BfArM
Bundesinstitut für Arzneimittel und Medizinprodukte

Paediatric Use Marketing Authorisation (PUMA) from a National Competent Authority Point of View

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Why Push Paediatric Medicines in the EU?

- More than 100 million children in the enlarged EU to benefit from the availability of safe and effective medicines

- Healthcare professionals to benefit through the supply of medicines specifically developed for children; may take part in clinical research on medicines for children

Who will be Affected?

- Children and healthcare professionals
- All pharmaceutical companies seeking access to a single national or the EU market
- All NCAs and EMEA will have to change their working practices as a result of the Paediatric Regulation

The Paediatric Regulation – Key Measures (1)

- **New medicines/line-extensions of patent protected medicines:**
  Paediatric Investigation Plan, deferrals and waivers

- **Established medicines:**
  Paediatric use marketing authorisation (PUMA)

The Paediatric Regulation – Key Measures (2)

Established and new medicines:

- Establishment of Paediatric Committee
- Increase of robustness of pharmacovigilance
- EU inventory of therapeutic needs of children to focus research, development and authorisation

The Paediatric Regulation – Key Measures (3)

Established and new medicines:

- Free scientific advice for industry by EMEA
- Implementation of a European register of clinical trials in products for paediatric use in the European database
- Community research programmes financing supplementary research into products, not protected by a patent or supplementary protection certificate

The Paediatric Regulation – PUMA a new type of Marketing Authorisation

- In order to establish incentives for authorised products no longer covered by intellectual property rights, it is necessary to establish a new type of marketing authorisation, the

PUMA

The Paediatric Use Marketing Authorisation (PUMA) (1)

- … shall mean a marketing authorisation granted in respect of a medicinal product for human use which is **n o t (!)** protected by a supplementary protection certificate or by a patent which qualifies for the granting of the supplementary protection certificate,….

The Paediatric Use Marketing Authorisation (PUMA) (2)

- …. covering exclusively therapeutic indications which are relevant for use in the paediatric population, or subsets thereof, including the appropriate strength, pharmaceutical form or route of administration for that product

The Paediatric Use Marketing Authorisation (PUMA) (3)

- provides incentives for off-patent medicines
- will utilise existing marketing authorisation procedures, but is specifically intended for medicinal products developed exclusively for use in children

The Paediatric Use Marketing Authorisation (PUMA) (4)

- the name of the product granted a PUMA can utilise the existing brand name of the corresponding product but will display a **symbol** (to be selected by the Commission on a recommendation by the Paediatric Committee) to aid recognition and prescribing

The Paediatric Use Marketing Authorisation (PUMA) (5)

- companies can capitalise on existing brand recognition while benefiting from the data protection for a new marketing authorisation (10 years)
- may refer to data contained in the dossier of a medicinal product which is or has been authorised in the Community
- may refer to data published in literature

The Paediatric Use Marketing Authorisation (PUMA) (6)

- Submission of an application for a PUMA shall in no way preclude the right to apply for a marketing authorisation for other indications

The Paediatric Use Marketing Authorisation (PUMA) (7)

- A PUMA application needs to be accompanied by the particulars and documents necessary to establish safety, quality and efficacy in children, including any specific data needed to support an appropriate strength, pharmaceutical form or route of administration, in accordance with an agreed paediatric investigation plan

Chances and Advantages of PUMA (1)

- The new type of marketing authorisation, the PUMA, **allows ten-years of data protection** for innovation (new studies) on off-patent products
- **Amended data requirements** for PUMA applications to attract SMEs including generics companies

Chances and Advantages of PUMA (2)

- Reference in the original explanatory memorandum to the establishment of an EU paediatric study program MICE **to fund research leading** to the development and authorisation of off-patent medicines for children, now to be covered by the multi-annual Community Framework Programmes for Research, Technological Development and Demonstration Activities

Chances and Advantages of PUMA (3)

- For the first time it will be possible to submit new data in an otherwise generic-type application
- Application in the Centralised procedure possible
- For products authorised for paediatric use: in case of withdrawal of such products from the market, the original MA shall transfer the MA to an interested third party or allow the latter to use pharmaceutical, pre-clinical and clinical documentation of the product

Rewards and Incentives

- Data and marketing protection periods referred to in Regulation (EC) 726/2004 and Directive 2001/83/EC applicable to a PUMA

The National Competent Authority's Point of View

- … fully endorses the intentions of the Paediatric Regulation
- … supports the assessment of paediatric data with contributions/participation to the Paediatric Working Party

The National Competent Authority's Point of View

- … takes an active role in the present Paediatric Work-Sharing Program of CMD(h)
- … will use their best endeavours to contribute to the success of the measures described in the Paediatric Regulation

The Paediatric Work-Sharing Program - Basic Principles -

- To make paediatric data available from products already on the market
- Harmonised European assessment in Work-sharing procedure
- Data should be included in SPC in Section 4.1 or Section 5.1
- Public assessment reports will be published on HMA website to make data available for medical professions

The Paediatric Work-Sharing Program - Basic Principles -

- … has been initiated under the Dutch EU-presidency in November 2004
- … is based on Mutual Recognition
- … follows the 90-day type II variation procedure (without official procedure numbers)
- … is based on two Member States that volunteer to assess the data and prepare and Assessment Report for the other MS

The Paediatric Work-Sharing Program
- Status -

- … “first wave” included 27 products (13 nationally authorised, 14 MRP)
- … has reached the “second wave”

The Paediatric Work-Sharing Program

- … rapporteurs draft assessment reports on the data on children/adolescents and distribute them to the other MSs
- … MAH's may give comments on the assessment report and recommendations.
- …. if the need arises, break-out sessions can be organised

The Paediatric Work-Sharing Program

- … MSs should come to a harmonised conclusion; in case of no agreement, involvement of CMD(h) for the specific issue at stake
- … national authorities will take outcome of the assessment as basis for further regulatory actions (in consultation with MAH's)

The Paediatric Work-Sharing Programme - Next Steps -

- New list of products prepared from FDA, MHRA websites and based on information of companies that they have paediatric data
- MSs are asked to select products from this list based on proposed selection criteria
- Requests for 10 new products sent out in March 2006
- Meeting with assessors in April 2006


Thanks a lot for
your attention!