CDDF 10TH ALPINE CONFERENCE
CURRENT AND FUTURE CHALLENGES OF INNOVATIVE ONCOLOGY DRUG DEVELOPMENT
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Challenges of getting biomarkers to the market

A whole ecosystem approach

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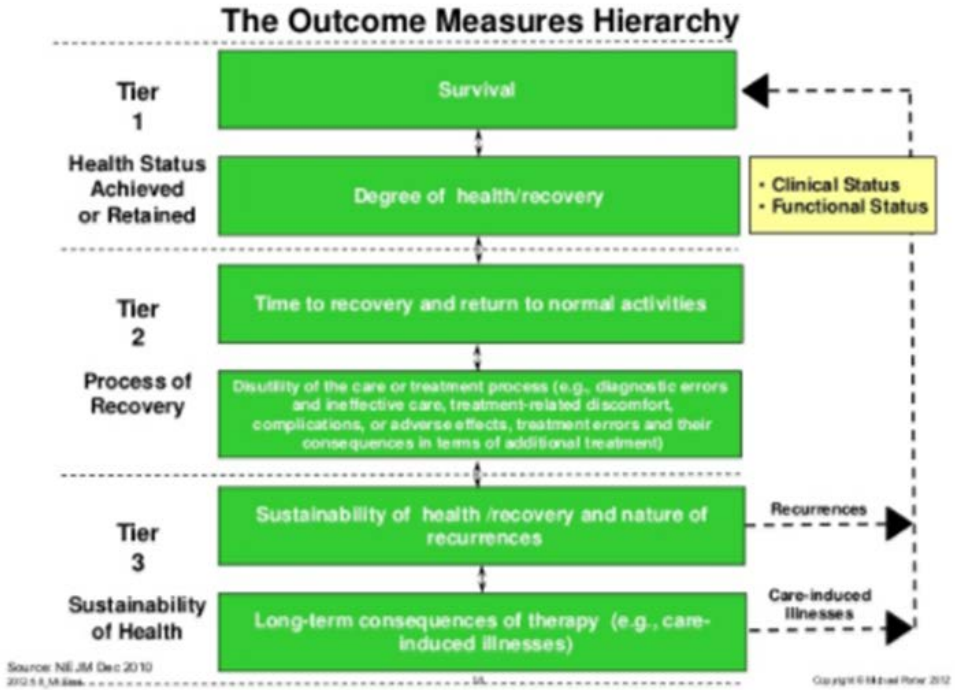
Agenda

- Systems challenges
- Development challenges
- Market access challenges
- Some ways forward

Redefining health care: recent trends



Source: Michael Porter, Value of Health Care

Current trends in healthcare and challenges for biomarkers

Current trends	Biomarkers development
Outcomes	
• Focus on outcomes that matter to the patients: survival, degree of health	• Focused on surrogate endpoints/intermediary outcomes
Value demonstration	
• Need to demonstrate the impact and effectiveness	• Patients benefit from pathway improvement, not from the test
Data	
• Adoption of VBHC incentivises the harmonisation of data and processes	• Need for custom data domains – data managed separately in trials
Evidence standards	
• Strong need for evidence base and clinical utility measure	• Quicker discovery pace, lack of standards to qualify novel biomarkers

Challenges for biomarker companies - development

- Funding and facilities
  - Bootstrapping and working with grants vs. large company investment/VC funding?
- Access to validated, clinical samples with outcomes
  - Heterogeneity in population genomics
  - Validation: Real-world evidence
- IP acquisition
  - Balance academic publication vs. R&D & validation investment

