Managing uncertainty over the life-span of drug development and use: Recent EMA developments

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How can we address the access vs. evidence trade-off?

Competing objectives

- Allow timely access for patients to address unmet medical need: “the safest drug that arrives too late is of no benefit to a patient”
- Provide an environment supportive of innovation
- Provide ‘complete’ information on benefits, risks, relative effectiveness
From magic moment to life-span management

Current model of licensing
“The Magic Moment”

Adaptive Licensing

← Access vs. Evidence →

Knowledge, investment

Time (years)
Drivers of adaptive pathways

Why change a ‘tried and tested’ concept?

• Patient expectations: demand for timely access and emphasis on unmet medical need
• Emerging science: fragmentation of treatment populations and early disease interception
• Healthcare systems under pressure: rise of payer influence
• Pharma/investors under pressure: sustainability of drug development
A systems approach

Comprises the entire life-span:

Development → licensing → coverage → utilization → monitoring

Adaptive Licensing → Adaptive Pathways
What will change with adaptive pathways?

Transition from ...

Magic moment → life-span management
Prediction → monitoring
RCT only → toolkit for evidence generation
Big populations → small populations
Focus on licensing → focus on patient access
Open utilisation → managed utilisation
From prediction to monitoring

Realised versus inherent risk

• 1950/60s: thalidomide (phocomelia; 10,000 cases) high-visibility, low background event!

• 2005: natalizumab (PML; 3 cases)

• 2009: Pandemrix (narcolepsy; 15 cases), but...

• high-background or low visibility events (e.g. MI in diabetics)?
What needs to be in place to enable adaptive pathways? 1/2

• Culture of collaboration with patients and physicians to agree on level of unmet need and acceptable uncertainty

  EMA initiatives: pilot programs to elicit patient preferences

• Collaboration of sponsor, regulators, payers/HTA bodies throughout the life-span of a product

  EMA initiatives: ample experience with parallel scientific advice with HTA bodies
What needs to be in place to enable adaptive pathways? 2/2

- Rapid learning systems for data generation across whole life-span → to minimise realised risk (as opposed to inherent risk)

  EMA initiatives: Risk management plans, data infrastructure / analysis projects

- Tools to provide reasonable assurance of appropriate Rx

  EMA initiatives: ?? (Risk minimisation activities)
Conclusion

• We are on a trajectory to more adaptive pathways

• The speed of change will depend on how fast preconditions can be met

• Adaptive pathways are likely the best (only?) way to address the access versus evidence trade-off

• **EMA initiatives:** ‘Adaptive Licensing Pilots Project’; to date: 28 products submitted, 9 selected for pilot – watch this space!
Thank you

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Discussion slides – will not be presented during main talk
From RCT to toolkit for evidence generation

Current scenario:
Post-licensing treatment experience of many patients does not contribute to evidence generation

Adaptive Licensing:
After initial license, patient experience is captured to contribute to real-world information
From licensing focus to patient access

Drug candidates

- Does the drug do more good than harm in a defined group of patients?

Regulatory Agency

- What are the health and cost consequences associated with this drug relative to other interventions?

Payer

- How does the drug perform relative to other interventions in this patient?

Prescriber

- Am I willing to take the risks for the benefits? (Am I able to pay for this out-of-pocket?)

Patient (as payer)

Market and patient access