

Medical Research Agency

<https://abm.gov.pl/en/news/52,Medical-Research-Agency-will-donate-PLN-100-million-for-research-on-rare-disease.html>
27.04.2024, 05:17

Medical Research Agency will donate PLN 100 million for research on rare diseases

There are several thousand rare diseases and up to 8% may be affected. population. 80 percent. of which are genetic. Next year, the Medical Research Agency plans to allocate PLN 100 million to expand access to innovative therapies for these diseases, among others.

According to the definition, a rare disease is a disease with an incidence of one in 2,000. births or less frequently. " However, many of these are ultra-rare conditions that occur at a rate of once in a million births or even less frequently. They share common features: severe course, high cost of diagnosis and treatment. They also often mean a significant reduction in life expectancy, physical and sometimes intellectual disability," says prof. Anna Latos-Bieleńska, head of the Department of Medical Genetics at the Medical University of Adam Mickiewicz in Poznań. The Karol Marcinkowski University of Poznań.

More than 50 percent. Rare diseases are diagnosed in childhood, 30% in childhood. patients die before the age of 5. These conditions account for approx. 30 percent. hospitalizations in children's wards and 10-25 percent. chronic diseases in adults.

Rare diseases have been identified as a priority area for public health action in the European Union. " And while they're rare, the fact that we know 6,000 to 8,000 of them will make up to 6 to 8 percent ill. EU population. 80 percent. of these are genetic disorders, which indicates the direction of scientific research in this area," adds prof. Latos-Bieleńska.

The researcher gave a short lecture on rare diseases, as part of a panel devoted to them, during the conference "Health priorities of Poles in 2021". The event was organized by the Medical Research Agency. Although rare diseases are so numerous, there is little knowledge about them among doctors and the public. " Therefore, many patients are doomed to what we call <<diagnostic odyssey>>. I know of cases like this where it took 20 years for the proper diagnosis to be established. The average is 5 years," explains prof. Latos-Bieleńska.

Talking about the plans of the Medical Research Agency, Krzysztof Górski, director of the MRA Project Funding Division, said that rare diseases have been at the heart of the Agency's work since its inception.

Medical Research Agency has held two editions of non-commercial clinical trial competitions to date, this year's had three rounds. Out of 300 projects submitted, 19 were related to rare diseases. In the end, 10 received funding. " This gives a total of about PLN 130 million. Projects related to rare diseases were additionally awarded by us, we paid more attention to them than to others"- emphasizes the director Krzysztof Gorski.

In connection with the finalization of the National Plan for Rare Diseases, whose task is, among others.

Improving diagnostics and access to treatment of these diseases, as well as seeing the need to further stimulate the development of Polish scientists in this area, the Agency is planning in early 2021 (in January or February) to announce a competition for non-commercial clinical trials dedicated exclusively to rare diseases. The assumed budget of the competition is PLN 100 million. "With it, we want to achieve results of two kinds. Short term: each project should develop at least two new diagnostic and therapeutic regimens for the disease entity concerned. In the six years following the completion of the project, we would like to achieve long-term results, i. e. to improve the care of patients with rare diseases, and we also want to maintain cooperation between the entities involved in the project," says Gorski.

This stimulation of scientific collaboration should also take place with the involvement of the patient community. ABM wants them to be part of research consortia.

"The National Plan for Rare Diseases has already outlived many health ministers and was born in pain. That is why we welcome the initiative of the Medical Research Agency to support the area of rare diseases" - says Szymon Chrostowski, President of the Management Board of the Foundation Winning Health. Chrostowski, who represents one of the patient organisations, expressed great satisfaction with the plans to involve this community in clinical trials for rare diseases.

"I am very pleased that this plan includes research to improve diagnosis in rare diseases. This is an extremely important problem. Of course we are aware that in rare diseases we do not catch up with the pace of development of new therapies that is available to patients with more common diseases, but the initial issue is the diagnostic problem, the aforementioned "diagnostic odyssey" - comments prof. Katarzyna Kotulska-Jóźwiak from the Department of Neurology and Epileptology of the Children's Memorial Health Institute.

The need for greater control of the number of patients than so far has been pointed out by prof. Wojciech Młynarski, head of the Department of Pediatrics, Oncology and Hematology at the Medical University of Lodz. " It is worth thinking about setting up a register for a rare disease. Centers that deal with them sometimes have registries, but informal ones. Formalizing this under the auspices of the Agency would stimulate research on rare diseases," emphasizes Prof. Młynarski.

[Previous Page](#)

[Next Page](#)