NICE National Institute for Health and Care Excellence

NICE, societal values and a systems approach

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Welcome to the new NICE

Our name has changed to the National Institute for Health and Care Excellence, to reflect our new role and responsibilities.

Read more about who we are and what we do

Find out about our new role in social care

NICE National Institute for Health and Care Excellence

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Patients and the public

Putting patients and the public at the centre of NICE's work

Patients, carers, service users and members of the public are crucial to NICE, playing a key role in shaping our recommendations. Join NICE's meetings held in public.

Medicines and prescribing

Safety, efficiency and effectiveness in the use of medicines

We now offer a comprehensive suite of advice and support for delivering quality, safety, and efficiency in the use of medicines.

Medicines and prescribing from NICE

Consultations

Guidance in development

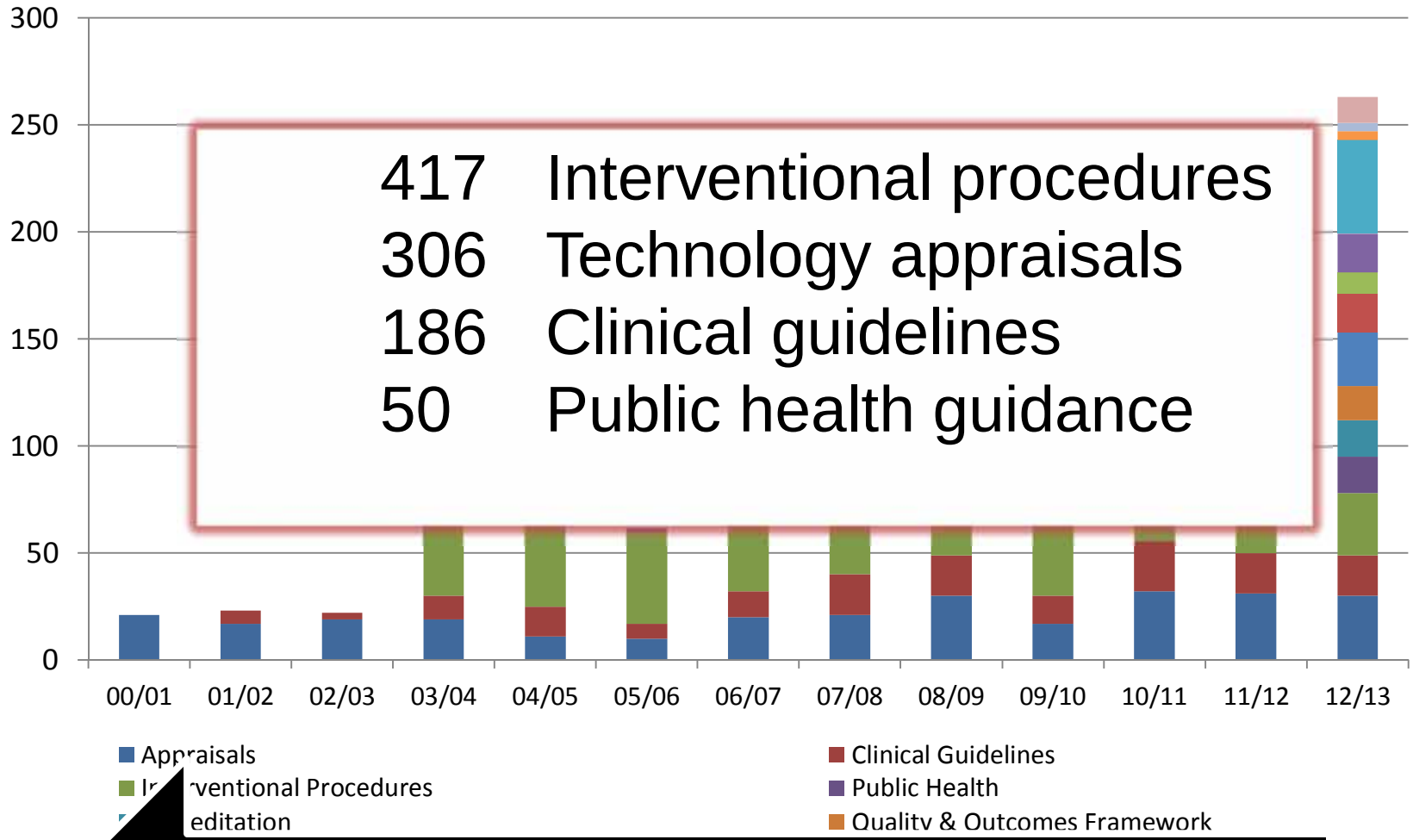
There are consultation stages during the guidance development process when stakeholders and interested members of the public can comment on draft guidance.

NICE

NICE

- Independent : non departmental public body
- ~600 staff
- 2014/15 budget: ~£65 million
- Provides national guidance and advice to improve health and social care.
- No price negotiation
- Not reimbursement
- Operates as network

A brief history of NICE



2014 Social Care. Highly Specialised Technologies. Briefings. VBA

NICE

Fund within 3 months

guidance and information programmes

Technology Appraisals

Are recommendations on the use of new and existing medicines and treatments within the NHS in England and Wales. Potential topics are identified by the National Institute for Health Research Horizon Scanning Centre.

Find out more

Clinical Guidelines

Recommendations based on the appropriate treatment and care of people with specific diseases and conditions within the NHS in England and Wales. Clinical guidelines are based on the best available evidence.

Find out more

Public Health

Guidance makes recommendations for populations and individuals on activities, policies and strategies that can help prevent disease or improve health.

Find out more

Quality Standards

Are a concise set of statements designed to drive and measure priority quality improvements within a particular area of care. Topics will be referred to NICE by the NHS Commissioning Board for health-related areas, and by the Department of Health and Department for Education for non-health areas such as social care.

Find out more

Quality Outcomes Framework (QOF)

Rewards practices for the provision of 'quality care' and helps to standardise improvements in the delivery of clinical care. NICE is responsible for managing an independent and transparent approach to developing the QOF clinical and health improvement indicators.

