

W3

DATE & LOCATION

09.00 – 11.00

Wednesday 4 October 2017

Conference Centre Room 1

(online update: 29 Sept 2017)

Personalising healthcare: How rare diseases pave the way

Current efforts in personalised medicine aim at bringing scientific insights into the clinic to effectively identify disease and predisposition for disease, prescribe the right therapy and determine the right dose for the right patient, and to better deliver timely and targeted prevention. This session brings together stakeholders to exchange knowledge and develop strategies, policies and guidance that pave the way to personalised medicine in Europe, using rare diseases as a model.

Advances in genomics and other "omics" technologies have significantly improved our understanding of the pathogenesis of rare diseases. This has opened avenues for piloting new, personalised diagnostic methods and therapies. The impact of "omics" is reinforced by the combination of these data with Real-World Data (RWD). Notably, it has been recognised that large data sets of detailed phenotypes integrated with genetic data help adjust dosage and select therapy. RWD is also vital for post-authorisation evidence generation. The blurred boundary between clinical care and research in rare diseases makes them an excellent candidate for piloting integrated bench-to-bedside pipelines to ensure the rapid translation of research findings into clinical support for personalised medicine.

This workshop will follow an interactive format, with talks on rare diseases research and care in Europe, the use of "omics" & RWD, health data infrastructures and health data policy.

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Moderated by **IRENE NORSTEDT**, Head of Innovative and Personalised Medicine Unit, DG RTD, European Commission