

On the approval of the rules for the use of advanced therapy medicinal products as part of the exemption from the standard procedure for the market authorization of a medicinal product, as well as the list of medical facilities eligible to provide treatment under the Hospital exemption

Unofficial translation

Order № KR DSM-240/2020 of the Minister of Healthcare of the Republic of Kazakhstan as of December 8, 2020. It is registered with the Ministry of Justice of the Republic of Kazakhstan on December 10, 2020 under № 21748

Unofficial translation

In accordance with paragraph 7 of Article 243 of the Code of the Republic of Kazakhstan “On Public Health and the Healthcare System” as of July 7, 2020, I hereby **ORDER**:

1. To approve the appended rules for the use of advanced therapy medicinal products as part of the exemption from the standard procedure for the market authorization of a medicinal product, as well as the list of medical facilities eligible to provide treatment under the Hospital exemption.

2. In accordance with the procedure established by the legislation of the Republic of Kazakhstan, the Department of Science and Human Resources of the Ministry of Healthcare of the Republic of Kazakhstan shall ensure:

1) the state registration of this order with the Ministry of Justice of the Republic of Kazakhstan;

2) the posting of this order on the website of the Ministry of Healthcare of the Republic of Kazakhstan after its official publication;

3) the submission of information on the implementation of the measures provided for in subparagraphs 1) and 2) of this paragraph to the Legal Department of the Ministry of Healthcare of the Republic of Kazakhstan within ten working days of the state registration of this order.

3. Control over the execution of this order shall be entrusted to A.Giniyat, deputy minister of healthcare of the Republic of Kazakhstan.

4. This order comes into effect ten calendar days of its first official publication.

*Minister of Healthcare of
the Republic of Kazakhstan*

A.Tsoi

Approved by Order
№ KR DSM-240/2020
of the Minister of Healthcare
of the Republic of Kazakhstan
as of December 8, 2020

Rules

for the use of advanced therapy medicinal products as part of the exemption from the standard procedure for the market authorization of a medicinal product, as well as the list of medical facilities eligible to provide treatment under the Hospital exemption

Chapter 1. General provisions

1. The rules for the use of advanced therapy medicinal products as part of the exemption from the standard procedure for the market authorization of a medicinal product, as well as the list of medical facilities eligible to provide treatment under the Hospital exemption (hereinafter referred to as the Rules) are developed in accordance with paragraph 7 of Article 243 of the Code of the Republic of Kazakhstan “On Public Health and the Healthcare System” as of July 7, 2020 (hereinafter - the Code) and establish the procedure for the use of advanced therapy medicinal products as part of the exemption from the standard procedure for the market authorization of a medicinal product, and also approve a list of medical facilities eligible to provide treatment under the Hospital exemption.

2. The following terms and definitions are used in these Rules:

1) informed consent - a procedure for a person to voluntarily confirm his/her consent to receive medical care and (or) participate in a specific study after receiving information on all aspects of medical care and (or) research that are important to know to make a decision;

2) bioethical examination - consideration of a biomedical research and issuance of a substantiated opinion of the Commission on Bioethics from the standpoint of ethical acceptability, safety for participants and the feasibility of this research;

3) medicinal product - a product that is or contains a substance or a combination of substances coming into contact with the human body, intended for the treatment, prevention of human diseases or the restoration, correction or change of his/her physiological functions through pharmacological, immunological or metabolic effects, or for diagnosing diseases and human conditions;

4) hospital exemption - the procedure for admitting an unregistered medicinal product for clinical use on an exceptional basis in one medical facility for individual indications;

5) sponsor – an individual or legal entity who initiates a clinical trial and is responsible for its organization and (or) financing;

6) research director - a person responsible for the overall conduct of a clinical study of safety for human health and the environment;

7) research center - an organization where preclinical (non-clinical) research, clinical research of drugs, medical devices, clinical and laboratory testing of medical devices for in vitro diagnostics are carried out;

8) clinical trial - a study with human participation as a subject, which is conducted to identify or confirm the safety and effectiveness of products, methods and technologies for the prevention, diagnosis and treatment of diseases;

9) clinical trial protocol (hereinafter referred to as the protocol) - a document describing the goals, design, methodology, statistical aspects and organization of the study;

10) medical facility - a healthcare entity whose main activity is the provision of medical care;

11) advanced therapy medicinal products (hereinafter referred to as ATMP) – medicinal products obtained by biotechnological or bioengineering ways, which offer new opportunities for the treatment of diseases and injuries, including agents for gene therapy, somatic cell therapy, tissue engineering;

12) expert organization - a state expert organization in the field of turnover of medicinal products, medical devices;

14) subject (research subject) - an individual participating in a clinical trial as part of a group receiving an investigational product, or as part of a control group.

Chapter 2.

Procedure for the use of advanced therapy medicinal products as part of the exemption from the standard procedure for the market authorization of a medicinal product

3. Advanced therapy medicinal products are used without conducting a clinical trial as part of the exemption from the standard procedure for the market authorization of a medicinal product, given:

- 1) a positive opinion of a local bioethics commission;
- 2) a scientific basis for the use of an advanced therapy medicinal product for the direct benefit of the patient;
- 3) obtained informed consent of the patient or his/her legal representative for the use of the advanced therapy medicinal product.

The doctor who prescribes the advanced therapy medicinal product shall see to that the specified conditions are met and, based on the results of the use of the advanced therapy medicinal product as part of the exemption from the standard procedure for the market authorization of the medicinal product, submits a report to the state expert organization in the field of turnover of medicinal products and medical devices and the Bioethics Commission in within fifteen working days of the end of the patient's treatment with ATMP.

