

# Personalised medicine research and the PERMIT recommendations

## What is personalised medicine?

Personalised medicine uses a person's individual characteristics (genetic and other) to arrive at the right diagnosis, the right treatment, at the right dose, to the right patient, at the right time.

Recent advances in personalised medicine research have shown new possibilities for better diagnosis, for better predicting the way people respond to specific interventions, and for better understanding of how disease will evolve in an individual. This has enormous value for patients, as it can reduce trial-and-error treatments, enable better choices for determining which medications or which preventive measures would be most effective, and it can help also manage rising healthcare costs.

Research on personalised medicine covers four stages:

- 1.The design, establishment and management of the division of patients into subgroups and the validation of these subgroups; and the generation and management of data.
- 2.The application of artificial intelligence methods to divide the patient group into subgroups.
- 3.The test of the treatment before trials may be carried out in humans.
- 4.The evaluation of treatments in randomised clinical trials[1]

[1] A clinical trial where participants are divided by chance into separate groups that compare different treatments or other interventions.



## What is the PERMIT project?

PERsonalised MedicIne Trials (PERMIT) is a project funded by the European Union's Horizon 2020 research and innovation programme. Coordinated by ECRIN, it aims to improve personalised medicines research by developing recommendations for the research community that can be widely accepted.

Over 2,5 years, different experts came together to develop these recommendations. Pan-European research infrastructures, research institutes, health technology assessment authorities, patient representatives, medicine agencies, ethics committee representatives, and field experts participated in a series of workshops to develop the recommendations that will ensure scientific excellence, validity, strength, and replicability of results generated by personalised medicine research.

The European Patients' Forum (EPF) was involved in this project to ensure that the patient voice was present in most workshops, milestones and deliverables, but also worked on a few materials in lay language for the patient community.

# Recommendations that matter to patients

- 1. Transparent and reliable reporting and data sharing** must be a requirement for all researchers to improve the quality, credibility, and responsiveness of research. Negative research findings should also be published, to help ensure that efforts are focused on the most promising research.
- Regulators, such as medicines agencies, should ensure that **preclinical models are clinically relevant and based on patients' preferences.**
- 3. Active patient involvement** in the preclinical phase should be facilitated and incentivised through public funders.
- 4. Fundamental principles of clinical evidence generation equally apply to personalised medicine**, as they do in other domains. Trials in personalised medicine should be planned and conducted with the same level of methodological requirements (e.g., use of clinically relevant and patient-important outcomes, etc.) as in any other domain.
- A **control group** (which does not receive the new treatment being studied) should be preferably included in clinical trials of personalised medicine. This group is compared to the group that receives the new treatment to see if the new treatment works.
- 6. Patients should be randomly assigned** to one of the two groups, the control group or the group receiving the new treatment. Randomisation should remain the preferred design feature to allow a fair comparison of interventions and should be favoured as much as possible.
- Individuals receiving the new treatment should **be compared to controls that were recruited during the same time period.**
- Clinical trial tests should use **clinically relevant and patient-important outcomes.**
- It is extremely important to **provide stakeholders, including patients and the society, with a comprehensive evaluation of the benefits and risks of a personalised medicine approach.** In order to determine if a personalised medicine strategy is the best approach, it should be **compared to the standard of care** (including a non-personalised approach whenever this is the case) using randomised controlled trials.
- As with non-personalised medicine clinical trials, incorporation of **patient-reported outcomes and patient-relevant endpoints** will be key to ensuring that the research actually brings treatments that provide added value for the patients compared to what exists.
- Trials comparing a personalised strategy to a non-personalised strategy **should consider the entire personalisation strategy.** This can allow decision making with full knowledge of potential benefits, risks and costs. Performing the full evaluation can shed light on all the limitations (including time and necessary resources) of the personalised strategy.



# Why is it important?

## Why do the recommendations matter?

- The recommendations are now published to support investigators to better plan and execute their research across personalised medicine research in all stages and across all disease areas.
- When the recommendations are properly implemented by researchers, they will help yield better quality results that translate to better quality personalised medicine.
- If the quality of personalised medicine is better, this means that patients should have access to solutions that are effective, safe, and better tailored to their needs.
- Many of these recommendations also point to importance of improving patient involvement in the design and execution of these research programmes.
- Personalised medicine at its core is about putting patients at its centre, and these recommendations are a reminder that this should be done as early as possible to make personalised medicine research a success.



## Why do the recommendations make research better?

- Personalised medicine is a field that is moving fast and a lot of designs and new methodologies are being used. The recommendations help to guide investigators to better design and implement personalised medicine research programmes, and can help other stakeholders who fund, evaluate, or publish this research.
- PERMIT aimed to bring all stakeholders to the table to reach consensus on what are the best methodologies to be applied in this field across the full pipeline of personalised medicines research.