Precision Medicine & CDx Market Access: A NICE perspective

World CDx – London 2017

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Adviser, NICE Office for Market Access
Agenda

• NICE
• Assessment of the clinical and cost effectiveness of companion diagnostics
• NICE evaluation options for companion diagnostics
  • Technology Appraisals programme
  • Diagnostics Assessment programme
• Support for companies
  • NICE Office for Market Access
  • NICE Scientific Advice
The National Institute for Health and Care Excellence (NICE) is the independent organisation responsible for providing national guidance and advice to improve health and social care.

1999
- The National Institute for Clinical Excellence established
- Special Health Authority, to reduce variation in the availability and quality of NHS treatments and care

2005
- Expanded to include the functions of Health Development Agency
- Name changed to the National Institute for Health and Clinical Excellence

2013
- NICE re-established as a non-departmental public body
- Took on responsibility for developing guidance and quality standards in social care; name changed to reflect new responsibilities
NICE - Aims

- Speed the uptake by the National Health Service (NHS) of interventions that are both clinically effective and cost effective
- Encourage better and more rational use of available resources by focussing the provision of health care on the most cost-effective interventions
- Encourage more equitable access to healthcare (reduce postcode lottery of care)
- Encourage the creation of new and innovative technologies
Highly Specialised Technologies*
Interventional Procedures*
Technology Appraisals*
Diagnostics Assessment Programme (DAP)*
Medical Technologies Evaluation Programme (MTEP)*

NICE Office for Market Access
NICE & NICE Scientific Advice

PASLU = patient Access Scheme Liaison Unit
*Denotes guidance producing programmes within CHTE for different technologies and value propositions
NICE perspective

- Companion diagnostics are tests intended to assist health care professionals in making treatment decisions for their patients.
- They do so by elucidating the efficacy and/or safety of a specific drug or class of drugs for a targeted patient group or sub-groups.
- There are two main groups of companion diagnostics that include:
  - Tests that have been developed after a drug has come to market (complementary)
  - Tests that are being developed in conjunction, or as a companion to the drug
Companion diagnostics

• Test developed in conjunction with a pharmaceutical

• Includes IVDs and molecular technologies

• Included in the Summary of Product Characteristics and Marketing Authorisation

• Typically evaluated in the NICE technology appraisal programme
Complementary diagnostics

• Test developed after adoption of pharmaceutical and offers potential to improve targeting of existing drugs

• Includes IVDs, molecular technologies, devices

• Identify sub-groups who may gain more benefit

• Monitor treatment and tailor the prescription

• Typically evaluated in the NICE diagnostics assessment programme
Methods in technology appraisals

Technology appraisals methods guide states:

• Costs of CDx testing incorporated into evaluation of clinical and cost effectiveness

• Sensitivity analysis to assess impact of CDx cost on cost effectiveness of pharmaceutical

• Diagnostic accuracy can be examined and incorporated in cost effectiveness analysis

• Potential issues of alternative CDx can be highlighted in guidance without assessment of evidence
<table>
<thead>
<tr>
<th>Treatment</th>
<th>Condition</th>
<th>Marker</th>
<th>NICE TA</th>
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<tr>
<td>Imatinib</td>
<td>Chronic myeloid leukaemia</td>
<td>Philadelphia chromosome (bcr-abl). Kit (CD 117)</td>
<td>50, 70, 241, 251, 86, 196, 209</td>
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<td>GIST</td>
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<td>Trastuzumab</td>
<td>Breast cancer</td>
<td>HER-2 (protein)</td>
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<td>Metastatic gastric cancer</td>
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<tr>
<td>Bevacizumab</td>
<td>Breast cancer</td>
<td>HER 2 (protein) (negative)</td>
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<td>Cetuximab</td>
<td>Metastatic colorectal cancer</td>
<td>KRAS</td>
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<td>Panitumumab</td>
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<td>Gefitinib, Erlotinib</td>
<td>Non-small-cell lung cancer</td>
<td>EGFR TK mutations</td>
<td>192, 258, 310</td>
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<td>Afatinib</td>
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<td>Vemurafenib</td>
<td>Malignant melanoma</td>
<td>BRAF V600 mutation</td>
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<tr>
<td>Crizotinib</td>
<td>Non-small-cell lung cancer</td>
<td>Anaplastic lymphoma kinase fusion (ALK) genes</td>
<td>296</td>
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Methods in the diagnostics assessment programme

Assessments of multiple companion diagnostic test options will generally be undertaken in the NICE diagnostics assessment programme.

