

Innovation, regulation and increased payer demands

Ansgar Hebborn

Global Payer & HTA Program Policy, F. Hoffmann-La Roche AG, Basel, Switzerland

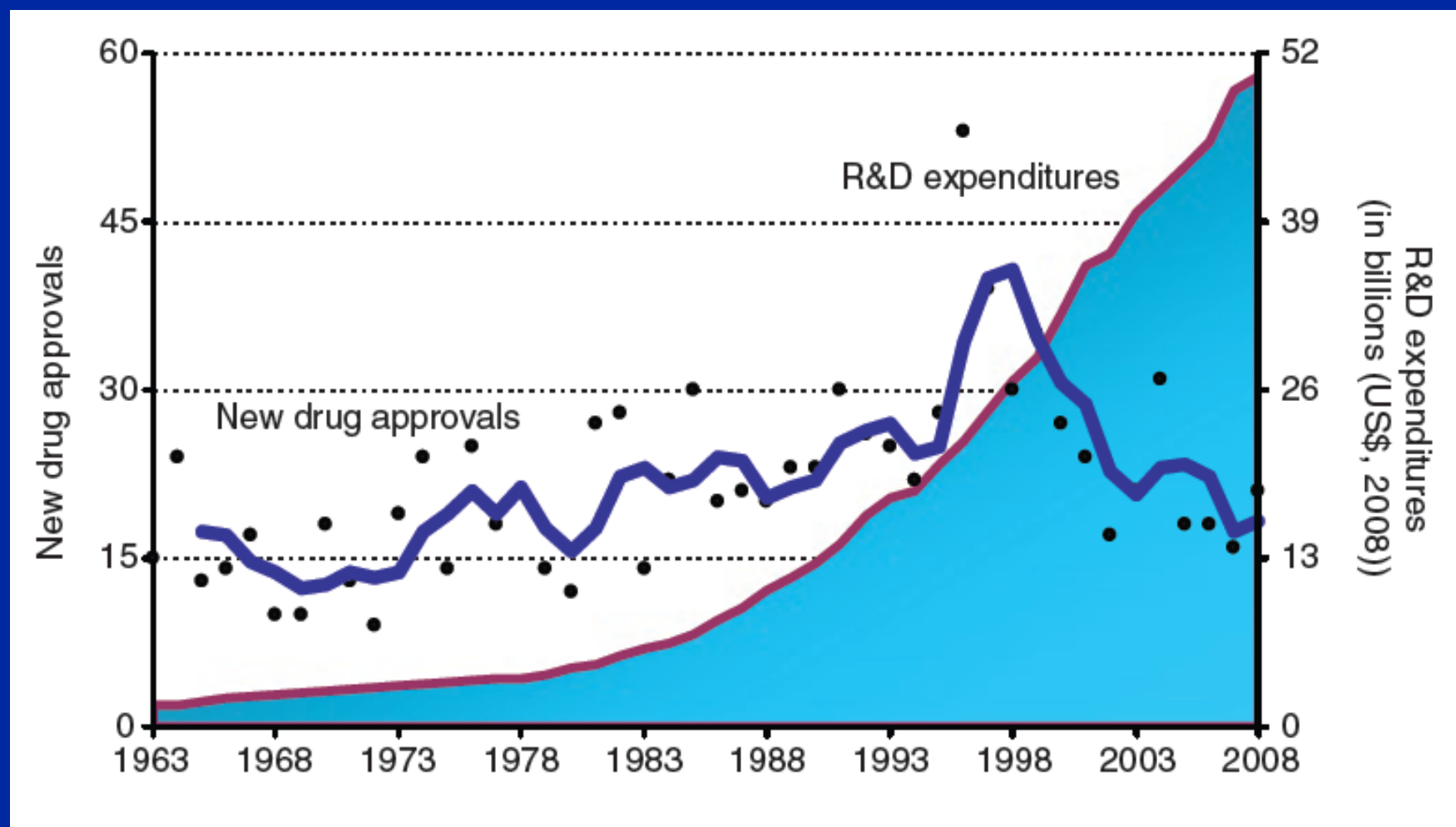
London, 18 February 2011



What is the issue?

Continuously declining R&D efficiency

R&D investments increasingly challenged



Improving R&D efficiency

Strong voices in support of public/private partnerships

PERSPECTIVES

nature
REVIEWS DRUG
DISCOVERY

OPINION

Cutting the cost of drug development?

