ARMG Publishing "Thinking ahead" sciendo
Health Economics and Management Review, Issue 4, 2022
ISSN 2786-4634 (print) ISSN 2786-4626 (online)

GENE AND CELL THERAPIES OVERVIEW UNDER THE LIGHT OF HEALTH ECONOMICS

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Type of manuscript: research paper

Abstract: *With the increase in drug development studies for rare diseases, gene therapies have recently come to the fore more frequently. In addition to orphan drugs used in the treatment of rare diseases, advanced therapy medicinal products have been developed. Advanced therapy medicinal products are a fast-growing field. Although it is not a treatment method used only in the field of rare diseases, it is also used in the fields of oncology and cardiovascular diseases, musculoskeletal diseases. Regenerative medicine can be promising in cases where advanced therapy medicinal products are difficult and clinically uncertain. There are various cell therapies related to regenerative medicine and cell-based therapies are one of them. Gene therapies, cell-based therapies, advanced therapy medicinal products and regenerative medicine products have high producer price and high production cost. Because all these treatments have limited clinical evidence and high costs, they are difficult to evaluate in terms of health technology assessment (HTA), and special considerations are needed for evaluation. As a solution, costs should be limited and clinical developments should be provided in cooperation with the society. SAVE (equivalent to young life saved) is recommended to evaluate the lifetime health profiles of curative treatments such as gene therapies. In order to reduce the budgetary burden of gene therapies, outcome-directed entry agreements with income-based payments are recommended. Compulsory use of gene therapies and non-reimbursement of these drugs can lead to catastrophic health expenditures. Various payment methods are offered to avoid catastrophic health expenditures. Income-based payment and outcome-based payment are some of these methods. It is also advocated that high prices should be accepted by the society, since gene therapies to be applied in the treatment of rare diseases will be applied to a small population. Both the support of the society to accept the high price of gene therapies, the support of the producer and the support of the payer are important in the development of gene therapies and their supply to the market.*
Keywords: Gene therapies, regenerative medicine, advanced therapy medicinal products, market access, cell-based therapy.
JEL Classification: I11, L11, M31

Received: 12 August 2022 **Accepted:** 19 September 2022 **Published:** 31 December 2022

Funding: There is no funding for this research.
Publisher: Sumy State University

Cite as: Karahan, E. B., & Kockaya, G. (2022). Gene and Cell Therapies Overview Under the Light of Health Economics. *Health Economics and Management Review*, 4, 15-22. https://doi.org/10.21272/hem.2022.4-02

Introduction. The "Orphan Drug Act", which was first approved in the United States of America in 1983 and quickly became widespread, was defined and transformed into a concept that supports the idea and definition of "Rare Diseases" to encourage drug development in this field (Kerpel-Fronius et al., 2020; Maldonado et al., 2021). This concept advocates the equality of individuals with rare diseases, regardless of the level of prevalence of the disease and of access to treatment and health services (Kerpel-Fronius et al., 2020).

In the following years, drug development for rare diseases increased significantly (Kerpel-Fronius et al., 2020). Miller et al. (2021), indicated that a total of 5,099 orphan drug applications were made, 307 in the 1980s, 660 in the 1990s, 1,153 in the 2000s and 2,979 in the 2010s. In addition to orphan drugs, advanced therapy medicinal products (ATMPs) have been developed and used to treat rare diseases.

ATMPs, also known as gene therapies, are used for rare diseases, and they are also used in these fields; oncology and cardiovascular diseases, musculoskeletal diseases, immune diseases, etc. (Kamusheva et al., 2021; Kim et al., 2021; Kockaya et al., 2020; Qiu et al., 2021; Spoors et al., 2020). Increasing government support for the ethical acceptance of gene therapy for cancer treatment in the field of oncology and against the backdrop of rising cancer prevalence may accelerate the growth of the gene therapy market (Kockaya et al., 2020).

