# Regulatory Science towards drug innovation & regulatory optimization

Christine Gispen-de Wied
Scientific Programmanager
Medicines Evaluation Board Netherlands
Vice-chair Regulatory Science Network Netherlands


BJCP British Journal of Clinical Pharmacology
Additional
exceptiona
drugs in Eu
Arna H. Arnardottir,[1] Flora
Sabine M. J. Straus,[2,3] Han
Peter G. M. Mol[1,2]
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PRIME: priority medicines
PRIME - PRIORITY MEDICINES
WHAT THIS S
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%). This was similar
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Cox-regression
g type was
CONCLUSION
The EC/CA procedure is not associated with a higher probability of DHPCs despite limited clinical development data. These data do not support the view that early drug approval increases the risk of serious safety issues emerging after market approval.

# We still struggle with the definition

FDA U.S. FOOD & DRUG ADMINISTRATION

Regulatory Science is the science of **developing** new tools, standards, and approaches to assess the safety, efficacy, quality and performance of FDA-regulated products

Regulatory Science is the **scientific** and technical **foundation** upon which regulations are based in various industries-particularly those involving health and safety

# MEB definition

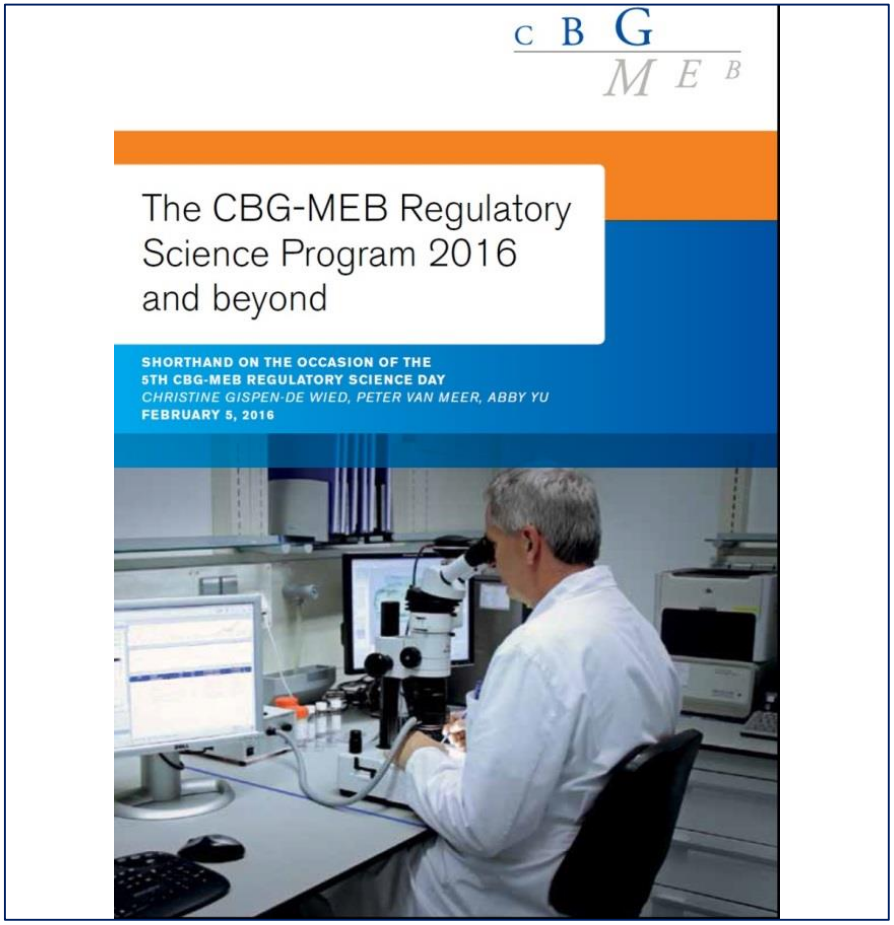

*'Towards drug regulation that maintains its sustainability and is efficient in bringing drugs timely to patients'*

'Regulatory science is the science of developing and validating new standards and tools to evaluate and assess the benefit/risk of medicinal products, facilitating sound and transparent regulatory decision making'

**'Through analysis of regulatory frameworks itself and their effectiveness, however, regulatory science can also advance knowledge of these systems in general'**

# Starting 2007

# Topics at the heart of the MEB



# 1. Drug development & Innovation: D&I

| Domain | Program(s) | Projects | University |
|---|---|---|---|
| 1. D&I | 1.1. 3R Support | 1.1.1. Registry for efficacy models | UU |
| | | 1.1.2. Two species selection | UW / RIVM |
| | | 1.1.3. Predicting carcinogenicity | UL |
| | | 1.1.4. In vitro vaccine models | UU |
| | | 1.1.5. Ancillary 3R projects | |

