THE INHERENT IMPRECISION IN PRECISION MEDICINE: how do we deal with the resulting uncertainties?

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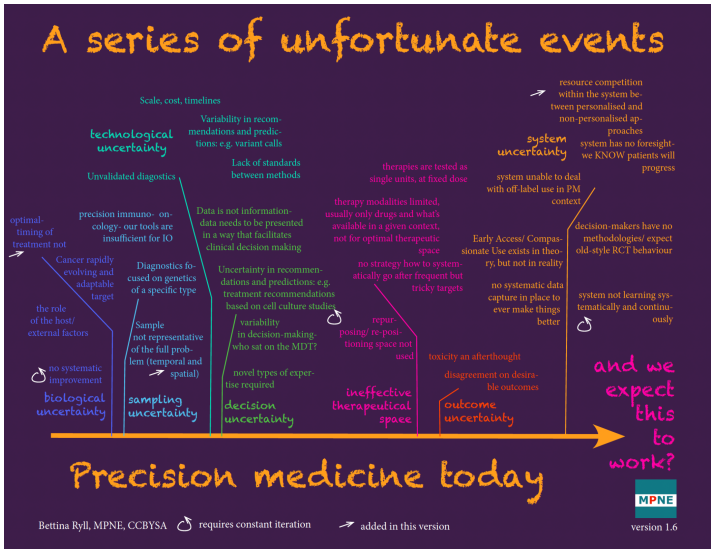
Precision Medicine is described as 'The right treatment to the right patient at the right time'. Conceptually deceivingly simple, precision medicine challenges the way we generate, evaluate and ultimately, act upon clinical evidence: broad implementation becomes the prerequisite for rather than the consequence of successful evidence-generation, constituting a veritable paradigm shift for all stakeholders involved. This panel will look at the multiple sources of uncertainty within precision medicine- from biological heterogeneity over diagnostic and clinical limitations to system-inherent and human factors and discuss pragmatic mitigation strategies as well as ways to systematically reduce these uncertainties with time. With its reliance on molecular diagnostics to identify the appropriate treatment, precision medicine represents an interesting challenge for HE and HTA as diagnostics and therapies do not only need to be considered in isolation but also with regards to interdependencies.

With the upcoming EU partnership for Personalised Medicine, the topic is particularly timely for the European audience.

Building on an earlier public discussion this issue panel will look at the sources of uncertainty in precision medicine[6], a group of ongoing national and EU-funded implementation initiatives in precision medicine centred around DRUP[7]-Like Clinical Trials, and previous work of the HTAi-DIA working group, taking a systematic approach to identifying and mitigating sources of uncertainty for decision-makers[8].



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[1] Melanoma Patient Network Europe
[2] PCM4EU, PRIME-ROSE
[3] HTAi-DIA working group
[4] Oslo University Hospital
[5] MSD
[6] https://www.linkedin.com/posts/bettinaryll_for-anyone-in-precision-personalised-medicine-activity-6870671252676235264-ZFeX, accessed 14th Nov 2023
[7] van der Velden, D.L., Hoes, L.R., van der Wijngaart, H. *et al.* The Drug Rediscovery protocol facilitates the expanded use of existing anticancer drugs. *Nature* 574, 127–131 (2019). https://doi.org/10.1038/s41586-019-1600-x
[8] Hogervorst MA, Vreman R, Heikkinen I, Bagchi I, Gutierrez-Ibarluzea I, Ryll B, Eichler HG, Petelos E, Tunis S, Sapede C, Goettsch W, Janssens R, Huys I, Barbier L, DeJean D, Strammiello V, Lingri D, Goodall M, Papadaki M, Toussi M, Voulgaraki D, Mitan A, Oortwijn W. Uncertainty management in regulatory and health technology assessment decision-making on drugs: guidance of the HTAi-DIA Working Group. Int J Technol Assess Health Care. 2023 Jun 16;39(1):e40. doi: 10.1017/S0266462323000375. PMID: 37325997.