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Researcher calls for one-person trials for personalized medicine

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"All things are poison, and nothing is without poison, the dosage alone makes it so a thing is not a poison." These were the words of Paracelsus, a Swiss physician who recognized back in the 16th century that every

medicine has its benefits and risks, and that for a medicine to work, the individual dose is crucial.

This is still highly relevant today, since most drugs on the market are "one size fits all," meaning patients with the same condition often receive the same dose, even though studies show that this is only optimal for a fraction of them. In some cases, doctors adjust the dose on a trial-and-error basis. This approach is inefficient and leads to unnecessary adverse reactions and increased healthcare costs.

By identifying the right drug, in the right dose, at the right time for each patient, precision medicine has the potential to make the healthcare system more efficient and to treat patients more effectively. Essentially, precision medicine combines a patient's genetic data with clinical, environmental and lifestyle information to guide decisions for the optimal prevention, diagnosis, and treatment of conditions.

Pharmacogenomics deals with the influence of a patient's individual genome on the effect of drugs. Using appropriate tests, physicians are able to determine in advance for patients individually which drugs are likely to work for them. The tests analyze key genes in our body involved in metabolism, transport, and elimination of the drug. Since genetics doesn't change over time, a pharmacogenomics report listing all the drugs likely to work for us is valid for our entire lifetime.

While such tests used to be expensive, they've become much cheaper today. There are also clear international clinical practice guidelines for these tests. Despite this, they're rarely carried out. Evidence as well as awareness is needed to convince regulators, clinicians and payers alike that using genetic information makes a difference to patient outcomes—and this evidence has to be continuously generated. Studies showing the effectiveness and cost-efficiency of this approach would be helpful here.

Women benefit particularly

This calls for committed citizens. I assume that the future of pharmacogenomics and precision medicine will be built on a critical mass of interested and committed stakeholders—including patients and clinicians informed enough to ask for it. The patients here partner closely with physicians and other stakeholders, and are involved in treatment decisions on their own health issues.

Marginalized groups that are currently under-represented in clinical trials would particularly benefit from such cooperation. Women too. There's a whole range of diseases whose incidence among women is the same as, or higher than among men—yet women are outnumbered in clinical trials, as an American study has shown. This is the case for cardiovascular disease, hepatitis, HIV, chronic kidney disease and digestive diseases.

A cohort as initial spark

Since genetic differences in pharmacogenes account for a quarter to half of an individual's response to medicine, such under-representation could mean that drugs may work less effectively in women than in men. This is why Women's Brain Project, an organization advocating women's neurological and mental health, is putting forward a new, personalized approach for women. This requires the active participation of female citizens, whose health data will help to optimize these treatments.

Data is the foundation for patient-centricity and precision medicine. However, as long as data is stuck in "silos" or in organizations not incentivised to share, it will never reach the very recipients that would benefit from it. Cause of Health, a Swiss initiative in which I'm involved, aims to network this health data. It will build a cohort of

people to provide health data, and will address, among other things, questions about pharmacogenomics. The participants will remain the owners of their data and receive a copy of their own pharmacogenomics report. This will empower the individuals to be more involved in their treatment decisions, and to benefit from the advantages of precision medicine and pharmacogenomics, from better treatment options and fewer side effects.

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