

A new methodology for HTA Ultra Orphan Drugs – the experience of AGNSS, NHS

ECRD 2012

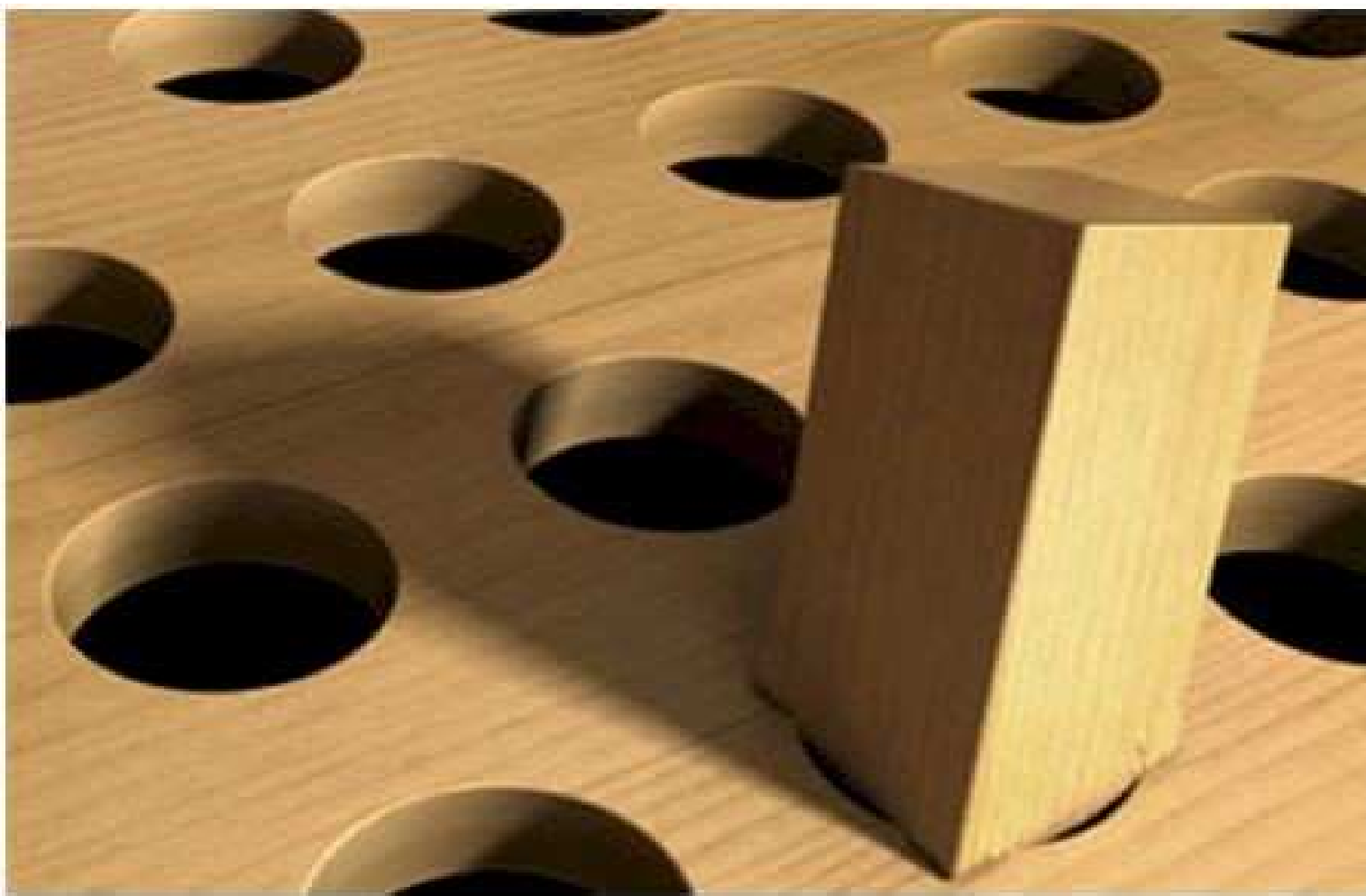
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- Why England has a national decision-making process for **very** rare diseases
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NICE ...



Or ...

AGNSS?



