

EMA-HTA Workshop
Parallel Advice in Scientific Drug Development

The HTA View
Professor David Barnett

The HTA/Regulatory divide?

Regulatory perspective

Efficacy
Safety

HTA perspective

Clinical effectiveness
Cost effectiveness

The same evidence can lead to different decisions

HTA

Core Principles

Obtaining value for money in the delivery of healthcare

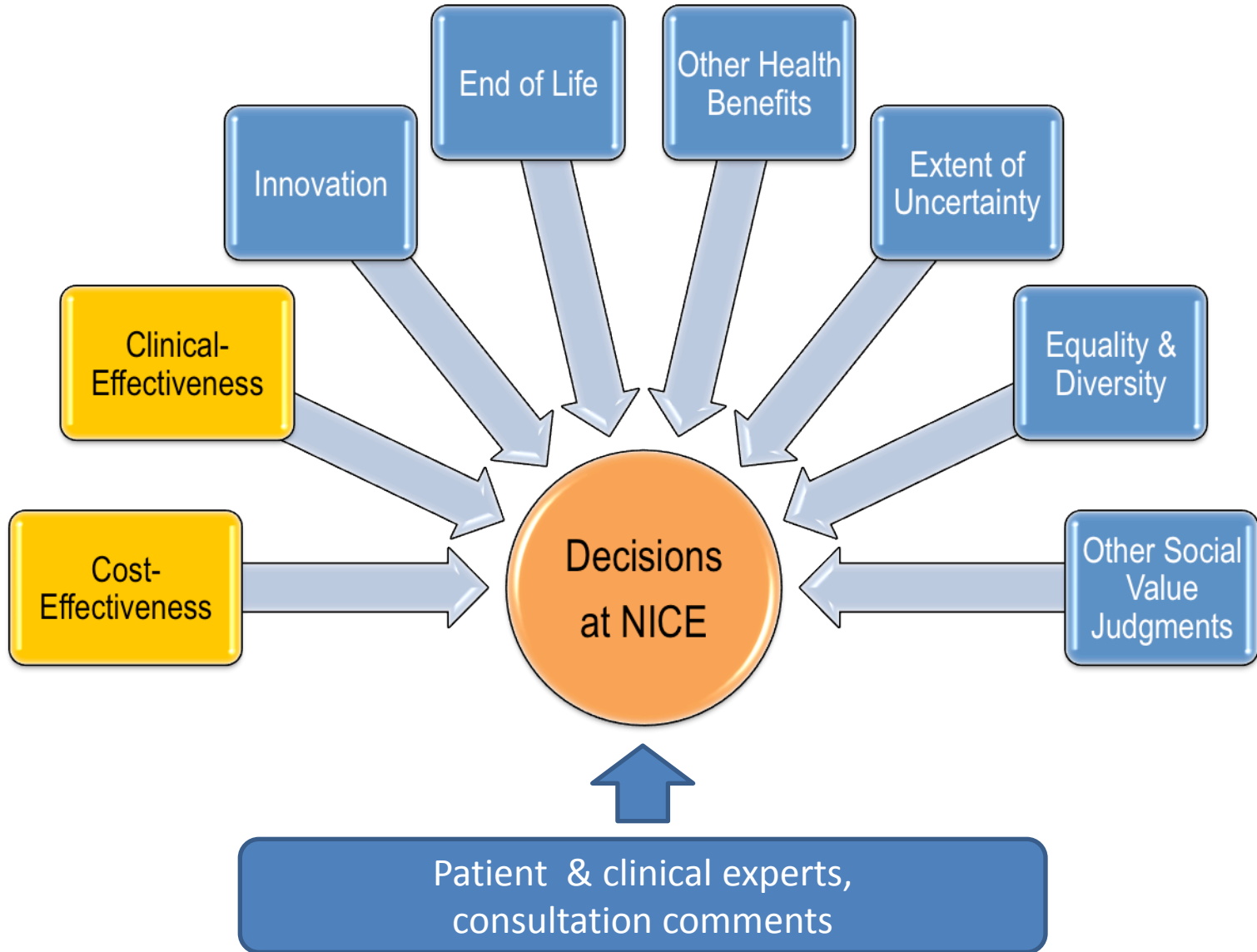


Working within a defined budget
Central Government or Private Payer



Taking into account opportunity costs
What will be displaced





The Evidence for HTA

- Clinical effectiveness
 - Comparators
 - Placebo
 - Standard of Care (SOC)
 - Licensing status
- Outcomes
 - Survival
 - Quality of Life (patients & carers?)
 - Relevance to patients
 - Surrogates
 - Long term (lifetime benefits)
- Cost effectiveness
 - Full pathway of care for drug use
