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STATE REGULATION OF RELATIONS IN THE FIELD OF CIRCULATION OF MEDICINES INTENDED FOR THE TREATMENT OF RARE DISEASES IN THE REPUBLIC OF KAZAKHSTAN

Abstract. The article deals with the problems of state regulation of relations in the sphere of orphan medicines circulation in the Republic of Kazakhstan. Definitions of the terms «orphan drugs» and «rare (orphan) diseases» are given. The analysis of foreign experience of state stimulation of orphan drug manufacturers is carried out. The reasons for the lack of incentive mechanisms for orphan drug manufacturers in the Republic of Kazakhstan are considered. The legislative base in the field of orphan drugs and rare diseases list is analyzed. The procedures of expertise and registration of orphan drugs are considered. Proposals are made to improve the regulation of relations in the sphere of orphan medicines circulation. For example, the establishment of a rare diseases laboratory in Kazakhstan and the creation of continuity between the child and adult orphan drug distribution system. As a result of the study, a number of conclusions have been made that can contribute to the implementation of the policy in the field of state regulation of relations in the sphere of orphan drugs.

Key words: Medicines, rare diseases, orphan drugs, government regulation, state examination and registration.

Intruduction. Medicines are currently one of the most important arsenals of therapeutic and preventive care. Every year, drugs are becoming increasingly important in the structure of medical services, allowing, in particular, to prevent or treat diseases, maintain a high quality of life in chronic diseases, alleviate the suffering of dying patients, and generally reduce the percentage of disability, increase life expectancy, and improve quality life of the population. Therefore, in society there is a great need for medicines, which continues to increase every year. Based on this, the importance of state regulation of relations in the field of circulation of medicines is growing, as the state is one of the main participants in the pharmaceutical industry.

So, for example, the global cost of prescription drugs in 2019 amounted to 871 billion US dollars. In the next few years, sales will only grow, and by 2024, the figure could reach 1.2 trillion US dollars. One of the fastest growing segments of the global drug market is the orphan drug market. According to experts, the market volume of orphan drugs by 2024 will be $ 262 billion [1]. This is not surprising, the orphan drug market is currently attracting even more interest from large pharmaceutical companies that seek to add orphan products to their portfolio and capture new sources of revenue.

Consider the definitions of the terms "orphan drugs" and "rare (orphan) diseases."

Orphan medicines (orphan medicinal product) - medicines intended solely for the diagnosis or pathogenetic treatment (treatment aimed at the mechanism of the development of the disease) of rare (orphan) diseases [3]. Orphan drugs have long been considered commercially unattractive. The main reason for the unattractiveness was a small number of sick people, some kind of rare disease, uantitatively almost unchanged. Also, pharmaceutical companies require significant investments to conduct clinical research, development and production of orphan drugs. In order to mitigate risks and provide an

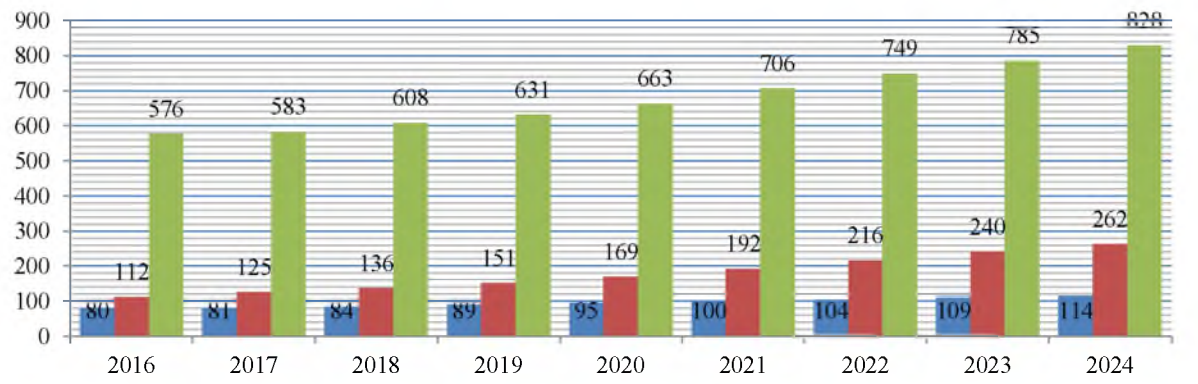

Figure 1 – Worldwide sales of prescription drugs in 2016-2019 and forecast up to 2024 (in billions of US dollars) [2]

