IMPLEMENTATION OF EMICZUMAB AS A TOOL FOR THE TREATMENT OF HEMOPHILIA IN UKRAINE

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Introduction. Hemophilia belongs to the group of hemorrhagic diatheses, the common feature of which is increased bleeding. Patients with hemophilia have a congenital blood coagulation disorder due to a deficiency of the coagulation factors FVIII (hemophilia A), FIX (hemophilia B), or FXI (the rarest type — hemophilia C). This causes a violation of the formation of a stable blood clot in the lumen of the bleeding vessel. The type and severity of the course of hemophilia is inherited by an autosomal recessive trait, inoculated with the sex X-chromosome. [1]

In recent years, world medicine has seen significant changes in the management of patients with hemophilia, in particular, innovative drugs have become available that enable patients to live a normal, fulfilling life.

The prevalence of hemophilia in various countries of the world, including Ukraine, is approximately the same - 24.5 cases of hemophilia A per 100,000 newborn boys, and hemophilia B - almost 5 times less. There are about 418,000 patients with severe forms of hemophilia A and B in the world. However, if the number of patients with mild and moderate hemophilia is added to the total number of these patients, it will exceed 1.1 million. There are more than 2,000 patients registered in Ukraine with hemophilia (most of them have a severe form of the disease).

Taking into account the hereditary genesis of the disease, today many works

are devoted to the search for points of influence on the genes responsible for the development of the disease. In general, the development of hemophilia therapy can be divided into several stages:

1. The use of concentrates of blood coagulation factors or recombinant factors, which to this day remains the basic method of treatment.

2. Introduction of non-factorial drugs.

3. Study of drugs that act at the genetic level (clinical studies are currently underway), when using these drugs, the transition of hemophilia from a severe form to a mild one is possible.

Aim. To assess the prospect of introducing Emicizumab for the treatment of hemophilia A in Ukraine.

Object and research methods. We worked out and analyzed the domestic and foreign literature on research on the role and effectiveness of Emicizumab. The search included articles from international medical libraries such as Cocraine, PabMed, etc.

The results. Previously, only plasma-derived coagulation factor concentrates and recombinant coagulation factors were used to treat patients. That is, the therapy was based on the introduction of a deficient blood coagulation factor into the patient. Prophylactic use of factor VIII concentrate involves intravenous administration of the drug 3 times a week, which corresponds to 156 injections during the year. In hemophilia B, coagulation factor IX is administered 2 times a week, which corresponds to 104 injections per year. This creates a great burden on the sick child and his family. Long-acting drugs with a longer half-life are now also available, which reduces the frequency of their use, but the method of administration of the drug remains the same.

Very recently, the era of so-called non-factor drugs, which differ in a fundamentally new mechanism of action, began - biospecific monoclonal antibodies, to which emicizumab belongs. These agents start the blood clotting mechanism and thereby eliminate the factors associated with increased bleeding. The great advantages of non-factor drugs are the duration of action (up to 28 days), which

reduces the frequency of use, and a more convenient method of administration (subcutaneous injection).

In the USA, the drug emicizumab (emicizumab) was approved, which can reduce the amount of bleeding in hemophilia A by 87%. The registration certificate was issued to the company Genentech (part of Roche). The drug is approved as a prophylactic. It should be injected subcutaneously once a week. The high efficiency of the new drug is confirmed by clinical studies among patients with hemophilia who have stopped responding to standard therapy. In particular, in 62.9% of patients receiving emicizumab, the number of bleeding episodes decreased to zero. Another study found that with the new therapy, 87% of participants under the age of 12 stopped bleeding by 38 weeks. [2]

Currently, about 30 children in Ukraine receive Emicizumab, which became available to patients last year. It should be noted that even during this short period, the quality of life of children and their families significantly improved compared to children receiving traditional treatment. This is due to both a more convenient subcutaneous method of administration and a lower frequency of medication use.

Biospecific antibodies are a new method of treating hemophilia A, introduced very recently. Great Britain has the greatest experience in the use of non-factorial drugs, where they are used in a large number of patients. In other European countries, the availability of Emicizumab is not much higher than in Ukraine, and in some it is at the same level or even lower.

The inclusion of Emicizumab in the nomenclature of medicinal products to be purchased under the budget program in Ukraine (for children) was preceded by trouble work. However, the pharmacoeconomic analysis showed the obvious advantages of Emicizumab for patients with hemophilia, because against the background, at first glance, expensive therapy, the need for the use of additional treatment methods, the number of hospitalizations, and therefore the burden on the health care system decreases. Therefore, today we can say that the availability of modern methods of treatment of hemophilia A in Ukraine is approximately at the same level as in other developed European countries. [3]

Conclusions. Currently, Emicizumab is not included in the list of medicines that will be purchased under the budget program in Ukraine for adults, but a group of experts is currently working on this issue. We hope that in the near future Emicizumab will also be purchased for patients older than 18 years under managed access contracts.

REFERENCES

1. https://umj.com.ua/uk/publikatsia-178680-gemofiliya-perspektivi-likuvannya-za-dopomogoyu-gennoyi-terapiyi

2. https://www.vz.kiev.ua/novyj-preparat-dlya-likuvannya-gemofiliyi/

3. https://www.uzhnu.edu.ua/uk/news/haemoph.htm