 - Patient access schemes/discounts
 - Relevance to 'jurisdiction'

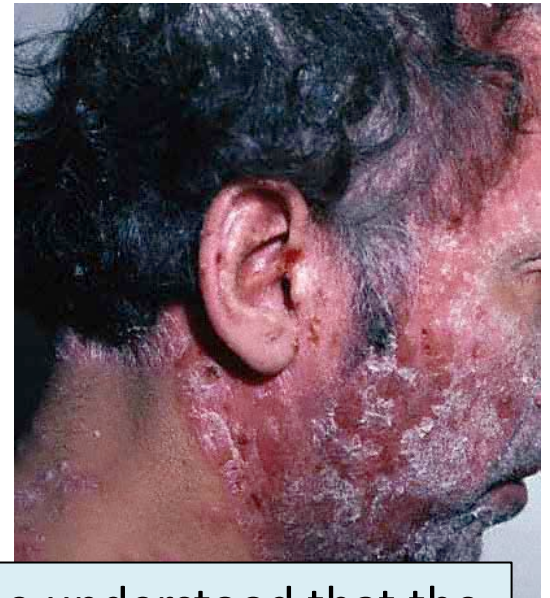
All evidence is important:

- RCTs
- Observational studies
- Registries
- Case series
- Clinical opinion
- Real life experience
- Patient reported outcomes

Psoriasis – Anti-TNFs

Impact of Psoriasis on QoL

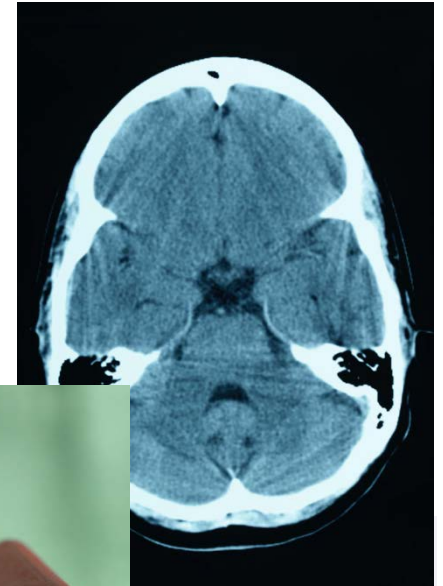
- Clinical trials use extent of psoriasis measured by PASI score (psoriasis area and severity index) as a proxy for effects on quality of life.
- Patients identified that the location (e.g. face, palms, perineum) was most significant on QoL irrespective of total area covered.



“Appraisal Committee understood that the effect of psoriasis on patients’ quality of life is related both to the degree of skin involvement and to the body sites affected” (PASI & DLQI)