opportunity to earn money, many states compensate for the large costs of pharmaceutical companies through allocated preferences, as well as create a favorable legislative framework that helps the orphan drug market to function effectively [4].

Main part. Rare diseases, orphan diseases, orphan diseases (English rare disease, orphan disease) - diseases affecting a small part of the population [5]. Most of the rare diseases are usually of a genetic nature, often occur in childhood, approximately 1/3 of sick patients do not live to the age of five. There is no specific general prevalence of a rare disease in a population at which it would be considered rare. A disease can be rare in one state and at the same time can be spread in the territory of another state [6]. The World Health Organization defines the boundaries of a rare disease if it affects from 6.5 to 10 people per 10,000 people.

When it comes to rare diseases, first of all, we should talk about rarely used medical technologies that dramatically affect the patient. Rarely used technologies have a high level of evidence of effectiveness and vital necessity. Without the use of rare technologies, the patient's inevitable disease progression occurs, complications worsen and develop, which leads to death for the patient. For their designation in the world use the term, "rarely used medical (orphan) technology."

Medicines are developed and circulated on the free market, however, being one of the most important factors for the success of medical interventions, they require the mandatory participation of the state in regulating circulation. The life cycle of a drug consists of several links, each of which is to some extent regulated by the state. The regulatory impact consists both in the creation of certain legislative and organizational-legal documents, and in the direct implementation of services (examination, registration, licensing) and the monitoring of the activities of pharmaceutical and medical organizations (control, supervision).

In the Republic of Kazakhstan, on the part of the state, much attention was paid to solving problems and regulating the drug supply of frequently occurring socially significant diseases. For example, infections, cardiovascular diseases, diabetes, etc. In the past 10 years, the state has begun to actively regulate relations in the field of rare diseases and orphan drugs. Until 2009, state regulation of medicines intended for the treatment of rare diseases was formal. The growing social significance of the problems of patients with rare diseases has made it possible to change the situation for the better.

Unlike Kazakhstan, many foreign countries much earlier began to develop government policies in the field of orphan drugs. So in 1983, the first country to adopt the Orphan Drug Act was the United States of America [7]. Americans were the first to define the boundary of the spread of a rare disease; in the USA, a rare disease is defined as a disease that affects less than 200,000 people, or about 1 in 1,500 people [8]. According to the enacted law, pharmaceutical companies producing orphan drugs provide a number of incentives. For example, pharmaceutical companies can sell orphan drugs without competition for 7 years. Until 2017, federal tax credits accounted for 50% of the cost of conducting clinical trials, now they are 25%. The state provides federal grants for clinical trials of new therapies for the treatment or diagnosis of rare diseases [9].

Manufacturers of orphan drugs do not experience regulatory restrictions when setting prices; prices are determined by market conditions. In 1997, the US Congress exempted pharmaceutical companies that

produce orphan drugs from FDA (Food and Drug Administration) fees [10]. Pharmaceutical companies are entitled to an expedited process for reviewing applications for the sale of orphan drugs. The FDA provides pharmaceutical companies with grants for the development of orphan drugs, and also reduces the cost of the drug registration process. The goal of the grant program is to fund clinical trials that will accelerate the emergence of promising drugs.