Literature Review. Within the scope of the research, 15 articles including the latest situation of gene therapies in various countries of the world, limited and expensive access to existing therapies, ethical limitations and limits of gene therapies, and market access examples of gene therapies were determined. As a result of the examination of the determined articles, the necessary information was compiled and prepared.

a) It has been obtained from the following source that gene therapy affects genetic and multifactorial diseases at the DNA/RNA level, and cell therapy affects diseases at the cellular level;

– Health Policies About Gene & Cell Therapies in France, Germany, Italy, United Kingdom, Spain, Portugal and Turkey (Kockaya et al., 2020)

b) Producer selling price of RMs was found to be USD 110,920–814,780;

– Regenerative Medicine in South Korea: Bridging the Gap Between Authorization and Reimbursement (Kim et al., 2021)

c) The US list price of Zolgensma®, used in gene therapy, was $2.1 million;

– Development and Use of Gene Therapy Orphan Drugs—Ethical Needs for a Broader Cooperation Between the Pharmaceutical Industry and Society (Kerpel-Fronius et al., 2020)

d) The incremental cost-effectiveness ratio (ICER) threshold is the method used in the management of gene therapies, derived from the following source;

– Do Advanced Therapies Have a Future in the Low- and Middle-Income Countries - The Case of Bulgaria, Romania, and Poland (Kamusheva et al., 2021)

e) Obtained from the following source where cell-based therapies (CBTs) are classified by risk-based classification method in Taiwan;

– Perspectives on Challenges to Cell Therapy Development in Taiwan: Strengthening Evidential Standards and Ways Forward (Abolarinwa et al., 2021)

A New Look at the Treatment of Rare Diseases: Regenerative Medicine. Many challenges are associated with ATMPs. The uncertainty of the reimbursement status, marketing, pricing, and clinical value of the products in a real-world setting are difficult to assess (Kamusheva et al., 2021; Qiu et al., 2022). But ATMPs are a rapidly growing field, which has the potential to transform the treatment of pathologies for which traditional treatment approaches are currently used (Kamusheva et al., 2021; Kim et al., 2021; Kockaya et al., 2020; Qiu et al., 2021).

Hanna et al. (2016) indicated that the number of ATMPs clinical trials has increased steadily over the past 15 years (Figure 1).

ISSN 2786-4634 (print) ISSN 2786-4626 (online)

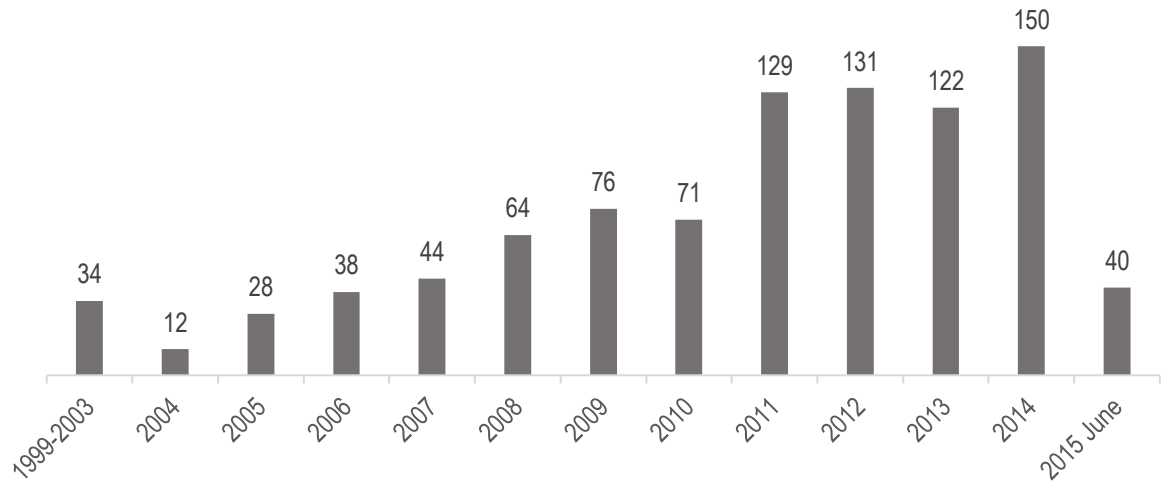

Figure 1. Number of ATMP trials registered by year between 2004 and June 2015 and by range: 1999–2003, 2004–2010, and 2011–2015

Sources: developed by the authors based on (Hanna et al, 2016).