Michael D. Rawlins

The cost of drug development has risen markedly in the past 30 years, with studies now reporting values exceeding US \$800 million. As these spiralling costs threaten to make the development of new drugs increasingly unaffordable for both developing companies and consumers, it is clear that efforts should be made to address this problem. All aspects of the drug discovery and development process should be examined for potential cost savings, but I focus here in particular on the current regulatory requirements.

“The time is now right for an exhaustive examination of the requirements of drug regulatory authorities. There needs to be a rigorous examination of the ‘rituals’ associated with drug development. Every step in the drug development pathway should be tested against two separate criteria: is there a clear evidence-base to support the continuing inclusion of the measure in the requirements of regulatory authorities?; and does each regulatory requirement offer value for money?”

Rawlins, Drug Disc 2004

Initiatives, new tools and technologies

But how fast can they deliver?

The Innovative Medicines Initiative

IMI Public Web

IMI JU Official Website

For the official IMI JU website click [here](#)

Innovative Medicines Initiative Joint Undertaking

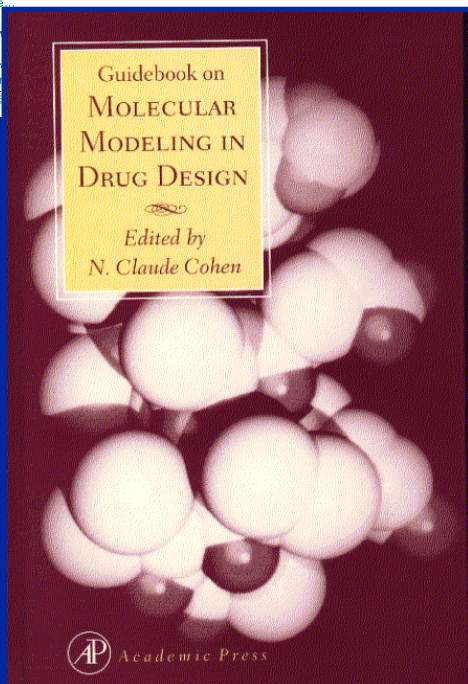
The IMI is a unique partnership between the European Community and the European Federation of Pharmaceutical Industries and Associations (EFPIA).

The aim of IMI is to support the faster discovery and development of better medicines for patients and to enhance Europe's competitiveness by ensuring that its biopharmaceutical sector remains a dynamic high-technology sector.

IMI Objectives

The vision of IMI is to create Biomedical Research & Development leadership for Europe to benefit patients and society.

IMI IN 3 MINUTES
(YouTube video)



CRITICAL PATH INSTITUTE
collaborate · innovate · accelerate

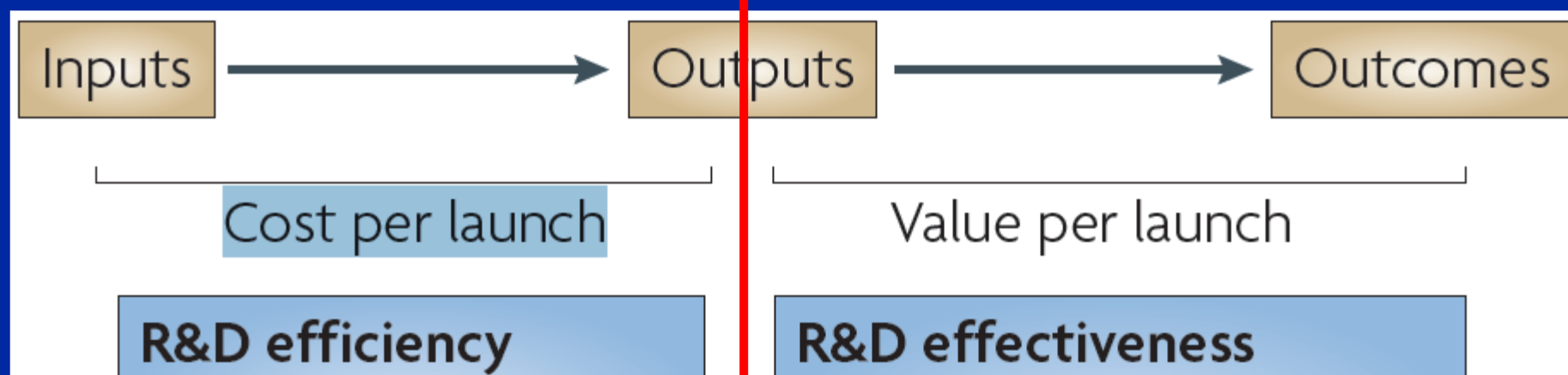
"C-Path has created neutral ground that allows FDA scientists to work collaboratively with industry and academic partners on improving the process."
- Janet Woodcock, MD