The Advisory Group for National Specialised Services (AGNSS)

- AGNSS provides a single source of **advice to Ministers** on:
 - which **services** at which centres should be nationally commissioned
 - a small number of highly specialised **new drugs and technologies** that are not suitable for consideration by NICE

AGNSS Membership

Chair: Professor Michael Arthur

- Patient members
- Lay members
- Local commissioners
- Health economist
- Ethicist
- General Practitioner
- Psychiatrist
- Geneticist
- Pharmacist
- Public Health
- Physician
- Paediatrician
- Pathologist
- Surgeon
- Regional Chief Executive
- Regional Finance Director
- Regional Medical Director
- Health Technology Assessment Director

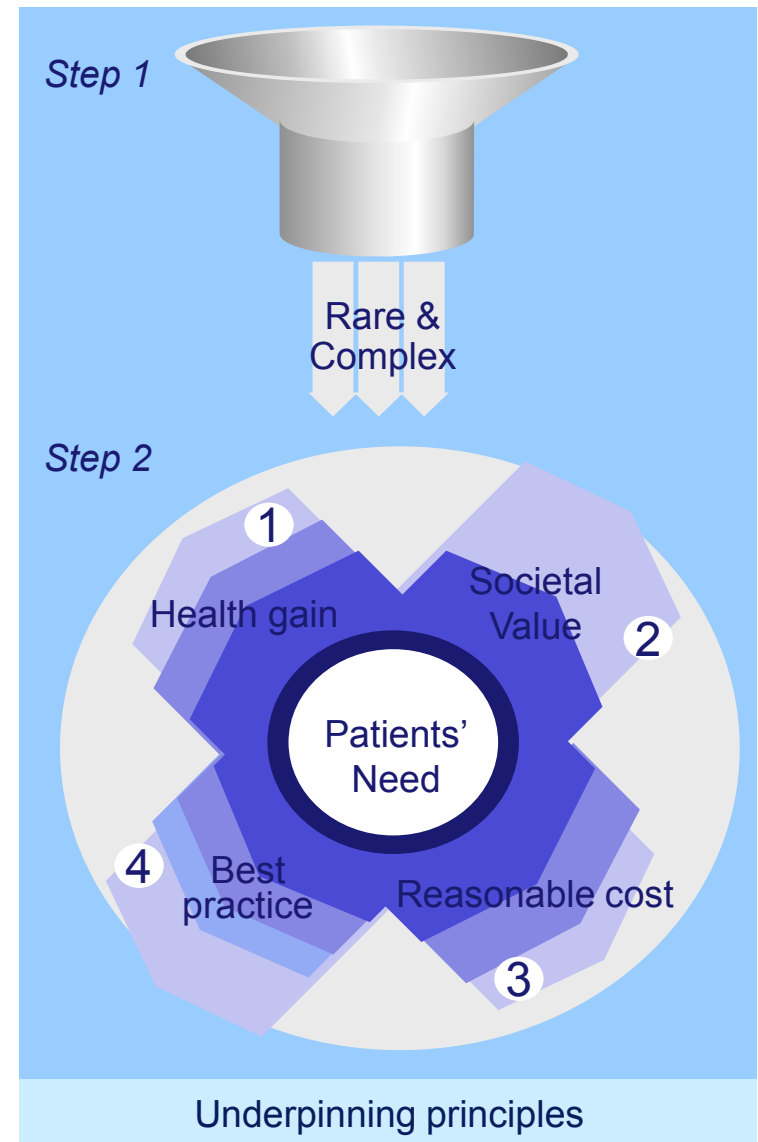
Observers: National Specialised Commissioning Team
Department of Health, Devolved Administrations

Developing the AGNSS decision-making framework

Specialised Services

- Builds on:
 - NICE Citizen’s Council findings
 - Other NHS decision-making frameworks

