



Session:
**How to improve the actual
use of effective treatments?**

Moderator: Sytse Buruma

Goal of the session

- How to use innovative models to deliver effective treatments to the right patients?

Goal of the session

- How to use innovative models to deliver **effective treatments to the right patients?**

'Effective treatments to the right patients'

- Appropriate use / doelmatig gebruik: the right medicine for the right patient, used at the right time in the right way
- Answer to many dilemma's that we discuss today, as it improves both the effectiveness and cost-effectiveness of treatments
- How can we make this a reality in 2030?

Rob Vries – Hub4Organoids

- One way is to determine in advance if a specific patient will respond to a drug
- Patients will only be treated with an orphan drug if we have determined that they will respond to it

Rob Vries – Hub4Organoids

- Rob Vries – Hub4Organoids; a foundation that grows mini organs from living tissue (organoids).
- Living replicas of organs that maintain the same genotype and phenotype as the diseased tissue
- Drugs can be tested on these organoids; a quick and easy way to determine if a drug will benefit a patient, with minor disruption for the patient

Pete Chan - Raremark

- Another approach: instead of finding the right drug for a patient, we can find the right patient for a drug
- Pete Chan – Raremark; a patient-powered research network in rare diseases
- During development: accelerate trial recruitment and bring patient experience into the process

Pete Chan - Raremark

- Raremark's expertise in asking patients for lived experience could have implications for appropriate use
- At the end of the day, patients are the real experts on a disease and the implications of a treatment
- How can we make sure that data and insights of patients will play a larger role in determining appropriate use?