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R&D efficiency and effectiveness

The two dimensions of R&D success



Adopted from Paul et al., Drug Disc 2010

requires effective payer support
(successful pricing,
reimbursement, and funding
negotiations)

Regulators have started to position themselves *EMA and the “dynamic” aspect of its role*



The drug regulator's role...

..to protect public health
against unsafe or ineffective drugs
against the consequences of untreated
disease

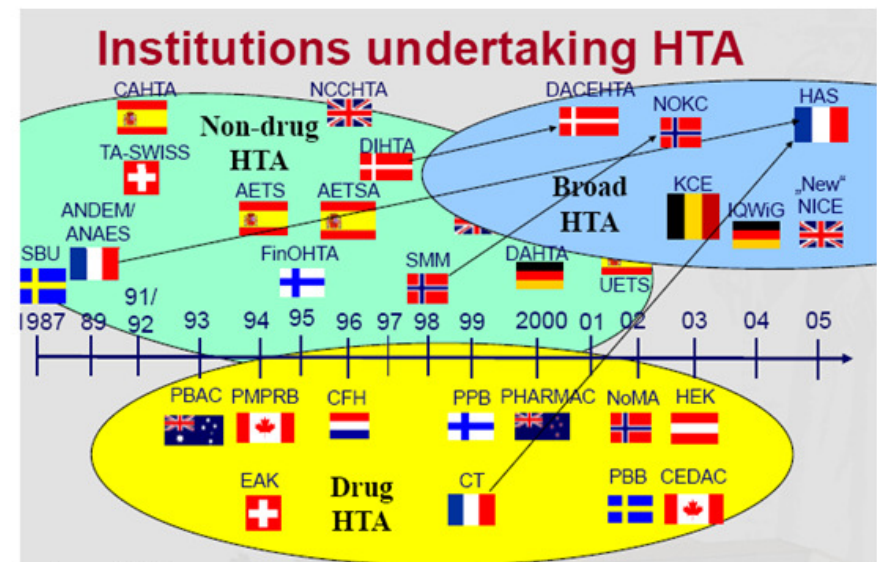
This role translates into a mandate to
support the development of beneficial
drugs

HTA and the diffusion of its gatekeeping role

Not without impact on R&D effectiveness

EUnetHTA Partners

- **62* partners (59 original partners)**
 - **34 Associated Partners** (national/regional HTA agencies, MoH, research institutions, international organisations (Cochrane Collaboration))
 - **29* Collaborating Partners** (e.g. WHO, OECD, CoE)
- **25 EU countries** (÷ Slovakia)
- **2 EEA** (Norway, Iceland)
- **Switzerland**
- **4 institutions in countries outside EU** (Israel, Australia, Canada, U.S.)
- *as of January 2008 (4 organisations became Collaborating Partners in 2007, www.eunetha.eu)*



Busse 2009

Regulatory authorities and HTA agencies

IQWiG and EMA - a “very different” remit?

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

An Agency of the European Union

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The Agency provides the Member States and the institutions of the EU the best-possible scientific advice on any question relating to the evaluation of the quality, safety and efficacy of medicinal products for human or veterinary use referred to it in accordance with the provisions of EU legislation relating to medicinal products.

IQWiG Institute for Quality and Efficiency in Health Care

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IQWiG is an independent scientific institute that investigates the benefits and harms of medical interventions for patients. We regularly provide information about the potential advantages and disadvantages of different diagnostic and therapeutic interventions.



