



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Regulatory agency representative

Regulatory aspects

Presented by Francesco Pignatti on 29 September 2016
Office for Oncology, Haematology and Diagnostics

An agency of the European Union





Regulatory challenges

Addressing the challenges of personalised medicine

Making the most of observational studies and data sharing

Developing models of interaction to generate relevant data

Frameworks for patient involvement in benefit-risk assessment



Addressing the challenges of personalised medicine

The issue: Generating sufficient clinical efficacy/safety data for cancer drugs developed based on multiple assays to select the right treatment or combinations no longer feasible with large randomised controlled trials in unselected populations

- Analytical and clinical validation of a biomarker assays
- Methods for assessing evidence from clinical trial data in small populations; facilitate patient entry into clinical trials
- Addressing the combinatorial complexity
- How to address remaining/future questions (e.g., dose, treatment optimisation, long-term follow-up)
- Viable models for conducting biomarker studies post-approval



Making the most of observational studies and data sharing

The issue: Observational studies, modelling and extrapolation and other non-RCT data will likely be used as supportive data but these are not collected or shared systematically and no agreement on optimal methods on how to manage bias

- Develop common principles and frameworks;
- Develop of high quality databases and access to anonymised data electronic health records
- Optimal long term follow up for appropriate long term patient benefits/safety monitoring



Endpoints

The issue: Conventional endpoints (e.g., overall survival) are difficult to interpret outside RCTs. Evidence will have to be collected across a variety of corroborative endpoints

- Methods for dealing with the uncertainty from multiple endpoints, establishing the surrogacy and clinical relevance
- Developing best analysis practices and instruments for data collection of patient reported outcomes e.g., health-related quality of life



Models of interaction

The issue: single-drug, single-target, single-diagnostic clinical development is inefficient. Need to adapt clinical drug development to meet the individual patient characteristics

- Opportunities for greater co-operation among stakeholders
- Identify optimal models for interactions between patients, industry, regulators, HTA, and academics; encourage a systematic early dialogue
- Develop infrastructure and collaboration models for integrated industry – academic, adult – paediatric clinical development
- Develop models for pursuing development of abandoned medicines (including funding)



Data Sharing

The issue: Although much progress has been made, only a fraction of clinical data are shared.

Develop sustainable models of data sharing, incl. industry and academic trials

Develop common principles and legal frameworks; develop of high quality databases

Access to anonymised data electronic health records

- detect and analyse safety issues in the real world
- facilitate data collection in clinical trials



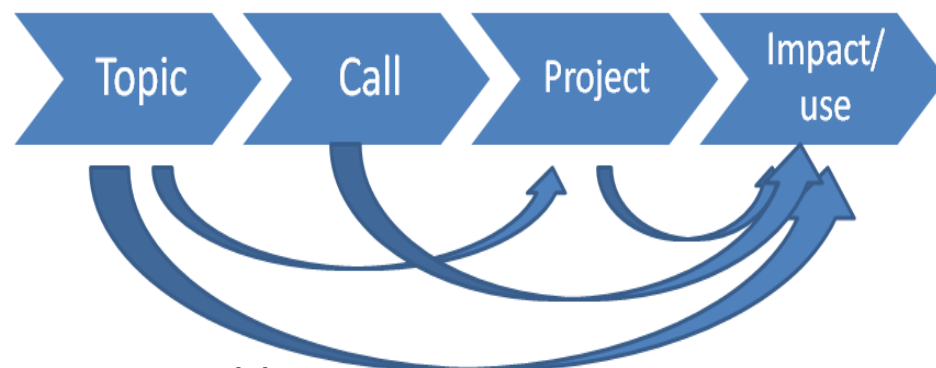
Benefit-risk analysis

The issue: Gain a better understanding of benefit-risk profiles of cancer drugs; patient and other stakeholder's (doctors, regulators) preferences; associated factors

- Exploring quantitative benefit-risk modelling for the comparative analysis of drugs and other treatment modalities for a given indication
- Exploring benefit-risk analysis methods to improve shared decision-making



Regulatory science projects



Considerations: as strategic and effective as possible

- Resource implications
- Conflict of interest – perceived or real
- Alignment with Network Strategy 2020
- Impact on EU public health
- Existing opportunities for regulatory interaction
- Not at the bidding stage – no competitive (dis)advantage



Summary

- Clinical trial designs and evidentiary standards in personalised medicine are changing
- Frameworks for early and more systematic interaction
- Continue development of data sharing frameworks
- Establish systems for monitoring efficacy and safety of drugs in real-life
- Better understanding of benefit-risk of cancer drugs and how they compare to each other
- Make use of existing opportunities for regulatory interaction



Thank you for your attention

Further information

francesco.pignatti@ema.europa.eu

European Medicines Agency

30 Churchill Place • Canary Wharf • London E14 5EU • United Kingdom

Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555

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