At the federal level, criteria for the interchangeability of drugs are established, which focus on the identical pharmacokinetic and pharmacodynamic properties of individual species, taking into account their dose, strength of action, method of use, safety, effectiveness and permitted use. Each state has the right to reduce the number of criteria for interchangeability and thereby increase the range of drugs considered interchangeable. A register of interchangeable drugs is maintained. The country has created a network of regional centers of excellence for research on rare diseases [11]. Financing of the drug supply for patients with rare diseases occurs at the expense of the state or insurance companies.

In Japan, the first Orphan drug law was passed in 1993.

To meet the criteria for an orphan drug, a drug must meet the following requirements:

- the drug should be used to treat a rare disease or condition that affects less than 50,000 people;
- the drug must treat diseases or conditions for which there are no other methods of treatment in Japan, or the proposed clinical drug is superior to drugs available on the Japanese market;
- the applicant must have a clear plan for product development and scientific justification in support of the need for the use of the drug in Japan [12].

Based on US experience, Japan has created its incentive system for pharmaceutical companies. Thus, the Ministry of Health, Labor and Welfare of Japan holds special free consultations for manufacturers of orphan drugs. Pharmaceutical companies may receive financial assistance from the government of Japan to collect additional data, such as clinical trials, additional studies, etc. The applicant can also receive financial assistance up to 50% of the cost of clinical trials, tax benefits in the amount of 6% of research costs and 10% of the company's income. An application for registration of orphan drugs is considered according to the accelerated method, for a period of not more than 10 months. Pharmaceutical companies are granted exclusive marketing rights for 10 years. The cost of the purchase of orphan drugs for patients with rare diseases is borne by the state.

In Australia, the orphan drug policy began in 1997 with the adoption of the relevant law. A program to provide patients with rare diseases was created, guaranteeing a wide selection of orphan drugs. Orphan drugs are controlled by the Australian Therapeutic Goods Administration (TGA). The criterion for the prevalence of a rare disease is not more than 2000 people or commercial non-viability of the drug.

The main feature of the Australian program is that it is based on the close cooperation of the TGA with the US FDA. The Australian program takes into account American drug evaluation experience, and also takes into account orphan drugs that do not meet the FDA criteria.

A feature of the Orphan Drug Policy in Australia is:

1. Good legislative framework;
2. Refusal of registration fees;
3. Five-year marketing exclusivity for orphan drugs.

Regarding the financing of orphan drugs, the TGA covers all costs associated with the drug registration procedure. In Australia, the research and development of orphan drugs is not supported by government grants or tax incentives. Due to the high cost of orphan drugs, benefits are provided to citizens to make some drugs more affordable [13].

In the countries of the European Union (hereinafter referred to as the EU), orphan legislation is based on Decree of the Parliament and the Council of the EU of December 16, 1999 No. 141/2000 on orphan medicines adopted pursuant to the decision of the Parliament and the Council of April 29 of 1999 No. 1295/1999 / EU on a joint program on rare diseases as part of public health action [14]. The borderline for the spread of a rare disease in the EU is different, usually 5 cases per 10 thousand people, in Sweden and Denmark 1 case per 10 thousand people. The European Medicines Agency (EMA) has set up a specialized body, the Orphan Medicines Product Committee (COMP), in London, which is responsible for regulating relations in the field of orphan medicines. EMA reviews the dossier for orphan drugs within 90 days, and the European Commission must decide not later than 30 days to use them [15].

The EU gives preferences to companies developing orphan drugs, in particular, it compensates for the costs of the marketing approval process. By approving the orphan drug, EMA gives the manufacturer the

exclusive right to sell the orphan drug for 10 years and 12 years if the orphan drug is intended for pediatrics. The term may be reduced to 6 years if the sale of the orphan drug brings profit to the manufacturer. When considering applications for granting the status of orphan drug, the services of the relevant state bodies can be provided free of charge or with certain benefits. The EU also provides free assistance in the preparation of the protocol, a 50% reduction in fees at the pre-registration stage, a reduction in all fees by 50% during the first year after the approval of the orphan drug.