Given the uncertainty of the clinical value of ATMPs and the difficulties in this area, and in cases where treatment with conventional treatment methods is difficult, regenerative medicine (RM) can be considered a promising approach for treating rare diseases (Kim et al., 2021; Kockaya et al., 2020; Qiu et al., 2021; Qiu et al., 2022).

According to the 21st Century Cures Act of the United States, certain cell therapies, gene therapies, and tissue engineering products are considered RM advanced therapy (RMAT) if they are intended to treat, modify, reverse, or cure serious or life-threatening diseases and have the potential to meet unmet medical needs (Qiu et al., 2021; Qiu et al., 2022).

RMAT uses stem cells and progenitor cells to repair or regenerate damaged functional cells and tissues (Kim et al., 2021; Qiu et al., 2021; Qiu et al., 2022).

Various cell therapies also exist with regard to RM (El-Kadiry et al., 2021; Qiu et al., 2022). Cell-based therapies (CBTs) are a subset of RMAT, which consist of living cells being transplanted into a donor or recipient to meet unmet medical needs (Abolarinwa et al., 2021; El-Kadiry et al., 2021; Qiu et al., 2022). According to the American Gene and Cell Therapy Association, gene therapy affects genetic and multifactorial diseases at the DNA/RNA level, while cell therapy affects diseases at the cellular level (Kockaya et al., 2020). The potential of these treatments has gone beyond scientific research and has reached commercial and wider market use (Abolarinwa et al., 2021; Kockaya et al., 2020). For example, in Taiwan, regulatory agencies in jurisdictions have consistently introduced regulations and established and published guidelines to evaluate CBTs based on their perceived risks, and they have used a risk-based classification method to classify them as medical procedures or biological drug products (Abolarinwa et al., 2021). However, this is not the case in South Korea. In a South Korean survey, PBCAC committee members raised the issue of increased production costs and proposed value-based pricing to bridge the gap between approval and reimbursement within the scope of their special pricing policy for RM (Abolarinwa et al., 2021).

Like any innovative health product, RMs have a very high manufacturer sale price (USD 110,920–81(5),780) (Kim et al., 2021). In particular, the use of living cells is one of the main factors that increases the cost of production (Kim et al., 2021). The cell growth medium used to grow and maintain cells accounts for 36% of the production cost (Kim et al., 2021). The high cost of production is also a problem when it comes to the reimbursement of RMs (Kim et al., 2021).

Gene Therapies in Rare Diseases. With the development of advanced therapies, interest in gene therapies (GTs) as treatments for rare diseases has increased (Kamusheva et al., 2021; Qiu et al., 2022). The limited clinical evidence and high upfront costs of GTs have made evaluating them in terms of health technology assessment (HTA) challenging; therefore, special considerations are needed (Kamusheva et al., 2021; Qiu et al., 2022).

With the introduction of the first GT in 2012, alipogene tiparvovec (Glybera®) was approved in Europe, and recommendations continue to be published for reducing uncertainties in the payer decision-making process of GTs (Qiu et al., 2022).

The increase in the presence of orphan drugs in the market and the very high prices of GTs will cause an increase in the financial burden of drug supply, and it will be necessary to sell them at lower prices for the treatment of other diseases (Kerpel-Fronius et al., 2020). To maintain the orphan drug principle, considering the characteristics of orphan GT products, ethical, scientific, and financial arrangements should be made by drug developers and various health institutions, and a successful solution should be obtained (1-2). This solution should limit costs and provide clinical developments in cooperation with the community (Kamusheva et al., 2021; Kockaya et al., 2020).

For example, Onasemnogene Abeparvovec (Zolgensma®), a GT product that has been released recently, will be used in the treatment of spinal muscular atrophy (SMA) and will be closely monitored with the aim of solving problems, such as one-time treatment, long-term efficacy, and safety uncertainty (Kerpel-Fronius et al., 2020). Building on this example, a new type of enduring contractual collaboration between manufacturers and the community is expected to be proposed to share the financial and scientific burdens as well as the benefits of the clinical and regulatory development of advanced orphan GT products in the USA (Kerpel-Fronius et al., 2020). Considering the fact that companies in the USA determine their own list prices, the list price of Zolgensma® of USD 2.1 million for one-time treatment has caused a huge reaction from the public (Kerpel-Fronius et al., 2020). The power of public pressure over the reimbursement policy has prevailed, and the drug is now reimbursed in most European countries (Kerpel-Fronius et al., 2020).