IQWiG's understanding of its responsibility

Peter Sawicki, Brussels, 17 April 2007

Evidence in the pharmaceutical policy

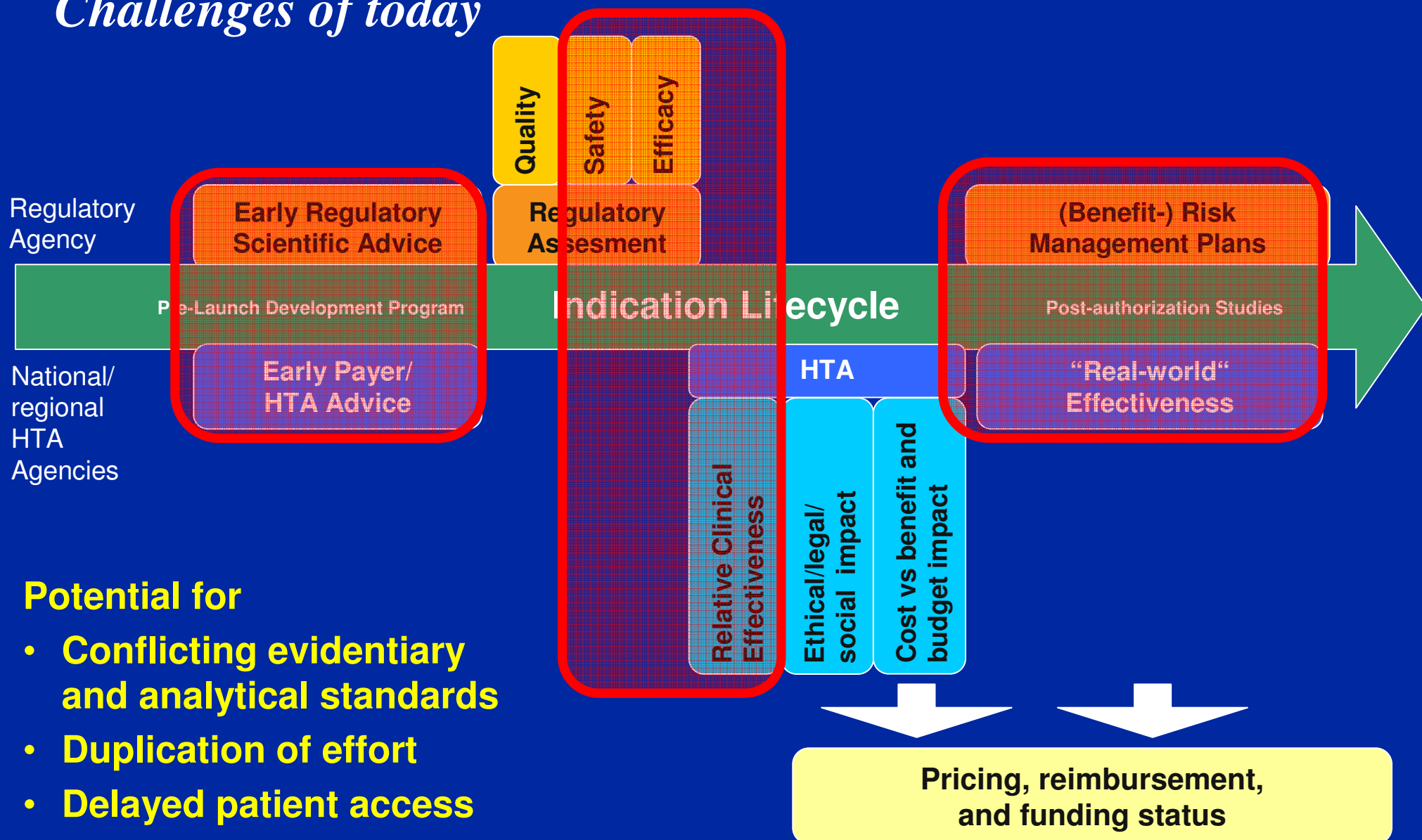
IQWiG resume after two years of work:

It is very difficult to protect the public and the individual patient from the threats to health and to the health-budget by the wide use of drugs with an unknown benefit - harm relation.

Regulatory-payer interface along the product lifecycle

Roche

Challenges of today



What can be done?

What are we aiming for?

“Reasonable” clinical evidentiary and analytical standards

- Validity
- Relevance
- Transparency
- Predictability
- Feasibility

...but that's easier said than done

Regulators and payers have started to collaborate

Isolated pilots or phase I of constructive engagement?

NHS
National Institute for Health and Clinical Excellence

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Who we are

What we do

Scientific advice consultancy service



16 February 2010
EMA/98431/2010
j.no.7-204-05-4/1
Press office

Press release



Voluntary parallel scientific advice with NICE and the MHRA

European Medicines Agency and EUnetHTA Joint Action start collaboration on European Public Assessment Report (EPAR) contribution to relative effectiveness assessments

Federal Register/Vol. 75, No. 180/Friday, September 17, 2010/Notices

57045

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Centers for Medicare and Medicaid Services

Food and Drug Administration

(Docket No. FDA-2010-N-0308)

Parallel Review of Medical Products

AGENCIES: Centers for Medicare and Medicaid Services; Food and Drug Administration, HHS.