Features of regulation of orphan drugs in foreign countries

Indicators	Countries			
	USA	Japan	Australia	The EU
Legislative act	Adopted in 1980	Adopted in 1993	Adopted in 1998	Adopted in 2000
The boundary of the spread of a rare disease	7.5 people per 10 thousand	4 people per 10 thousand	1 person per 10 thousand	5 people per 10 thousand
Exclusive right	7 years	10 years	5 years	10 years
Financial incentives for manufacturers	Clinical Studies 50%	yes	no	Varies by country
Express Registration	yes	yes	yes	yes
Scientific Advice	yes	yes	yes	yes
Reduction of registration fees	yes	yes	yes	Full or partial

As already mentioned, in the Republic of Kazakhstan in recent years the state has been actively working to improve the provision of medicines for patients with rare diseases and improve their quality of life. One of the main problems of providing medicines for patients with rare diseases until 2009 was that the legislative framework governing situations with rare diseases and orphan drugs, as well as expensive technologies for their treatment, was absent. Such concepts as “rarely used medicine” were not legislatively fixed, that is, medical technology, “rare disease”, there was no criterion for classifying diseases as rare diseases.

The first step to rectify the situation was the adoption on September 18, 2009 of the Code of the Republic of Kazakhstan "On the health of the people and the health care system, in it such concepts as" rare diseases "and" orphan drugs "were fixed at the legislative level.

Rare diseases - rare serious diseases that threaten a person’s life or lead to disability, the frequency of which does not exceed an officially determined level.

Orphan (rare) drug - a drug intended for the diagnosis, etiopathogenetic or pathogenetic treatment of rare diseases, the frequency of which does not exceed an officially determined level in the Republic of Kazakhstan.

In the Republic of Kazakhstan, in order to fall under the category of orphan drug, a drug should rarely be used or used in less than 1 person per 10 thousand people. Also, medicines must have a high level of evidence of effectiveness and vital necessity. In the Republic of Kazakhstan, orphan drugs are not produced, the need for orphan drugs is met through imports. Having considered the experience of the USA, Japan, Australia and the EU, two main reasons can be identified for which the production of orphan drugs in the Republic of Kazakhstan is unprofitable for both domestic and foreign pharmaceutical companies:

1. A small number of patients with rare diseases. In Kazakhstan, out of 18.6 million people, less than 1% of the population has rare diseases. Pharmaceutical companies invest the same amount of money in the development, testing and registration of commonly used and rarely used drugs. For the production of large and small batches of drugs, the costs are also approximately the same, but the price of one conventional tablet of orphan drug varies markedly. For example, take a certain amount of X that is spent on developing a medicine, whether it is orphaned or not, X is divided by one hundred people with a rare disease and we get one price of the drug, if X is divided by a million people, a more reasonable price will be obtained. The manufacturer needs to cover the costs of production, and make a profit. For this reason, the costs of orphan drugs can be huge.

2. In the Republic of Kazakhstan, there are no mechanisms to stimulate and encourage manufacturers of orphan drugs. There is no exclusive right to orphan drugs, the state does not apply financial incentives

to pharmaceutical companies with the ability to produce orphan drugs. Scientific consultations are not held, registration fees are not reduced. In developed countries, the production and sale of orphan drugs stimulates the state. Typically, the state takes upon itself the financing of special programs, compensating manufacturers for the production of expensive orphan drugs, so that pharmaceutical companies are interested in investing money not only in production and sale, but also in the development of new drugs. The state also gives tax preferences and increases the term of exclusivity of the orphan drug.