- **Assessment of clinical effectiveness:**
  - End-to-end studies if available
  - Evidence on test accuracy from existing or new systematic reviews
  - Evidence on the efficacy of treatment from existing or new systematic reviews
  - Expert elicitation
  - These clinical effectiveness measures feed into the economic modelling

- **Assessment of cost effectiveness:**
  - Economic analysis combines the costs and outcomes for different alternatives and is used to calculate cost effectiveness
  - Looks at the differences in costs and health outcomes between two or more options
  - The analysis models the underlying care pathway and the role of the diagnostic tests in this pathway, and data sources provide values for parameters such as test accuracy, health related quality of life etc
  - Uncertainties can arise from assumptions made, selective use of data sources to provide values for clinical effectiveness and costs, and the precision of each input. These are dealt with using sensitivity analysis.
Examples of CDx in DAP

- Therapeutic monitoring of TNF-alpha inhibitors in Crohn’s disease (DG22)

- High-throughput non-invasive prenatal testing for fetal RHD genotype (DG25 links to TA156)

- Gene expression profiling and expanded immunohistochemistry tests for guiding adjuvant chemotherapy decisions in early breast cancer management (DG10)

- EGFR-TK mutation testing in adults with locally advanced or metastatic non-small-cell lung cancer (DG9)
Companion Diagnostics - Diagnostics Assessment Programme

- Companies/clinicians notify NICE
- Test
- Medical Technologies Advisory Committee
- Selected for Diagnostics Assessment Programme
- Not selected for guidance production

DAP process allows for various Companion Diagnostic options (e.g. other proprietary and “in-house” tests) to be included in the assessment.

Assessment may include separate cost effectiveness determinations for the pharmaceutical in combination with each of the alternative Companion Diagnostic options.
The Diagnostics Assessment Process

**Guidance Topic Routed into DAP from MTEP**

**Scoping (12 weeks)**
- Utilising input from stakeholders and specialist to lock down the question the NHS needs answering
- Inclusion of all relevant technologies

**Assessment (28 weeks)**
- Production of systematic review of clinical and cost effectiveness by External Assessment Group
- Stakeholder comments

**Guidance Production (23 weeks)**
- Production of draft recommendations
- Public consultation and finalisation of recommendations
- Resolution period & guidance publication

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NICE National Institute for Health and Care Excellence
Centre for Health Technology Evaluation programmes

- Technology Appraisals*
- Diagnostics Assessment Programme (DAP)*
- National Technologies Evaluation Programme (MTEP)*
- Science Policy & Research

NICE

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The Office for Market Access (OMA)

- **Covers all** life sciences products:
  - **Pharmaceuticals and biopharmaceuticals** including:
    - Orphan & ultra-orphan products
    - Cell/gene therapies
  - **MedTech** including:
    - Medical devices
    - Diagnostic tests
    - Digital (if patient benefit)

- Our team of experts are available to answer initial enquiries or refer you to other system partners where appropriate
  - Offered free of charge.
- Every company’s needs are different, so we offer bespoke services tailored to requirements
  - Fees are charged on a not-for-profit basis (varying in scale to reflect resources required).
Office for Market Access (OMA)

NICE OMA - ‘SAFE HARBOUR PRINCIPLE’
From multi stakeholder to focused perspective
Multifaceted opportunity for exploration of key system access questions

Initial discussion & exploration (free)

Fee for service (not for profit)

- Multi stakeholder
- Focused stakeholder
- Engagement Meetings under safe harbour principles
- EAMS Meetings

NICE Scientific Advice*

* Can be reached through OMA or directly
OMA engagement meetings (under safe harbour principles)

Engagement meeting

Multi-stakeholder engagement meeting
Why seek Scientific Advice?

• Understand the perspective of decision makers
• Understand pros and cons of different trial/study options
• Maximise relevance of trial programme outputs
• Explore alternative strategies to address data gaps
• Integrate cost effectiveness considerations into early decision making
• De-risking strategy
Things to consider...

➢ Build early engagement into your development plans

➢ Engage with NICE and other key stakeholders to understand our perspective and needs

➢ Use the feedback to refine your evidence-generation plans and market access strategy

Get in touch:

WWW.nice.org.uk/OMA

OMA@nice.org.uk
Thank you

Questions?