



**Prof. Wojciech Fendler, MD, PhD**

Medical doctor President of the Medical Research Agency Author of several hundred scientific publications in renowned journals, including *Diabetes Care*, *Science Translational Medicine*, *Nature Communications*, and *Cell Reports*. He has served, or is currently serving, as a director or scientific supervisor in more than 20 projects funded by national and European Union sources. He is the recipient of numerous prestigious awards, including scholarships from the Polpharma Scientific Foundation and the Ministry of Science and Higher Education (MNIŚW), the START scholarship of the Foundation for Polish Science (twice), and the 2020 National Science Center Award in the life sciences.

wojciech.fendler@abm.gov.pl



**Joanna Kornacka, PhD, Eng.**

Doctor of Engineering and Technical Sciences and Senior Specialist for Clinical Research at the Medical Research Agency. She combines engineering expertise with medicine, supporting the development of clinical trials and projects aimed at improving healthcare quality.

joanna.kornacka@abm.gov.pl

# FROM AN IDEA TO THERAPY

Clinical trials are a crucial stage in translating scientific advances into innovative therapies. Here, responsibility for human health and life is present at every step.

**Wojciech Fendler**

Medical Research Agency, Warsaw  
Medical University of Łódź  
Dana-Farber Cancer Institute, Boston

**Joanna Kornacka  
Justyna Kutyba  
Elżbieta Bylina  
Zuzanna Nowak-Życzyńska**

Medical Research Agency, Warsaw

Without clinical trials, we would not have pharmacies on every corner with shelves filled with proven medications. We would not have detailed package leaflets explaining dosage and warning of potential side effects, and physicians would be forced to administer treatments “on a hunch,” as they did centuries ago. Clinical trials are the foundation of modern medicine, as they make it possible to verify the effectiveness of new drugs, therapies, and medical procedures.

These processes are complex and lengthy, governed by numerous procedures – not out of a preference for bureaucracy, but to maximize patient safety, ensure ethical standards, and properly assess whether a new therapy is truly effective.

## Why Do Clinical Trials Take So Long?

Clinical trials typically take 10–15 years from the initial concept to drug approval (Fig. 1). Each clinical trial is carefully designed by interdisciplinary teams

of specialists, including physicians, pharmacists, statisticians and ethics experts. Before a patient receives an investigational product, the substance undergoes preclinical testing in laboratories and animal models. Only after positive results are obtained can research involving human participants begin. The development of new drugs includes assessments of safety and dosage, verification of efficacy, and, finally, detailed observation in the target population. Every clinical trial must obtain, among other approvals, the consent of an independent bioethics committee – a panel of experts who evaluate whether the trial is safe, justified, and conducted with full respect for patients’ rights.

Trials involving patients include participant recruitment, implementation of medical procedures in accordance with the protocol, systematic monitoring of safety and progress, and analysis of results (Fig. 2). In some cases, this process may begin with the so-called phase 0, which involves preliminary pilot studies on very small groups of healthy volunteers. This allows for an early assessment of the drug’s behavior in the human body and comparison with the results of preclinical studies. Although phase 0 has no therapeutic purpose and is not mandatory, it provides preliminary pharmacokinetic and pharmacodynamic datasets (i.e., data regarding the fate of the drug in the body and its effects on the body), which can help accelerate decisions about further project development. All stages are meticulously documented and subjected to audits and quality control, both internal – by the sponsor or research center – and external, e.g., by the Office for Registration of Medicinal Products, Medical Devices and Biocidal Products (*Urząd Rejestracji Produktów Leczniczych, Wyrobów Medycznych i Produktów Biobójczych* – URPL), bioethics committees, or international regulatory institutions. Only a positive result in a given phase allows progression to the next phase, which significantly prolongs



Fig. 1 Diagram illustrating the stages of drug development, from laboratory research through the phases of clinical trials to registration and market authorization (Patient in Clinical Trials. Information leaflet; *Pacjent w badaniach klinicznych. Folder informacyjny*)