Find out more

Clinical Commissioning Group Outcomes Indicator Set

CCG OIS is part of the NHS Commissioning Board's systematic approach to promoting quality improvement. NICE is responsible for developing indicators for the CCG OIS from quality standards

Find out more

Medical Technologies Evaluation Programme

Selects and evaluates new or innovative medical technologies (including devices and diagnostics). Sponsors can notify medical devices and diagnostics that meet the eligibility criteria directly to the programme for topic selection consideration.

Find out more

Diagnostic Assessment Programme

Focuses on the evaluation of innovative medical diagnostic technologies in order to ensure that the NHS is able to adopt clinically and cost effective technologies rapidly and consistently.

Find out more

Interventional Procedures

IP assesses the safety and efficacy of (mainly) new procedures that are used for diagnosis or treatment that involve incision, puncture, entry into a body cavity or the use of ionising, electromagnetic or acoustic energy.

Find out more

Evidence summaries: New medicines

Summaries of the best available evidence for selected new medicines, or for existing medicines with new indications, to inform local NHS planning and decision-making.

Find out more

Evidence summaries: Unlicensed/off-label meds

Providing summaries of the best available evidence on selected unlicensed and off-label medicines, designed to meet demand for information to inform local NHS planning and decision-making.

Find out more

Highly Specialised Technologies

NICE liaises closely with AGNSS on highly specialised technologies. The work of the Topic Selection team will continue to feed into the NICE Highly Specialised Technologies programme when it launches in April 2013.

Routing

Clinical Guidelines

- A number of **equivalent technologies** available
- The equivalents have been **available in clinical practice** for some time
- Benefits best evaluated in the **context of a care pathway**.

Technology Appraisals Guidance

- New treatments with **significant impact** on NHS, or **policy priorities**
- Clinical and cost-effectiveness
- **Companion diagnostics** suitable if an appraisal of the pharmaceutical that they are intended to enhance is appropriate
- 3-month **funding direction**.

Interventional Procedures Guidance

- **Safety and efficacy** of novel procedures
- **New device** in a novel procedure where safety and efficacy are still unknown
- Comparative effectiveness and health economic considerations are **not relevant** at this point.

Diagnostics Guidance

- **More cost/more benefit**
- Complex care pathways
- Recommendations on the basis of **clinical utility and cost- utility** analysis
- Gold standard or **established comparator** to enable an assessment of potential benefit
- **Multiple or single** products.

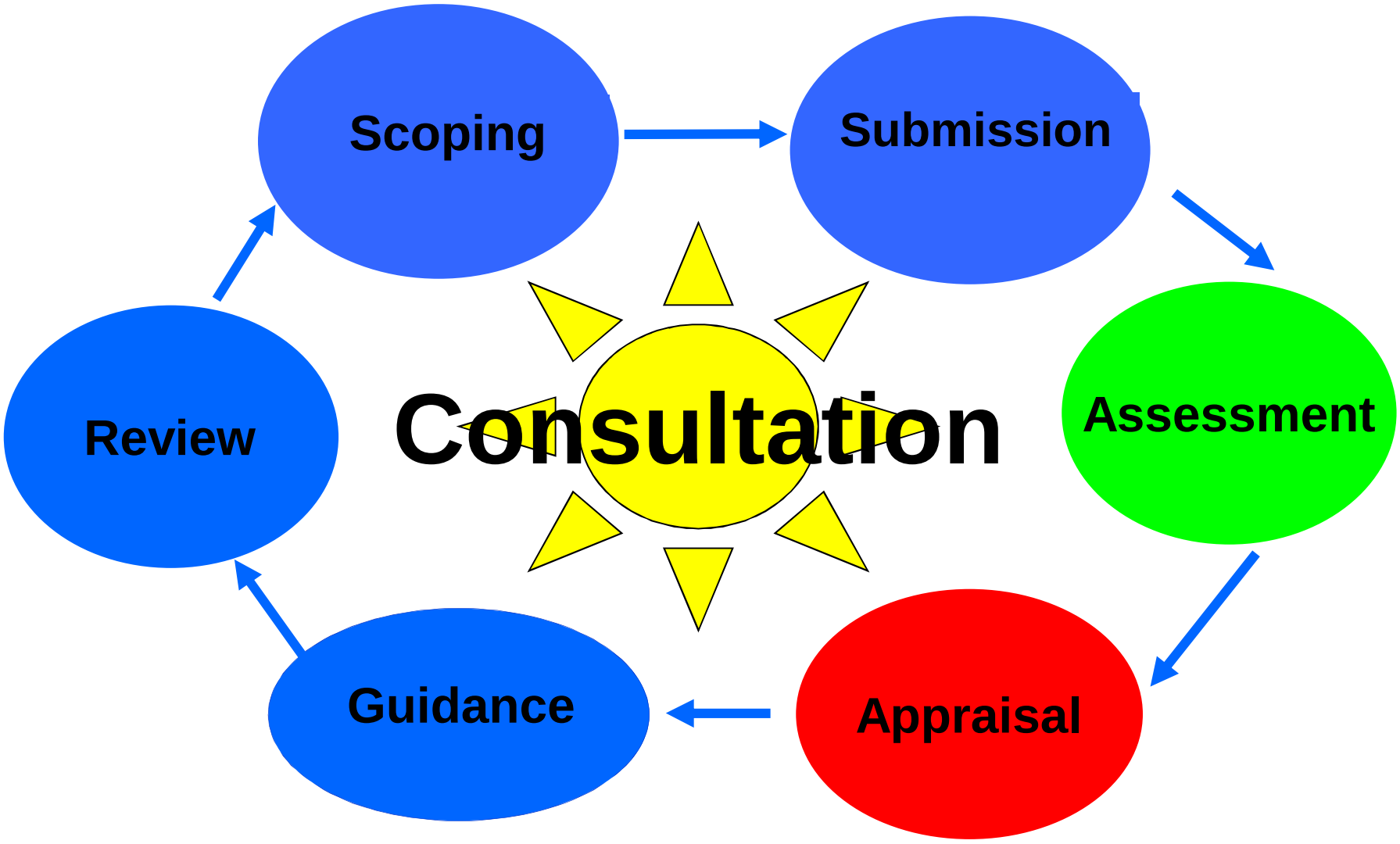
Medical Technologies Guidance

- **Single** product
- Innovative **devices and diagnostics** (early stage evidence)
- **More benefit/same cost OR same benefit/less cost**.