It is important to evaluate the social perspective as well as the relationship between HTA institutions and other stakeholders regarding the obstacles and difficulties that arise in these situations (Kamusheva et al., 2021; Qiu et al., 2021). The European Medicines Agency (EMA) will have a major role and influence in the HTA process in terms of providing scientific advice, empowering market innovative treatments, and satisfying the market access process (Kamusheva et al., 2021; Qiu et al., 2021). It has been reported that this may create difficulties for middle-income countries (Romania, Bulgaria, and Poland, for example) due to their limited financial resources with regard to the early adoption of ATMPs (Kamusheva et al., 2021; Qiu et al., 2021).

According to the National Institute of Health and Clinical Excellence (NICE), a specific HTA methodology should not be adopted for ATMPs (Kamusheva et al., 2021). In addition to the difficulties ATMPs face in demonstrating their value, the STD methodology should consider the specific characteristics of ATMPs (Kamusheva et al., 2021). The accreditation process and incremental cost-effectiveness ratio (ICER) thresholds are some of the specific methods used in ATMP management (Kamusheva et al., 2021).

Several challenges exist on the producer and payer side, including uncertainty regarding the value and long-term benefits of GTs, limited clinical outcomes, high costs, difficulties in conducting traditional randomized clinical trials, limited enrolment in the RCT patient population, the heterogeneity of the patient population, and the lack of comparators (Kamusheva et al., 2021).

The economic evaluation approach needs to be changed, as the scarcity of long-term clinical trial data is one of the main problems in the process of performing cost-effectiveness analysis (CEA) and cost utility analysis (CUA) (Kamusheva et al., 2021). There are two perspectives to consider in CEA because of the long-term utilities expected from GTs: that of the community and that of the health insurance fund (Kamusheva et al., 2021). Considering the specific characteristics of ATMPs, methods have been developed that emphasize the importance of long-term and evidence-based results (Kamusheva et al., 2021). The SAVE (saved young life equivalent) is recommended to evaluate the lifetime health profiles of curative treatments, such as GTs (Kamusheva et al., 2021). Patient involvement is a key element in the entire HTA process for ATMPs (Kamusheva et al., 2021).

Jorgensen et al. highlighted the need to produce real-world evidence to reduce uncertainty, as well as to implement outcome-managed entry agreements with income-based payments to reduce the budgetary burden of ATMPs (Kamusheva et al., 2021).

Sabatini et al. (2022) indicated that there may be outcome-based agreements to reduce the concerns of the payer and the manufacturer in the introduction of gene therapies.

Ethical Needs and Limitations of Gene Therapies. The need for higher-priced orphan drugs for small patient populations of individuals with rare diseases is now widely accepted as ethical (Kim et al., 2021). It has led to the emergence of orphan drug legislation, which encourages and supports pharmaceutical companies in developing drugs for rare diseases (Kerpel-Fronius et al., 2020). For example, Zolgensma® was approved based on a single trial with a small number of patients and quick follow-up. Although rapid approval has opened the way for the possibility of purchasing the drug for severely ill patients, the available data do not provide sufficient clinical information about the long-term therapeutic benefits and safety of Zolgensma® GT (Kerpel-Fronius et al., 2020).

ISSN 2786-4634 (print) ISSN 2786-4626 (online)

Collaboration between society and stakeholders is highly important in the acceptance and follow-up of GTs and in providing real-world data. As the price of orphan GTs increases, there may be a decrease in the treated patient population. The obligatory use of orphan GTs by patients who cannot choose another type of treatment and the fact that these drugs are not reimbursed may cause catastrophic health expenditures. However, at some point, the high prices of orphan drugs must be accepted by society because of the small patient populations of rare diseases. However, increased costs and adverse effects associated with GTs may hinder market growth.

