Challenges Experienced or Anticipated by Other Regulators and Payers … And Proposals For Dealing With Them

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Position Paper of the European Social Insurance Platform (ESIP) on Personalised Medicine
www.esip.eu

Disclaimer

Currently, in Europe, there is no single payers' voice.

The opinions expressed here, and any mistakes, are exclusively mine.

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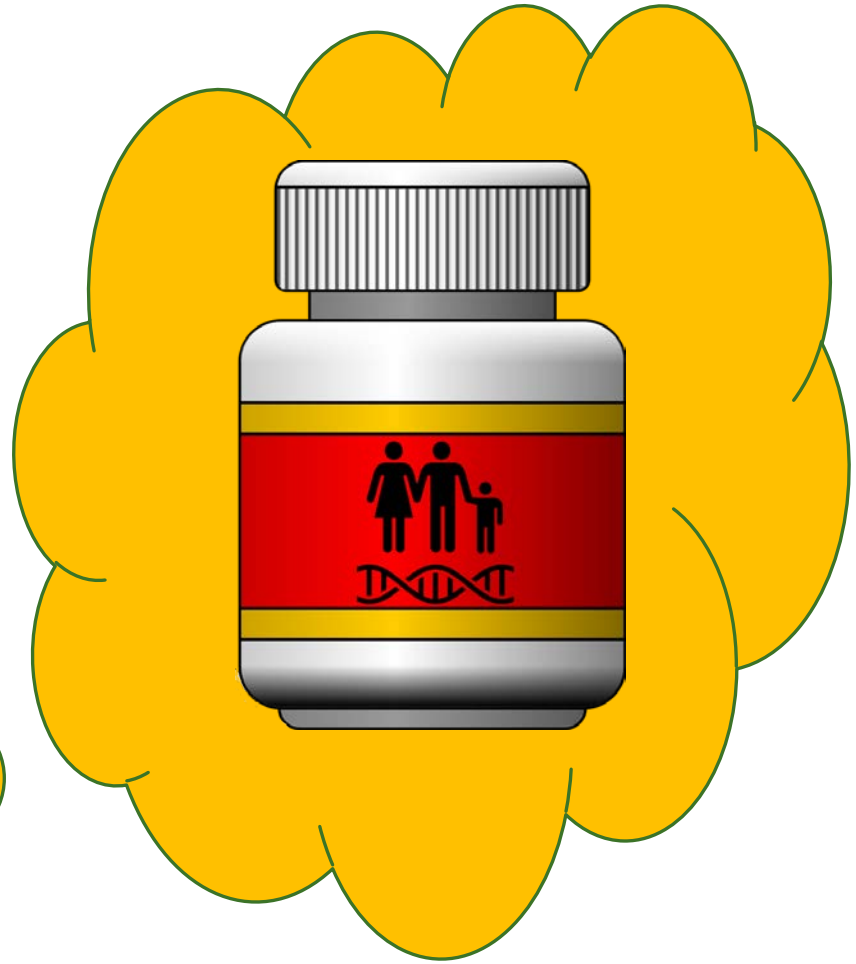
	Development	Marketing Authorisation	HTA	Reimburse-ment	Market Performance
Industry	Do or buy? Coordinate product development	Filing for MA	Dossier/PE model	Dossier, PE model, prices,	Co-Marketing, logistics, registries,
Clinicians /Scientists	Academic & commercial research	Expert advice	Expert advice Guidelines	Expert opinion	Managing the individual patien and registries
Regu--lators	guidelines, scientific advice	MA for medicine consult for device			Pharmacovigilance,, Postmarketing commitments, registries
HTA	Horizon scanning, guidelines, Early Dialogue, parallel SA		Assess dossier/PE model	inform	Assess outcome data, review
Reim-bursers	Make and/or coordinate reimbursement decisions, negotiate prices and/or packages/rebates,				administer databases, review decisions
Patients	Natural histry of Diease, Disease Burden, PRO				Use of Medicine

Payers' Concerns about „Personalised Medicine“



Convincing Evidence of Real, Patient-Relevant Benefit

- “Orphanisation”- Deteriorating levels of evidence, increasing prices?
- Are biomarkers the “ weakest link”?
- Early Access?

How to Hit a Moving Target?

