

Researcher explores drug repurposing for treatment of rare diseases

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In two publications in *Nature Reviews Drug Discovery*, Anneliene Jonker (TechMed Center) highlights the importance of health care for rare diseases, and the innovation needed in that. The personalized medicine program manager shows how drug repurposing can be a solution for rare diseases, as well as how developments in innovative therapies for rare diseases are influenced by personalized medicine.

Around the world, there are many different definitions for when a disease is rare. As a result, no one knows exactly how many [rare diseases](#) there are. This makes it difficult to come up with good treatments efficiently. By thinking about that definition and, for example, merging certain subgroups, you can maximize the impact of treatments.

Describing the [first publication](#), Jonker says, "Around the world, there are about ninety different definitions, the World Health Organization is also working on such a definition. But how this definition is influenced by looking more and more at the individual, as happens with personalized medicine, we didn't really know. In doing so, we looked at how we increasingly break down [common diseases](#) into small subgroups."

"That way, sometimes you move towards a rare entity. For example, a more common type of cancer can become a [rare disease](#) because we divide it up so much with specific biomarkers. Finally, we looked at shared molecular features, which allow some different rare diseases to still be treated in the same way."

In her [second publication](#), Jonker looked at drug repurposing, the reuse of drugs for a different disease. "The best-known example is Viagra; it

was developed as a drug for [cardiovascular disease](#), but is now better known as a remedy for [erectile dysfunction](#)."

Developing drugs for a rare disease is difficult. There are few people to conduct [clinical trials](#) with and also little expertise on the course of the disease. As a result, it takes a lot of money and time to develop a drug for a rare disease. By reusing a drug, you need much less money and time for development. This can be a godsend in rare diseases.

To this end, Jonker mapped what initiatives there have already been worldwide to make reuse possible. "If you know what all is possible, as a developer you can create a better route for the development of drug reuse," she said.

For her publication, she and an international group of experts created an overview of initiatives including checklists to see when is the best time to make use of them. In this way, drug reuse could shorten the development of a drug for a rare disease from an average of 30 years to around 10 years.

More information: Daniel J. O'Connor et al, Defining rare conditions in the era of personalized medicine, *Nature Reviews Drug Discovery* (2023). [DOI: 10.1038/d41573-023-00145-2](https://doi.org/10.1038/d41573-023-00145-2)

Anneliëne Hechtelt Jonker et al, IRDiRC Drug Repurposing Guidebook: making better use of existing drugs to tackle rare diseases, *Nature Reviews Drug Discovery* (2023). [DOI: 10.1038/d41573-023-00168-9](https://doi.org/10.1038/d41573-023-00168-9)

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