The United Kingdom (UK), which has regional structures and regional bodies that play a strong role in deciding the ethical adoption of GTs, provides an interesting case study. STD reports have been published on Trimvelis, Kymriah, Yescarta, Holoclar, Epifix, and Spherox by the NICE in the UK (Kockaya et al., 2020). According to the data obtained from these reports, all the treatments were recommended by the NICE (Kockaya et al., 2020). In the UK, final pricing and reimbursement (P&R) decisions are made by the Department of Health (DoH) following NICE guidelines. The NICE's decisions are based on value and cost-effectiveness recommendations (Kockaya et al., 2020). The cost-effectiveness value is calculated in terms of QALY rather than the therapy area (Kockaya et al., 2020). Given that the QALY takes into account not only the number of life years but also the patient's quality of life, a value of GBP <30,000 is considered cost-effective (Kockaya et al., 2020). Under the NHS, there is an indefinite period of admissibility when the special commission threshold is GBP 30,000/QALY (Kockaya et al., 2020). In a small patient population, whose threshold increase may be GBP 50,000/QALY, end-of-life treatments are also considered if they increase the survival rate by a minimum of 3 months (Kockaya et al., 2020). Considering this issue, the threshold value for cost-effectiveness may be very high due to the fact that GTs are long-term and high-cost therapies. The fact that the threshold value is too high makes it difficult for either the patient receiving the treatment or the state to pay.

Results. Market Access Examples. Access to gene and cell therapies is an important problem in emerging countries. Access to gene therapies is dependent on HTA and decisions made by payers (Tunis et al., 2021). For example, Turkey, as an emerging country, does not have any approved gene and cell therapies. In Turkey, patients who need such treatment should somehow obtain the drugs from abroad through out-of-pocket payment (Kockaya et al., 2020).

Pharmacoeconomic analyses are required for reimbursement in Bulgaria (Kamusheva et al., 2021). According to the HTA legislation in Bulgaria, at least one positive decision issued by an HTA organization in England, France, Germany, or Sweden is required (Kamusheva et al., 2021). Only one HTA report is prepared and published for ATMPs in Bulgaria (Alofisel®) (Kamusheva et al., 2021). Nusinersen, a type of GT used in the treatment of SMA, is included on the reimbursement list in Bulgaria (Kamusheva et al., 2021).

In Romania, an HTA scoring system is active (Kamusheva et al., 2021). The inclusion of a new INN (international non-proprietary name) on the reimbursement list in Romania is subject to preliminary assessment by the NMMDA (Kamusheva et al., 2021). In this procedure, data needed to calculate therapy costs, summary characteristics of products, compensation statuses in the EU, the prices approved by the Ministry of Health, etc. are taken into account (Kamusheva et al., 2021). Only two ATMPs (Kymriah and Alofisel) have been evaluated by the Romanian HTA organization (Kamusheva et al., 2021).

In Poland, reimbursement decisions are made by the Ministry of Health as of 2021, provided that the Reimbursement Law is met (Kamusheva et al., 2021). There are no exceptions for rare diseases with regard to the cost-effectiveness threshold per QALY and no reimbursement for drugs with excessively high prices (Kamusheva et al., 2021). Three ATMPs (Kymriah®, Yescarta®, and Zolgensma®) were evaluated by the HTA organization in Poland, and only Zolgensma® was rated as a highly innovative product (Kamusheva et al., 2021).

In the pricing of products in South Korea, if the price of a drug is higher than its alternative, economic evaluation data for the drug must be listed in the NHI benefit package (Qiu et al., 2021). When pharmaceutical companies file reimbursement applications, HIRA and the Economic Evaluation Subcommittee review the cost-effectiveness data and ultimately decide on reimbursement pricing based on the relationship between PBCAC, the companies, and the National Health Insurance Service (Qiu et al., 2021).

In 2017, HIRA introduced exceptional criteria that are designed for products that have a positive impact on healthcare, and they may apply to RM. Currently, four cell therapies (Chondron®, Kaloderm®, Cupistem®, and Keraheal-Allo®) are officially approved in South Korea, and the prices of Cupistem® and Keraheal-Allo® are lower than those of alternative drugs (Qiu et al., 2021).

In the UK, however, free pricing is applied throughout the country, and although limitations are set for returns on capital and sales, reimbursement is not guaranteed, especially for innovative treatments (Kockaya

et al., 2020). Therefore, in the UK, a separate price list with the official and reimbursement prices should be established to allow the DoH to contact manufacturers when the treatment is low cost, so they can negotiate (Kockaya et al., 2020).