Genomics analyses from single tumor-biopsy specimens may underestimate the mutational burden of heterogeneous tumors. Intratumor heterogeneity may explain the difficulties encountered in the validation of oncology biomarkers owing to sampling bias,[29] contribute to Darwinian selection of preexisting drug-resistant clones,[12,30] and predict therapeutic resistance.[13] …"

Intratumor Heterogeneity and Branched Evolution Revealed by Multiregion Sequencing

Gerlinger et al., N Engl J Med 2012; 366:883-892, March 8, 2012,DOI: 10.1056/NEJMoa1113205

Complexity

- HTA: Co-Assessing Diagnostic & Therapeutic – Different procedures, different licensing agencies, different criteria for licensing, different timelines?
- Managing Access at National level (if reimbursement procedures are different for diagnostic and medicine)
- Coordinating Responsibilities for
  - Patient Data Protection
  - Registries and Accessibility of Data
  - Outcome Assessment and Re-Evaluation

The Silo Mentality

Diagnosis in Hospital: Test must be cheap, cost of ambulatory therapy of little interest

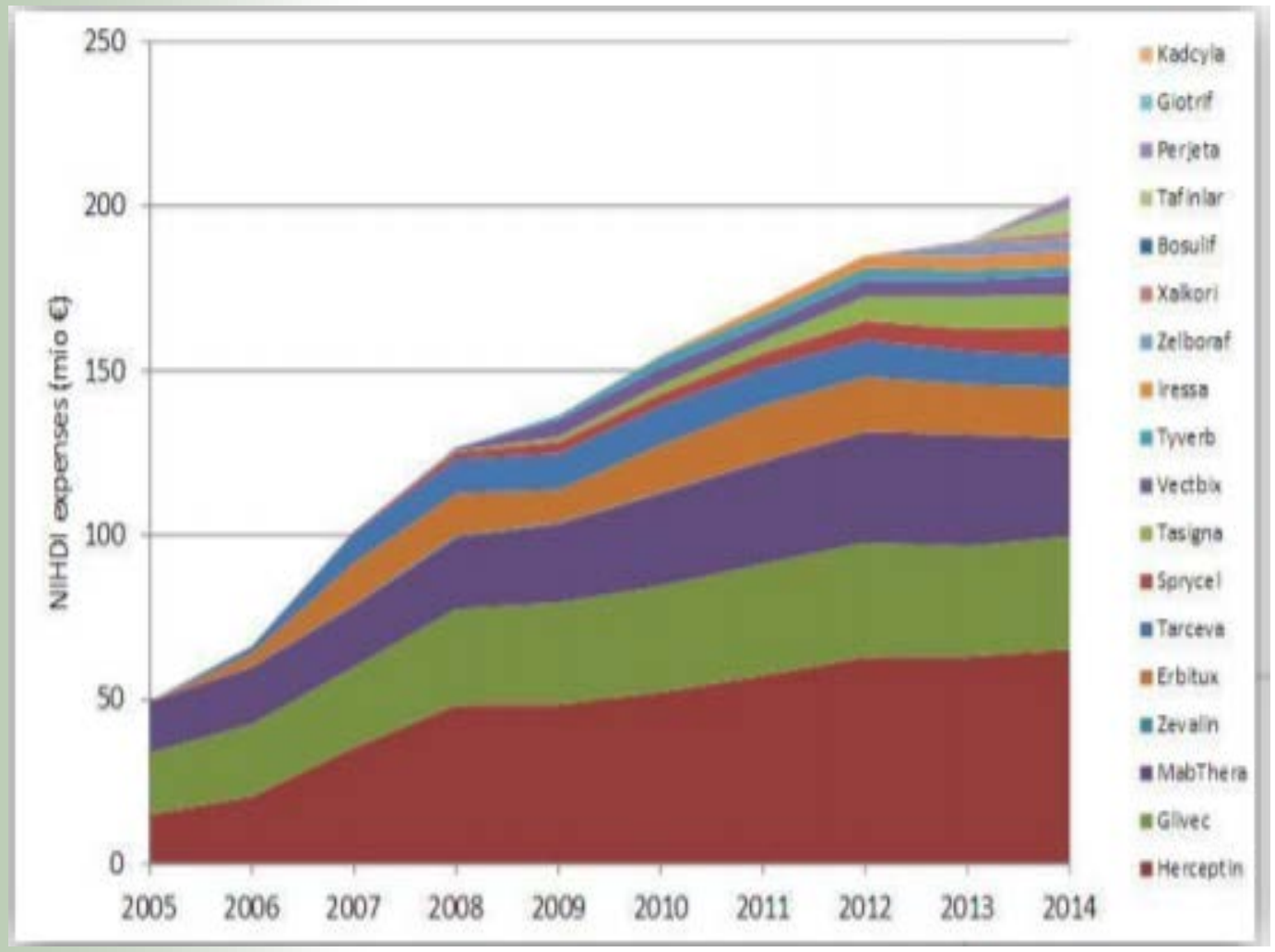
CONTRA

Ambulatory therapy: Test must be validated and very discriminatory (cost of little interest), as the cost of therapy is borne by health insurer

