipment and expertise that are often concentrated in large urban hospitals or academic centres. This geographic concentration can create significant barriers for individuals living in rural, remote or underserved areas, who may be forced to travel long distances or miss out on treatment altogether. When healthcare infrastructure and personnel are unevenly distributed, disparities in access and health outcomes are likely to increase, even when treatments are technically available within the system.

To address these challenges, national policies should include investment in healthcare infrastructure that is more evenly distributed across regions. This could involve expanding and upgrading regional facilities, equipping local hospitals to deliver certain types of innovative care under remote supervision and integrating innovative treatments into primary and secondary care settings where feasible. Training programmes should also be strengthened to ensure that healthcare professionals have the necessary skills to deliver new and complex treatments safely and effectively. Since a shortage of qualified staff can slow the implementation of new therapies, targeted investment in ongoing education and professional training is essential, as are supportive working conditions to attract and retain staff in underserved areas. In situations where specialised care cannot yet be provided locally, patients should receive clear information on where and how they can access the treatment, as well as logistical and financial support to ensure timely and equitable treatment.

Additionally, the increasing role of digital and data-driven healthcare highlights the need to promote digital accessibility and literacy. The use of AI-based diagnostics, digital therapeutics and telemedicine can enhance healthcare delivery, but only if they are implemented in ways that do not reinforce the digital divide. Many individuals in low-resource areas, older populations or communities with limited education may lack reliable internet access, appropriate digital devices or the skills needed to engage with these technologies. This can limit access to care, delay diagnoses and deepen healthcare inequities. To ensure that digital transformation reduces rather than reinforces disparities, governments should invest in broadband infrastructure, affordable internet access and digital devices, and the development of digital and health literacy programmes. These programmes should be tailored to the needs

of different communities and include simple, understandable communication. By addressing these infrastructural, professional and digital barriers, health systems can ensure that medical innovation is not only available in theory but also truly accessible and beneficial to everyone.

Grounded in Article 3 of the Convention on Human Rights and Biomedicine, this White Paper explores the ethical and policy challenges related to equitable and timely access to appropriate innovative treatments and technologies in healthcare. It aims to support policy makers, regulators, healthcare professionals and researchers by highlighting key barriers – affordability, availability, accessibility and acceptability – and by identifying five priority areas for action. The recommendations of this White Paper are intended to guide future policies and practices to ensure innovation strengthens health equity rather than exacerbates existing disparities.

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