These findings show that the UK government provides effective HTA reports and that treatments are readily available to the public in terms of their supply, prices, and reimbursement (Kockaya et al., 2020).

A regulatory pathway also exists for gene and cell therapies in France. The EMA (European Medicines Agency) is the regulatory authority for ATMPs (Kockaya et al., 2020). Pricing, reimbursement, and market access in France are overseen by the Transparency Commission (TC) (Kockaya et al., 2020). The TC draws conclusions from actual benefits (SMR) and improvements in actual benefits (ASMR) (Kockaya et al., 2020). It is reported that the French National Health Authority published a HTA report for Holoclar cell therapy in 2016 (Kockaya et al., 2020). HAS (French National Authority for Health); Glybera has published HTA reports for Holoclar, Yescarta, Kymriah, and Zalmoxis (Kockaya et al., 2020; Jørgensen et al., 2020).

In Germany, the Paul Ehrlich Institute plays a role in the management of ATMPs (Kockaya et al., 2020). Clinical benefit assessment based on input from the Institute for Quality and Efficiency in Healthcare (IQWiG) is carried out by the Federal Joint Committee (G-BA) (Kockaya et al., 2020). This assessment forms the starting point for negotiating pricing, reimbursement, and market access for new treatments (Kockaya et al., 2020). The IQWiG has released HTA reports for Glybera, Provenge, Kymriah, Yescarta, and Zalmoxis (Kockaya et al., 2020).

In Germany, the clinical data for Glybera did not support the additional benefits and remained an unquantifiable category, and only one direct hospital negotiation was performed in Berlin in 2015, where the price was EUR 900,000 in an agreement with a German employee health insurer (DAK) (Kockaya et al., 2020).

In Italy, the main decisions are implied by the AIFA, including approval, inclusion, and negotiations (Kockaya et al., 2020). The AIFA has published HTA reports on Strimvelis, Holoclar, and Zalmoxis (Kockaya et al., 2020). The study found that the Italian government has made an agreement to reimburse Strimvelis at a price of EUR 594,000 which is significantly less than that of most long-term therapies (Kockaya et al., 2020). The minimum total cost for the Ablatherm high-intensity focused ultrasound (HIFU) system and other gene treatments was found to be EUR 2,938.60, and the maximum total cost was EUR 4,610.57 (Kockaya et al., 2020).

In Spain, the Spanish Agency of Medicines and Medical Devices (AEMPS) is the main regulatory authority at the national level (Kockaya et al., 2020). Although pricing, reimbursement, and market access are approved by the AEMPS, the decentralization in Spain empowers the regional authorities to engage in second-price negotiation, and variation in decision making and budget constraints add the risk of market access delays (Kockaya et al., 2020).

The findings suggest that gene and cell therapy products in Spain have achieved EMA approval; however, they have still not participated in or made any public statements about ongoing gene and cell therapies (Kockaya et al., 2020).

Spain's HTA program is differentiated from the reimbursement process, but they are closely related (Kockaya et al., 2020). The AEMPS, regional authorities, and the Ministry of Health-DG Pharmacy (HM-DG Pharmacy) are involved in the HTA of pharmaceuticals. Non-pharma assessments can be performed by both regional HTA bodies and through cooperation within the framework of the Spanish Network for HTA, which brings together regional HTA agencies for cooperation on a national level (Kockaya et al., 2020).

When the cases of Bulgaria, Romania, and Poland are examined, the main common difficulties identified in terms of the reimbursement of ATMPs are as follows: high prices, high cost-effectiveness ratios, restricted budgets, low levels of evidence/uncertainty regarding the available evidence, a paucity of centers for excellence and experts, a lack of specific methodological criteria for the HTA of ATMPs, and limited possibilities for follow-up through prospective electronic registries (Kamusheva et al., 2021).

Examples of market access are summarized in table 1.