ACTION: Notice; request for comments.

SUMMARY: The Food and Drug Administration (FDA) and the Centers for Medicare and Medicaid Services (CMS) are conducting a pilot project for a parallel review of medical products. The pilot project will be conducted for a period of 18 months, from September 1, 2010, to August 31, 2012. The pilot project will be conducted for a period of 18 months, from September 1, 2010, to August 31, 2012. The pilot project will be conducted for a period of 18 months, from September 1, 2010, to August 31, 2012.

peter.beckerman@fda.hhs.gov, or Tamara Syrek Jensen, Centers for Medicare and Medicaid Services, 7500 Security Blvd., Baltimore, MD 21244, e-mail: Tamara.Syrekjensen@cms.hhs.gov.

For device sponsors interested in requesting voluntary parallel review: Markham C. Luke, Center for Devices and Radiological Health, Food and Drug Administration, 10903 New Hampshire Ave., Silver Spring, MD 20993-0002, 301-796-5550, e-mail: markham.luke@fda.hhs.gov.

SUPPLEMENTARY INFORMATION:

I. Background

Pilot project of joint scientific advice meetings arranged by the TLV and the MPA

Collaboration between the Dental and Pharmaceutical Benefits Agency (TLV) and the Medical Products Agency (MPA) offers new opportunity for joint scientific advice meetings.

Background

The TLV and the MPA have received inquiries from the pharmaceutical industry regarding the possibility of arranging joint scientific advice meetings. In the light of these inquiries, a pilot project of joint meetings attended by representatives from both authorities will be conducted during the period September 1st 2009 to June 30th 2010.

The aim of providing joint scientific advice is to fulfil the Government's instructions to the agencies to contribute to a rational and cost-effective use of pharmaceutical products as well as meeting inquiries from the pharmaceutical industry.

Procedure

The application procedure is in principle the same as for regular scientific advice meetings at the MPA. The application form at the MPA website for scientific advice meetings has been updated with a section to request the participation of the TLV in the meeting.

Specific questions directed to the MPA and the TLV respectively should be included in the application and it is of importance that it is clearly stated which questions are directed to which authority.

The request for a scientific advice meeting should be submitted via e-mail to the MPA registrar.

The relevant background documentation should be submitted to the MPA no later than three weeks ahead of the planned meeting.

The meetings will be held at the premises of the MPA and will usually be 1 hour and 30 minutes long. Representatives from both the MPA and the TLV will be present.

Comprehensive stakeholder involvement

The European pilots

The screenshot shows the EMA website with the following elements:

- Header:** EMA logo, "EUROPEAN MEDICINES AGENCY", "SCIENCE MEDICINES HEALTH", "An Agency of the European Union", and the European Union flag.
- Navigation:** Home, Find medicine, Regulatory, Special topics, Document library, **News & events**, Partners & networks, About us, Quick links.
- Left Sidebar:** News and press release archive, Committee meeting reports, Calendar, Statistics, What's new, Media centre, RSS feeds, Newsletters, FAQ on the new website.
- Breadcrumbs:** Home > News and Events > News and press release archive
- Main Article:**
 - Title:** Pilots of multi-stakeholder consultations in early-stage drug development
 - Actions:** Email a friend, Print page, Help
 - News Section:**
 - Headline:** Pilots of multi-stakeholder consultations in early-stage drug development
 - Date:** 25/10/2010
 - Text:** Healthcare institutions from Europe have launched a new pilot process testing multi-stakeholder consultations in early-stage drug development. The purpose of the stakeholders required to demonstrate the pilot initiative representatives, Italy, the Netherlands, the Agency.
- Related information:**
 - Working with health technology assessment bodies at the Agency
 - European Healthcare Innovation Leadership Network

“...committed to addressing the complementary goals of improving patient health outcomes and enhancing the climate for innovation while acknowledging pressures to control healthcare costs”

Comprehensive stakeholder involvement

“Balancing validity, relevance, and feasibility”