Given the above, government measures are needed to create conditions for pharmaceutical companies that could organize the production of orphan and other medicines in the Republic of Kazakhstan. In 2025, the full functioning of the single market for medicines of the countries of the Eurasian Economic Union (hereinafter - the EAEU) will be implemented. With the market expanding to 183.4 million people, the number of patients with rare diseases will increase, and the number of orphan drugs consumed will increase accordingly. Therefore, in a competitive environment, Kazakhstan needs to be the first of the EAEU countries to create favorable conditions for manufacturers of orphan pharmaceutical companies. Government incentives will create the opportunity to produce original drugs or "generics" for rare diseases at a lower price.

I would like to note that before creating incentive mechanisms for pharmaceutical companies, it is necessary to improve state regulation of relations in the sphere of circulation of orphan drugs. Today, Kazakhstan faces difficulties in compiling lists of orphan drugs and rare diseases. For example, if in the world there are about 7 thousand rare diseases, then in the list of rare diseases there are only 57 nosologies. Accordingly, if a citizen of the Republic of Kazakhstan becomes ill with a disease that has not previously been registered in Kazakhstan, then he will have bureaucratic difficulties in the treatment and access to free orphan drugs. In the list of orphan drugs, not all orphan drugs are also present.

In the Republic of Kazakhstan, the list of orphan drugs was first approved on June 10, 2009 by order of the Minister of Health of the Republic of Kazakhstan "On the Procedure for the Formation of the List of Orphan Medicines". The list was formed in order to ensure accessibility, create a unified order and approve the principles for the formation of orphan drugs. The procedure for the registration of medicines in the list was determined and the stages of consideration by the formulary commission of proposals for the inclusion of orphan drugs in the list were determined. This list has lost its force and has been revised and amended several times. For example, on December 7, 2009, the order of the Minister of Health of the Republic of Kazakhstan No. 831 "On approval of the List of orphan (rarely used) drugs in the Republic of Kazakhstan" was issued, which stated that orphan drugs from the list can be purchased and used in medical practice if not in the state register medicines of the Republic of Kazakhstan. It was also spelled out that the decision on the use of orphan drugs is made by the head of the healthcare organization on the recommendation of the formulary commission of the healthcare organization (or the department head). In order to import an orphan drug that has not been registered in the state register of medicines at a time, you must obtain permission from the state authority in the field of drug circulation.

The procedure for registration and examination of orphan drugs is spelled out in orders No. 735 "Rules of state registration, re-registration and amendments to the registration dossier of a medicinal product, medical devices and medical equipment" and No. 736 "On approval of the Rules for the examination of pharmaceuticals, medical devices and medical technicians "of November 18, 2009. Orphan drugs that are not registered (if justified) in the country of the manufacturer or country of the holder of the production license and registration certificate for the drug are not subject to state registration in Kazakhstan. Also, orphan drugs do not pass state expertise on effectiveness, safety and quality.

If there is insufficient information on the results of preclinical (nonclinical) and clinical trials, by agreement with the applicant, the state body carries out state registration of orphan drugs in the following cases:

– if on the day of filing the application for registration, the level of scientific knowledge does not allow to collect more detailed information;

– if obtaining complete information is contrary to generally accepted principles of medical ethics.

When examining orphan drugs, a positive safety opinion is issued against the obligation of the applicant on the following conditions:

– fulfillment within a certain time frame of a certain research program, the results of which will be the basis for reassessing the benefit-risk ratio;

– the use of the drug under the strict supervision of a physician;

– immediate notification of the state body of any side effects that occurred with the use of the orphan drug, and the measures taken.

In cases where the orphan drug is not registered in Kazakhstan, but is included in the treatment diagnosis protocols, then the state body has the right to allow one-time import of the drug for a particular patient.

Thus, the market for orphan drugs is one of the fastest growing young markets in the pharmaceutical sector. The state occupies an important place in resolving problematic issues that arise both for manufacturers of orphan drugs and for citizens with rare diseases. Since 2009, the Republic of Kazakhstan has been pursuing an active state policy and legal regulation of relations in the sphere of circulation of orphan medicines. The globalization of the drug market is forcing most countries, including Kazakhstan, to implement national drug policies and legislation in accordance with international standards. Therefore, it is necessary to take into account the experience of developed countries in carrying out state policy of regulating and stimulating the market of orphan drugs.