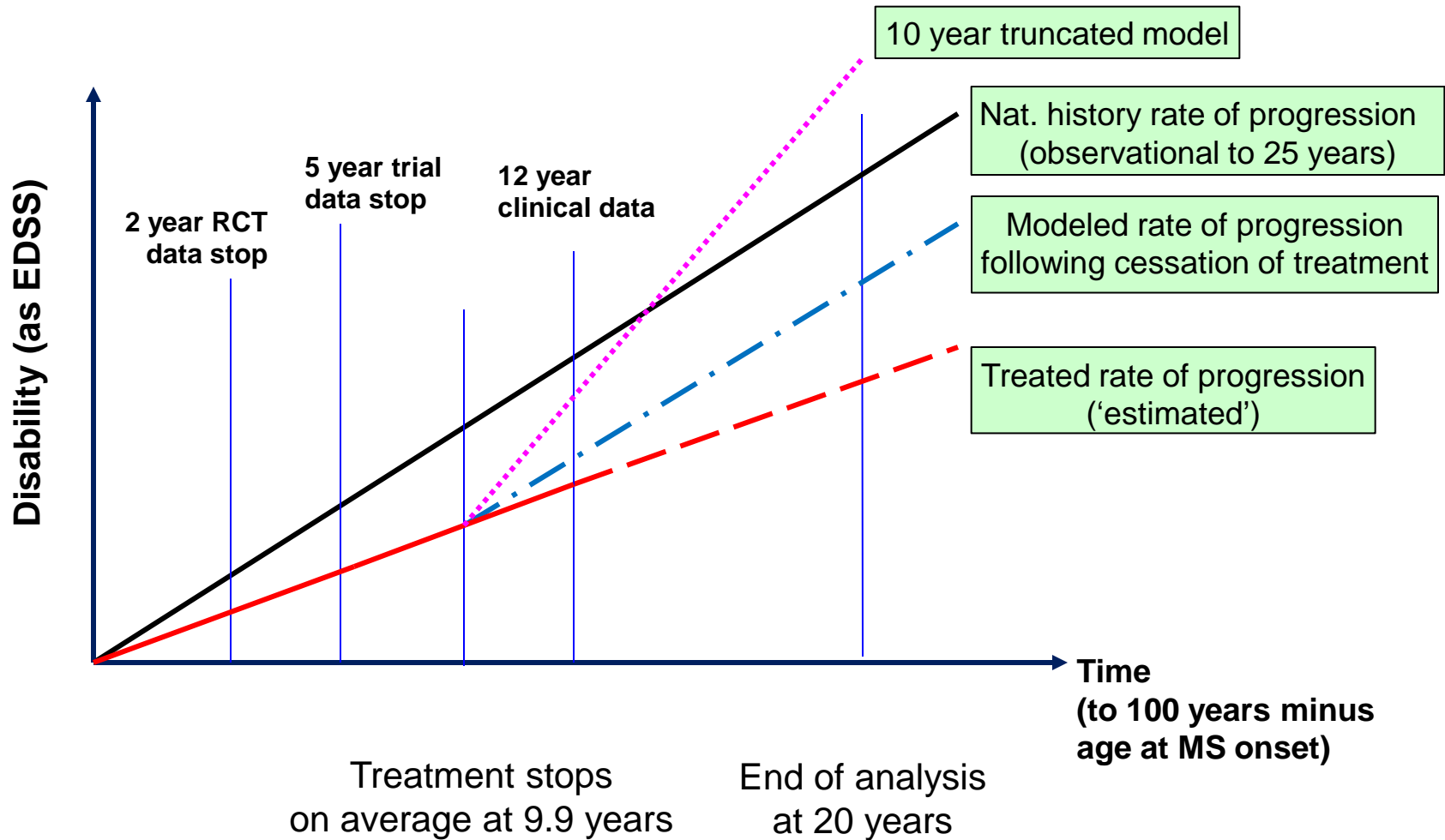
Beta-interferon for multiple sclerosis

- The appraisal
 - 2 years
 - 2 appeals
- Patient reported outcomes
 - Flares & remissions
 - Disability long term
 - Cognition
- Cost effectiveness modeling
 - extrapolation of trial data to long term effects on disability



Beta-interferon for multiple sclerosis

What Happens in the Model - Extrapolation



Lucentis - ranibizumab (VEGF inhibitor)

Age-related macular degeneration

- Quality of life
 - Monocular versus binocular vision
- Cost effectiveness
 - One eye versus two eyes
 - Costs of blindness
 - Societal benefits
- Patient access scheme

Monocular versus binocular vision

Evidence suggested that loss of sight in one eye impacts little on quality of life.