4. To obtain an opinion on the use of ATMP as part of the exemption from the standard procedure for the market authorization of a medicinal product, an applicant from among the medical facilities specified in paragraph 19 of these Rules shall submit to the local bioethics commission:

- 1) an application for the use of ATMP as part of the exemption from the standard procedure for the market authorization of the medicinal product;

2) data of scientific and clinical trials on the proposed ATMP and the nosologies for the treatment of which it is used, and (or) systematic reviews summarizing the results of clinical trials at the republican and (or) international levels;

3) information on the qualifications of specialists involved in the production and treatment processes;

4) the informed consent form of patients for whom ATMP is used as part of the exemption from the standard procedure for the market authorization of a medicinal product or their legal representatives.

5. As part of the exemption from the standard procedure for the market authorization of a medicinal product, ATMPs are used, which are produced in healthcare entities having appropriate conditions in accordance with the requirements of the Standard of Good Manufacturing Practice (GMP) (hereinafter referred to as the GMP standard) of the standards of good pharmaceutical practices in accordance with subparagraph 9) of Article 10 of the Code.

6. The local bioethics commission considers the application's materials for the use of ATMP as part of the exemption from the standard procedure for the market authorization of a medicinal product in the manner prescribed by standardized operating procedures approved by the Central Commission on Bioethics.

To issue an opinion on the use of ATMP as part of the exemption from the standard procedure for the market authorization of a medicinal product, the following requirements are met:

1) ATMPs are made for a specific patient according to the individual prescription of the attending physician;

2) ATMPs are not manufactured commercially and are made from cells, tissues, or other biological materials. By origin, ATMPs are classified as autologous, allogeneic, or xenogenic;

3) ATMPs are used in the medical facility in which they were prescribed. The use of ATMP, monitoring of results is carried out personally by the doctor who prescribed the treatment;

4) the medical facility has qualified specialists with the skills and abilities to apply ATMP ;

5) the medical facility has premises, equipment and necessary infrastructure for the application of ATMP;

6) the medical facility has conditions to ensure proper compliance with bioethical principles when applying ATMP.

When using ATMP in another medical facility related to the patient's place of residence or other circumstances, the prescription of ATMP is officially confirmed by this facility's attending physician, who directly administers the ATMP. In this case, the monitoring of effectiveness and registration of side effects is the responsibility of the attending physician.

7. The term for the local bioethics commission's consideration of the materials of the application for the issuance of an opinion on the use of ATMP as part of the exemption from the standard procedure for the market authorization of a medicinal product and making a decision to issue or refuse to issue an opinion does not exceed fourteen working days of payment for expert work and the submission of a full list of documents.

8. The local bioethics commission asks the sponsor or applicant for oral or written explanations in case of incomplete disclosure of information on the planned use of ATMP in the submitted documents, and also involves national experts who are not part of the Commission. The applicant is given no more than thirty working days to provide explanations

9. The local bioethics commission communicates its decision to the sponsor or applicant of the clinical trial, who, in case of disagreement with the decision of the LBC, lodges an appeal. An appeal by an applicant who does not agree with the results of the bioethical examination is considered by the commission within a period not exceeding thirty calendar days, with the participation of the applicant himself/herself and the involvement of independent experts. Based on the results of the consideration of the appeal, the Commission makes a final opinion.

10. Based on the results of the bioethics examination, the Local Bioethics Commission makes a decision and issues an opinion of the bioethics examination in the manner approved by the research center, on the basis of which the Local Bioethics Commission was set up.

11. A medical facility applying ATMP as part of the exemption from the standard procedure for the market authorization of a medicinal product shall ensure that all steps in the application of ATMP are documented.

12. Electronic systems are used for registration and processing of data, provided that the reliability, control and protection of these systems are guaranteed against loss of or damage to data, copying, transferring data to other storage systems, while ensuring the availability and legibility of data, the ability to print.

13. The ATMP label (or attached ATMP Instructions for Use) includes the following information:

- 1) the name of the medicinal product,
- 2) the name of the medical facility where the medicinal product was prescribed,
- 3) identification code of the medicinal product and (or) packaging,
- 4) the name of the patient to whom the medicinal product was prescribed,
- 5) doctor's prescription and doctor's registration number,
- 6) the name and quantity of active substances,
- 7) type of cells or tissues,
- 8) dosage form,
- 9) a list of excipients, including canning systems,
- 10) the shelf life of the medicinal product,

- 11) special storage conditions,
- 12) the results of examination for transfusion infections.
- 14. The cells that make up the ATMP are harvested from voluntary and non-remunerated donors.
- 15. ATMP is transported in accordance with the requirements for storage conditions according to the instructions of the ATMP manufacturer.
- 16. The medical facility using ATMP appoints a pharmacovigilance officer for the ATMP , who ensures:
 - 1) the analysis and comparison of all received information on suspicious adverse reactions ;
 - 2) the provision of a report on the effectiveness and safety of ATMP to the manufacturing organization within the first two years of the administration of ATMP.
- 17. The medical facility informs the manufacturing organization of any serious adverse reactions during treatment with ATMP.
- 18. The informed consent of the patient (his/her legal representative) to the use of ATMP indicates the essence of the proposed treatment, the general characteristics of ATMP, the expected results and possible risks of treatment, as well as the potential advantages of ATMP over traditional methods of treatment.

Chapter 3. List of medical facilities eligible to provide treatment under the Hospital exemption

19. Medical facilities eligible to provide treatment under the Hospital exemption include: national centers, scientific centers and clinical research institutes, university hospitals.