### Start of clinical research on a new drug: clinical trial (phases)

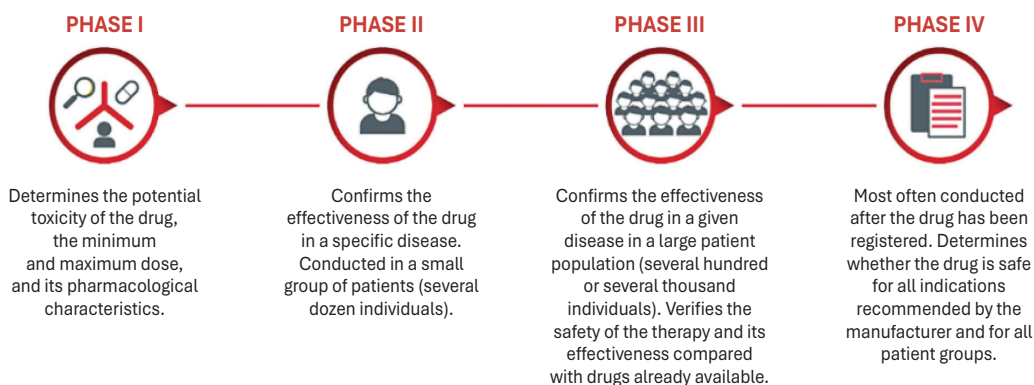


Fig. 2 Phases of clinical trials of a new drug (Patient in Clinical Trials. Information leaflet; *Pacjent w badaniach klinicznych. Folder informacyjny*)

the overall process but is necessary to ensure patient safety and the reliability of the results obtained.

## How Does the Safety Monitoring and Quality Control System Look Like?

Modern clinical trials are founded on full respect for the dignity and rights of participants, who are treated as partners in the research process. In practice, this means that each patient receives reliable information,

provides informed consent, and is allowed to withdraw from participation at any time without consequences for further treatment. Until a few decades ago, such standards were not in place; the absence of clear regulations led to abuse and a loss of trust. That is why international rules, such as the Declaration of Helsinki and Good Clinical Practice (GCP), are in force today, guaranteeing patient safety and transparency throughout the entire process. Importantly, participation in a clinical trial is not an experiment in the uncontrolled sense, but the most regulated and standardized process of investigating new



### Justyna Kutymba, PhD

Doctor of Medical and Health Sciences and Senior Specialist in Clinical Research and Strategic Projects at the Medical Research Agency. She combines clinical, scientific, and administrative experience, focusing on improving the quality of clinical trials and implementing innovative solutions in the healthcare sector.  
justyna.kutymba@abm.gov.pl



### Elżbieta Bylina, PhD MD

Director of the Center for Clinical Research Development at the Medical Research Agency. She is a graduate of the University of Warsaw and completed postgraduate studies in clinical trials management organized by the Medical University of Warsaw. From 1995 to 2021, she was affiliated with the Maria Skłodowska-Curie National Institute of Oncology – National Research Institute. She is a lecturer in postgraduate programs and a speaker at sector conferences on the coordination and management of clinical trials in the research centers.  
elzbieta.bylina@abm.gov.pl



**Zuzanna Nowak-Życzyńska, PhD, Eng.**

Director of the Department of Science and Evaluation at the Medical Research Agency. Geneticist and biostatistician. Over the past four years, she has focused her work on supporting research projects in the field of medicine. [zuzanna.nowak-zyczynska@abm.gov.pl](mailto:zuzanna.nowak-zyczynska@abm.gov.pl)

medical solutions. Each research project undergoes multi-stage evaluation, receives a positive opinion of the ethics committee, and obtains the consent of the URPL, while participants are fully informed about the purpose, risks, and procedures involved.

An important component in ensuring transparency is the registration of clinical trials in international databases, such as the European Clinical Trials Information System (CTIS) or the globally recognized ClinicalTrials.gov. Registration provides access to key information about ongoing projects, including their objectives, methods, recruitment status and results. This allows everyone – from physicians and researchers to patients and their families – to verify what studies are being conducted, their goals, and their current stage. Furthermore, any changes that could affect participant safety must be immediately reported to the supervisory authorities, and the trial may be modified or even discontinued. Decision to halt or modify a trial can be made by the researchers, the sponsor, or, in the case of larger projects, by an independent monitoring committee. Regulatory authorities may also suspend trials if participants are deemed to be at excessive risk or if there are serious breaches of protocol that would invalidate potential results. Entries in international registries not only inform patients and physicians but also alert other research teams to potential risks. This multi-level control system ensures that innovative therapies introduced in clinical trials meet high safety standards.

