



PROTECT



Pharmacoepidemiological Research on Outcomes of Therapeutics by a European Consortium

Contribution from PROTECT to regulatory practice: from science to process improvement

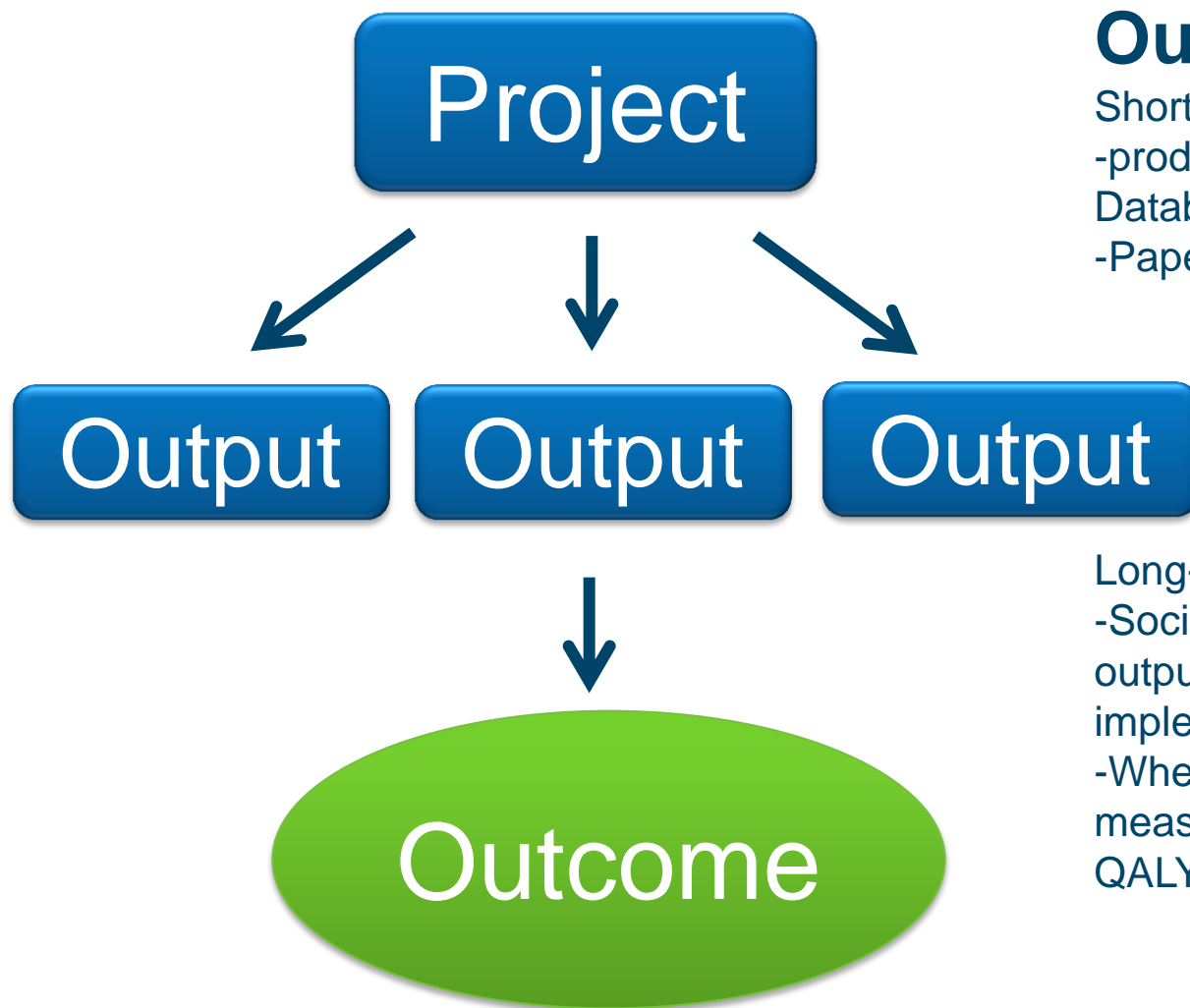
PROTECT SYMPOSIUM

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Xavier Kurz



Translation of outputs into outcome



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,...)

