Federal agency for medicines and health products

Innovation and access to new medicines
HMA subgroup on Timely access
Compassionate use

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1: Mandate (1/2)

Innovation and access to new medicines is one of the key priorities of the HMA Multi-annual Work Plan :

- **Early detection of promising drugs**
- **Guiding an appropriate clinical development**
- **Tailored access trough clinical trials and/or CU programs**
- **Efficient and fair regulatory process**
- **Integration with Price and reïmbursement decisions**
- **Integration in entire life cycle of medicines**

1: Mandate (2/2)

Main actions :

- **Explore flexibilities that EU regulatory framework offers for**
  early access of innovative products at national level
  - Clinical trials , Compassionate Use , off-label use
  - Contributing to further optimisation (conditional approval / accelerated assessment / PRIME
- **Harmonising requirements of registries and defining circumstances for Real World Data**
- **Improve the involvement of patients , health care professionals and academic communities**
- **Reinforce the collaboration with HTA, P&R to allow optimal market access**

2 : Use of national flexibilities for early access

Main actions :

- Inventory of all activities of NCA's at global/national level
- Update reporting STAMP achievements to HMA
- CU programs :
  - general document to be presented at HMA
  - suggestions for update HMA website
  - analysis of existing CU programs
  - sharing of best practices
- Cooperation in off-label use , sharing common view .

- Collaboration with EMA (national innovation offices , optimising exisiting procedures , Prime ..)

3 : CU program published on the HMA website

- **Link :** http://www.hma.eu/fileadmin/dateien/HMA_joint/02-_HMA_Strategy_Annual_Reports/08_HMA_Publications/2016_05_HMA_Compassionate_use_program.pdf

- **Current situation :**

  - **Many member states involved with link to their respective NCA websites**
  - **However :**
    - **Information for some of them is only available in the national language**
    - **Some member states have CU programs published on their website but are still lacking at the HMA website**
    - **Referring to the literature some member states seem to have CU programs which are not yet accessible via website(s) ?**

3 : CU program published on the HMA website

- **New suggestions :**

	Reference included in HMA document?	Relevant info found on the website (in English)?	Compassionate use programs (cohorte)	who can apply?	list of approved CUPs	number of approved CUPs	named patient / urgent need for specific patient	who can apply?		Compassionate use programs? (IRDR source)
Austria	yes	yes	yes	Manufacturer, if also the sponsor of approved clinical trials for the respective medicinal product, or the applicant for a marketing authorization	yes	14 (from 2010)	yes: no notification or approval by agency (except for gene technology) - responsibility of treatting physician	medical doctor, dentist or veterinarian		yes
Belgium	yes	yes	yes	Future marketing autorisation holder, sponsor clinical trial, minister of social affairs	yes	29 (from 2014)	In urgent cases where there is a high risk for the patient. A notification to the agency and the ethics committee of the site concerned is strongly recommended, but is not required to start the treatment.	the sponsor (with declaration from physician)		yes

4 : Next steps

- Document to be proposed at next HMA WG

- Suggestions for improvement of the current website to be validated by HMA WG

- Further analysis identifying best practices
  - Recommendation to NCA's to publish the authorised CUP's on their website
  - Worksharing on B/R ?
  - Hurdles UMN related to reïmbursement issues
  - ....

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