Future-proof HTA

The need for innovative HTA methods for more complex and personalized medicine

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New realities?

Changing HTA paradigms (future-proof HTA?)

- **Personalized treatments**
  - Smaller populations
  - Combinations of treatments, different sequences
  - Companion diagnostics (genetic testing)

- **Real world evidence (RWE)**

- **Internationalization**
  - Clinical assessments on an European level for single technologies (pharma and medtech)
About the HTx project

• Horizon 2020 project supported by the European Union, kicking-off in January 2019 and lasting for 5 years.
• Facilitate the development of methodologies to deliver more customized information on the effectiveness and cost-effectiveness of complex and personalised combinations of health technologies.
• Provide methods to support personalised treatment advice that will be shared with patients and their physicians.
• In close collaboration with the European Network for HTA (EUnetHTA) and its stakeholders pilot the implementation of these methods in Europe.
HTx – Participants

- Utrecht University (project coordinator) (UU) Netherlands
- University of Copenhagen (UoC), Denmark
- University of Oulu (UoO) Finland
- University of York (UoY) UK
- Medical University of Sofia (MUS) Bulgaria
- University of Bern (UBERN) Switzerland
- Universidad Politecnia de Madrid (UPM) Spain
- European Organisation for Research and Treatment of Cancer (EORTC) Belgium
- Dental and Pharmaceutical Benefits Agency (TLV) Sweden
- National Health Care Institute (ZIN) Netherlands
- National Institute of Health and Care Excellence (NICE) UK
- Syreon Research Institute (SRI) Hungary
- Synapse research management (SYNAPSE) Spain
- EURORDIS Rare Diseases Europe (EURORDIS) France
- University of Maastricht (UM) Netherlands
Learning healthcare systems + Data + Infrastructure

Stakeholders
- HTA
- Payers
- Healthcare Providers, clinicians
- Regulators
- Patients/consumers
- Pharma/medtech
- Technology industry/SMEs

Methods
- Advanced analytics
- RWE methods development
- Rapid updates systems

Combination
- Personalised Medicine (incl. diagnostics)
- Treatment pathways

Interventions
- Outcomes in priority disease areas
- Chronic diseases, multimorbidity
- Orphan diseases

Therapeutic areas

Systems
- Technology Appraisal and Clinical guidelines synergies
- Implementation
- Payment models
- Policy ‘sandbox’

Projects and products/knowledge

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Methods

• Prediction modelling on the basis of data using different study designs (RCT, RWD etc) (WP2)

• Health-econometric tools to take into account effects and costs (WP2)

• Develop AI/ML methods to forecast individual patient treatment outcomes (WP3)

Focus on combinations (and/or sequences) of health technologies not evaluated in RCT as such
Case studies

• Proton therapy for head and neck cancer

• Monitoring and treatment pathways in diabetes (T1DM and T2DM)

• Pharmacological treatments for relapsing multiple sclerosis (MS)

• Different treatment modalities in patients with myelodysplastic syndrome (MDS)

Including framework to generalize results to other indications and settings
Implementation and transferability

- Developing PROMS that are fit for purpose
- Link to flexible funding and reimbursement models
- International consensus on RWD and resulting HTx models between HTA, regulators and guideline developers
- Transferability of case study findings and methods across participating countries
- Develop and disseminate training materials to patients
How can success of HTx be measured?

- Clear methods developed for certain disease area’s;
- Are practically used in healthcare practice
  - By HTA organisations to facilitate HTA for personalised treatments (including support appropriate use);
  - By healthcare providers as part of new guidelines
  - For individual patients and their clinicians
- Provides a general framework that can help other groups to develop methods for specific disease areas
- Has a clear link to national reimbursement and pricing processes.