PLN 100 million for innovative projects in the area of rare diseases

Over 100 million PLN will be distributed by the Agency for Medical Research for the realization of 12 projects connected to rare diseases. This will provide an opportunity to develop new treatments for conditions that remain difficult to diagnose and treat due to their rarity.

The Medical Research Agency's competition for non-commercial clinical trials related to rare diseases attracted 39 applications. After a thorough analysis of each of them, independent experts selected 12 that will receive funding over the next few years in the total amount of approximately PLN 102 million. The list of beneficiaries of the competition has just been presented by Radosław Sierpiński, PhD, President of the Agency and Sławomir Gadomski, Deputy Minister from the Ministry of Health.

As Radosław Sierpiński, Ph. D. , assured, within the next few weeks all agreements with beneficiaries will be concluded, which will enable the transfer of first amounts of money allowing for the purchase of research equipment.

"The essence of the competition is to support non-commercial scientific institutes and academic centers in finding new treatments for rare diseases using medicinal products that are already available on the market," says President Sierpinski. The aim of the Agency is to help those patients for whom the current treatment options often do not offer any solutions. In this aspect, we place great hope in the internationalisation of the conducted research and establishing cooperation with foreign centres," he added.

Rare diseases, life-threatening or chronically disabling, occur once in every 2,000 people or less. In contrast, conditions that affect one person in 50,000 or fewer are called ultra-rare. Until now, their severe and chronic course has also been accompanied by lack of public awareness and de facto exclusion from the health care system, associated with very high costs of diagnostics and medical care.

To improve the situation of this group of patients, the Ministry of Health has developed a National Plan for Rare Diseases. It envisages, among other things, the creation of expert centres, broad access to medicines and the creation of a Register of Rare Diseases. Additionally, a passport will be developed for patients with rare diseases that often require complex treatment and interdisciplinary care. As assured by Deputy Minister Sławomir Gadomski, the government should approve the entire project by the end of the holidays.

"The Medical Research Agency was established relatively recently , but the effects of its activities are already impressive, as exemplified by the competition just concluded, implementing the objectives of the National Plan for Rare Diseases. More than PLN 100 million for non-commercial clinical trials is an enormous and unprecedented amount. Poland, from a country only observing world breakthroughs in

the treatment of rare diseases, is becoming a leader in the development of innovative therapies" - emphasized Deputy Minister Gadomski.

The winning projects touch the areas of oncology, cardiology, urology, gynaecology and obstetrics, clinical neurology and haematology. However, as the president of ABM stressed, the real beneficiaries of the competition will be the patients themselves. The primary goal of all research teams is to develop treatments that are universally accessible and have measurable effects.

Among the distinguished initiatives was the "BraimTOR" project coordinated by Joanna Trubicka, MD - professor at the Institute "Child Health Center Pomnik". Her team focuses on pediatric oncology and neurology. Childhood cancers, although classified as rare diseases, are the second most common cause of death in the youngest patients. They are sometimes caused by deregulation of the mTOR pathway, which also causes other conditions such as drug-resistant epilepsy or neurodevelopmental disorders of the autism spectrum. The Institute's BraimTOR experts hope that repamycin may improve the prognosis for this group of patients. ABM funding will allow 100 patients between the ages of 2 and 18 to receive appropriate therapy.

Another beneficiary of the MRA competition, the "REGBONE" project, implemented at the Institute of Mother and Child in Warsaw, focuses on the treatment of ultra rare primary bone tumors in children and adolescents. About 20 patients a year do not respond to first-line treatment, with a prognosis of 15 to 20 percent. As Prof. Anna Raciborska from the Institute of Mother and Child explained, the scientists want to test the effectiveness and safety of regorafenib in a group of 30 patients aged from 2 to 21 years.

Scientists from the Medical University of Gdańsk have received funding for the development of the "POLPHITT" project, which deals with the treatment of an extremely rare liver tumour in children and adolescents - embryonal hepatoma and hepatocellular carcinoma. Approximately 45 patients under the age of 18 are to be studied for the effectiveness of reducing chemotherapeutic treatment while intensifying the use of new, innovative drugs. As emphasized by Prof. Piotr Czauderna from the Medical University of Gdansk, the research will be conducted on a global scale, in cooperation with research centres in the EU, Japan, USA and Canada.

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