**GOOD JOB – WORKED
WELL!**



**ULTIMATE JUDGE OF SUCCESS IS WHETHER THE
EXCELLENT RESEARCH RESULTS (OUTPUTS) ARE
CONVERTED INTO OUTCOMES FOR INNOVATION
AND PUBLIC HEALTH**



PROTECT Impact assessment Objectives

1. To develop a conceptual framework for the review of the potential impact of outputs of regulatory science projects and the prioritisation of their implementation into regulatory practice

Using the PROTECT project as an example:

2. To test this conceptual framework to the outputs of PROTECT.
3. To make recommendations to EMA and its committees for an appropriate action on PROTECT results.

Scope: Regulatory science

EMA definition: Range of scientific disciplines that are applied to the quality, safety and efficacy assessment of medicinal products and that **inform regulatory decision-making throughout the lifecycle of a medicine**. It encompasses basic and applied medicinal science and social sciences, and contributes to the **development of regulatory standards and tools**.

European Medicines Agency process for engaging in external regulatory sciences and process improvement research activities for public and animal health EMA/14946/2013.

http://www.ema.europa.eu/docs/en_GB/document_library/Other/2013/03/WC500139888.pdf

FDA definition: Science of developing **new tools, standards, and approaches** to assess the safety, efficacy, quality, and performance of all FDA-regulated products.

Advancing Regulatory Science. -Moving Regulatory Science into the 21st Century.

http://www.fda.gov/ScienceResearch/SpecialTopics/RegulatoryScience/default.htm?utm_campaign=Goo

Questions to be addressed

- When are results matured enough to form a basis to implement changes in regulatory or clinical practice?
- To what extent should results/recommendations from regulatory science projects be systematically 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 implementation?

Proposed criteria

Domain	Indicator	Description
Description	Process	Changes in process to be reflected in guidelines or procedures
	Behaviour	Impact on behaviour of individuals or targeted entities
	Outcome	Positive or negative impact reflected in actions
Evaluation	Impact of change	Evaluation of the level of benefits brought by the change in each dimension
	Maturity	Need for further development or verification before use
	Feasibility	
	- Resources	Amount of resources needed for implementation
	- Acceptability	Acceptability by stakeholders
	- Compliance	Alignment with the legislation
	Timing of implementation	Timing with which the deliverable can be implemented

Scoring

- • Semi-quantitative: zero, low, medium, high
- Weighting possible according to stakeholders' perspective
- Criteria divided in two categories:

