

Methodological Guidance for the Life Sciences Industry.

An Industry Perspective

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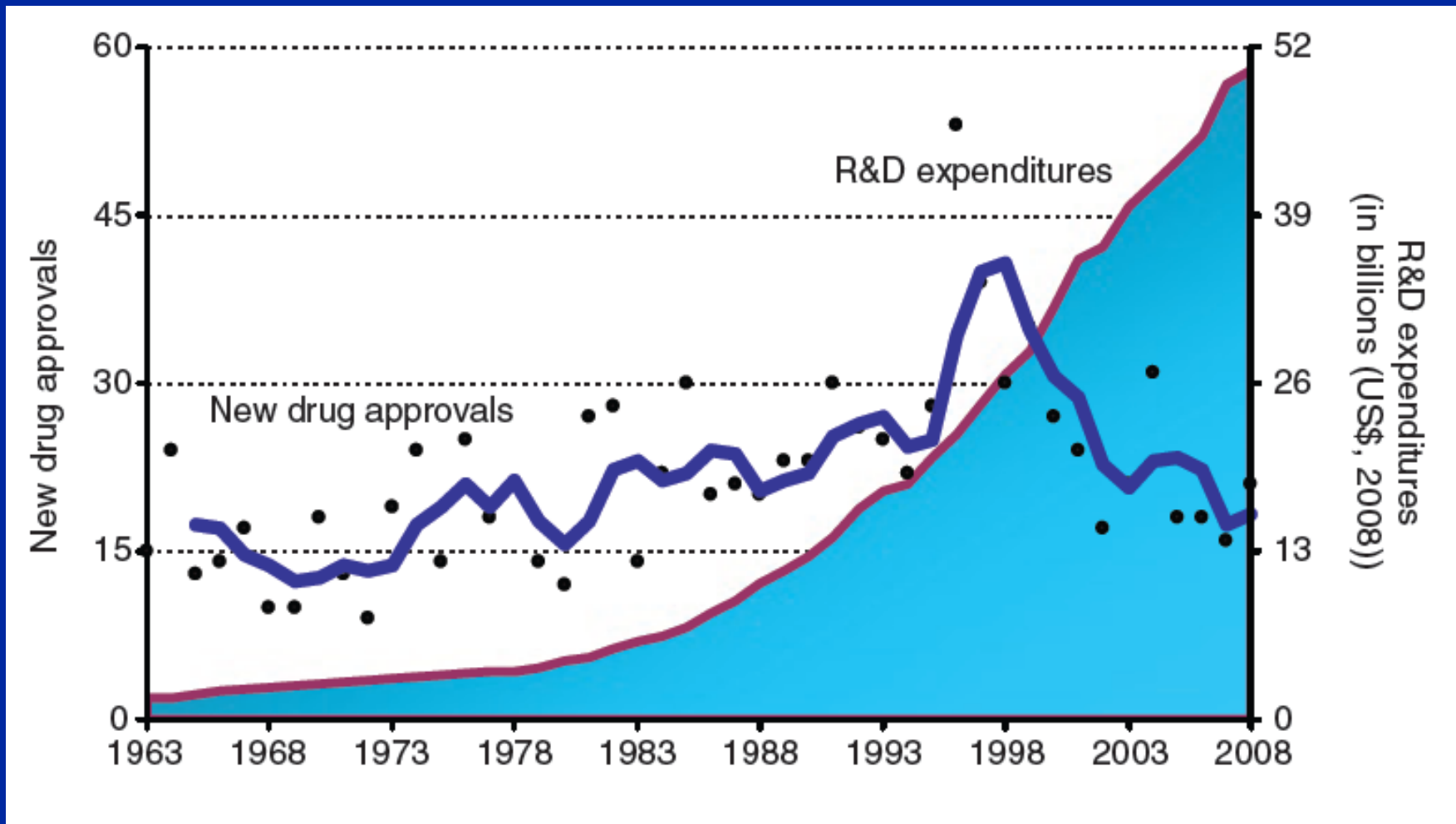
Rio, 27 June 2011



What is the issue?