# 2. Regulation & Decision Making: R&DM

| Domain | Program(s) | Projects | University |
|---|---|---|---|
| 2. R&DM | 2.1. Adaptive pathways | 2.1.1. Best model registry orphan drugs | UU |
| | | 2.1.2. Legal basis stakeholder driven applications | RUG |
| | | 2.1.3. Personalized medicine and biomarkers | UU |
| | | 2.1.4. Levels of knowledge vs acceptable risks | UU |
| | 2.2. Safe (Bio) Generics | 2.2.1. Interchangeability | UM / RUN |
| | | 2.2.2. Personalized modelling | UM / RUN |
| | 2.3 Optimized clinical trials | 2.3.1. Enriched methods | RUG / UU / UVA |
| | | 2.3.2. Trial characteristics | UVA |
| | | 2.3.3. Efficacy vs Effectiveness | UVA |
| | | 2.3.4. Ethical bridging in the Health Chain | UU |

# 3. Consumer Use & Safety

| Domain | Program(s) | Projects | University |
|---|---|---|---|
| 3. CU&S | 3.1. Patient safety | 3.1.1. Communicating risk | RUG |
| | | 3.1.2. Minimizing risk | EUR |
| | | 3.1.3. Prevent medication errors | EUR |
| | | 3.1.3. Tailored management of biologics | UU |
| | | 3.1.4. Signal detection | EUR |
| | | 3.1.5. Ancillary Patient Safety Projects | |
| | 3.2. Fair Access | 3.2.1. Medicines shortages | UU |
| | | 3.2.2. Human rights perspective | UU |

Federatie voor Innovatief Geneesmiddelonderzoek Nederland

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Taal:

RSNN

Regulatory Science Netwerk Nederland

**1. Inleiding**

Regulatory science is een wetenschappelijke discipline, die zich bezig houdt met de meest recente wetenschap en technologie rondom geneesmiddelenontwikkeling en innovatie. Regulatory Science onderscheidt zich van de 'klassieke' academische wetenschappen in die zin dat vragen veelal gegenereerd worden vanuit overheid en industrie. Deze vragen richten zich op het verhogen van de efficiëntie van het regulatoir systeem en verbetering van de effectiviteit ervan op basis van wetenschappelijke onderzoeksresultaten. Regulatory Science geeft inzicht in (verbeterde) methodes om de baten-risico balans te kunnen bepalen, hoe een transparante manier van besluitvorming bevorderd kan worden en hoe dit vertaald kan worden naar beleid binnen de grenzen van het eigen systeem. Regulatory Science wordt daarom wel gekenmerkt als discipline waar niet alleen de hypothese, maar ook de toepassing centraal staat. Inherent aan deze omschrijving is dat het regulatoire systeem zelf ook onderdeel is van het onderzoeksgebied: levert het systeem wel op wat de samenleving ervan verwacht? Regulatory science richt zich op

# Topics listed

- Small population guidance
- Biomarkers
- Real World Data and its use in regulatory decision making
- Advanced therapy medicinal products and regulatory challenges as regards their manufacturing
- Disease interception

# Regulatory Science at the broad regulatory level

rivm
Rijksinstituut voor Volksgezondheid en Milieu

Inspectie voor de Gezondheidszorg
Ministerie van Volksgezondheid, Welzijn en Sport

Lareb
Nederlands Bijwerkingen Centrum
Netherlands Pharmacovigilance Centre

ccmo


Zorginstituut Nederland
Inzicht in kwaliteit van zorg

# Topics listed

- Safe, new medicines, fast
- The use of observational data
- Alignment of knowledge needed and available in the health chain: CCMO-MEB-ZIN; towards efficient use of data.

# Summarized

- Biomarker identification at different levels
  - patients/gender, disease progression, interception
- Understanding real world data
- How to use data across the health chain?
  - from initiating trials to reimbursement
- Translational justification; 3R
- Optimized clinical trials
  - new methods
- Trustworthy medicines
  - generics + biosimilars
- ATMP manufacturing
- The broader perspective of global drug development
  - Fair access: rights, ethics and obligations

# Alignment with IMI projects

# Projects at the core of our network

## Regulators, HTAs and payers

### Interested in applying?

Find out about our latest funding opportunities and read our tips for applicants.

IMI projects cover the whole medical research and drug development process ranging from understanding the causes of diseases, through drug discovery, all the way to patient access to innovative medicines. Scientific knowledge derived from many of our projects is of direct relevance to regulatory authorities, health technology assessment (HTA) bodies and payers. For example:

- PROTECT focused on the assessment of the benefits and risks of medicines and has had an impact on regulatory practice in a number of ways.
- WEB-RADR has developed apps to aid in the collection of reports of adverse reactions.
- GetReal has generated tools and guidance on the integration of real world evidence in drug development.
- ADAPT-SMART is creating a multi-stakeholder platform for discussions on 'medicines adaptive pathways to patients' (MAPPs).

Many more projects are developing tools and methods with a potential regulatory impact, such as tests and measures to evaluate medicines safety or efficacy, or alternative clinical trial designs. The

# Concluding remarks

- Regulatory science is not recognized similarly by different stakeholders in the field
- Many data in the public domain can be used and 'flagged' as of regulatory impact
- To pave the way forward, all stakeholders should try to create Open Space for scientific crosstalk