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Transfer of research results into the market: How to optimise a safe, fast and economic process to implement personalised medicine approaches?

Working Group III

Panel:

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Leading questions:

- Technological challenges for Personalised Medicine research. Data generation and analysis.
- Methodological standards for Personalised Medicine studies.
- Which issues related to market and regulation have to be addressed first in order to facilitate market access of Personalised Medicine approaches?
- Entrepreneurial challenges common to all the previous questions.

Technological challenges for Personalised Medicine research. Data generation and analysis:

- Changes in personalized medicine on cancer (currently 400 actionable hotspots, 20 targeted treatments)
  - single gene -> cluster of somatic mutations -> whole genome sequencing
- Quality of data, reproducibility of analyses
- Pan European interoperable standards for data collection (-omics, imaging, electronic health records)
  - use for patient stratification, secondary use for trials ? GDPR ?
- Need for training data scientists, machine learning summer school

Methodological standards for Personalised Medicine studies:

- Stratification (secondary use of data ?) and validation cohort : sample size ?
- Multi-omics, imaging, exposome etc
- Machine learning stratification: reproducibility ?
- Translational step: which treatments to be tested ?
- Multi-arm trial testing treatment in each cluster, and comparison with standard of care
- What is acceptable for clinical trial authorization ? for market access ? for reimbursement ? for funding ? for publishing ?
- Regulation considers the products (diagnostics, health products)
- Research Use Only ? for diagnostics if no impact on treatment decision

Which issues related to market and regulation have to be addressed first in order to facilitate market access of Personalised Medicine approaches?

- What will be marketed ? Diagnostics ? Health product ?
- Regulatory challenges : in vitro diagnostics regulation, clinical trial regulation
- IVD requires CE if impacts treatment decision
- Need to adapt legislation ?
- Regulatory awareness before planning the development
- Need for evidence for superiority and cost-effectiveness vs. standard of care, for reimbursement decision by HTA and adoption by medical community. Need for monitoring and comparative effectiveness trials
- Scientific advice group ? Cochrane group on PM (including HTA experts) ?
- Clinical trial for joint marketing authorization plus HTA decision ?
- Public or PPP funding also needed when no economic model (and no marketing authorisation needed)
- IP protection

Entrepreneurial challenges common to all the previous questions:

- Challenge for SMEs : market access, post-marketing monitoring
- Fragmentation of EU market (vs. US)
- Availability of venture capital in the EU
- Low awareness of users (healthcare professionals)
- Change in the business model and value chain : from pharma / IVD companies -> data- and AI-driven stratification, research for healthcare optimization