### Development of Non-Commercial Clinical Trials in Poland

Clinical trials can be funded by both the pharmaceutical industry and public institutions. The role of the state in this process is not merely supplemental, but

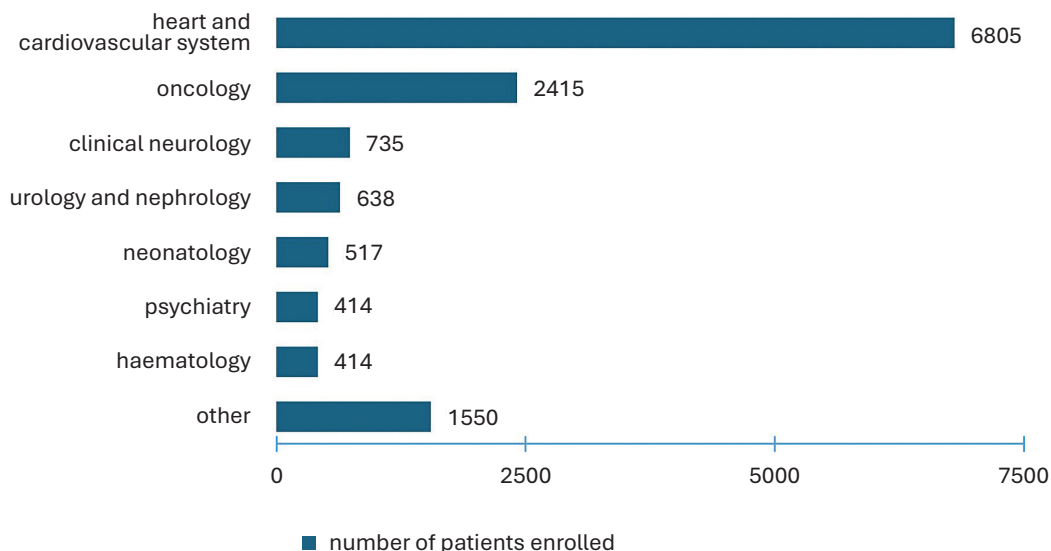
a prerequisite for ensuring equal access to treatment and fostering scientific development. For this reason, in 2019, the Medical Research Agency (*Agencja Badań Medycznych – ABM*) was established as a public institution to address an existing gap in the healthcare system – namely, non-commercial clinical trials.

The agency’s role is to finance and support projects that are not motivated by profit, but by gaps in knowledge identified by scientists, as well as by the social and health-related needs of citizens. Such studies aim to expand medical knowledge, verify systemic solutions, and ultimately lead to changes in reimbursement, treatment availability, and clinical guidelines, even when they address rare diseases. Thanks to non-commercial clinical trials, governmental decisions can be based on solid evidence, which is often of limited commercial relevance in scenarios which do not have high priority from the commercial perspective.

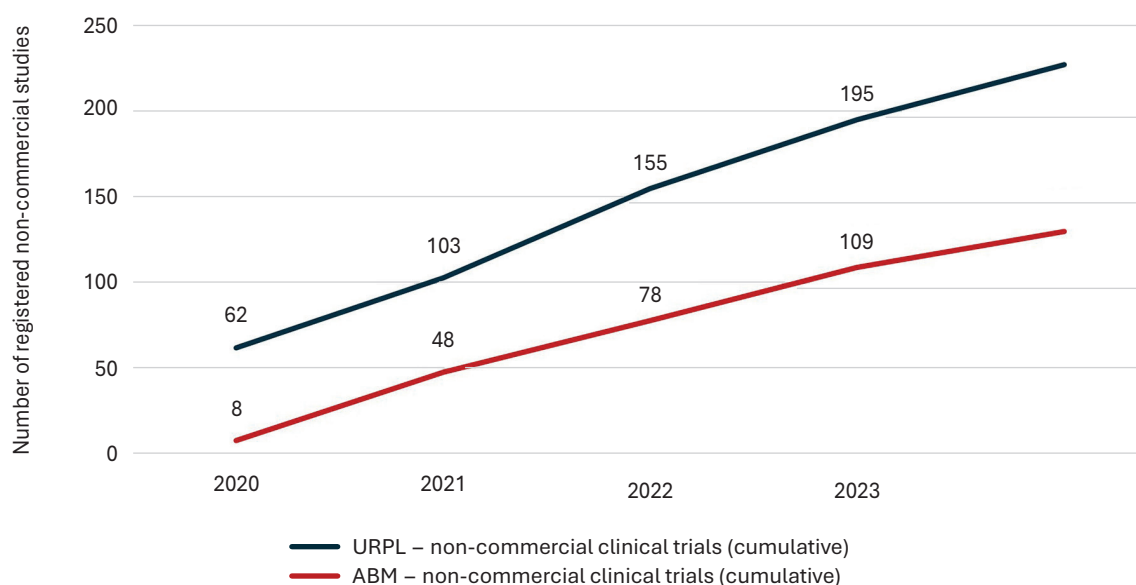
Importantly, non-commercial research in our country is rapidly growing in significance. In 2018–2019, as many as 970 commercial trials and only 26 non-commercial trials were registered in the country, with the latter accounting for 2.6% of all projects. In 2020–2021, the number of registered non-commercial trials had risen to 66, with a market share of 5.6 %, representing more than a twofold increase.