Based on Oxford Cancer Biomarkers insights

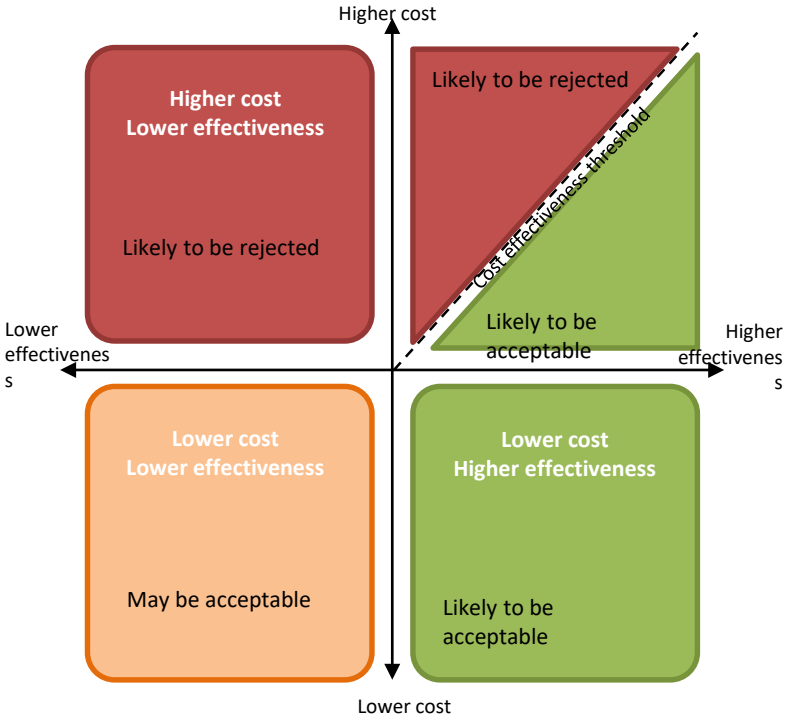
The journey to market is designed for medicines

	Review of…	Flow of information	Biomarkers
Regulatory	• Safety • Manufacturing and clinical production standards	Is there a need? Is the product deemed safe in this indication?	• No separate framework for qualification of novel technologies unless medical device/medicine
Scientific advice	• Clinical performance	Should it be included in treatment guidelines?	• Evidence standards to be defined
Pharmaco-economics	• Correlation between clinical performance and potential price	Should we pay for this?	• Need whole of pathway approach – see next slide
Pricing and reimbursement	• Price negotiation with manufacturers	Any price adjustment?	• Unclear and challenging timelines for reimbursement frameworks
Purchasing and payment	• Medicines procured as efficiently as possible (tenders) and providers are reimbursed	Nominated tariffs and usage monitoring	• Effort needed to connect clinical diagnostic labs to where outcomes benefits can be seen

Demonstrating the value of tests: NICE approach

- META-Tool was developed as a light version of the NICE scientific advice suited to medtech companies
- Process includes a review of :
  - Product information
  - Regulatory and HTA requirements
  - Questions for economic evaluation
  - Value proposition
  - Clinical treatment pathway
  - PICO statement
  - Measuring clinical effectiveness
  - Economic data collection
  - Funding and commissioning
  - Adoption and impact

NICE Cost effectiveness framework



Adapted from NICE

Some thoughts for the future

- Strengthen collaboration with **providers** to access clinical samples – public and private
- Use **accelerators and academic incubators**, invite them and break silo between pharma and start ups
- Ensure early adoption by aligning with current practice first and focus on **pathway integration**
- Continued regulatory harmonisation efforts +++
- Start with **key opinion leaders**, engage in early dialogue with HTA agencies
- Find support to develop systems perspective **economic analysis** of benefit of test

Annex: Categories of biomarkers

- **Susceptibility/Risk biomarker** - A biomarker that indicates the risk for developing a disease or sensitivity to an exposure in an individual without clinically apparent disease.
- **Diagnostic biomarker** - A biomarker used to identify individuals with the disease or condition of interest or to define a subset of the disease.
- **Monitoring biomarker** - A biomarker used to detect a change, over time, in the degree or extent of disease, safety indicator, or exposure.
- **Prognostic biomarker** - A biomarker used to identify likelihood of a clinical event, disease recurrence or progression.
- **Predictive biomarker** - A biomarker used to identify individuals who are likely to experience a favorable or unfavorable effect from a specific intervention or exposure.
- **Pharmacodynamic biomarker** - A biomarker used to show that a biological response has occurred in an individual who has received an intervention or exposure.
- **Safety biomarker** - A biomarker used to monitor toxicity.