From fruit flies to patients in less than 5 years.

After our discovery in 2015 that the vitamin B5 derivative 4'-phosphopantetheine works protective in a *Drosophila* model for PKAN, my research group together with collaborators used this fundamental knowledge to explore possibilities to generate bypass molecules for defective CoA biosynthesis steps, like the vitamin B5 derivative "4'-phosphopantetheine" and successfully tested them in PKAN animal models. After crucial promising results in a CoA-biosynthesis impaired mouse model, (research group of prof. Hayflick and prof. Hogarth at the Oregon Health and Science University (OHSU), USA), we developed 4'-phosphopantetheine further into a medical product which fulfils all the standards allowing testing in a clinical trial. As a result, at the OHSU, a clinical trial with 4'-phosphopantetheine is now successfully finished and currently, my team is coordinating the first PKAN clinical trial in the Netherlands with 4'-phosphopantetheine.

Our goal is to finally bring 4'-phosphopantetheine to the market as a cost-effective product for PKAN. My lecture is about the challenges, hurtles, milestones and future steps of this mission.