Highly Specialised Technologies

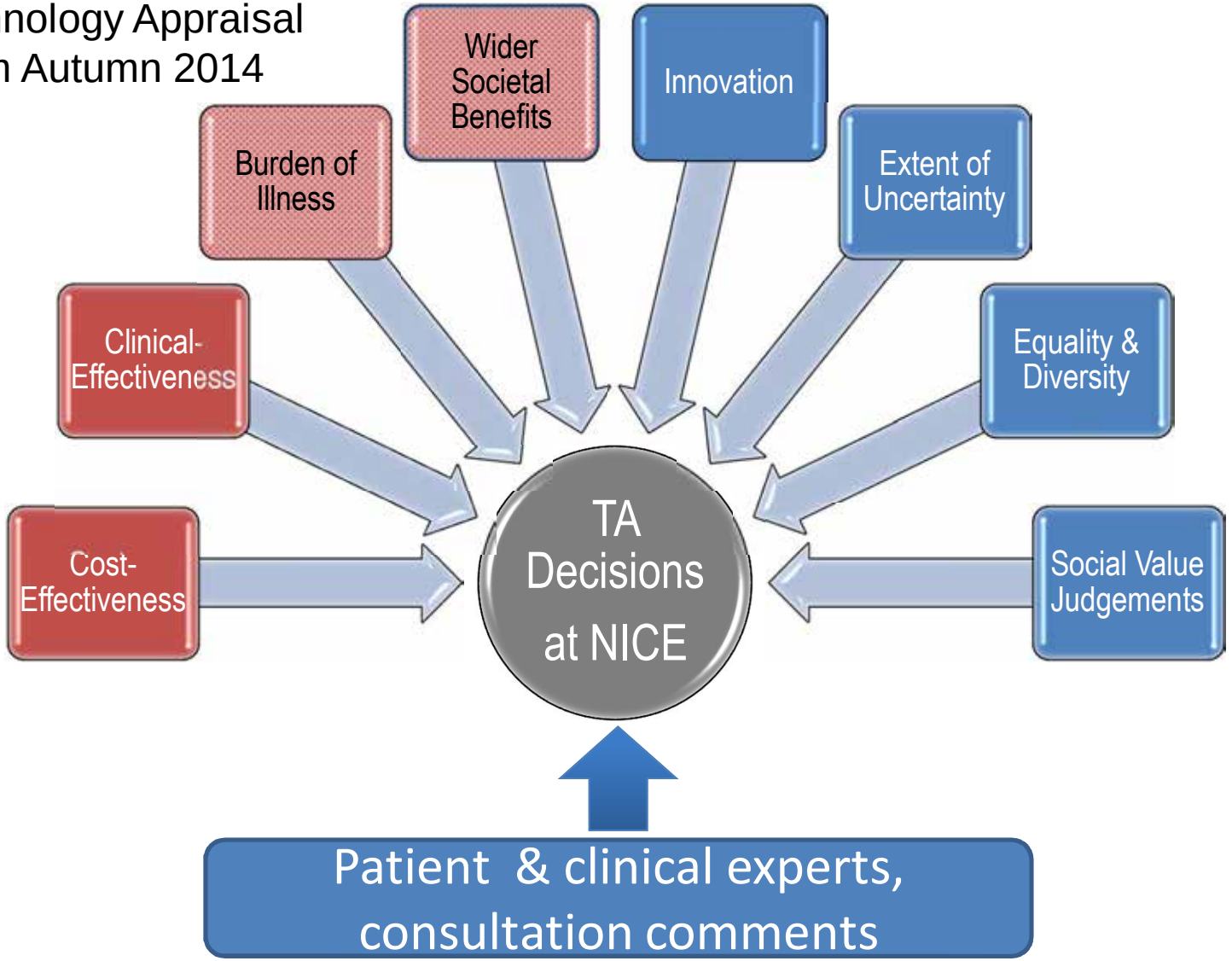
- Small distinct patient group
- High cost
- National commissioning

The Process


Scoping
Submission
Assessment
Appraisal
Guidance
Review
Consultation

NICE's Procedural Principles

Technology Appraisal
From Autumn 2014



What evidence does NICE use?

Patient and public involvement policy

NICE's approach to patient and public involvement is based on two key principles:

1. that lay people, and organisations representing their interests, have opportunities to contribute to developing NICE guidance, advice and quality standards, and support their implementation, and
2. that, because of this contribution, our guidance and other products have a greater focus and relevance for the people most directly affected by our recommendations.

Introduction

Since 1999 NICE has involved patients, service users, carers and the public, including voluntary, charitable and community organisations in its work. In April 2013 NICE's remit expanded to include producing guidance and quality standards on social care topics. To support this work we are building on existing relationships with organisations who work in social care and fostering new relationships with organisations with whom we have not worked previously.

Our guidance aims to address issues relevant to patients, service users, carers and the public, reflect their views, and meet their health and social care needs. Involving patients, service users, carers and the public adds value to the discussions of the independent committees and working groups that develop NICE guidance.

The value of this patient, service user, carer and public involvement has been shown many times in the guidance NICE has produced. NICE is committed to continuing and developing its patient and public involvement work, a commitment underpinned by this policy.

This policy:

- sets out NICE's commitment and approaches to patient and public involvement
- outlines the underlying principles of NICE's approach to involving lay people
- explains the support available to lay people and organisations involved with NICE's work.

More information

Download a PDF version of the Patient and Public Involvement Policy

Patient and Public Involvement Policy
PDF 260 kb

Lay contributor payments - policy principles
PDF 178 kb

Lay contributor payments - frequently asked questions
PDF 145 kb

Independent committees and working groups

Members of these groups have commented on the importance of this involvement:

Involvement of patients focused on the

Patient and publi

What questions are we actually asking ?