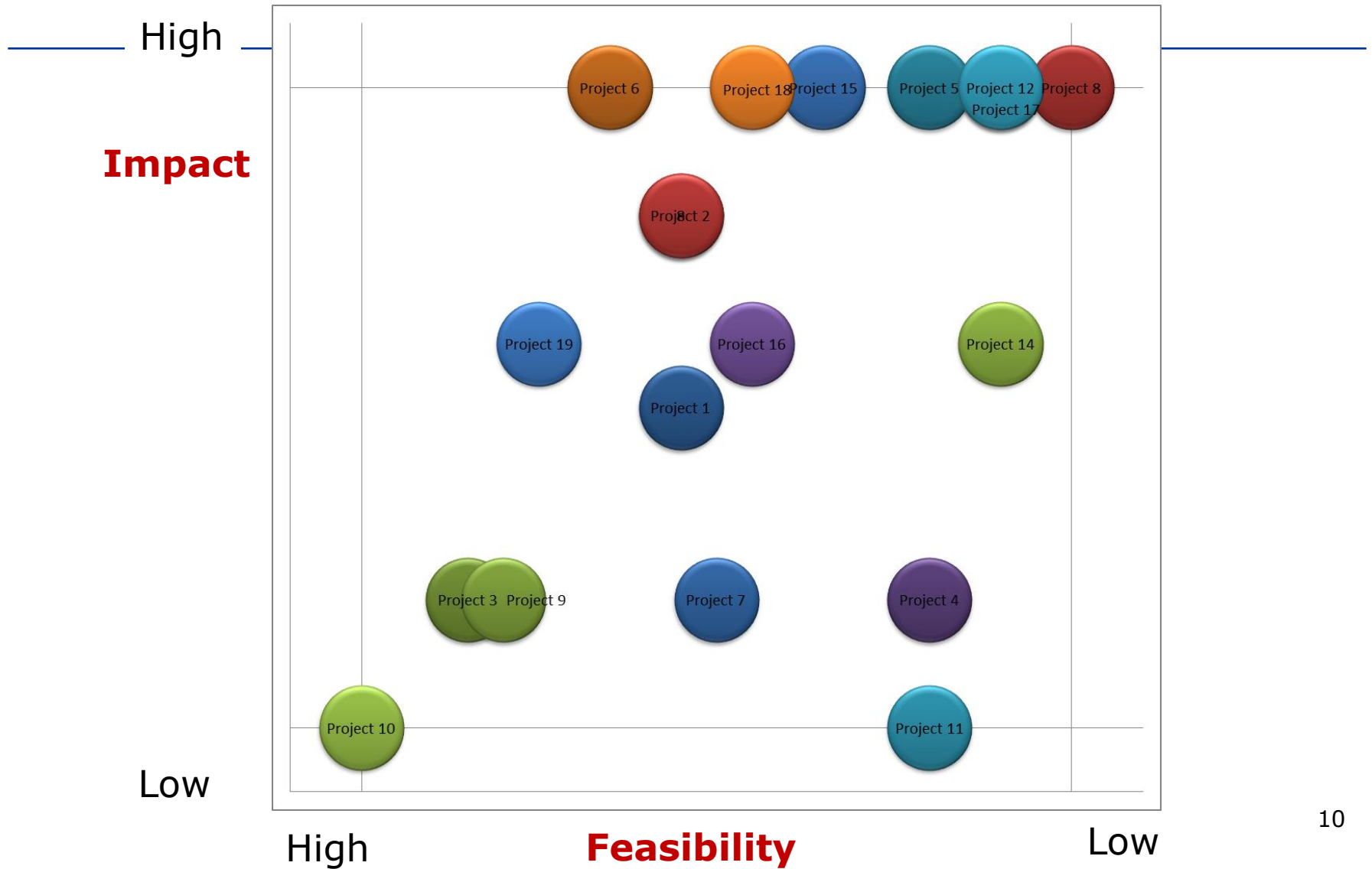
Feasibility category

- Impact of the implementation of the output in terms of resources (human, financial, infrastructure, IT or other resource needed)
- Acceptability by concerned stakeholders
- Compliance with the existing applicable legislation
- Evaluation of the timing for implementation (e.g. <6 m., 1 y., 2 y, >2 y.)

Impact category

- Evaluation of the level of benefit brought by the change in each indicator
- Deliverable maturity (inadequate, incomplete, nearly complete, complete)

