European Federation of Pharmaceutical Industries and Associations

european biopharmaceutical enterprises

Making the case for Personalised Medicine

The biopharmaceutical industry perspective






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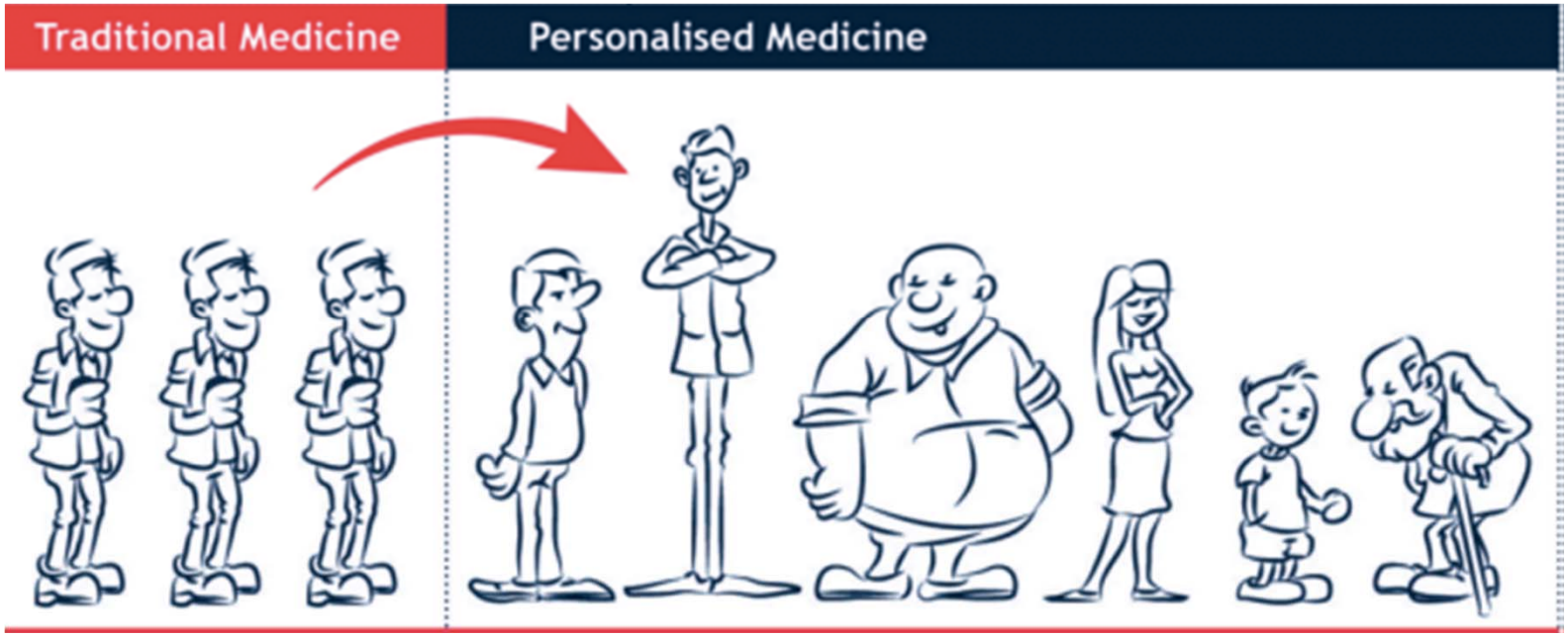
Who we are

EBE represents the voice of biopharmaceutical companies of all sizes in Europe and is a specialised group within EFPIA. EBE currently has 60 members.

EFPIA brings together 33 European national pharmaceutical industry associations as well as 41 leading companies undertaking research, development and the manufacture in Europe of medicinal products for human use.

The **EBE-EFPIA Personalised Medicine Working Group** promotes an appropriate regulatory framework for Personalised Medicine (PM) in Europe and advocates the positive contribution of PM to the sustainability and efficiency of the healthcare systems while providing treatment that is better tailored to patients.

Personalised medicine aims to deliver the right medicine to the right patient at the right time.