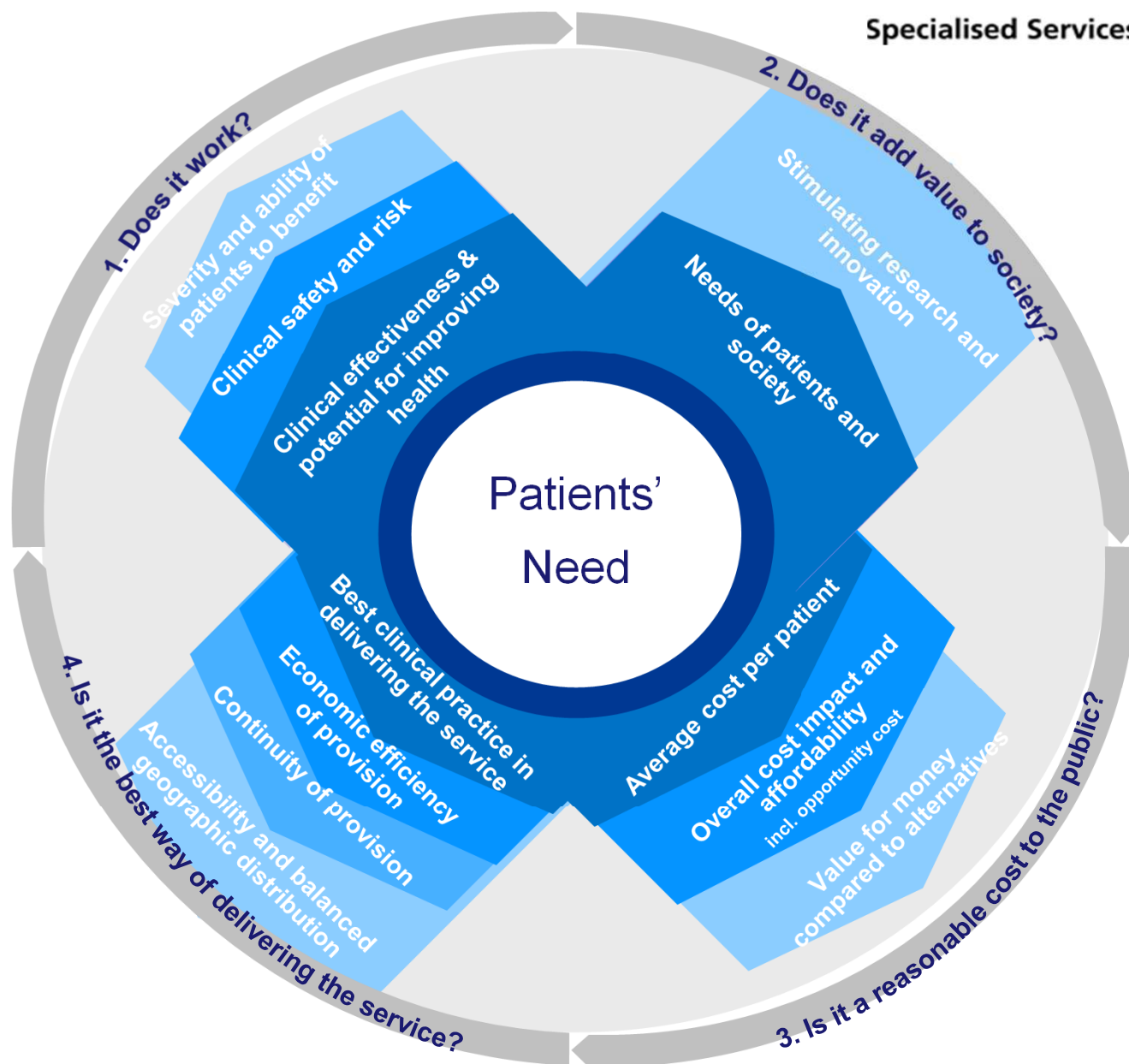
- Developed through interviews and workshops with over 80 stakeholders including:
 - Patient groups
 - NHS commissioners
 - National Clinical Directors
 - Royal Colleges
 - Health economists
 - Public health consultants
 - Medical ethicists
 - Department of Health
 - NICE



Decision-making framework: core criteria

Specialised Services

- All criteria will be considered as part of each evaluation and the outcomes will be documented as part of the rationale for the decision
- Following evaluation of the criteria, a holistic view will be taken across all criteria



New drugs and technologies

- Special considerations for new drugs and technologies
 - Products will
 - Be aimed at a **clinically distinct subgroup**
 - Ordinarily, have EU designated orphan drug status
 - Usually, be applying for market authorisation
 - AGNSS will not consider products which received market authorisation before 24 September 2010
 - AGNSS will not consider products which have been, or will be, appraised by NICE

Full AGNSS review - the elaprased pilot

- Pilot process
 - Scoping meeting
 - Modelling meeting
 - Company submission
 - Health Technology Appraisal
 - Patient submission
 - AGNSS discussion (included presentations from patient group, expert clinician, company, and York HTA team)

- Working with partners
 - Industry (ABPI and manufacturer), patient group, academic HTA team, NICE, Department of Health, clinical expert

