



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

# Impact assessment of regulatory sciences – application to PROTECT outputs

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ENCePP Plenary meeting

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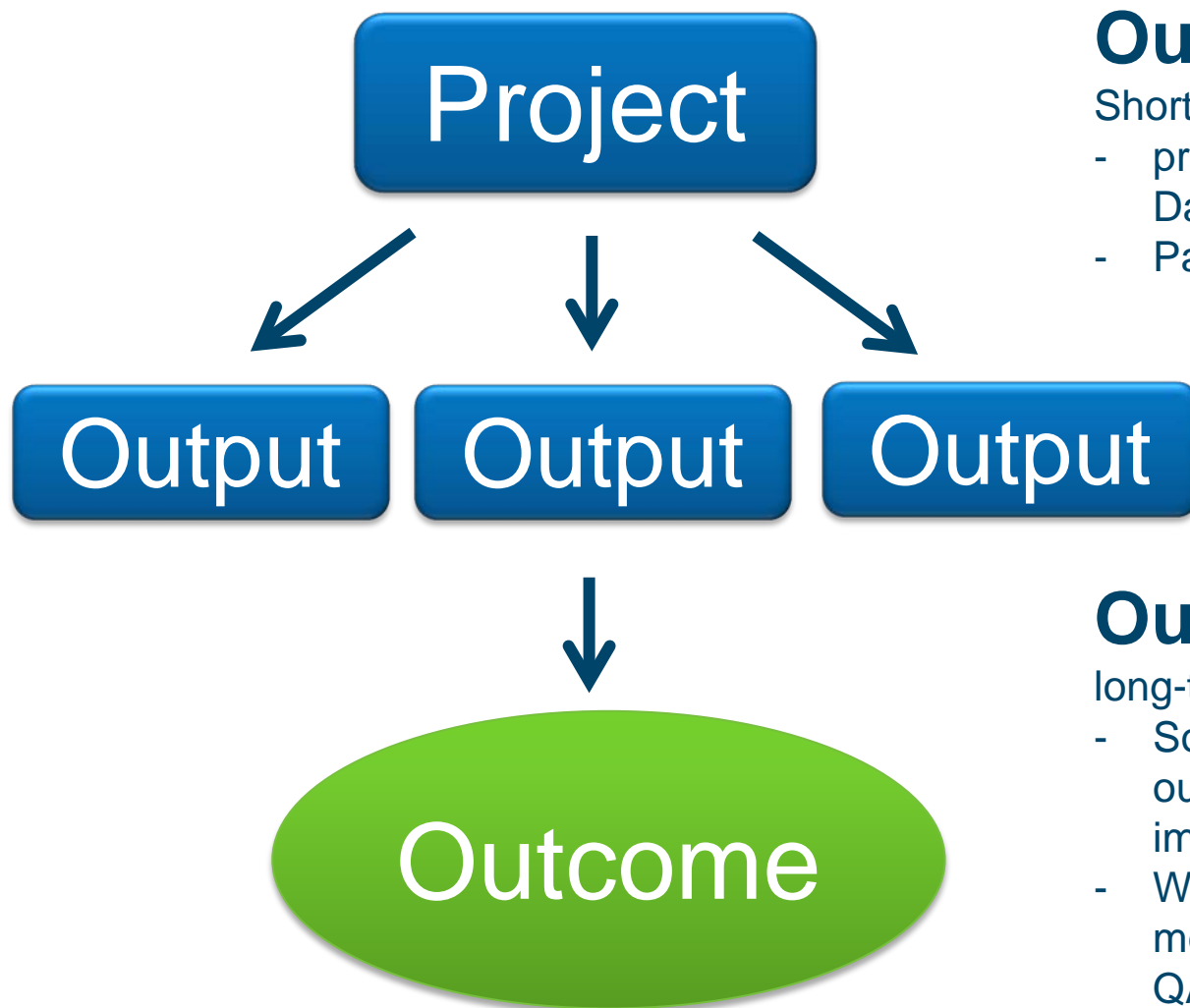
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# Background

- Regulatory agencies have primary responsibility to promote and protect public health through the evaluation and supervision of medicines
- To this end, many are engaged in research activities, notably regulatory sciences aiming to improve the evaluation of quality, efficacy and safety of medicinal products by:
  - Supporting research in areas of emerging and innovative sciences
  - Improving and evaluating the regulatory framework (methods and processes)
  - Developing and testing an infrastructure to build capacity for studies on drug safety and benefit-risk.