Visual representation



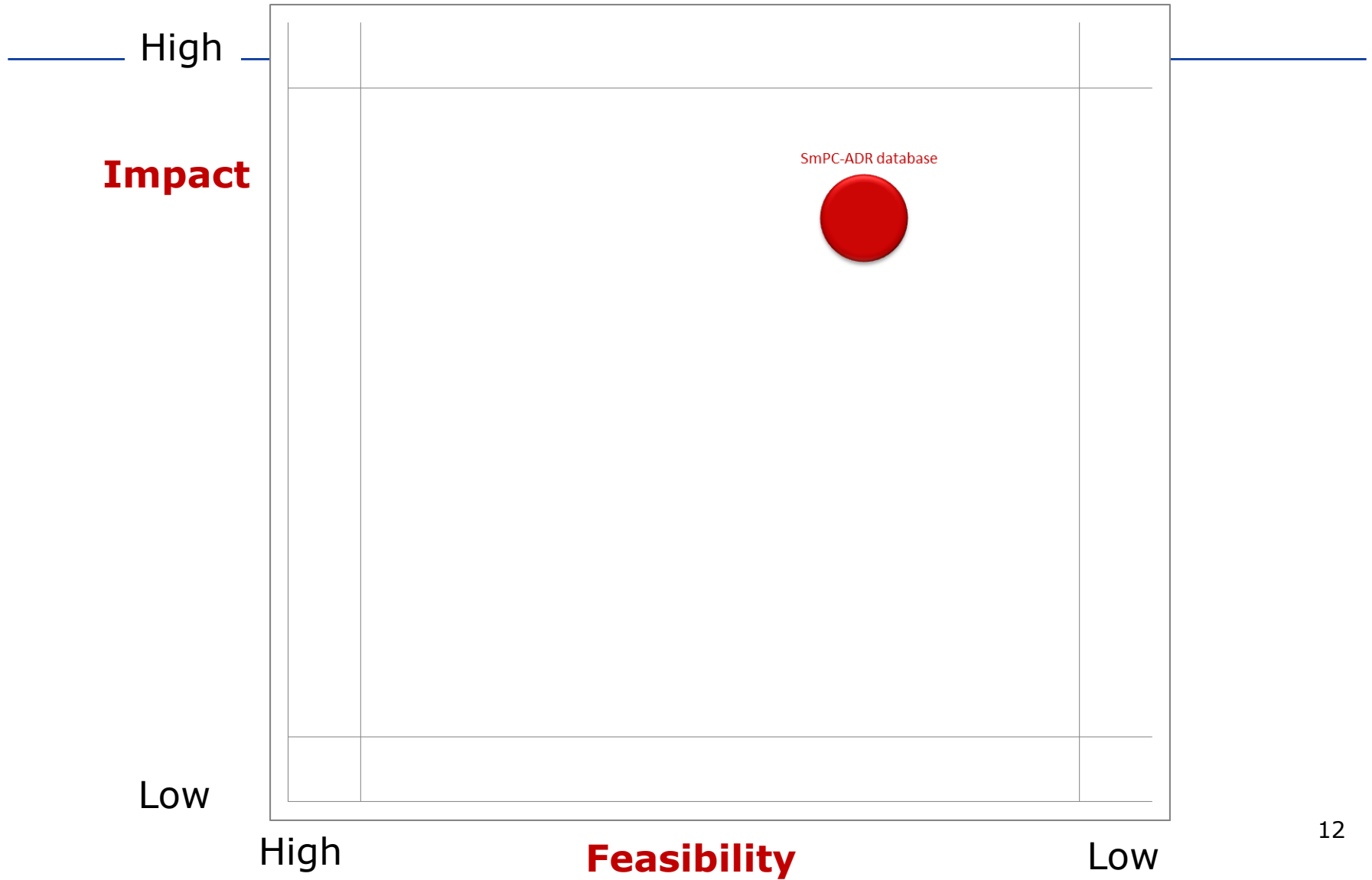
Example

PROTECT ADR database: Impact assessment

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

Last update: 30 June 2013

SmPC-ADR database



Planned PROTECT Deliverables

All planned deliverables:	101
“Final” deliverables:	42
WP2. Improving consistency between pharmacoepidemiological studies	7
WP3. Methods for signal detection	16
WP4. Direct-to-Patient Pharmacovigilance	7
WP5. Benefit-risk integration and representation	8
WP6. Replication studies	3
WP7. Training & Communication	1

Several outputs (reports, publications, databases, ...) for each deliverable

Next steps

1. Confirm evaluation criteria and relative weightings
2. Confirm scoring options
3. Identify which outputs are to be assessed as part of the prioritisation exercise.
4. Select documentation for each output (e.g. published article, executive summary)
5. Evaluate outputs against Scoring matrix
6. Prioritise implementation of outputs

Next steps

WE NEED YOU



**Thank
You**

Mahalo

Kiitos

Tack

Grazie

Toda

Obrigado

Thanks

Takk

Gracias

Merci

The deputy Co-ordinator, Liz Swain

WP co-leaders and deputy co-leaders project managers,
members of the External Advisory Board, all members of
the PROTECT Consortium

At IMI: Fatiha Sadallah, Antoine Juliens, Michel Goldman

At EMA:

Peter Arlett

Lucia Caporuscio

Henry Fitt

Thomas Lönngren

Urszula Piotrowska

Guido Rasi

Fergus Sweeney

Dagmar Vogl

Stella Blackburn

Hans-Georg Eichler

Katarina Lenova

Leszczynska Malgorzata

Stephanie Prilla

Judith Routledge

Panos Tsintis

Noël Wathion

Chairs of EMA Committees: June Raine, Tomas Salmonson,
Peter Bachman, and others...

Staff of the Monitoring and Incident Management Studies

The deputy Co-ordinator, Liz Swain

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