Early Dialogue between payers and developers of medicinal products – payers' perspective

AD SCHUURMAN, HEAD OF THE BUSINESS CONTACT CENTRE & INTERNATIONAL AFFAIRS

NATIONAL HEALTH CARE INSTITUTE

ANNA BUCSICS, ADVISOR TO ESIP AND MEDEV

Commission Expert Group on Rare Diseases Meeting Nov 13, 2015,

The opinions presented here do not represent an official ESIP opinion.
Special thanks to the members of the MEDEV Committee and (former) Colleagues at EUnetHTA
Their contributions are gratefully acknowledged.
Any mistakes in the presentation are exclusively those of the authors

Key Points of the Joint Position of the European Social Insurance Platform (ESIP) and Association Internationale de la Mutualité (AIM) on Access to innovative medicines

- Steering pharmaceuticals R&D on the basis of needs
- Ensuring a central role for Health Technology Assessment (HTA) in market access and pricing and reimbursement decisions
- Strengthening national pricing and reimbursement mechanisms in an EU context
- Increasing transparency around innovative pharmaceuticals within the EU
- Support for industrial competitiveness must not be allowed to supersede public health interests
- Supporting innovation in the context of sustainable health systems

http://www.esip.eu/files/ESIP-AIM%20Joint%20position%20on%20access%20to%20innovative%20medicines.pdf

Steering pharmaceuticals R&D on the basis of needs – benefits of Early Dialogue

- Communicating research priorities for individual medicines
- Helping to clarify expected value and required proof of value
  - to inform „stop-or-go“ decisions
  - To inform trial design for HTA and reimbursement decision-making
- To reduce the uncertainty for all stakeholders and to streamline the processes for making medicines available to patients

Comprehensive Lifespan Approaches: MAPPs and MoCA

MAPPs is a prospectively-planned lifespan approach, encompassing:

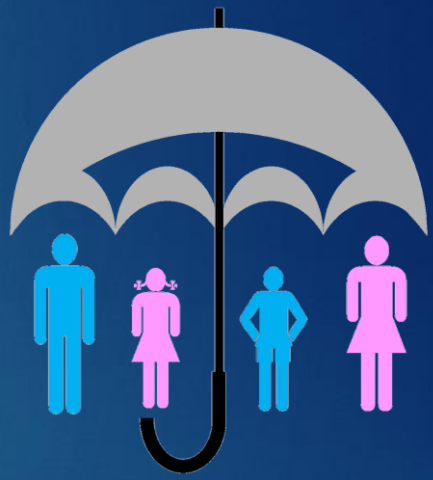
- **An iterative development plan (e.g. either by gradual expansion of the target population, perhaps starting from a population with high(est) medical need, or progressive reduction of uncertainty after initial authorisation based on surrogate endpoints)**
- **Engagement with HTAs and other downstream stakeholders,** with proposals for how the demands of these stakeholders can be met.
- **Monitoring, collection and use of real-world data, post-authorisation,** as a complement to RCT data, to inform updates to the regulatory label and to the positions of other stakeholders.

(www.ema.europa\adaptive pathways, accessed 19.10.2015)

MoCA is a oluntary engagement at all stages of the orphan drug de-velopment process, cross-border, on a continuum

- Horizon-scanning & early dialogue
- Clinical development
- Early Access Programmes
- Therapeutic Scientific Compilation Reports
- Patient selection
- Transparent Value Framework
- Pricing
- Using existing specific tools & processes for OMPs
- Possible collective value assessment for individual purchasing agreements

Payers' Perspective



THERE IS NO SINGLE PAYERS' VIEW ON THE SEVERAL DIFFERENT APPROACHES TO IMPROVE ACCESS TO MEDICINES.

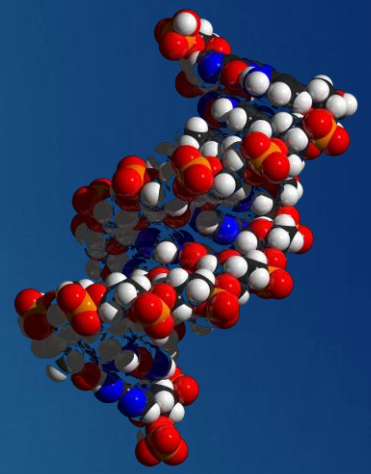
THIS PRESENTATION AIMS TO INCLUDE AS MANY POINTS AS POSSIBLE. IT IS BASED ON THE ITEMS OF THE EUNETHTA CORE MODEL™ CHECKLIST

Concerns about Early Dialogue

Although all parties reiterate that early dialogue/parallel scientific advice is voluntary and nonbinding, there are concerns that advice-givers will be bound, even if the advice given is obsolete by the time the product is assessed for reimbursement

MAPPs is a "moving target"

- Scope is not clear – will it be restricted to products addressing urgent unmet need or to all products?
- Will this pathway be the default for "personalized/precision medicine"?
- Experience with follow-through on commitments makes payers skeptical
- Are payers' concerns adequately addressed?