The year 2021 was a record-breaking year in this respect, with 729 clinical trials reported, 41 of which were non-commercial projects. Of particular note, 40 of these were financed by ABM. Funded trials primarily focus on rare diseases, psychiatry, neurology, cardiology, oncology and hematology. Their purpose is to test how medicines and medical devices perform in practice, as well as to develop new diagnostic and treatment approaches. For example, studies are investigating whether ambroxol, a drug commonly used in cough syrups, can benefit patients with the rare Gaucher’s disease, and whether pembrolizumab,

Number of patients recruited in clinical trials in 2020–2025 by therapeutic indication (based on ABM project reports)



Cumulative number of registered non-commercial studies in Poland (2020–2024)



an immune-supporting drug, can help patients with rare and treatment-resistant adrenocortical carcinoma. These projects are currently ongoing, and the first results are expected no earlier than 2026.

Data for 2020–2024 confirm this trend: of 227 non-commercial studies registered in Poland, 130 were conducted with ABM support, accounting for almost 60%. This demonstrates the critical role of the Medical Research Agency in developing a research sector focused on patient needs and economic benefits arising not from the sale of medicines, but from the improved functioning of society.

## Taking Notice of Those Who Are Overlooked: The Role of Non-Commercial Trials

Non-commercial clinical trials are projects conducted by physicians and scientists, rather than by pharmaceutical companies. Their goal is not to develop a new drug, but to determine whether existing therapies can be effective in treating other diseases, to compare different treatment methods, or to tailor therapies to specific needs of patients. For example, further research may reveal that a drug previously used to treat leukemia may also be beneficial in treating a rare form of cancer in children, or that two similar drugs are equally effective, but one is cheaper and more readily available. This was the case with semaglutide (Ozempic), which was initially used to treat diabetes and has now been shown to be effective in treating obesity and heart disease. Similarly, empagliflozin, another antidiabetic drug, has been shown to be effective in treating heart failure and kidney diseases. In Poland,

ABM-funded trials investigate, for example, the use of bortezomib in children with acute leukemia and valacyclovir in pregnant women to prevent CMV infection in newborns.

This type of social-scale optimization does not necessarily align with the commercial interests of pharmaceutical companies, but it is of enormous importance for the healthcare system and for patients. It allows, for instance, reduced therapy costs, improved the treatment comfort, and increased access to effective interventions. Moreover, it generates invaluable data that supports better health policy planning and long-term improvements in the quality of medical care.

It is thanks to such research that an evidence-based healthcare system is being built – independent, transparent, and focused on real societal benefits. Poland ranks fifth in the European Union in terms of the number of clinical trials conducted, reflecting the growing potential of the domestic research environment. A high ranking promotes increased patient access to innovative therapies and strengthens Poland's role in international scientific cooperation.

ABM pays particular attention to patient groups that are often overlooked by commercial sponsors because they do not guarantee a rapid return on investment. These groups include children with rare bone cancers, patients with tuberous sclerosis, pregnant women at risk of CMV infection, and individuals requiring innovative therapies such as CAR-T. These examples illustrate research endeavors that would be difficult to pursue without public funding. Since its inception, the agency has financed more than 150 non-commercial clinical trials, investing over PLN 1.5 billion and providing care to tens of thousands of patients across Poland. The funded projects

include both large-scale trials conducted simultaneously in multiple hospitals and smaller studies evaluating new treatments for rare diseases.