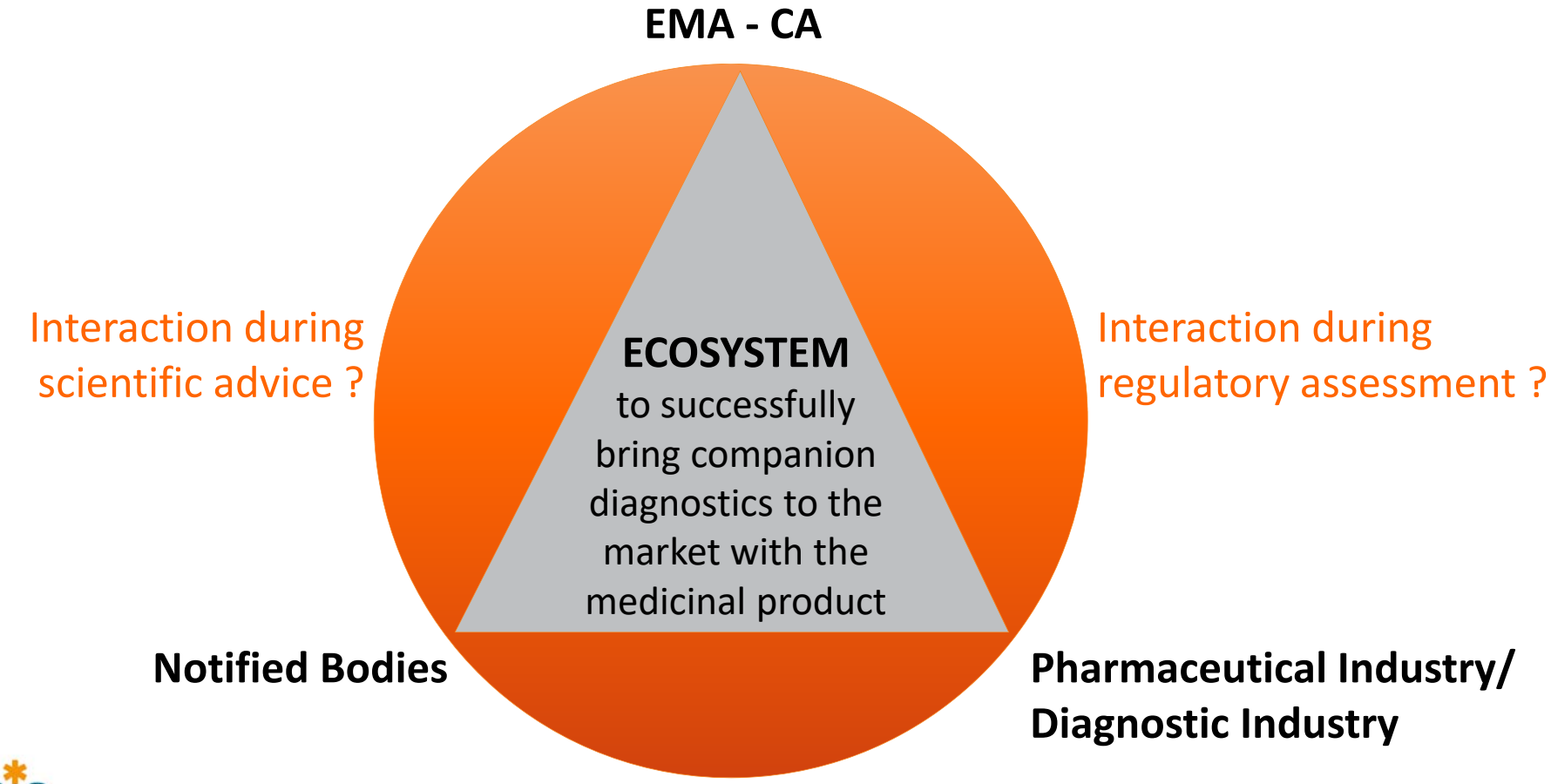
It holds great promise, however some gaps still need to be filled …

Personalised medicine raises challenges

1	**Regulatory challenges** following the adoption of the new In-Vitro Diagnostic Medical Devices Regulation (IVDR)
2	Limited **patient access** to personalised medicine in Europe
3	Need for a European **framework supportive of emerging innovation**

1 - Key regulatory challenges (1/3)

Interaction between EMA- National Competent Authorities (CA) and Notified Bodies during development and authorisation procedure needs to be clarified



Key regulatory challenges (2/3)

Labelling

How will labelling decision be coordinated between the medicinal product and its companion diagnostic?

- Label of a companion diagnostic in its 'intended use' refers to the INN of the medicinal product.
- What would be the appropriate level of description of companion diagnostic test performance in the product information of the medicinal product?

Transitional period during the implementation of the IVDR

Importance to have transparency on development of guidance documents to understand early on the impact for the biopharmaceutical industry.