Scoping

- Consultation
- Submissions

Assessment

- Representation on advisory bodies
- Testimony at committees
- Social value judgments

Guidance

- Public consultation
- Appeal rights for patient organisations

Patient and public: technical

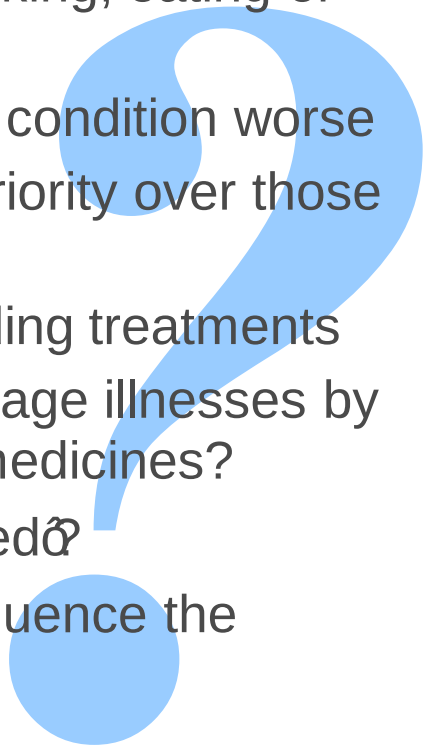
- Patient-report outcomes
- QOL
- Economic modeling
  - Utilities: published
  - Tariffs
- Some qualitative reviews
- Issues
  - Resources
  - Timing
- Going forwards
  - Health and social care: unified metrics?
  - Improved elicitation
  - Real-world data
  - MCDA? : but whose weights?
  - Modeling of the decision-making process? : Janus

Value judgements

- Guidance is based on the best available evidence
- May not be very good and is rarely complete
- Have to make judgements about
  - what is good and bad in the available science: **scientific value judgements**
  - what is good for society: **social value judgements**

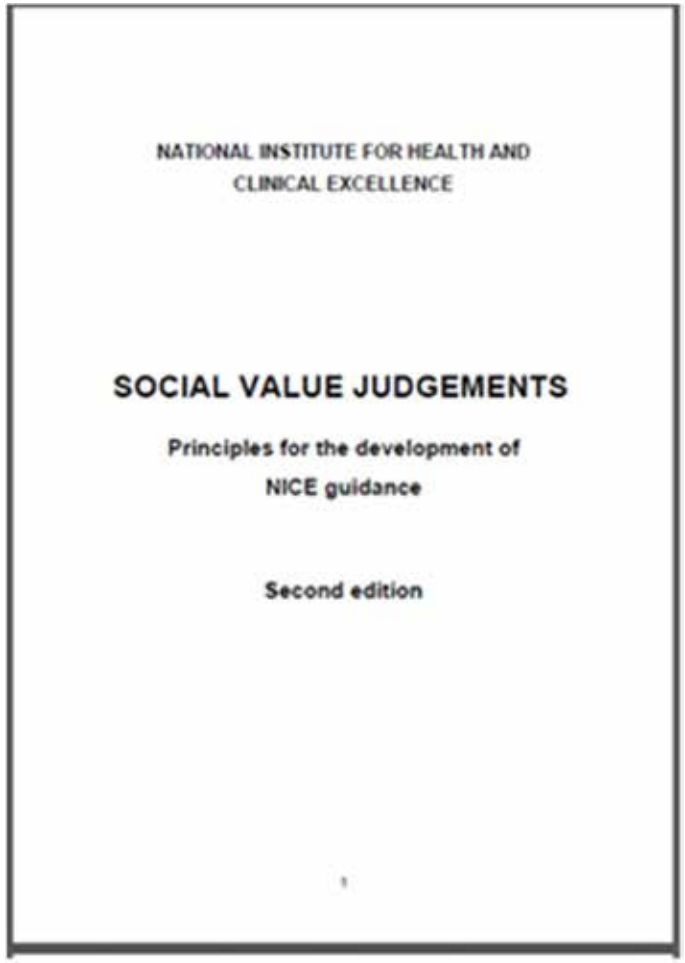
Social value judgements

- societal aspirations, preferences, culture and ethical principles
  - Should ñdeservingnessò ever be a criterion? Think about illnesses such as those brought on by smoking, eating or drink
  - Failing to comply with treatment, making a condition worse
  - Should getting people back to work be a priority over those with no work ?
  - Should age ever be a factor in recommending treatments
  - Are high cost medicines that extend end stage illnesses by only a few weeks valued more that other medicines?
  - How to interpret the concept of ôclinical needõ?
  - Should the nature of a condition should influence the decision?
  - Risks versus benefits?
  - Should we pay more today for tomorrowôs innovations?



Social value judgements document

The judgements that NICE and its advisory bodies should apply when making decisions about the effectiveness and cost effectiveness of interventions, especially where such decisions affect the allocation of resourcesé é


NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

SOCIAL VALUE JUDGEMENTS

Principles for the development of NICE guidance

Second edition

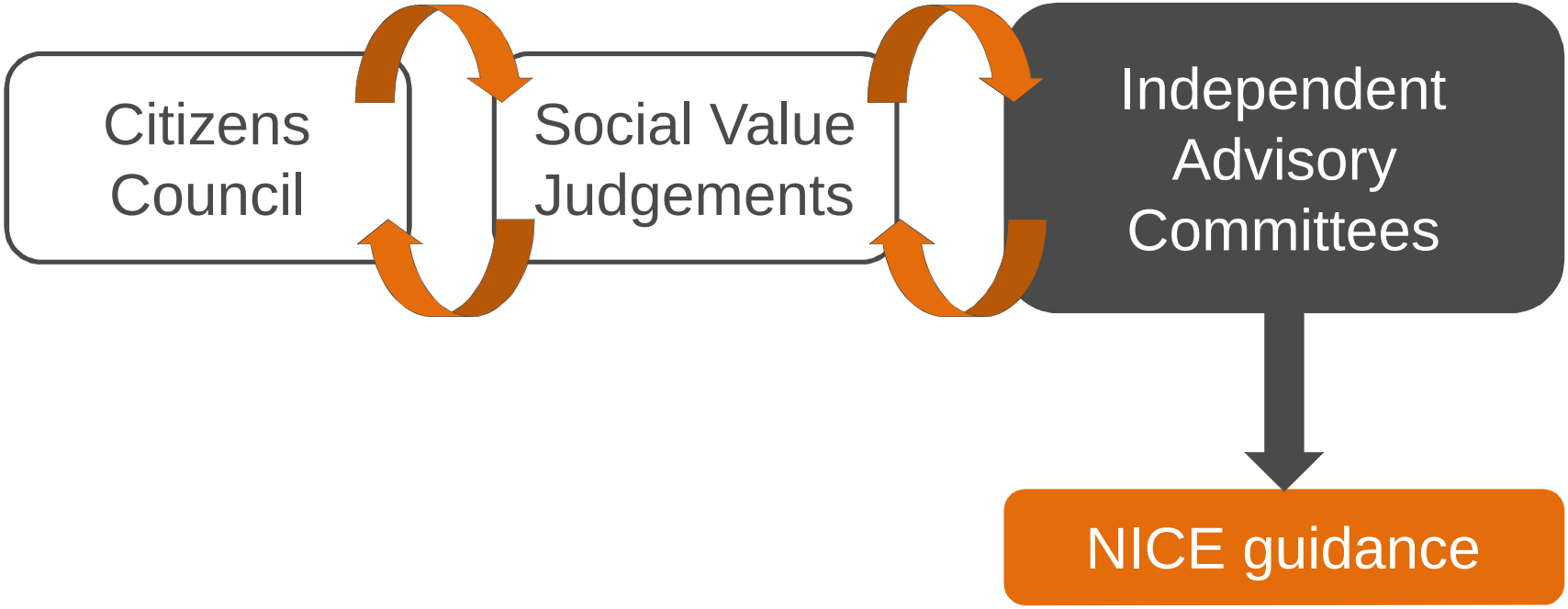
Social value judgements document

- ethical principles concerning decisions on healthcare (moral principles, justice)
- fundamental principles underlying NICE and its processes (legal obligations, guidance process)
- principles NICE applies when developing guidance (how to make decisions)
- responding to comments and criticisms (duty to)
- how NICE aims to avoid discrimination and promote equality (race, disability, age, etc)
- reducing inequalities

Social value judgements document

- Currently in the process of updating the document
- Project includes:
  - academic literature review
  - stakeholder workshops
  - 2014 Citizens Council meeting
  - public consultation
- Updated document due Spring 2015

Applying social value judgements



The Citizens Council

Citizens Council membership

- 30 people broadly representative of UK adult population
- But not working in health or social care industries or for patient groups etc.
- Completely new Council is recruited every 3 years (no rolling membership)

The role of the Citizens Council

- Set up to explore and understand the **social, moral and ethical views** of the general public
- Explores value judgements:
  - based on **personal** beliefs about what is right or wrong, beneficial, important, useful, beautiful, desirable, constructive, etc.
  - shared by members of a particular society or a value system i.e. **cultural value**
    - **ñsocial value judgementsò**

Using the outputs

- Main output is the independent report, which captures the Councilôs exploratory discussions and the range of opinions and social values held
- Used in two main ways:
  - **in relation to the specific topic explored**
    used to inform that area of NICEôs activity
  - **as whole, across all topics**
    used to inform NICEôs Social Value Judgements document

The Citizens Council ómethodô

- Operates through a two-day meeting, roughly once per year
- Similar to a ñCitizenôs Juryòformat:
  - One topic per meeting
  - Presentations from experts in the topic area
  - Group discussion and deliberative activities
- Independently facilitated
- Aim is to explore the *breadth and depth* of opinions (rather than reach consensus)

Examples of previous topics

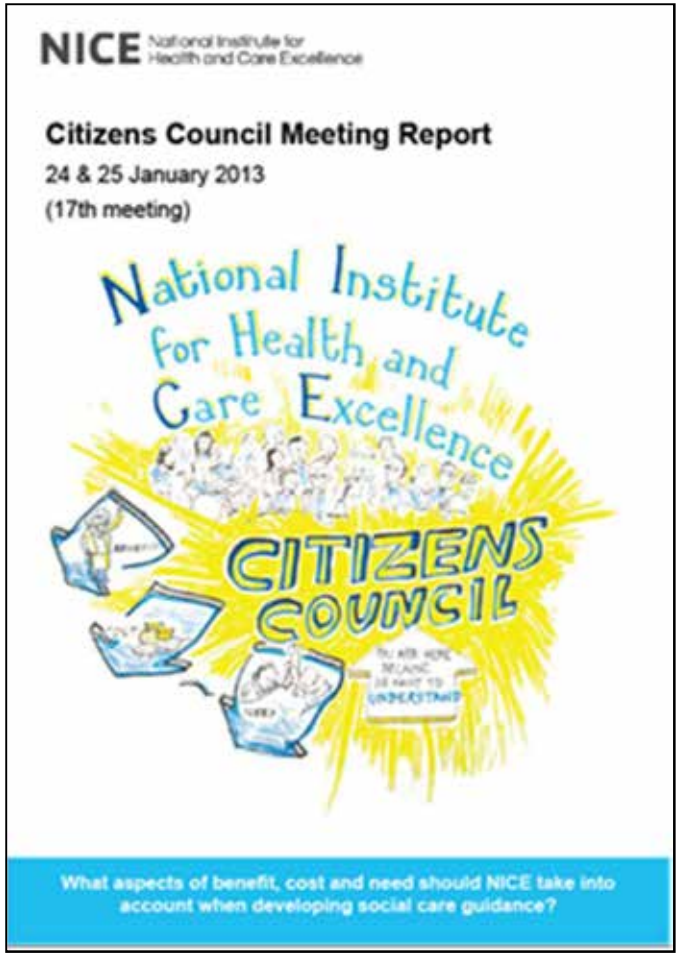
- **Clinical need:** What should NICE take into account when making decisions about clinical need? (November 2002)
- **Age**: Are there circumstances in which the age of a person should be taken into account when NICE is making a decision about how treatments should be used in the NHS? (November 2003)
- **Quality adjusted life years (QALYs) and severity of illness**: Should NICE and its advisory bodies take into account the severity of a disease when making decisions? (February 2008)

Reports: http://www.nice.org.uk/aboutnice/howwework/citizenscouncil/reports.jsp

January 2013 topic

Social Care:

What aspect of **benefit**, **cost** and **need** should NICE take into account when developing **social care guidance**?