## Rare Diseases: Social Responsibility in Practice

One of the clearest examples of social responsibility in science is research in the field of rare diseases. Conducting studies in this patient population presents particular challenges, primarily due to the limited number of patients and the often heterogeneous course of disease. Despite these difficulties, such studies are essential for the development of effective therapies in areas where standard treatments are lacking. They frequently represent the only opportunity for patients to gain access to modern therapies, and their implementation is critical to improving the quality of life of individuals affected by rare diseases. Due to small patient populations and the often high costs of therapy development, this field is frequently neglected by the private sector.

This is where support from the state budget becomes crucial. In Poland, research activities related to drugs for rare diseases are carried out by the National Center for Research and Development (STEP mechanism and the planned Medistrateg program) and by ABM (Transmed program; [abm.gov.pl/pl/o-nas/dzialalnosc/plan-finansowy-i-plan-d/plan-dzialalnosci/2025/2929,2025.html](http://abm.gov.pl/pl/o-nas/dzialalnosc/plan-finansowy-i-plan-d/plan-dzialalnosci/2025/2929,2025.html)).

Growing government involvement and cooperation between science and business make it possible to implement innovations that improve access to effective therapies and enhance the quality of treatment. This provides patients with faster access to modern medicines and medical technologies, enables hospitals to introduce more advanced diagnostic methods, and allows physicians to conduct research that would often be impossible under purely commercial conditions. Such partnerships also facilitate the transfer of discoveries from laboratories to clinical practice, which directly translates into improved patient prognoses.

## Placebo: Ethics and Safety

The use of placebos in clinical trials is controversial; however, they are currently employed only in situations where no effective treatment exists. The vast majority of trials compare the new therapy with the standard of care. In such cases, the placebo and the investigational drug are administered as additions to the standard treatment. As a result, patients assigned to the placebo group receive at least the same level of medical care as patients with the same condition who are not participating in the trial. In some instances, clear evidence of the superior effectiveness of the

investigational product emerges during the trial. In such cases, the study may be terminated early or participants in the placebo group may be transferred to the group receiving the investigational product. This approach minimizes risk to patients and maximizes the likelihood of rapid access to effective treatment (R. Zaręba, *Stosowanie placebo w badaniach klinicznych – aspekty prawne i etyczne* [The Use of Placebos in Clinical Trials: Legal and Ethical Aspects], 2024).

## Who Can Take Part in a Trial and Under What Conditions?

Participation in a clinical trial is always preceded by a thorough qualification. Inclusion and exclusion criteria describe who may participate, taking into account factors such as age, health status, disease progression, and previous treatment. Before enrollment, each participant receives detailed information about the potential benefits, risks, and available alternatives. Consent to participate must be given by in a fully informed and voluntary manner, and participants have the right to withdraw from the trial at any stage without providing a reason.

Patients interested in participating in clinical trials can gain access to innovative medical solutions and play an active role in the advancement of medicine. One of the key sources of information is the official *Pacjent w Badaniach Klinicznych* (Patient in Clinical Trials) portal ([pacjentwbadaniach.abm.gov.pl/pwb/](http://pacjentwbadaniach.abm.gov.pl/pwb/)), operated by the Medical Research Agency and its partners. This website serves as a comprehensive compendium of knowledge on clinical trials. It provides information on how to search for a clinical trial, guidance on participation, answers to frequently asked questions, and an overview of available clinical trial search engines.

To access clinical trials, the patient or caregiver should first consult the attending physician, who is usually the most reliable source of information. If answers cannot be obtained in this way, it is advisable to use available clinical trial registries, which contain basic data on trial objectives, participant population, and the location of study centers. It may also be helpful to follow information published by patient organizations, associations, and healthcare institutions. In addition, it is worth reviewing materials provided by the National Bioethics Center ([kcb.abm.gov.pl/](http://kcb.abm.gov.pl/)), which address, among other topics, the ethical and legal aspects of participation in clinical trials.

## Real change

Clinical trials are the bridge between scientific discovery and tangible benefits for the patient. Out of concern for patient well-being, science fulfils its social responsibility – in practice, not merely in declarations. ■

### Further reading:

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