Key regulatory challenges (3/3)

Framework needed to discuss delineation of EMA, CA and NB responsibilities

EMA announced the publication of a "*Concept paper on the need to develop a guideline on co-development of biomarker-based companion diagnostics and medicinal products in the context of drug development"* (in preparation, draft planned to be published in Q2) – may provide some clarification

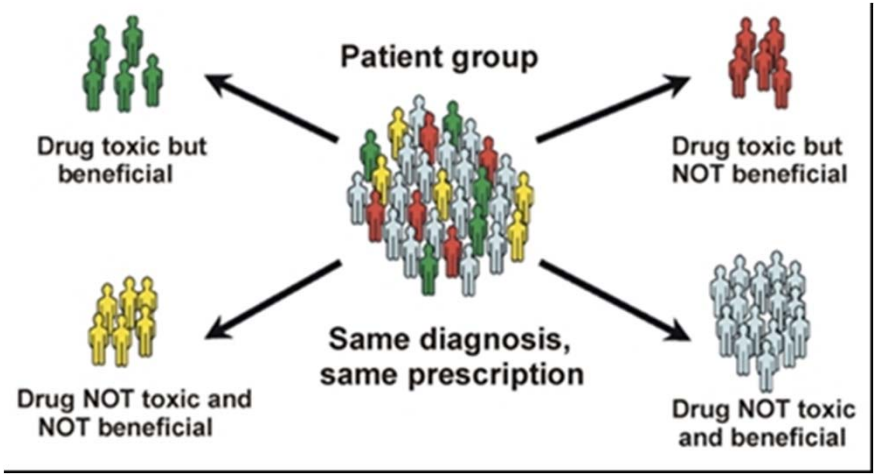
EBE-EFPIA propose a SPECIFIC WORKSHOP to discuss the challenges in detail

- NBs and companion diagnostic developers should be invited in addition to medicinal product manufacturers and CAs to ensure all relevant stakeholders understand the status and can provide input
- Stakeholders could prepare proposals on specific topics for this workshop

2 - Access to PM in Europe

Personalised medicine brings benefits to society

Examples: selection of optimal therapy, reduce trial-and-error prescribing, reduce drug-related adverse reactions, improve health outcomes, etc.



However, uncertainties remain and decision-makers fear the impact of Personalised Medicine on sustainability of the healthcare systems

EBE-EFPIA aim to launch a project (Q3 2017) showing that Personalised Medicine brings positive development in clinical outcomes, and also will generate efficiencies and incentives for healthcare systems in the long term, improving patient access.

3 - Need for a European framework supportive of emerging innovation (1/2)

Framework adapted to the use of multi-marker signatures

Current environment: 'one drug – one test' paradigm
Evolution to tests with multiple markers leading to a treatment pathway

→ Need for adaptive pathways and adapted value assessment, as well as education for patients on test panel (signature) approaches

Use of electronic health record systems

Health systems are transitioning towards tracking electronic patient data from diagnosis over treatment to outcomes in a continuous way

→ Need for integrated electronic patient health data systems with inter-operability standards;
will support generation of real world data for regulatory submissions

Need for a European framework supportive of emerging innovation (2/2)

Importance of data privacy and informed consent in healthcare research

General Data Protection Regulation adopted in 2016. It delegates key aspects of data privacy and informed consent to Member States.

- Potential for development of divergent requirements with the
- Potential for unintended negative consequences for use or re-use of electronic health records or 'omic' data in clinical practice, research, drug development and reimbursement, and the
- Potential to impede other EU legislation (e.g. cross border health care directive).

→ EFPIA and EBE work with stakeholders to raise awareness

Conclusion

- **Personalised Medicine: EBE and EFPIA work to address current challenges that hamper personalised medicine from becoming reality**
  - EBE & EFPIA advocate for clarification of the regulatory environment and the interaction mandated by the IVD regulation between EMA, National Competent Authorities, Notified Bodies, and other stakeholders to bring Personalised Medicine to patients
  - EBE & EFPIA will demonstrate benefit to patients in terms of clinical outcomes and access, and to healthcare systems
  - EBE & EFPIA promote a system supportive of innovation, including
    - changing the "one test – one medicinal product" paradigm,
    - improving and implementing electronic Health Records,
    - use of Real World Data in personalised medicine regulation that acknowledge data privacy needs
- **Support by all stakeholders is necessary to effect change, which EBE and EFPIA seek to facilitate**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you

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