Conclusion. As a result of the study, we can draw a number of conclusions about state regulation of relations in the field of circulation of medicines intended for the treatment of rare diseases in the Republic of Kazakhstan.

Orphan drugs, having a number of specific features (a small number of patients, large investments, difficulty in conducting clinical trials), are an integral part of the pharmaceutical market, requiring special support from the state. The great social significance of orphan drugs requires the government to quickly solve problems by creating conditions conducive to increasing the social and economic efficiency of the functioning of the orphan drugs market.

As foreign experience shows, medicine is not only new innovative technologies, it is primarily innovative technologies in medical management, which allows minimizing government costs and increasing the return on the use of new technologies. The state is able to include regulatory mechanisms, reducing the cost of treating patients with rare diseases and increase the efficiency of investing in this area. The problem of development of orphan technologies concerns not only the life and health of a particular person, it is also associated with the development of the market for innovative technologies in healthcare and related fields with the speed of innovation. Therefore, developing orphan technologies, the state invests in the health care of the future. Without the formation of a state policy in the field of orphan technologies, it is impossible to overcome the lag and develop innovations in healthcare.

State regulation of relations in the sphere of circulation of orphan drugs should primarily be aimed at solving the problems of patients with rare diseases. It is necessary to establish an effective system for the diagnosis of rare diseases in order to quickly provide them with orphan drugs. It is necessary to create your own laboratory for rare diseases so as not to waste time on diagnosis abroad. Incorrect diagnoses and untimely provision with orphan drugs leads to sad consequences for people suffering from rare diseases. Proceeding from this, the state should build an effective system for providing the population with orphan drugs, without bureaucracy and formalism.

It should be noted that the solution of the above issues will ensure the implementation of a policy in the field of protecting the health of citizens, the use of effective methods for the prevention of rare diseases, the detection of diseases at an early stage, diagnosis, treatment, as well as the healing of patients, mortality reduction, fairness and equal access to health care for all to citizens.

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ҚАЗАҚСТАН РЕСПУБЛИКАСЫНДА СИРЕК КЕЗДЕСЕТІН АУРУЛАРДЫ ЕМДЕУГЕ АРНАЛҒАН ДӘРІЛІК ЗАТТАР АЙНАЛЫМЫ САЛАСЫНДАҒЫ ҚАТЫНАСТАРДЫ МЕМЛЕКЕТТІК РЕТТЕУ

Аннотация. Мақалада Қазақстан Республикасында орфандық дәрілік заттар айналымы саласындағы қатынастарды мемлекеттік реттеу мәселелері қарастырылған. «Орфандық дәрілік препараттар» және «сирек (орфандық) аурулар» терминдеріне анықтама берілді. Орфандық дәрілік заттарды өндірушілерді мемлекеттік

ынталандырудың шетелдік тәжірибесіне талдау жүргізілді. Қазақстан Республикасында орфандық дәрілік заттарды өндірушілерді ынталандыру тетіктерінің болмауының себептері қарастырылды. Орфандық дәрілік заттар мен сирек кездесетін аурулар тізбесі саласындағы заңнамалық база талданды. Орфандық дәрілік препараттарды сараптау және тіркеу процедуралары сарапталды. Орфандық дәрілік заттар айналымы саласындағы қатынастарды реттеуді жетілдіру бойынша ұсыныс берілді. Аталған ұсыныс Қазақстанда сирек кездесетін аурулар бойынша зертхана құру және балалар мен ересек орфандық дәрілік заттарды тарату жүйесі арасында сабақтастық құру мәселесі ретінде көрсетілді. Жүргізілген зерттеу нәтижесінде орфандық дәрілік препараттар саласындағы қатынастарды мемлекеттік реттеу саласындағы саясатты іске асыруға ықпал ететін бірқатар қорытындылар жасалды.

