Relation between pharmaceuticals regulatory framework and timely access of patients to medicines: Reflection on difficulties and opportunities

Pharmaceutical Committee:
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D5: Medicinal products – authorisations, European Medicines Agency
2015: a landmark for the EU pharmaceutical legislation (1/3)

1965: 50 years since first Community rules on medicinal products. **Directive 65/65/EEC**

- prevent a recurrence of the thalidomide disaster
- safeguard public health by not allowing medicinal products ever again to be marketed without prior authorisation.

• 1995: 20 years since:
  
  - The establishment of the EMEA
  - new European procedures: centralised procedure and mutual recognition procedure
2005: 10 years since most provisions of the 2004 review of the Directive and Regulation took effect:

- New rules for approval of generics (8+2 yrs data exclusivity)
- Scope of centralised procedure
- Quicker approval process
- Stricter pharmacovigilance
- Increased transparency
- Compassionate use
- Support of SMEs

In addition legislation on **orphans** and **paediatrics**
2015: a landmark for the EU pharmaceutical legislation (3/3)

• Furthermore:
  • In 2008 Strategic Commission communication on "Safe, Innovative and Accessible Medicines: a Renewed Vision for the Pharmaceutical Sector"
  • And new legislation on falsified medicines (2011) and reinforced pharmacovigilance (2010, 2012)
Earlier Access to innovative Medicines

- Since the 1965 first pharmaceutical law a lot has been achieved:
  - rigorous evaluation of safety, quality, and efficacy of medicines
  - Streamlined and efficient procedures for the evaluation and authorisation of medicines and post-authorisation monitoring
  - Incentives (market and data exclusivity, orphan and paediatric medicines) to encourage innovation
  - flexibilities (conditional MA, exceptional circumstances, accelerated assessment) for faster approval of medicines
- Still one issue continues to be raised: Earlier access to innovative medicines for patients
Earlier Access to innovative Medicines

Several discussion fora and proposals have emerged the last years, including:

- **Council** conclusions on the "Reflection process on modern, responsive and sustainable health systems" and Reflection process under the **Council Working Party on Public Health at Senior Level (SLWP): Subgroup on cost-effectiveness of medicines**- Coordinator: The Netherlands; Members: Belgium, Cyprus, Greece, Poland, Spain, European Commission

- **EMA**: Adaptive licensing initiative

- **The Innovative Medicines Initiative (IMI)**: Medicines Adaptive Pathways to Patients (MAPP)

- **International developments on adaptive approaches to authorisation of medicines**
Council conclusions from December 2013 on the "Reflection process on modern, responsive and sustainable health systems"

- Council Conclusions encourage the Commission and MS to “continue reflection, on a voluntary basis, on aspects that may have an impact on availability, accessibility, prices, costs, patient safety and innovation of pharmaceuticals and medical devices and, where relevant, on systems that facilitate access, while fully respecting areas of Member States' competence”
Council Working Party on Public Health at Senior Level (SLWP)

• Reflection Process on modern, responsive and sustainable health systems: **Cost effective use of medicines**

• **One of the subjects considered:**
  - Can *time to market be shortened* without damaging the safety of pharmaceuticals?

• **Recommendation of SG:** To further explore the area of regulation and market access for pharmaceuticals

• **At the SLWP meeting of 18 February 2014 it was considered that discussion on this topic could be continued at the Pharmaceutical Committee**
EMA-adaptive licensing pilot

• EMA Road map to 2015
  • [...] a key issue for regulators will be whether a more ‘staggered’ approval’ (or progressive licensing) concept should be envisaged for situations not covered by conditional marketing authorisations [...] 

• "Adaptive licensing is a prospectively planned, flexible approach to regulation of drugs and biologics. Through iterative phases of evidence gathering to reduce uncertainties followed by regulatory evaluation and license adaptation, AL seeks to maximize the positive impact of new drugs on public health by balancing timely access for patients with the need to assess and to provide adequate evolving information on benefits and harms so that better-informed patient-care decisions can be made".

EMA-adaptive licensing pilot

• Other features of adaptive licensing:

  • ‘evidence versus access balance’
  • earlier regulatory approval to align with patient needs for timely access and for data to inform medical decisions
  • Stepwise learning while accepting acknowledged uncertainty about product’s efficacy and safety
  • progressive reduction of uncertainty
  • staged approach to collection of evidence and consequent adaptation of MA
  • More timely and open dialogue between sponsors, regulators and payers
EMA-adaptive licensing pilot

- **EMA** is planning to initiate soon a **Pilot Project on Adaptive Licensing** in order to address a range of **technical and scientific questions** and refine the understanding of AL.

- The **Commission** services will need to examine the **legal and policy aspects** related to AL in collaboration **with the Member States** and by consultation of relevant stakeholders, as necessary.
International developments-among others FDA Breakthrough Therapies

• In July 2012 the FDA Safety and Innovation Act (FDASIA) was signed. FDASIA provides for a new designation - **Breakthrough Therapy Designation**. A breakthrough therapy is a drug:

  • intended alone or in combination with one or more other drugs to treat a **serious or life threatening disease or condition** and

  • **preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies** on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development.

• If a drug is designated as breakthrough therapy, FDA will expedite the development and review of such drug.

Regulatory flexibilities under the current EU legislation for timely access

- Approval with conditions
- Conditional approval*
- Exceptional circumstances*
- Accelerated assessment
- Compassionate use
- Treatment on a ‘named-patient basis’

From 2003-2013: 56 conditional/exceptional circumstances MA over a total of 550 MA i.e. 10%
Discussion

The Commission services want to discuss with the Member States the relation between pharmaceuticals regulatory framework and timely access of medicines to patients, specifically regarding the following points:

1. Analyse the perceived problem and the reasons for it and to what extent they are related to marketing authorisation procedures or other policy areas

2. Examine whether current approaches to marketing authorisation meet the objective to ensure timely access of patients to new medicines

3. Study if there are ways to improve the situation within the current legal framework

4. Analyse the perceived merits and weaknesses of an adaptive licensing approach from the regulatory/policy point of view, including the acceptable levels of uncertainty, possible change of paradigm and the consequences of shifting evidence gathering to the post-authorisation phase

5. Examine how AL fits within the current legal framework and principles of legislation

6. Consider whether any action would be useful or necessary