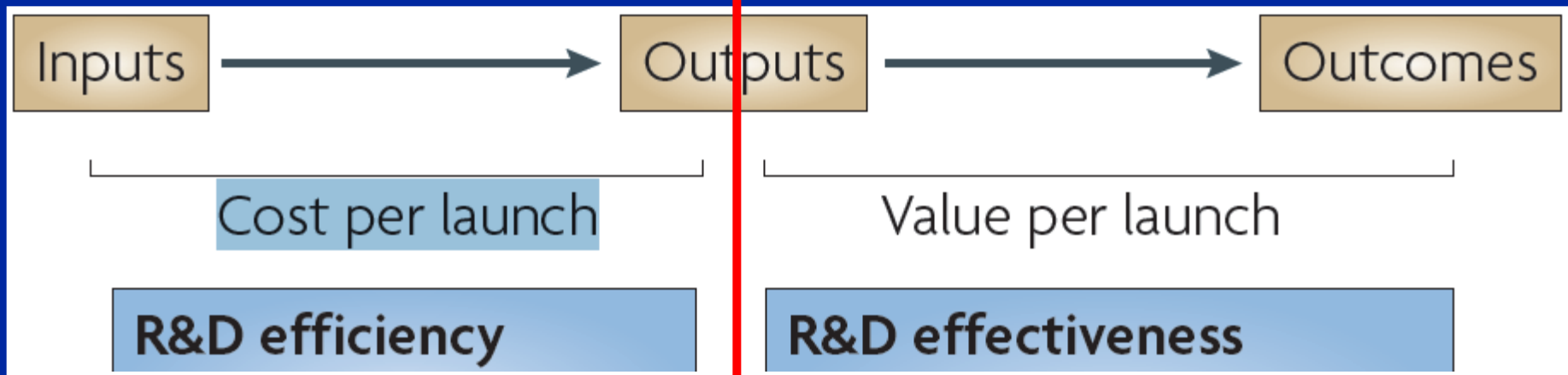
Technology development decisions

R&D investments increasingly challenged



R&D efficiency and effectiveness

The two dimensions of R&D success



Adopted from Paul et al., Drug Disc 2010

requires constructive and efficient interaction with regulatory authorities throughout development process

requires that patients and clinicians understand the value of a therapy as well as successful pricing, reimbursement, and funding negotiations

What can be done?

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 agencies, payers and early advice

Isolated pilots or phase I of constructive engagement?

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Scientific advice consultancy service

NICE offers product-specific scientific advice to pharmaceutical companies and device manufacturers about products they have in development that may be referred for a technology appraisal.

Based on a [briefing book](#), written questions submitted by the company and a face-to-face meeting, NICE will provide an advice report. There is a fee for this service.

Advice given by NICE will be in response to the written questions asked and documentation submitted and cannot account for future changes and developments in scientific knowledge or appraisal requirements.

Why seek advice?

NICE's technology appraisal processes require certain types of evidence about the product being appraised. A company may wish to seek advice from NICE to ensure that their clinical- and cost-effectiveness studies can produce evidence that is relevant for a NICE technology appraisal.

More information about [NICE's technology appraisal methods](#).

When can advice be sought?

A useful time for requesting scientific advice could be during phase II studies before the planning of phase III studies. The earlier in the development process the less specific the advice can be, and the later in the process the less likely it is that companies will be able to make changes to the design of clinical trials.

What advice can be requested?

Scientific advice can be given on many issues connected with the development of evidence for post-regulatory

Multi-stakeholder consultations

Isolated pilots or phase I of constructive engagement?



Voluntary parallel scientific advice with NICE and the MHF

Federal Register / Vol. 75, No. 180 / Friday, September 17, 2010 / Notices 57045

<http://www.cdc.gov/niosh/docs/2010-167/>.

Background: The NIOSH Alert: Preventing Occupational Exposures to Antineoplastic and Other Hazardous Drugs in Health Care Settings was published in September 2004 (<http://www.cdc.gov/niosh/docs/2004-165/>). From that time until June 2007, approximately 60 new drugs have received FDA approval and approximately 60 drugs have received special warnings (usually black box warnings) based on reported adverse effects.

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Centers for Medicare and Medicaid Services

Food and Drug Administration

(Docket No. FDA-2010-N-0306)

Parallel Review of Medical Products

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

ACTION: Notice; request for comments.

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.



eunethhta

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

Press release



European Medicines Agency and EUnethHTA Joint Action Plan to start collaboration on European Public Assessment Reports (EPAR) contribution to relative effectiveness assessments

CMTMP
Center for Medical Technology Policy

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.cmtmpnet.org

MPA and the Centers for Medicare and Medicaid Services (CMS) are establishing a pilot project of joint meetings for evaluating medical products of interest to such parallel review. The pilot project will serve the interests of patients, the public, and the industry.

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

The purpose of the joint scientific advice is to fulfil the Government's commitment to agencies to contribute to a rational and cost-effective evaluation of medical products as well as meeting inquiries from the industry.