Concerns about Assessing Efficacy

- Decrease in robustness of evidence/increased uncertainty
- Decrease in quality of evidence for efficacy/safety
- When is such evidence acceptable for
  - Marketing authorisation?
  - Reimbursement?
  - Rescinding of marketing authorisation and/or reimbursement conditions if claims/expectations/forecasts are not met?

The Real World Evidence/registries avalanche

- Postmarketing data are subject to various forms of bias which cannnot always be controlled for
- RWE mostly collected for what industry & prescribers think relevant
- Informs generally more about safety than effectiveness
- Contains generally no good QoL
- Or other parameters that help payers decide
- Cost-effectiveness will become an outcome - Validation of pharmacoeconomic models

Safety

- Acceptable risk in a new medicine licensed via MAPPs still depends on the potential benefit. If the adaptive pathway will be used for medicines to treat severe conditions, eg orphan diseases, acceptable risk may be greater, so better monitoring is welcome.
- For orphan medicinal products, the safety database is currently very limited. Ensuring that all patients treated with a new medicine can be followed up (eg by inclusion in a comperhensive registry) can enhance safety, if safety signals can be picked up earlier.
- **This does not necessarily apply to widely-used products, where common side effects can be detected in large clinical trials.**

Prices, Prices, Prices…

- Initial price - A low starting price will incentivize the industry to complete development a.s.a.p. and will give payers a better starting point for negotiations
- Adaptive pricing: expansion of indication, definitive evidence of benefit
- Evidence and Value-based pricing
- Fair price/return on investment
- Outcomes-based pricing
- Who pays for post-authorisation data collection?
- Reference pricing/confidential rebates
- Budget impact - Even cost effective treatments may blow up the bank vault…

Ethical Analysis

- "Randomise the first patient" – until the evidence unequivocal on risk vs. benefit, the comparator may be the best choice – even if it is a placebo – we simply do not know until we know!
- Difficult to recruit patients into clinical trials after a medicine is authorised as having a positive risk-benefit ratio
- Early access schemes should be restricted to therapeutic areas in which no (or only insufficiently effective) alternative therapies are available
- Do patients have an obligation to be included in registries?
- What are the consequences for patients if the company or the therapy does not deliver?

Organisational Aspects

- Who will be responsible for **registries**?
- Who has access to registry data?
- How can premature expanison of the indication (off-label-use) be prevented?
- How to organise **managed entry**?
- Who will prescribe/deliver the therapy?
- Arising **manufacturing issues** – can these be solved if Phase III is eliminated?

Social aspects

- How to consider publicly funded research/cooperation of patient organisations?

  It is not acceptable that a large part of the research being done uses public funding while the profits are accrued privately by industry selling the developed medicinal products to the same public

- Transparency of clinical trials data, of medicine prices & expenditures is necessary for trust – and also for robust assessment of post-authorisation data
- Disruptive aspects of restriction/delisting/deauthorising products which did not deliver

Legal Aspects

- Will any legal provisions be changed?
- Begin of data protection for products with a narrow indication could be a deterrent for companies – is this a problem?
- **How to ensure that commitments can and will be honored?**
- Current experience is not encouraging

Dutch experience: Outcome based deals rarely work

- Coverager with Evidence Devleopment findings since 2006: not really positive
  - Effect must be sufficiently big and credible
  - Products with greatest uncertainty least likely to yield positive results:
    - Oncology
    - Orphan drugs
- ...Lessons to be learned for everybody

Collaboration in adaptive pathways requires guarantees

- The house of the insured package should have a back door
- Product should not be part of package during adaptive period
- Doctors and patients should agree in writing to possible abrupt withdrawal
- Industry should undertake to recall all stock immediately when adaptive period is not prolonged
- Industry should demonstrate how data collection in adaptive period will accelerate proof of effectiveness

Looking for solutions to the problems

- The EU's Innovative Medicines Initiative authorised a Coordination and Support Action project (ADAPT-SMART) for the MAPPS/Adaptive Licensing pilot to address these issues
  - Work packages co-led by industry
  - Payers' involvement limited
- http://adaptsmart.eu/

ADAPTSMART

MoCA

- Is an informal process of exchange between patient groups, payers and companies
- Participants are also aware of the problems outlined
- Aims to find solutions through direct exchange, "learning by doing", "case by case"
- Was conceived to facilitate access through international coordination of national pricing/reimbursement discussions
- Will NOT duplicate MAPPs

Conclusions

- New outcomes based deals should be further explored
- We must try to stay away from previous mistakes
- All stakeholders should shoulder a tangible responsibility
- Keeping it simple is a challenge

Thank you very much for your attention and contributions to the discussion!