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European Confederation of Pharmaceutical Entrepreneurs AISBL

Enhancing patient access to care: new approaches to pricing and funding

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About EUCOPE

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European Confederation of Pharmaceutical Entrepreneurs AISBL

The EU association representing mid-sized companies at national and EU level

900+
mid-sized innovative companies

EMIG · IML · BPI · BIO DEUTSCHLAND · france biotech (medtech | biotech) · Bio Biotechnology Innovation Organization

Innovative, mid-sized pharma and biotech companies many in the field of rare diseases

Founded in 2008 in Brussels with **10** member companies

100 **member companies in 2018**

Recognised stakeholder by EMA, HMA, European Commission and European Parliament

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http://www.eucope.org/en/eucope-members/

Focus on EU Advocacy, Regulatory and Market Access

EUCOPE Priorities for 2018

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- **Incentives and reimbursement** for **OMPs, gene therapies and personalized medicines** and fixed dose combinations, EU Commission incentive review, pediatrics review
- **Brexit (regulatory impact)**
- **Foster innovation by coordination on HTA:** avoiding national re-assessments / different comparators / participation in EUnetHTA / parallel advice
- **HTA and rare diseases:** No “one size-fits-all” approach
- **International Reference Pricing to reflect patient access as main objective**
- **No promotion of off-label use for cost containment purposes induced by law:** Respect the authorized indication
- **Restrict tendering** to non-innovative products and non-biologics
- **Joint Procurement / Joint Negotiations:** Only under specific circumstances, voluntary – not suitable for OMPs
- **EMA Transparency Policy:** Clear rules to protect CCI / trade secrets (CJEU intervention, EMA and NCA practice on FOI requests, TTIP)
- **New Clinical Trial Legislation:** implementation and database
- **Medical devices, IVDs :** New EU Regulations (implementation)
- **Serialization and Coding:** pragmatic, cost-effective approach needed
- **EMA Early Access: PRIME + Adaptive Pathways**

The Incentives analysis

Regulations: what could be changed?

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Paediatric Regulation (1901/2006)

- 6-month SPC extension
- 2-year orphan Market Exclusivity extension
- 10-year Regulatory Data Protection for Paediatric Use Marketing Authorisations

Orphan Drug Regulation (141/2000)

- **10-year Market Exclusivity linked to a single orphan designation**
- 1 Market Exclusivity per medicine per « designated » orphan condition (possibility for several MEs for a single orphan medicine)

SPC Regulation

- Supplemental Protection Certificate (max 5 years, max total exclusivity period of 15 years from MA)
- Introduction of an SPC manufacturing waiver for exports outisde the EU

The Incentives analysis
Why EUCOPE decided to get involved

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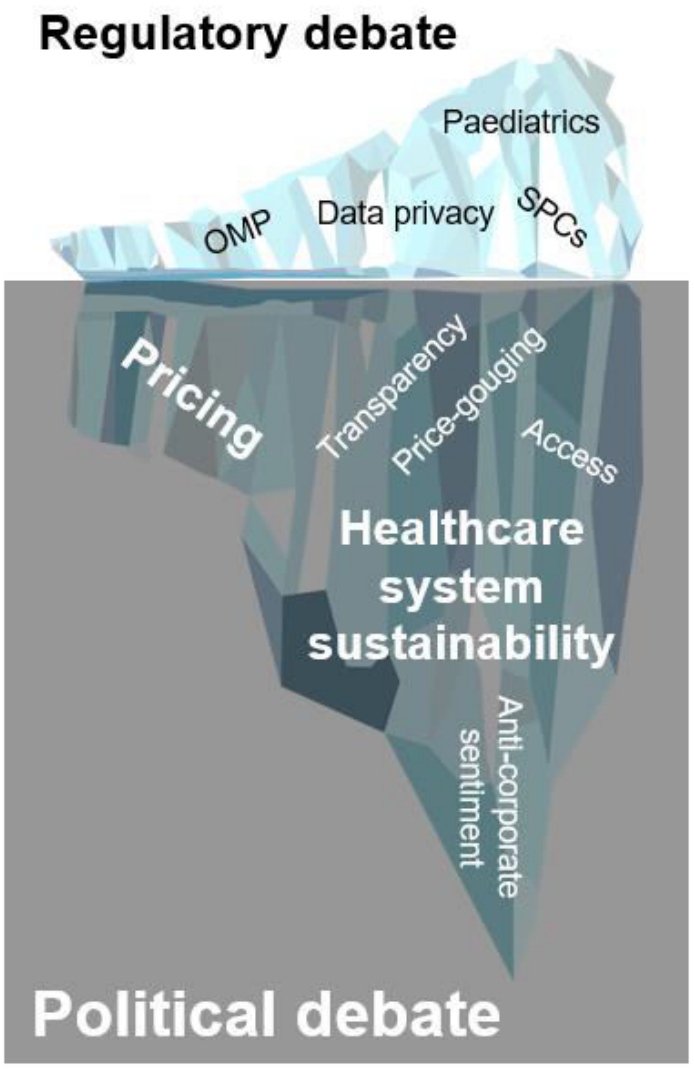

