







Criteria for Value Assessment of Personalized Medicines and Factors for Achieving Value-based Reimbursement in this Rapidly Advancing Field

Findings from the "HISCRENDIAG" EU project

Lieven Annemans, PhD, MMan, MSc
 Professor of Health Economics, I-CHER, Ghent University and Brussels University, Ghent, Belgium

HIScreenDiag – Project

Building a Tool to Evaluate and Improve Health Investments in Screening and Diagnosis of Disease


Lieven Annemans, Fernando Antofanizas, Cornelis Boersma, Katharina Fischer, Dolores Ibarreta, Jan Jacob, Renner Leidl, Daniele Paci, Katherine Payne, Maarten J. Postma, Roberto Rodriguez, Wolf Rogowski, William Sullivan, Dominique Vandijck



Finding 1: emerging economic research activity



Currently 182 papers in database developed by university of Rioja



Finding 2: but the current EU decision process is a jungle

- There are enormous differences in
 - Who triggers the health economic evaluation
 - Who participates in the assessment
 - The criteria for assessment
 - The way they are conducted
- Coverage decisions about genetic tests frequently appear to be made outside of the scope of national decision making bodies, presumably on a local decision making level.



→ Project resulted in a common toolkit

- To be applied for those screening/diagnostics that claim to have an added value
- HTA toolkit is built around 3 main elements:
 - Ten criteria for decision-making
 - Quality assessment
 - To assess the quality of the evidence that is submitted for each of the 10 criteria
 - Process of HTA
 - Step by step description of the process for submitting, assessing and appraising the genetic diagnostic



Example: 10 Criteria for decision making (all explained in 2-3 pages)

1. Current use of the technology (dissemination so far)
2. Epidemiology of relevant disease(s)
3. The exact technology and its characteristics
4. Safety/toxicity
5. Accuracy
6. Effectiveness/efficacy (clinical utility)
7. Costs & economic evaluation
8. Ethical aspects
9. Organisational aspects
10. Psychosocial



Ultimate aim of the toolkit

Endorsed by 106 experts/decision makers

- **Benefits for patients and population**
 - Enables incentives to private investments in genetic diagnostics
 - Transparency about which diagnostics are beneficial and value for money
 - Opens the possibility of including genetic diagnostics into the public health system, with important benefits in terms of equity in access.
- **Implementation at national and international level**
 - The toolkit could be easily implemented at national level.
 - Does not require specific competence/resources & can be used by local HTA agencies / decision makers, without strong initial investments.
- **Improvement of current decision-making processes**
 - Despite the resistance that national health authorities show in delegating to international bodies the assessment of new health technologies, the implementation of the tool would significantly increase harmonization.



ISPOR Personalized Medicine Special Interest Group



Thank You!

For more information, please contact:

Eric Faulkner

Chair, ISPOR Reimbursement & Development Working Group

efaulkner@rti.org

919-541-6772



Key Payer and HTA Agency Questions for Value Assessment of Diagnostics and Personalized Medicine

- What is the accuracy of the test? To what extent are responders overidentified (false positives) or missed (false negatives) by the test?
- Are the test results actionable? Is test interpretation clear?
- What is the clinical utility of the test?
- How many tests must be administered to identify one treatable patient or adverse event?
- How much more effective is the treatment in the responder population compared with standard-of-care alternatives?
- What is the proper comparison strategy for a PGx test and/or medicine if others do not exist for that indication?
- What is the budget impact of avoiding resource wastage by treating nonresponders with alternatives?
- What proportion of responders must be identified to make the PGx scenario clinically beneficial and cost-effective?
- Should all patients receive the test before accessing alternative medicines in the same position (e.g., first-line vs. second-line) or formulary tier?
- How do we handle the multiple diagnostic tests that may emerge after launch of a diagnostics-driven medicine?

Adapted from: Faulkner E, Annemans L, Garrison L, Helfand M, Holtorf A-P, Hornberger J, Hughes D, Li T, Malone D, Payne K, Siebert U, Teesse A, Veenstra D, and Watkins J. Challenges in the Development and Reimbursement of Personalized Medicine: Payer and Manufacturer Perspectives and Implications for Health Economics and Outcomes Research. (submitted) 2011.



ISPOR Personalized Medicine Special Interest Group



Chair:

Eric Faulkner, MPH, Director, Global Market Access, Quintiles Consulting, and Executive Director, Genomics Biotech and Emerging Medical Technology Institute, National Association of Managed Care Physicians, Assistant Professor, Institute for Pharmacogenomics and Individualized Therapy, University of North Carolina at Chapel Hill, Research Triangle Park, NC, USA

Leadership Committee Members:

Lieven Annemans, PhD, MSc; Finley Austin, PhD, BS; Pat Deverka, MD, MS; Lou Garrison, PhD; Mark Helfand, MD, MPH; John Hornberger, MD, MS; Dyfrig Hughes, PhD, MSc; Tracy Li, PhD; Dan Malone, RPh, PhD; Katherine Payne, PhD; Uwe Siebert, MD, MPH, MSc, ScD; Adrian Towse, MA; Dave Veenstra, PhD, PharmD; John Watkins, RPh, MPH; Dyfrig Hughes, Anke-Peggy Holtorf, PhD, MBA

Goal:

To develop good research practices in personalized medicine and inform appropriate health care decision and policy making using personalized medicine information

For more information, please contact: efaulkner@rti.org or 919-541-6772