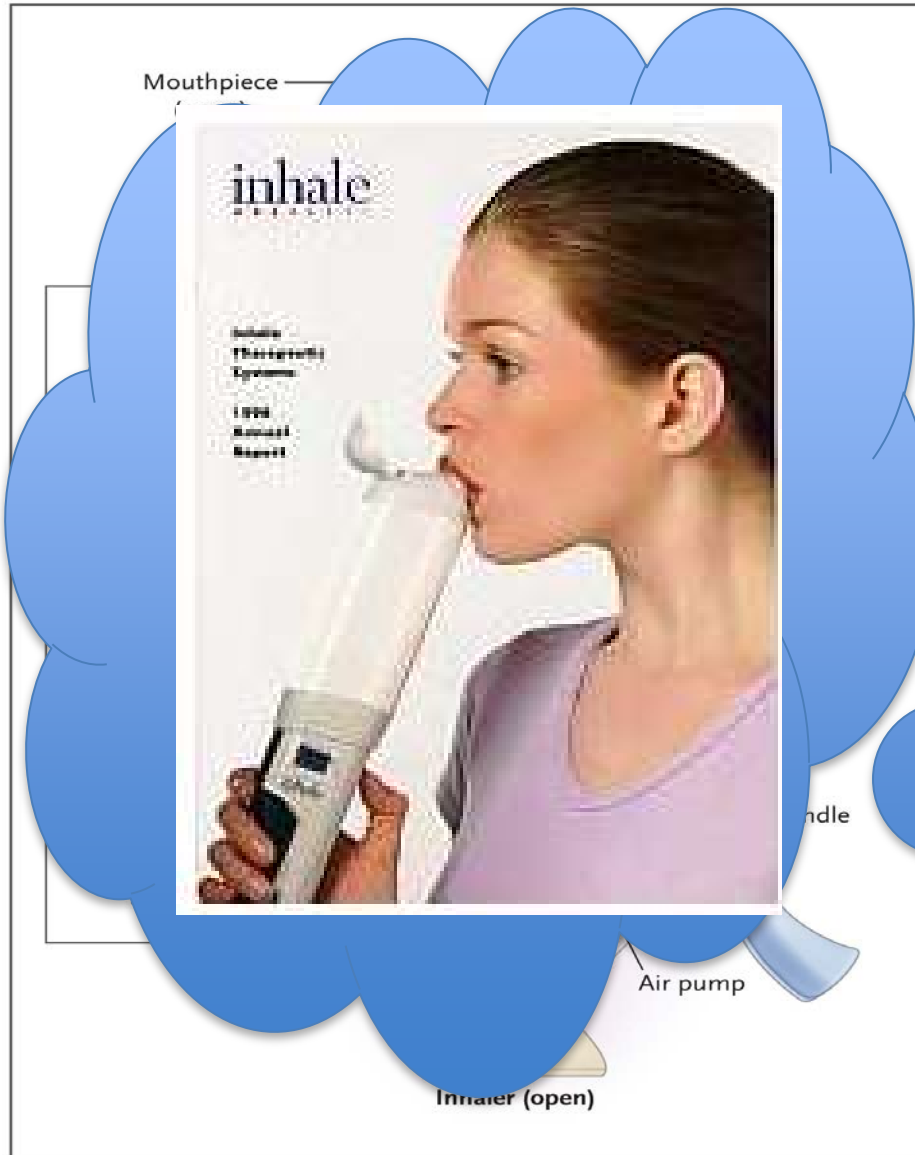
Patient organisation, patients and carers clearly indicated that there were significant negative effects of loss of binocular vision on daily activities and quality of life.