Example: Social care (2013)

ñ*What aspect of benefit, cost and need should NICE take into account when developing social care guidance?***ò**

- NICE should approach the development of quality standards and guidance for social care with ófresh eyesôï those of the service user
- NICE should produce new and original quality standards for social care that are authoritative and they must have ólegithô
- NICE standards and guidance should enable care to be built around each personôs individual needs
- NICE should consider integrating health and social care better to the point of producing joint health and social care guidance
- NICE standards and guidance should advocate that unpaid and informal carers are properly supported from an early stage and that these costs and benefits are taken account of in any calculations

NEWDIGS: New Drug Development ParadIGmS

A Systems Approach to Enhancing the Value & Sustainability of Pharma Innovation

PATIENTS

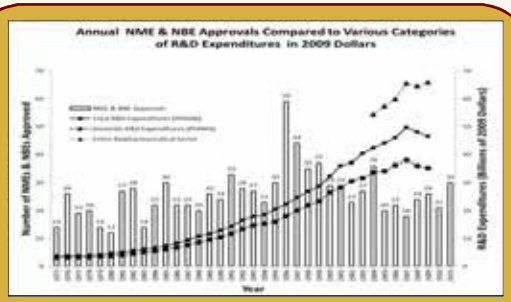
More treatments faster

"We simply don't have time to wait for the results [of clinical trials]. Our life spans are shorter than the [regulatory] approval process."

"Frustrated ALS Patients Concoct Their Own Drug," Wall Street Journal, April 15, 2012

PHARMAS

Unsustainable cost of innovation



Burrill & Co. Analysis for PhRMA 2006-2011

NEWDIGS Mission: Reliably & sustainably deliver new, better, affordable therapeutics to the right patients faster.

PROVIDERS

Need better benefit/risk information

"I rarely prescribe a new drug during the first 2 years it has been on the market. There is too much uncertainty about safety during this time."

Neurologist, Boston

REGULATORS

Competing demands: innovation & safety

"Our current regulatory model sets unrealistic expectations for the public that it is possible to eliminate all uncertainty about product safety prior to market approval."

Senior Official, FDA

PAYORS

Skyrocketing costs

"If companies want premium pricing for their drugs, they need to demonstrate premium value."

John LaMattina, PureTech Ventures

New Drug Development Paradigms (NEWDIGS)

- Collaborative innovation and learning environment
  - Think and Do Tank
  - Open and transparent
  - MIT neutral intermediary
- Systems engineering approach to designing, evaluating, and catalyzing change:
  - Coordinate the evolution of processes, technologies, policies, and people
  - Understand what tradeoffs are required to align stakeholders
  - Inform and enable change

NEWDIGS Global Collaborators (Partial List)

Regulators

- EMA
- FDA
- Health Canada
- HSA
- MHRA

Payers/HTA

- Aetna
- National Healthcare Institute*
- EUnetHTA
- HAS**
- Kaiser
- NICE

Academia

- MIT
- HMS + hospitals
- Sloan Kettering
- National U of Singapore

* Formerly Netherlands CVZ
** HAS: French National Authority for Health

Patients/Other

- ASCO
- Friends of Cancer Research
- Genetic Alliance
- NORD
- RWJF

Industry

- Bristol Myers Squibb
- GlaxoSmithKline
- Pfizer
- Sanofi

NEWDIGS: Linking Thought Leadership to Action

March 2012

STATE OF THE ART

nature publishing group

Open

See COMMENTARY page 378

Adaptive Licensing: Taking the Next Step in the Evolution of Drug Approval

H-G Eichler[1,2], K Oye[2,3,4], LG Baird[2], E Abadie[5], J Brown[6], CL Drum[2], J Ferguson[7], S Garner[8,9], P Honig[10], M Hukkelhoven[11], JCW Lim[12], R Lim[13], MM Lumpkin[14], G Neil[15], B O'Rourke[16], E P D Shoda[18], V Seyfert-Margolis[14], EV Sigal[19], J Sobotka[20], D Tan[12], TF Unger[18] and G Hirsch[2]

Traditional drug licensing approaches are based on binary decisions. At the moment of licensing, an experimen therapy is presumptively transformed into a fully vetted, safe, efficacious therapy. By contrast, adaptive licensir approaches are based on stepwise learning under conditions of acknowledged uncertainty, with iterative phas gathering and regulatory evaluation. This approach allows approval to align more closely with patient needs fo access to new technologies and for data to inform medical decisions. The concept of AL embraces a range of per Some see AL as an evolutionary step, extending elements that are now in place. Others envision a transformati framework that may require legislative action before implementation. This article summarizes recent AL propo discusses how proposals might be translated into practice, with illustrations in different therapeutic areas; and unresolved issues to inform decisions on the design and implementation of AL.

Clinical Pharmacology & Therapeutics (2012);
91 3, 426ï 437. doi:10.1038/clpt.2011.345

March 2014

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European Medicines Agency launches adaptive licensing pilot project