- The Incentives Assessment is a **regulatory AND political debate** identified as of **major importance** to the whole industry
- Other trade associations are involved; EUCOPE contributes the perspective of **SMEs and of OMP-focused companies**
- Some EUCOPE companies can be **disproportionately impacted**

European cooperation on HTA
background

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HTA network

- Voluntary cooperation of national authorities or HTA bodies
- Developing policy papers and identifying potential areas for cooperation
- Third parties include authorities competent for HTA of EEA/EFTA countries, stakeholders' representatives. Industry represented by EFPIA.

EUnetHTA Joint Action 3

- Scientific and technical cooperation mechanism supporting collaboration between HTA bodies
- Objective: increase the use, quality and efficiency of joint HTA work at European level
- 2 workstreams: implementation; structure and methodological consolidation stream to ensure scientific quality
- Running until 2020

Strengthening European cooperation on HTA beyond 2020

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Inception Impact Assessment (Sep 16)	Public consultation (Oct 16 – Jan 17)	Consultation Report (May 17)	EU Commission proposal on HTA (Jan 18)

EUCOPE's submission:

- Divergence of HTA procedures lead to considerable challenges for small to mid-sized companies (workload; hampered business predictability & innovation)
- further cooperation and alignment regarding HTA methodologies is desirable while it is essential to **ensure this cooperation does not lead to a delay in patients' access to new forms of treatment**.
- Favourable to joint tools (templates, databases, etc), Guidelines (e.g. for clinical or economic evaluations), Early dialogues, Joint clinical assessment (REA)
- **Voluntary participation with mandatory uptake** of joint work by the Member States
- **Secretariat**: EU Commission to ensure coherence and neutrality in the process
- **Funding:** the EU and Member States.

Alternative payment models
State of Play

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- **Increasingly personalised medicines** / drastic increase of the number of **clinical choices** open to patients suffering from **rare diseases**

- Affordability challenges for health systems across Europe: Member States' concerns about **sustainable market pathways** from appropriate utilisation of curative & high-budget impact medicines

- Ongoing initiatives to offer solutions to those challenges:
  - Discussions around **value-based pricing** (i.e. indication- and combination-based pricing)
  - **Real World Evidence** (RWE)
  - **Horizon scanning**
  - EC Proposal on HTA, including section on "emerging health technologies"

Alternative payment models
Challenges

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- Existing payment models (upfront costs) or pricing mechanisms (ERP) no longer fit for e.g. one-time curative medicines
- Limited knowledge and awareness of the diseases and therapies, in particular by payers
- Insufficient information to payers concerning budget impact at launch
- Assessment of long-term product value and value for money versus cost at launch
- Role of RWE still uncertain
- Insufficient data at scale to implement innovative pricing arrangements and outcomes certainty for next generation of innovative treatments

Alternative payment models

Ways forward – some recommendations

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- **Defining** breakthrough and curative medicines and **how to assess their value** across the stakeholder spectrum
- Encourage **early dialogue** between regulators and payers/HTA bodies with regards to therapies with high unmet needs
- Ensure **pricing incentives are aligned with value**
  - Price evaluation against the health benefit provided by the treatment
  - Removing disincentives such as ERP
  - Showcase the value of new experiences such as RWE
- Strive for broader support for **value-based healthcare**