Designing More Informative Clinical Trials
for New Indications of Oncology Drugs

Meeting Summary

November 12, 2009

8:00 a.m.–4:00 p.m. EST

World Trade Center Baltimore
Maryland Room, 21st Floor

www.cmtpNet.org

Representation of all relevant stakeholders

- Academia
- Patient Associations
- National Research Programs
- Payers (US CMS and private, NICE, PBAC, CADTH)
- Regulators
- Manufacturers

“Reasonable” evidentiary and analytical standards

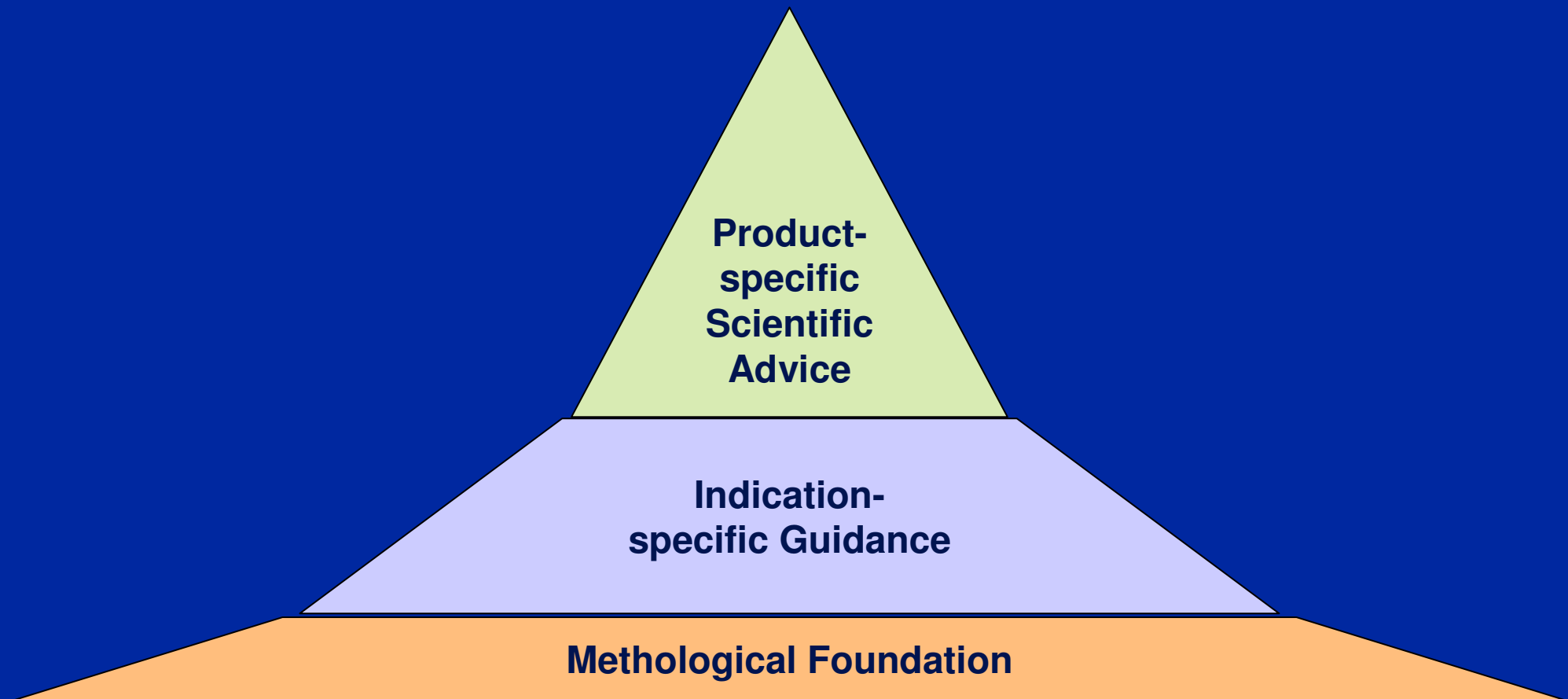
What does it need?

1. Sustainable platform(s) to promote inclusive involvement of stakeholders (manufacturers, patients/providers/regulators and payers) and (other) experts
2. Shared interest of all stakeholders to establish “reasonable” standards i.e. to balance relevance, validity, and feasibility
3. Transparency about the value of demanding additional evidence, the associated burden in terms of patient access delays as well as the longer term “dynamic” implications for the innovation process
4. Recognition of the global scope of technology development

...plus a much stronger sense of urgency

Good intentions exist - but action is needed

Subject layers of interest





We Innovate Healthcare