Challenges of Pricing and Reimbursement

- “Orphanisation”- Deteriorating levels of evidence, increasing prices?
- “Salami slicing” – no economies of scale
- Expensive therapies - used in combination
- Blockbuster tests and orphan prices raise the dangers of increasing inequality among MS and problems of sustainability
- “Lock-in” of diagnostics and therapies?
- How to make sure that post-marketing evidence is valued fairly?
- Can we expect pricing respite though generics/biosimilars?

Evolution of annual clear RIZIV / INAMI spending (hospitals and public pharmacies 2005- 2014) for anti-cancer personalised therapies in Belgium.



Source: Monitoring of Reimbursement Significant Expenses, INAMI, 2014

ESIP's Proposals to Ensure Realisation of the Potential Benefits of Personalised Medicine

The Statutory Social Insurers of Europe

esip
EUROPEAN SOCIAL INSURANCE PLATFORM

A Robust Scheme of Market Access at EU Level that Addresses the complexity of Personalised Therapies

- A comprehensive, consistent and transparent regulatory framework at EU level should govern market access for targeted therapies
- Strong clinical evidence should be required before accessing the market in order to demonstrate the safety and efficacy of targeted therapies
- Conditions of a conditional authorisation should be strictly enforced
- “Early access” must remain an exception based on solid grounds and be subject to strong rules
- European standards must guarantee uniform and reproducible outcomes of diagnostics
- No “lock-in” of diagnostics with therapies

Access to Comparative Data, Data Protection and Patients' Privacy

- The heavy use of data in the context of personalised medicine requires strong measures to protect patients' data and privacy.
- Competent authorities should have early and full access to clinical trials data and international registries.
- The work at EU level on coordinating quality standards for registries should continue to be supported to facilitate the exchange and comparability of data.

Strong Pricing and Reimbursement Policies to Ensure the Sustainability of Healthcare Systems in the Context of Expensive Personalised Therapies

- Evidence on patient-relevant benefit / cost-effectiveness needs to be collected before admission to reimbursement, through robust clinical studies
- Voluntary cooperation on HTA, including joint horizon scanning
- Policy measures aimed at ensuring the affordability of available therapies and preserving the sustainability of healthcare systems remain crucial
- Voluntary cooperation on joint procurement

Patient Empowerment and Healthcare Provider Information to Make the Best Use of Personalised Medicine

- Patients should be informed about any lack of robust evidence, the possible beneficial effects and the risks of side effects of personalised therapies (access to clinical data)
- The patient population must be clearly defined and the centres that administer these drugs must demonstrate explicit patient consent to the therapy and to the collection and use of their data
- Guidelines should be developed to ensure healthcare providers have the necessary knowledge, skills and competences to help patients reach an informed decision about the use of personalised medicine.
- Closer cooperation between national bodies (i.e. healthcare professionals' representatives and national public health authorities) responsible for elaborating guidelines, including therapeutic guidelines and guidelines for biomarker testing and reporting.

THANK YOU VERY MUCH FOR YOUR ATTENTION!

Further Reading:

Position Paper of the European Social Insurance Platform (ESIP) on Personalised Medicine
https://www.esip.eu/images/pdf_docs/ESIP_Position_Personalised-Medicine.pdf

Ermisch M, Bucsics A, Bonanno PVella, Arickx F, Bybau A, Bochenek T, Van de Casteele M, Diogene E, Fürst J, Garuolien\.e K et al.. 2016. Payers' Views of the Changes Arising through the Possible Adoption of Adaptive Pathways. Frontiers in Pharmacology. 7

Finlayson AE, Godman B, Paterson K, Aston E, Haycox A, Gustafsson LL, Ali R. 2013. Personalizing healthcare: from genetics through payment to improving care? Journal of the Royal Society of Medicine. 106:41–44.