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Pharmaceutical Committee

Update on the European Commission expert group on Safe and Timely Access to Medicines for Patients (STAMP)

18 October 2016

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STAMP meeting 28 June 2016

- **Repurposing** of established medicines
- Presentation of **Health Technology Assessment** Network paper on **synergies** between regulatory and HTA issues
- Updates on **early access initiatives** – adaptive pathways and PRIority Medicines (PRIME)
- **Off-label use** of medicinal products
- Update on **Council conclusions**

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Repurposing of medicines

- process of identifying a new use for an existing drug in an indication outside the scope of the original indication
- 10 March 2016 STAMP meeting considered that off-label use of medicines for patient access should be explored further and agreed that a questionnaire should be circulated to seek more information on 'important' authorised medicines widely used off-label
- 5 questions posed to Member States
- UK representative presented overview of replies to the questionnaire

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HTA reflection paper

- Aim to identify activities along the life-cycle of health technologies in which cooperation between regulatory and HTA bodies can contribute to facilitating efficient access to effective, safe, innovative, and added value technologies
  - On-going and new activities
  - To be addressed in both short and medium/long term
- Focused on pharmaceuticals
- Implementation of the activities identified - not in the scope of the Reflection Paper yet…

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HTA reflection paper - continued

3 areas for possible collaboration

- Pre-marketing phase
- Market entry
- Post-marketing launch phase

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HTA reflection paper - continued

- Finalisation of the Reflection paper by the HTA Network
- Planned adoption date 10 November 2016 (2nd annual meeting of HTA Network)
- Implementation of the identified areas for collaboration

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Adaptive Pathways

EMA Pilot

- Launched in March 2014 – closed 2016
- Relevant for medicines with the potential to treat serious conditions where there is an unmet medical need.
- Cooperation between a wide range of stakeholders

How AP fits within the current EU regulatory framework?

- **Scientific issues-EMA pilot project**
- **Legal-policy issues-28 MSs at Pharmaceutical Cttee**

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Adaptive pathways pilot - continued

- Presentation to STAMP of learnings from the pilot
- Report of the pilot published 3 August 2016
- Stakeholder workshop planned 8 December 2016

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PRIME scheme update

- Launched 7 March 2016
- Until June 2016
  - 35 applications evaluated
  - 16 orphan, 19 non-orphan
  - 6 eligible for PRIME scheme, 20 denied, 6 out of scope

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First 6 products granted eligibility

CCX-168
Treatment of patients with active ANCA-associated vasculitis (GPA and MPA)
Orphan

KTE-C19
ATMP
Treatment of DLBCL, PMBCL, TFL
Orphan

CTL019
ATMP
Treatment of paediatric patients with relapsed or refractory B cell acute lymphoblastic leukaemia
Orphan

Emapalumab
Treatment of primary haemophagocytic lymphohistiocytosis (HLH)
Orphan

Adacunumab
Alzheimer's disease

rVSVΔG-ZEBOV-GP, live attenuated
Vaccination against Ebola (Zaire strain)

EMA reflections on PRIME experience to June 2016

- ✓ Number of requests received confirms **high interest** from industry, particularly **SME**
- ✓ **Cross-committee collaboration** enables scrutiny from our scientific committees and oversight group to ensure consistency and discussing policy aspects of implementation.
- ✓ A number of products are in **late stage** of development -> this may be due to recent launch of the scheme.
- ✓ Next phase of the scheme: Support to applicants with **kick-off meeting** being organised.
- ✓ **HTA engagement** during development through parallel advice procedure will be of key importance.

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Off-label use of medicines

- Belgian Healthcare Knowledge Centre Report "*Towards a better managed off label use of drugs*"

- Presentation by contractor of draft final report on off-label use of medicinal products in the EU
  - Members of STAMP invited to review the draft report and to comment during the meeting or in writing

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Off-label use of medicines: next steps

- Final report to be finalised by end 2016
  - Scope: scientific (patient safety) and legal (regulatory framework)

- Reflection with Member States in STAMP (?) on:
  - Issues identified in the study
  - Possible future actions: need for coordination? Exchange of best practices? Guidelines?....

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Thank you for your attention

More information:

http://ec.europa.eu/health/documents/pharmaceutical-committee/stamp/index_en.htm

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