## Output =

Short-term result

- product, service, knowledge, e.g. Database, software, biomarker...)
- Paper, patent, ...

## Outcome =

long-term result/impact

- Social and economical impact of an output after (successful) implementation
- Where possible quantitative measurement (e.g. costs saved, QALYs gained, times shortened,...)



## Questions

- When are outputs matured enough to form a basis to implement changes in regulatory practice (OUTPUT → OUTCOME)?
- To what extent should outputs from regulatory science projects be validated, scrutinised and peer reviewed in the scientific community before their implementation?
- Should there be a trade-off between timing of implementation and scientific replication/validation?
- Which outputs should be prioritised for active implementation?
- What is the impact on resources?

**Can we define simple and standard criteria that would help prioritisation?**



## Proposed criteria (under discussion at EMA)

### 1. Domains

Intended target of research activity

Process: changes in process reflected in changes in guidelines, procedures, work instructions, training courses

Behaviour: behaviour of individuals or targeted entities affected by the deliverable

Outcome: actions implemented and final results

*Adapted from Coglianese C. Measuring Regulatory Performance-Evaluating the impact of regulation and regulatory policy, OECD, August 2012.*



# Proposed criteria (2)

## 2. Indicators

Impact of change: level of benefits brought by the change in case of implementation, considering affected stakeholders and estimate of public health impact

Maturity: stage of development in relation to intended application; eg.

- **inadequate**: output has not reached such a stage of development that it can be communicated to scientific community;
- **incomplete**: significant further development is still needed (e.g. independent confirmation, re-testing in another setting)
- **nearly complete**: need for peer review process or minor adjustments
- **complete**: no further development is needed



# Proposed criteria (3)

## 2. Indicators (2)

### Feasibility:

- impact of implementing the outcome in terms of resources (human, financial, infrastructure, IT or other resource needed)
- acceptability by concerned stakeholders
- alignment with applicable legislation.

### Timing of implementation

Delay within which the deliverable can be implemented, eg. <1 year, 1-2 years, >2 years.



# Proposed criteria (4)

## 3. Scoring

- Semi-qualitative, eg. +, ++, +++
- Weighting possible
- **Perspective may differ according to: academia, industry, regulators, patient, health care professionals,...**





## Example PROTECT Adverse Drug Reaction Database

- **Structured downloadable Excel database of all ADRs listed in section 4.8 of the SPC of Centrally-authorized products authorised in the EU, based exclusively on MedDRA.** Also includes information on gender, causality, frequency, class warning and source of information for ADRs for which additional information is provided in the SPC. (see <http://www.imi-protect.eu/adverseDrugReactions.shtml>)
- Created through a stepwise approach using automated mapping of ADR terms listed in section 4.8 of SPCs to MedDRA terminology, fuzzy text matching and expert review. Updated periodically.
- Intended result:
  - Improvement of the efficiency of signal detection by filtering or flagging electronic reaction monitoring reports (eRMRs) for signals related to unlisted reactions only (= OUTCOME)
  - Research purpose: evaluation of adjustment of statistical signals for known ADRs, and of the effect of background restriction on the performance of statistical signal detection (=PROCESS)



# Example

## PROTECT ADR database: Impact assessment

<b>Indicators</b>	
Intended target	
- Process	++
- Behaviour	-
- Outcome	+++
Impact of change	+++
Maturity	++
Feasibility	
- impact on resources	+
- acceptability	+++
- alignment with legislation	+++
Timing	++



# Summary

- Attempt to define criteria for prioritisation of regulatory science activities:
  - Identification of activities with highest impact
  - Efficient use of resources
- Work in progress
- Systematic analysis of PROTECT outputs is planned
  - Protocol being developed
- Application to other projects