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

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

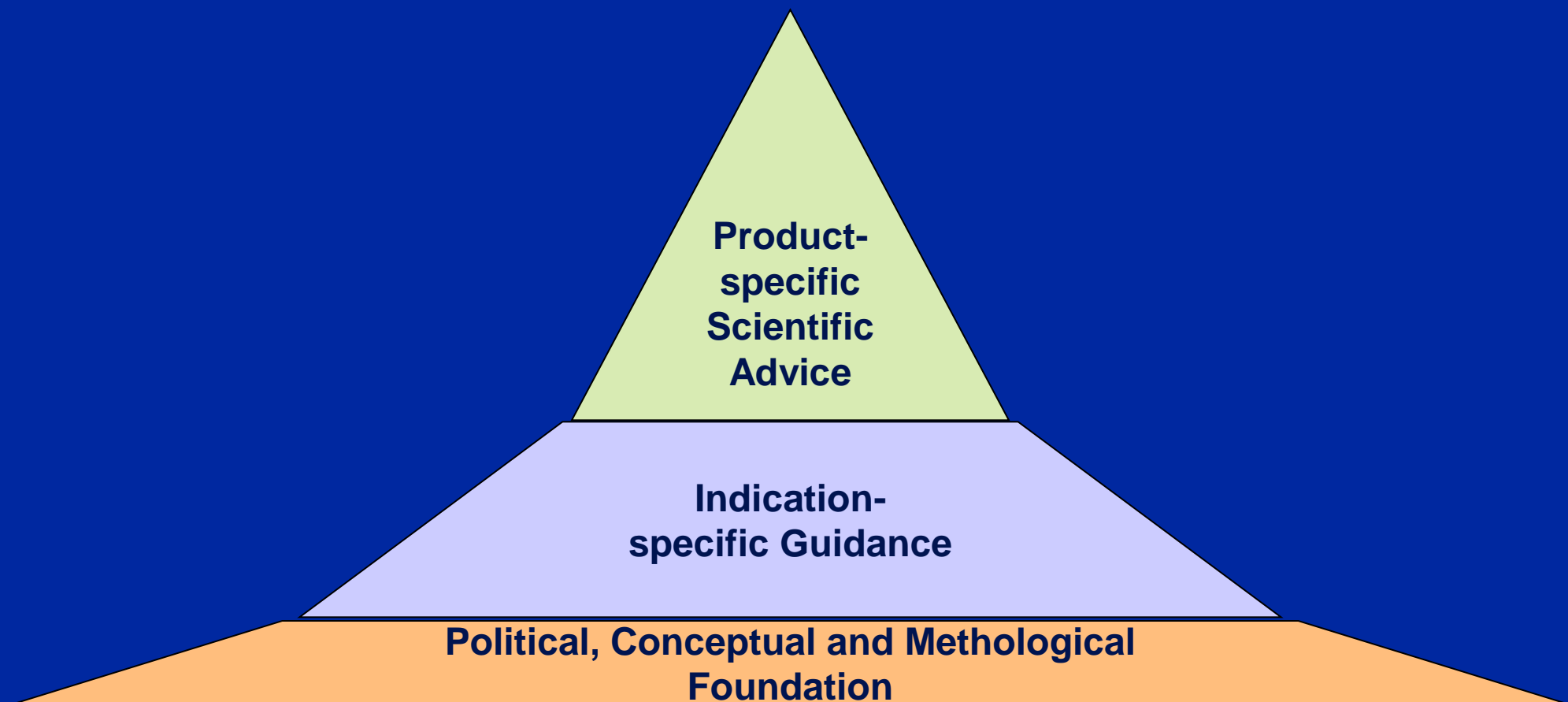
For a scientific advice meeting should be submitted via e-mail to the MPA and the TLV registrar.

The background documentation should be submitted to the MPA and the TLV **three weeks** ahead of the planned meeting.

The meeting will be held at the premises of the MPA and will usually be held for a maximum of 90 minutes long. Representatives from both the MPA and the TLV will be present.

Good intentions exist - but action is needed

Subject layers of interest



“Reasonable” evidentiary and analytical standards

What does it need?

1. A shared interest of all stakeholders to establish “reasonable” standards i.e. to balance relevance, validity, feasibility, and timeliness
2. Sustainable platform(s) and resources to promote inclusive involvement of stakeholders (manufacturers, patients/providers/regulators and payers) and (other) experts
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

But that's easier said than done...

Participation, governance, processes and funding

Some first thoughts

- 1. Broadest inclusion of decision makers (technology developer, patients, provider, regulators, HTA agency, and payers)***
- 2. Build trust and understanding***
- 3. Recognize self-interest as the primary motive for constructive participation***
- 4. Recognize competition as the major source of better ideas***
- 5. Recognize opportunities to collaborate whenever these emerge***



We Innovate Healthcare

Participation, governance, processes and funding

Some first thoughts

- 1. *Broadest of decision makers (technology developer, patients, provider, regulators, HTA agency, and payers)***
- 2. *Build trust and understanding***
 - Participants need a clear understanding of each others roles and responsibilities, how they share interests and how they are different.
- 3. *Recognize self-interest as the primary motive for constructive participation***
 - All participants will be inevitably „self-interested“, often labeled as „biased“. But there are more efficient ways to handle this than discrimination or exclusion.
- 4. *Recognize competition as a major source of better ideas***
 - This endeavour does not need a monopolistic position that cannot be contested by those with even smarter ideas.