Table 1. Market Access Examples Summarize

Countries	Products	Status
Bulgaria	Nusinersen®	Reimbursement in Bulgaria
Romania	Kymriah® and Alofisel®	Evaluated by HTA organization in Romania
Poland	Kymriah®, Yescarta® and Zolgensma®	Evaluated by the HTA organization in Poland
South Korea	Chondron®, Kaloderm®, Cupistem®, and Keraheal-Allo®	Officially approved in South Korea

Continued Table 1

Countries	Products	Status
Germany	Glybera®, Provenge®, Kymriah®, Yescarta®, and Zalmoxis®	Published HTA reports
Italy	Strimvelis®, Holoclar®, and Zalmoxis®	Published HTA reports
France	Holoclar®, Yescarta®, Kymriah®, and Zalmoxis®	Published HTA reports

Sources: developed by the authors based on (Kockaya et al, 2020).

Conclusions. The aim of this article was to reveal the difficulties of gene therapies and to offer suggestions to prevent the expensiveness and payment difficulties of the treatments, which are among these difficulties. In light of the data obtained for this research, the budgets set aside for orphan GTs in countries around the world should be reimbursed by the respective governments. However, the very high costs of production and transportation, the scarcity of R&D studies in this area, and the inadequacy of HTA reports constitute an obstacle. Low- and middle-income countries in particular should focus on implementing reliable and nationally oriented programs for HTA and consider the financial scope of gene therapies. Due to these factors, countries are hesitant to launch new products in the market and do not want to supply them. Market access agreements based on real-life data can be established to help end or improve this situation. The extraordinarily high list prices of GTs have alarmed decision makers around the world. More research is needed to improve drug development and to address the challenges at this stage to develop effective and safe treatments for all individuals with rare diseases.

Author Contributions: conceptualization, E. B. K., and G. K.; introduction, E. B. K.; resources, E. B. K.; writing-original draft preparation, G. K. and E. B. K.; writing-review and editing, G. K. and E. B. K.; project administration, G. K.

Conflicts of Interest: Authors declare no conflict of interest.

Data Availability Statement: Not applicable.

Informed Consent Statement: Not applicable.

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Дослідження генної та клітинної терапії в контексті економіки охорони здоров'я

Зі збільшенням кількості досліджень щодо процесу розробки лікарських засобів для лікування рідкісних захворювань генна терапія останнім часом все частіше виходить на перший план. Крім препаратів, які використовують для лікування рідкісних захворювань, розроблено препарати передової терапії. Лікарські засоби передової терапії є сферою, що швидко розвивається. Хоча це не метод лікування, який використовують лише для лікування рідкісних захворювань, його також використовують в онкології та для лікування серцево-судинних захворювань, захворювань опорно-рухового апарату. Регенеративна медицина може бути перспективною у випадках, коли лікарські засоби передової терапії є складними та клінічно невизначеними. Існують різні терапії, пов'язані з регенеративною медициною, і клітинна терапія є однією з них. Генна терапія, клітинна терапія, лікарські засоби передової терапії та продукти регенеративної медицини мають високу ціну від виробника та високу вартість виробництва. Оскільки всі ці методи лікування мають обмежені клінічні докази та високу вартість, їх важко оцінити з точки зору оцінки медичних технологій. Для їх оцінки потрібні особливі умови. Як рішення, витрати повинні бути обмежені, а клінічні розробки мають бути забезпечені у співпраці з суспільством. SAVE (еквівалент врятованого молодого життя) рекомендується для оцінки профілів здоров'я впродовж усього життя використання методів лікування, таких як генна терапія. З метою зменшення бюджетного тягаря генної терапії, рекомендують укладати угоди про вступ, орієнтовані на результат, із виплатами на основі доходу. Примусове використання генної терапії та невідшкодування вартості цих препаратів може призвести до катастрофічних витрат на охорону здоров'я. Автори пропонують різні способи оплати, щоб уникнути катастрофічних витрат на здоров'я. Оплата на основі доходу та оплата на основі результату є одними з цих методів. Також стверджується, що високі ціни повинні бути прийнятні в суспільстві, оскільки генна терапія, яка буде застосовуватися для лікування рідкісних захворювань, буде застосовуватися до невеликої кількості населення.

Ключові слова: генна терапія, регенеративна медицина, лікарські засоби передової терапії, доступ до ринку, клітинна терапія.