Press release

19/03/2014

European Medicines Agency launches adaptive licensing pilot project

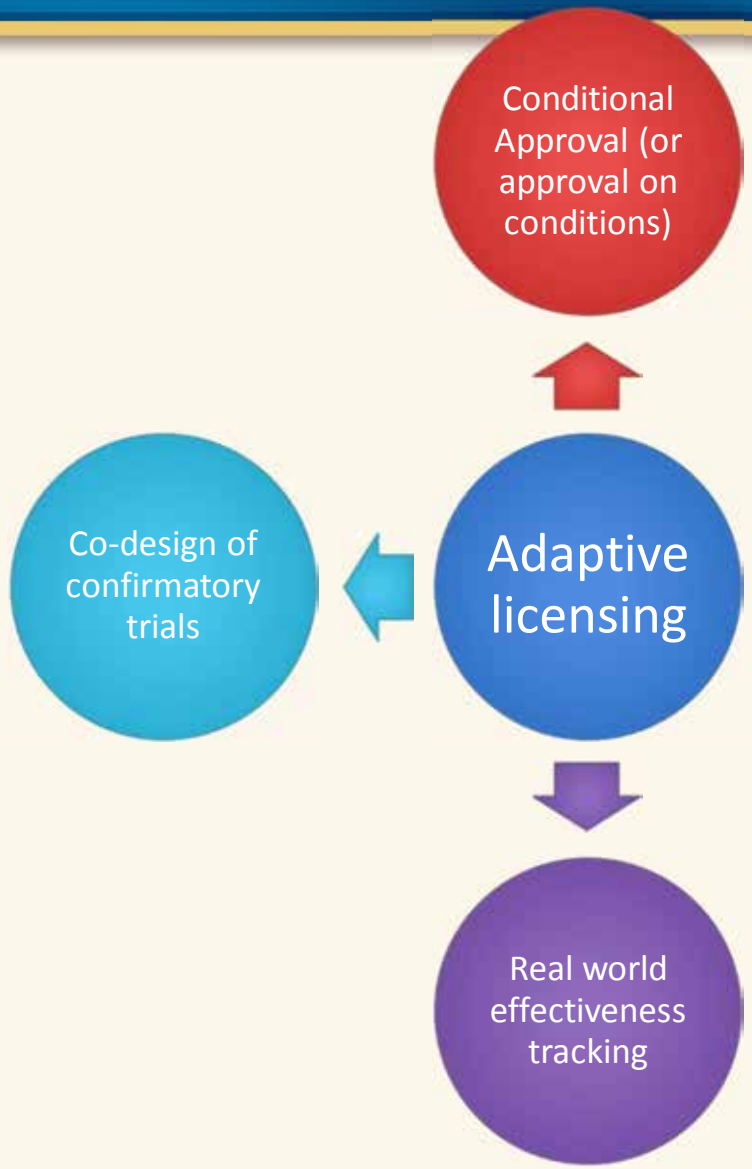
Improving timely access for patients to new medicines: pilot explores adaptive licensing approach with real medicines in development

The European Medicines Agency (EMA) is inviting companies to participate in its adaptive licensing pilot project. Companies who are interested in participating in the pilot are requested to submit ongoing medicine development programmes for consideration as prospective pilot cases.

A framework to guide discussions of individual pilot studies has been published.

The adaptive licensing approach, sometimes called staggered approval or progressive licensing, is part of the Agency's efforts to improve timely access for patients to new medicines. It is a prospectively planned process, starting with the early authorisation of a medicine in a restricted patient population, followed by iterative phases of evidence gathering and adaptations of the marketing authorisation to expand access to the medicine to broader patient populations.

Key elements of the concept



EMA, based on rapporteur evaluation of submission

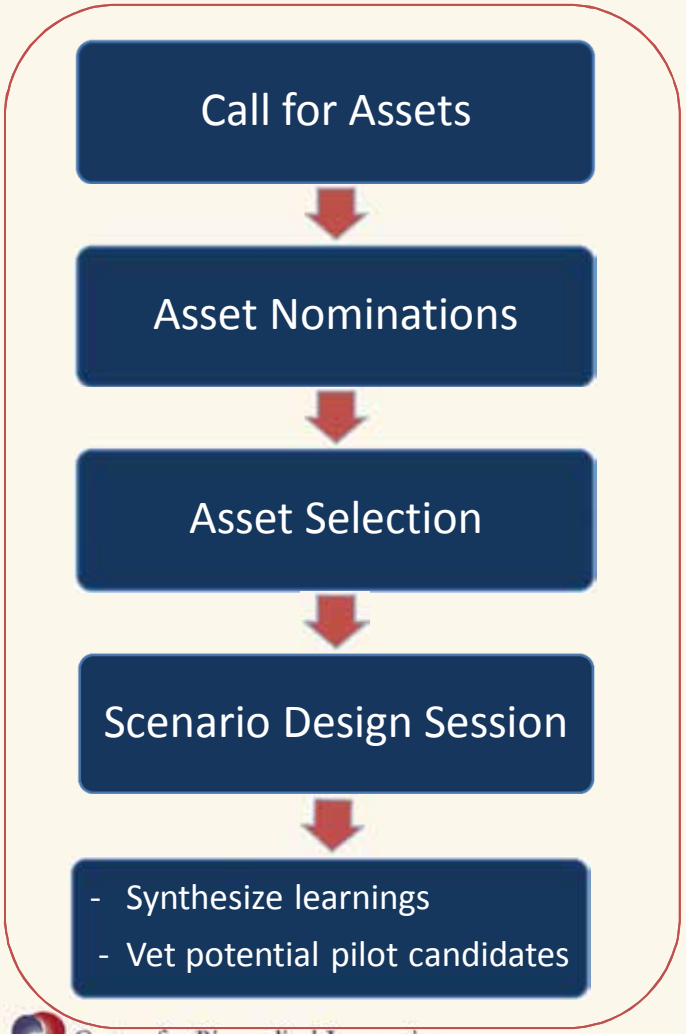
Stakeholders:
-Sponsor
-Regulator
-HTA
-Patients

Patients treated; sponsor reimbursed

Outcome, safety data collection via reliable network

Scenario Design Sessions: Interactive Multi-stakeholder Simulations as a Collaborative Learning Platform

Scenario Design Methodology



- 14 assets nominated since 2011 by 9 companies
- 13 assets evaluated in scenario design sessions
- 6 assets presented at 2 or more workshops

Adaptive Licensing: What Have We Learned?