Қазақстан Республикасында қазіргі кездегі өзекті мәселелердің бірі сирек кездесетін ауруларды емдеуге арналған дәрі-дәрмектерді тіркеу және сараптау тәртібін жетілдіру болып саналады. Тіркеу есірткінің өмірлік циклiндегі есірткі қолдануды қалыптастыру мен қолдану кезеңі арасындағы маңызды кезең, мемлекеттің ажырамас функциясы болып саналады және клиникалық тәжірибеде сатуға және қолдануға рұқсат беру туралы барлық зерттеулердің нәтижелерін жан-жақты бағалауға және шешім қабылдауға арналған. Дәрілік заттарды тіркеу мемлекет тарапынан фармацевтикалық нарықты реттеудің негізгі буыны саналады: біріншіден, сатуға мақұлданған дәрілік заттардың номенклатурасын атап өткен жөн, екіншіден, олардың тиімділігі мен қауіпсіздігі, үшіншіден, сапаның фармаңевтикалық аспектілері, сондай-ақ сату шарттары және т.б. Көп елде тіркеу нарыққа кіру ретінде анықталады.

Орфандық дәрі-дәрмекпен қамтамасыз етуді мемлекеттік реттеу тетіктерінің тиімдірек жұмыс істеуі үшін басқа елдердің жүйелеріне сәйкес келетін жетілген заңнамалық база қажет, оған клиникалық зерттеулер мен орфандық өндірушілерді ынталандыру кіреді. Мемлекет салық жеңілдігін беруі қажет, патенттік және маркетингтік құқықтарды қорғауы керек, клиникалық зерттеу бағдарламаларын қаржыландыруға көмек көрсетуі тиіс, ең бастысы, орфандық дәрілерді өндірушілерге әкімшілік және бюрократиялық кедергілер алып тасталуы тиіс. Мемлекет өндірушілерге қолайлы жағдай жасап, ЕАЭО елдерінің дәрі-дәрмектің бірыңғай нарығына шығу мүмкіндігімен фармаңевтикалық компанияларды Қазақстан нарығына тарта алады.

Мемлекеттік тіркеу емделушіге тиімді және қауіпсіз дәрі-дәрмектердің қолжетімді екендігінің кепілі болып саналады. Сондықтан, мемлекеттік тіркеу мемлекеттік стандарттарға, клиникалық сынақтарға сәйкес клиникалық сынақтарды өткізу, болашақ сериялық өндірістің қасиеттерін реттейтін нормативтік құжаттарды жасау жағдайын, сондай-ақ клиникалық қолдануға арналған нұсқаулықтардың болуын бақылауды қамтамасыз етеді. Біріншіден, дәрілік заттың тиімділігі мен қауіпсіздігін қамтамасыз ету үшін тіркеу, аз дегенде, клиникалық зерттеулерде белгіленген деңгейде болуы қажет. Сонымен қатар, тіркеу жан-жақты және профилактикалық сипатқа ие болуы тиіс, оның мақсаты – сатуға рұқсат беру мәселесін шешуде әр препарат үшін пайдасы мен тәуекелінің арақатынасын бағалау. Осылайша, препарат сапасының фармаңевтикалық аспектілеріне қатысты пайымдағанда, алдымен жеке үлгілердің сапасын тексеруден бөлек, оның өндірістік үдерісінің сенімділігін анықтау үшін тіркеу қажет.

Түйін сөздер: дәрі-дәрмек, сирек кездесетін ауру, орфандық препарат, мемлекеттік реттеу, мемлекеттік сараптама және тіркеу.