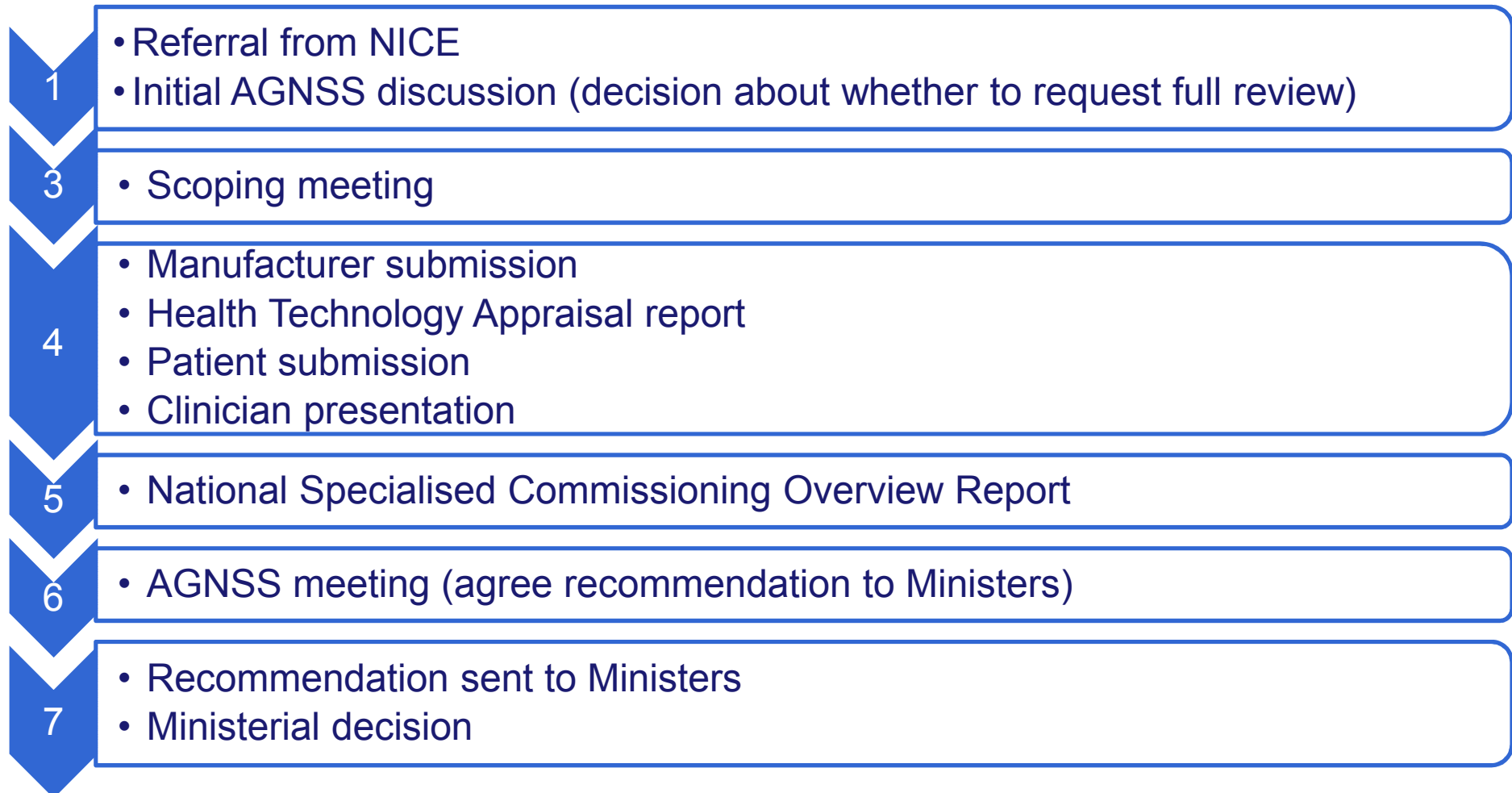
Lessons from the pilot

- AGNSS liked:
 - Patient submission and case studies
 - Presentations from key partners (clinician, patient, company, HTA team)
 - Information about the natural history of the disease and current treatment
 - Full HTA process

More lessons from the pilot

- AGNSS also wanted:
 - An overview of the submissions from the secretariat
 - More information about the range of benefits, including individual patient level outcome data
 - Greater clarity about what information is available for each of the criteria
 - More explanation of the evidence and its limitations
 - Greater clarity about assumptions being made when extrapolating from non-disease specific data
 - More focus on the wider benefits to patients, families and carers
 - Context around costs and benefits

Full AGNSS review process



Steps 3 - 6 are likely to take 9 months. The process could begin before an application for marketing authorisation is complete.

Manufacturer submission

- Builds on NICE Single Technology Appraisal submission template and lessons from the pilot

- Aims to:
 - Reflect criteria in decision-making framework
 - Minimise repetition
 - Encourage a broader societal perspective
 - Provide room for narrative alongside data
 - Expects manufacturers to build a health economic model

- Complemented by expert clinical input, patient submission and Health Technology Assessment report

Patient submission

- Builds on lessons from the pilot
- Aims to:
 - Describe the impact of a disease on patients and their families
 - Encourage a broader societal perspective
 - Provide room for narrative alongside data
 - Provide confidence that it reflects the experiences of the patient population
- Support available to patients/patient groups in developing submission
 - Literature review
 - Focus groups
 - Writing
 - Survey design
 - Interviews
 - Attend meetings

Challenges ahead

- Transition to new NHS arrangements
- Financial pressures
- Value-based pricing
- Stratified/personalised medicine