

од, суцільна вибірка рецептів для лікування ССЗ становила 10524 рецептів на суму 1172 969,21 грн.

За результатами АТC/DDD аналізу, пацієнтами було спожито 783 167 DDDS, з яких найбільшу кількість становлять препарати еналаприлу 142 384 DDDS (18,2%), клопідогрелю 124 625 DDDS (15,9%) та спіронолактону 113 274 DDDS (14,5%). Із суцільної вибірки проаналізованих рецептів, для пацієнтів із сільської місцевості сімейними лікарями було виписано 91,5% (9628 рецептів) і лише 8,5% (896 рецептів) для пацієнтів з міста, що свідчить про зростання доступності до лікарських засобів (ЛЗ) для цієї категорії населення.

Згідно проаналізованих даних, при старті програми у 2017 році 81,1% пацієнтів отримували ЛЗ без додаткової доплати. За період 2018- початок 2019 року питома вага пацієнтів, що отримували ЛЗ без доплати становила 60,7-61,8% відповідно, оскільки зменшилась кількість безоплатних ЛЗ в Реєстрі лікарських засобів, вартість яких підлягає відшкодуванню.

Висновки. Проаналізувавши вибірку більше 10 тисяч відпущених рецептів у програмі «Доступні ліки» за 2017-2020 роки на рівні конкретної аптеки, нами встановлено зростання кількості відпущених ЛЗ для лікування ССЗ для різних груп населення, визначено, які препарати займають домінуючу частку за частотою відпуску. Запропоновано аптечним закладам, враховуючи дані АТC/DDD аналізу, мати відповідні запаси препаратів еналаприлу, клопідогрелю та спіронолактону для безвідмовного забезпечення пацієнтів в урядовій програмі «Доступні ліки».

CONSOLIDATED ASSESSMENT OF ORPHAN PATIENTS REGISTRATION IN UKRAINE

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Introduction. WHO reports that nowadays in the EU, a disease is considered to be rare when the number of people affected is less than 5 per 10 000. There are between 5 000 and 8 000 rare diseases, most of them with a genetic basis. 1 A very rough estimate would be that one out of 15 persons worldwide could be affected by a rare (“orphan”) disease – 400 million people worldwide, of whom 30 million are in Europe and 25 million in the United States.

Rare diseases are serious chronic diseases, and may be life-threatening. In Ukraine rare disease is the illness caused less than 1 person per 2000 - the equivalent of approximately 1 patient in 10,000 persons.

In Ukraine, great importance is attached to the providing of orphan patients, in particular, the regulatory framework functions and some state guarantees are fulfilled, thus, the pharmaceutical providing of patients with certain nosologies of rare diseases.

In our previous publications, lists of nosologies (7 groups) and the level of satisfaction of demand (73-100%) in drugs and medical devices were described. At the same time some significant problems remain in the access of orphan patients to pharmaceutical supply. In particular therapy of some rare nosologies is not predicted by budgeting programs.

Research methods. List of rare diseases in Ukraine with registered patients has been assessed using operative data of public patients organizations.

Results of the research. The available data allowed to form a list of rare diseases, from a sample of patients who received therapy according to certain indications in health care facilities. This list has consisted of 22 diseases: Hemophilia, Cycoscidosis, Primary (congenital) immunodeficiency, Nanism of various origins, Juvenile rheumatoid arthritis, Phenylketonuria, Gaucher's disease, Mucopolysaccharidosis, Bullosis epidermolysis, Pulmonary arterial hypertension, Tyrosinemia, Pump's disease, Fabri's disease, Idiopathic family dystonia, Spinal muscle atrophy, Tuber sclerosis, Imperfect osteogenesis, Crown's disease, Wilson-konovalov's disease, Children's dialysis, Chronic viral hepatitis (children), Cerebral palsy (children)

Thorough analysis of the legislation allows to conclude, that patient with some diagnosis may not receive proper pharmacotherapy at of state funding. in particular, according to the current budget program approved by the order of the Ministry of Health of Ukraine № 2178, there is no provision for the purchase of drugs and medical devices under the following nosologies: Idiopathic family dystonia, Spinal muscle atrophy, Tuber sclerosis, Imperfect osteogenesis, all of wich are serious disease and require high-cost long-term therapy.

It should be noted that a serious difficult in collecting data about rare patients is an absence of Orphan patients' State Register (Orphans Register). Developing and implementation of The Orphans Register is provided by the National strategy for prevention, diagnosis and treatment of rare (orphan) diseases 2020-2025, development of which should begin at the initiative of the Minister of Health voiced in May 2020.

Conclusions. Rare diseases present fundamentally different challenges from those of more common diseases. This is most apparent during the clinical development stage when rarity significantly complicates the task. Problems include the small number of patients, limited clinical expertise and expert centres and others.

Despite the large number of qualitative developments in the issue of medical and pharmaceutical support of orphan patients in Ukraine, the issues of improving the pharmaceutical support of this category of patients is on time. It is fair to say, the same problem is inherent in the health care systems of other countries.

Often it is families of sufferers of rare conditions that end up bearing the burden of managing the costs of therapy and care, due to the lack of affordable treatments. This burden is excessive, and cannot be fairly managed in any way other than through the insurance provided by a universal health system. However, patients are finding that even where new therapies become available overseas, they cannot access them due to the high cost and lack of government funding.

There are community and other forms of charity in the world that provide programs to support the improvement of the quality of life of people with rare diseases, such as travel grants, education, necessity and necessity for the normal functioning of regional government, while the state will not provide support to ensure safety and time of therapy, especially at the expense of the budget for this category of patients.

Solving of existing issues is possible by cooperation of practical, scientific and educational potential in our country using most effective approaches from international practice.