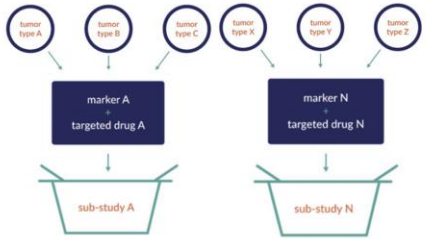
Regulatory Readiness – what is it and why should we care?

Figon DMD 27 September 2021

Welcome!

- Regulatory Readiness - what is it and why should we care?
- A session co-hosted by:

Innovation in the regulatory space…



Methods

…examples..

Tools

Biomarkers/outcome measures

Data sources

When are such innovations ready for regulatory use?

- Before innovations in methods, tools, data source, biomarkers etc. can be implemented in the regulatory d process, they first have to be assessed and convincing evidence must be provided for their suitability.
- But…. when is such a new innovation ready? And can we distinguish a certain levels of ‘readiness’? And why should you care?

Focus for today: registry data for the purpose of medicine development and authorisation

The agenda for today

- Introduction en relevance Regulatory Readiness
- Regulatory Readiness Levels as a tool to drive regulatory innovation - Sjaak Bot (RSNN, Janssen)
- The impact and use of quality registries - Can real-world data complement post-approval clinical trials? - Rawa Ismail (DICA, UU) en Michel Wouters (DICA, NKI)
- An academia-led registry for metachromatic leukodystrophy – the MLD initiative - Daphne Schoenmakers (AmsterdamUMC)
- Perspective of the regulator on readiness of registry data –Marjon Pasmooij (RSNN, MEB)
- Discussion