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ГОСУДАРСТВЕННОЕ РЕГУЛИРОВАНИЕ ОТНОШЕНИЙ В СФЕРЕ ОБРАЩЕНИЯ ЛЕКАРСТВЕННЫХ СРЕДСТВ, ПРЕДНАЗНАЧЕННЫХ ДЛЯ ЛЕЧЕНИЯ РЕДКИХ ЗАБОЛЕВАНИЙ В РЕСПУБЛИКЕ КАЗАХСТАН

Аннотация. В статье рассмотрены проблемы ғосударственноғо регулирования отношений в сфере обращения орфанных лекарственных средств в Республике Казахстан. Даны определения терминам «орфанные лекарственные препараты» и «редкие (орфанные) заболевания». Приведен анализ зарубежноғо опыта государственного стимулирования производителей орфанных лекарственных средств. Рассмотрены причины отсутствия механизмов стимулирования производителей орфанных лекарственных средств в Республике Казахстан. Проанализирована законодательная база в области перечня орфанных лекарственных средств и редких заболеваний. Рассмотрены процедуры экспертизы и регистрации орфанных лекарственных препаратов. Даны предложения по совершенствованию регулирования отношений в сфере обращения орфанных лекарственных средств. Например, создание в Казахстане лаборатории по редким заболеваниям и создание преемственности между детской и взрослой системой распределения орфанных лекарственных средств. В результате провиденного исследования сделан ряд выводов, которые могут способствовать

реализации политики в области государственного регулирования отношений в сфере орфанных лекарственных препаратов.

В Республике Казахстан одной из актуальных проблем, существующих в настоящее время, является совершенствование процедуры регистрации и экспертизы лекарственных средств, предназначенных для лечения редких заболеваний. Регистрация – это важный этап в жизненном цикле лекарственных средств, который лежит между этапами создания и этапами применения лекарств, она является неотъемлемой функцией государства и предназначена для комплексной оценки всех результатов проведенных исследований и принятия решения о возможности допуска к продаже и применения в клинической практике. Регистрация лекарственных средств является главным звеном регулирования государством фармацевтического рынка по многим его параметрам: во-первых, следует отметить номенклатуру допущенных к продаже лекарственных препаратов, во-вторых, их эффективность и безопасность, в-третьих фармацевтические аспекты качества, а также условия реализации и многое другое. В большинстве стран процедура регистрации определяется как допуск на рынок.

Для более эффективного функционирования механизмов государственного регулирования лекарственного обеспечения орфанными препаратами необходима хорошая законодательная база, соответствующая системам других стран, которая включала бы проведение клинических испытаний и стимулирование орфанных производителей. Государство должно предоставлять налоговые привилегии, защищать патентные и маркетинговые права, оказывать содействие в финансировании программ клинических исследований и, что самое важное, не ставить административных и бюрократических барьеров для производителей орфанных препаратов. Создав благоприятные условия для производителей, государство сможет привлекать фармацевтические компании на рынок Казахстана с возможностью выхода на единый рынок лекарственных средств стран ЕАЭС.

Государственная регистрация выступает гарантией того, что пациентам будут доступны эффективные и безопасные лекарственные препараты. Поэтому государственная регистрация предусматривает контроль за условиями проведения соответствующих государственным стандартам доклинических исследований, клинических испытаний, создание нормативных документов регламентирующих свойства будущей серийной продукции и также наличие инструкции по клиническому применению. Прежде всего, регистрация обязана обеспечить эффективность и безопасность лекарственного средства как минимум в пределах, установленных клиническими испытаниями. Помимо этого, регистрация должна нести в себе комплексный и профилактический характер, целью которой должна быть оценка соотношение пользы и риска по каждому препарату для решения вопроса о его допуске к продаже. Таким образом, касаясь фармацевтических аспектов качества препаратов, регистрация, прежде всего, нужна для определения надежности процессов их производства, но никак не на проверку качества отдельных образцов.

Ключевые слова: лекарственные средства, редкие заболевания, орфанные препараты, государственное регулирование, государственная экспертиза и регистрация.

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