- AL Is not a “regulatory pathway” in the traditional sense
  - Can not be driven by regulators alone – involves all stakeholders
  - Involves coordination across development, approval, reimbursement, and real world monitoring and product utilization
  - Can be implemented using existing statutory authorities

- Not one- size-fits-all model: design and implementation is highly context dependent
  - Eg, therapeutic area, jurisdiction (cultural, policy, healthcare system), asset-specific considerations (maturity of associated science/technology, associated marketplace)
  - Provides a structured framework for all stakeholders to work together to optimize the management of uncertainty related to evidence vs. access
  - Having appropriate stakeholders involved ensures that the relevant tradeoff decisions are tailored for the context

Some Important Considerations for Advancing Adaptive Licensing

- Acceptability of tradeoffs for all stakeholders, e.g.,
  - Sponsors & payers: economics
  - Patients: tolerance of uncertainties versus earlier access
- Legal issues
  - IP and market exclusivity
- Information and communication
  - Systems that deliver reliable and timely post market evidence
  - Effective and timely communication of emerging product information to key stakeholders

- Access control:
  - Assurance that use of product is in those for whom it is initially authorized
- Prospective planning throughout lifecycle, with pre-specified processes for iterative review, decision making aimed at expanding/contracting product use
  - Involve all stakeholders, not just sponsor/regulator
- Resource requirements, especially for regulators, payers, and sponsors
- Implications of AL for global development strategy for multi-national companies

Get Real

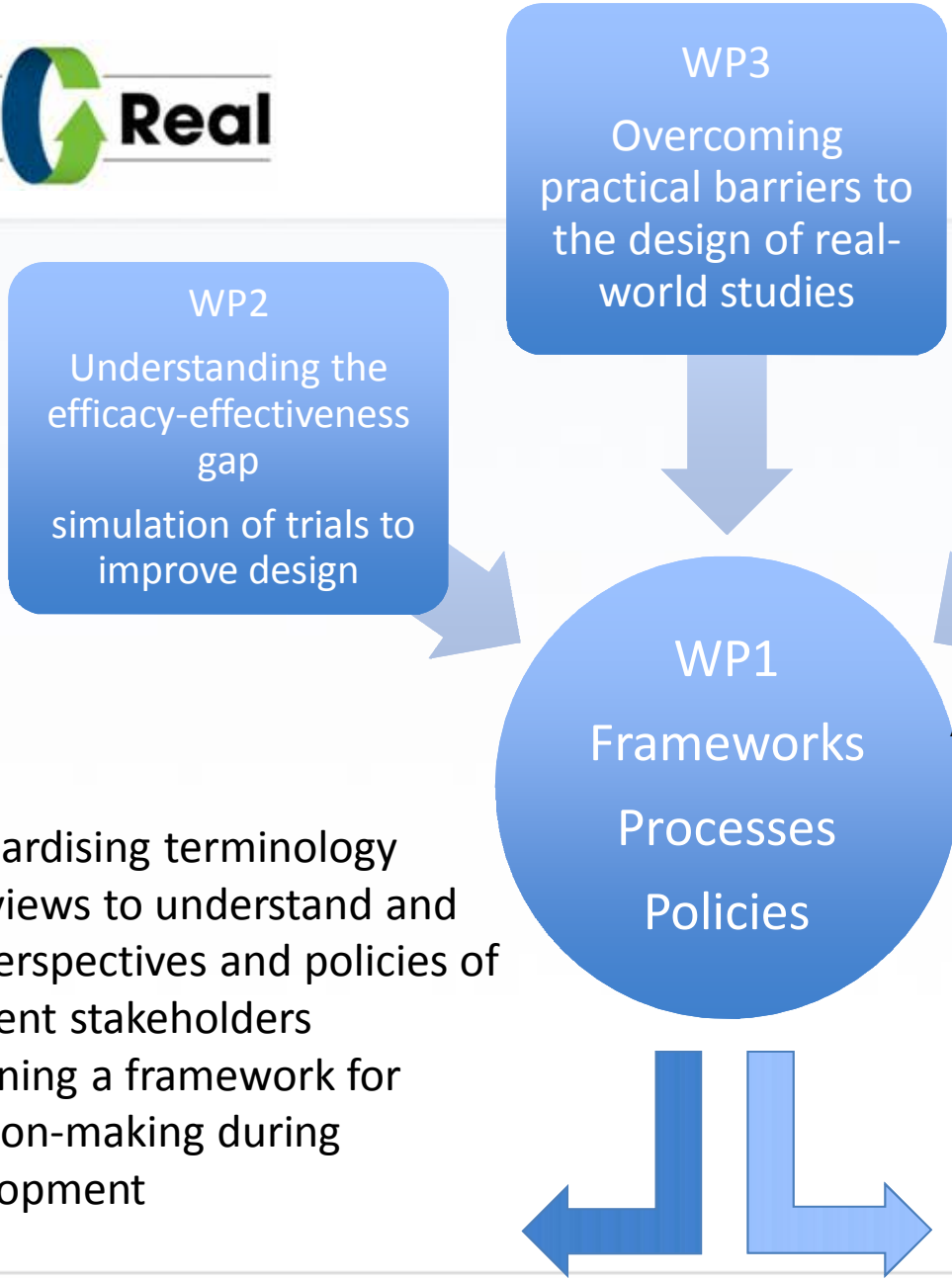
Real-Life Data in Drug Development

- **Innovative Medicines Initiative (IMI)**
  - Europe's largest public-private initiative
  - joint undertaking between European Union and European pharmaceutical industry association EFPIA.
- **GetReal**
- Understanding how real-world data can contribute to decision-making
  - October 2013 to December 2016 (39 months)
  - 29 partners
  - Total budget: ú18 million
    - 50% staff from the 15 participating pharma companies
    - 50% cash contribution from the EU to fund ópublicô sector

The research leading to these results has received support from the Innovative Medicines Initiative Joint Undertaking under grant agreement no [115303], resources of which are composed of financial contribution from the European Unionôs Seventh Framework Programme (FP7/2007-2013) and EFPIA companiesô in kind contribution.
www.imi.europa.eu

Get Real

Real-Life Data in Drug Development



- Standardising terminology
- Interviews to understand and the perspectives and policies of different stakeholders
- Designing a framework for decision-making during development

- 5 Case studies using drugs that had difficulty at regulation and HTA
  - 360 degree reviews
  - Re-designing development pathways to include real-world data
  - Simulation
  - Ascertaining impact on decision makers

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