enough to get
NHS drug'

iving drug

Inhaled Insulin

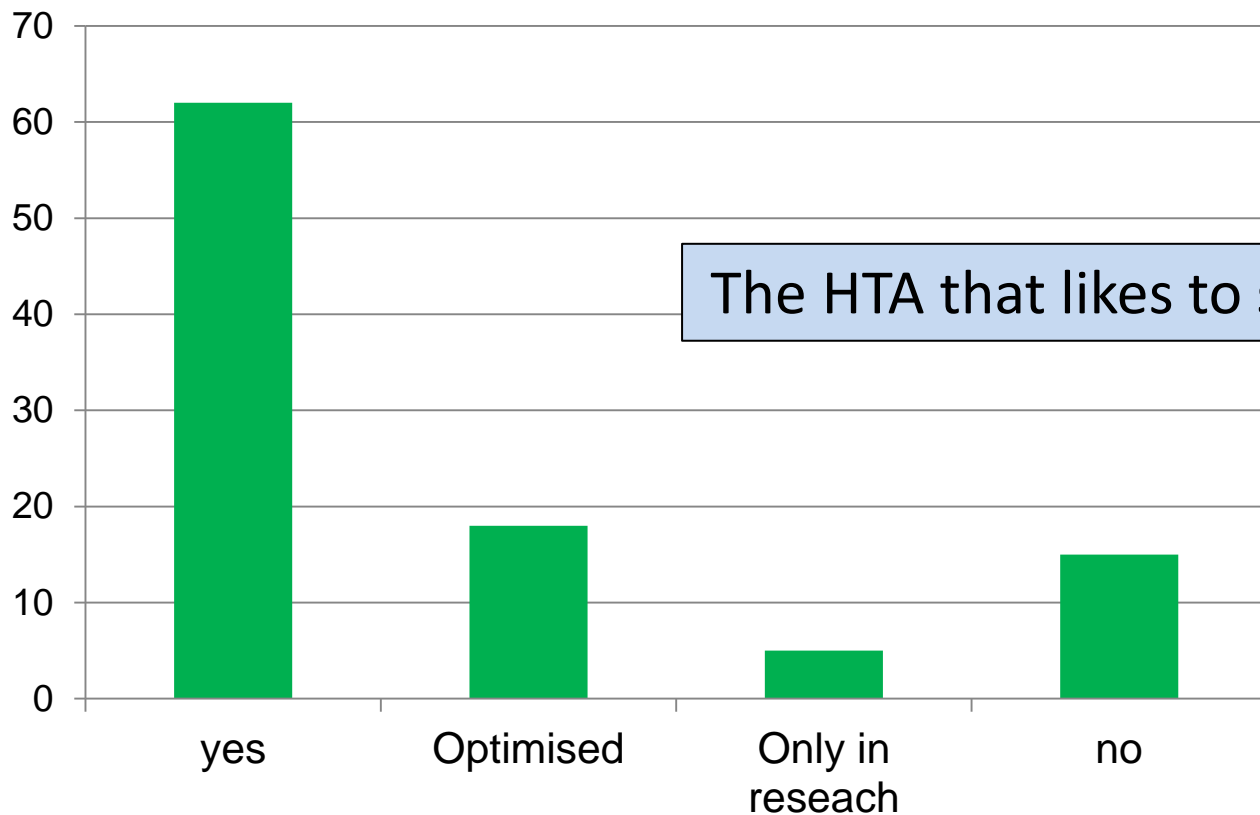
'Innovation' for Diabetes?



- Innovation
- Dosing variation
- Costs and benefits
- Needle phobia
- Patient choice
- Convenience/portability?

NICE Technology Appraisal Recommendations

291 appraisals published to June 2013



The HTA/Regulatory divide?

Regulatory perspective

HTA perspective

Not a divide but a continuum
of evidence development

Eff

Safety

ctiveness

Cost effectiveness

Parallel advice on evidence development at an early stage should reduce the likelihood of different decisions and provide a better pathway from laboratory to market for new medicines as well as the provision of 'value for money' in healthcare delivery