

# Is Delayed Access Due to Issues of HTA Capacity a Necessary Reality or an Unacceptable Problem?

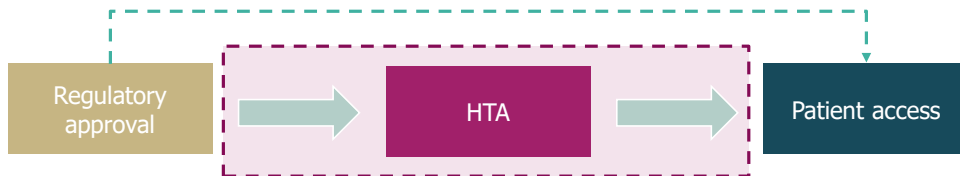
ISPOR Europe 2019 – Issue Panel IP2  
Monday 4<sup>th</sup> November, 11:00-12:00

Moderator: Matt Griffiths  
Panellists: Eric Low, Michael Drummond, Lesley Tilson



## HTA as a Delay to Patient Access

Ultimate desire to reduce the gap between regulatory approval and patient access (at 'appropriate' price)



**The HTA process can contribute to delays to patient access**

# HTA Capacity as a Contributor to Delay

Constrained capacity may result in delayed patient access via:



Extended timelines for conducting the assessment (initiation to recommendation), where there is flexibility in assessment duration



Delayed "initiation" of HTA (relative to 'ideal' timelines vs regulatory process), due to lack of capacity to plan into HTA body workflow

Tangible evidence for the role of HTA capacity constraints in delaying access?

The importance of the role of capacity-building when establishing nascent HTA bodies

NICE process review (2017) driven by recognised need to increase capacity

Anecdotal delays to initiation of submission review (e.g. SMC, NCPE)

# HTA Capacity as a Contributor to Delay

Exciting trends in medicine development and HEOR = increased demands on HTA body capacity?

## 1 Multi-indication therapies (e.g. immunotherapies, biologics)

- Pembrolizumab, nivolumab and atezolizumab

22 published NICE appraisals since January 2017

+

Further 14 scheduled appraisal publications between now and July 2021

=

An appraisal decision on one of these immunotherapies every **6-7 weeks over a 4 1/2 year period**

## 2 Increasingly complex assessments and decisions

Earlier regulatory approval



data uncertainty

More complex technologies



cell and gene therapies; combination therapies

## 3 Move towards re-appraisal by HTA bodies on the basis of real-world evidence?

# The Panellists



Eric Low

Patient Perspective



Professor Michael Drummond

Academic Perspective



Dr Lesley Tilson

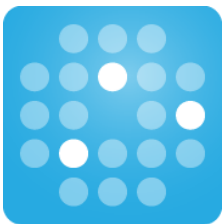
HTA Agency Perspective

- Are HTA capacity issues and the consequent risks of delayed access inevitable given the need for robust, locally-relevant appraisal?
- Are all stakeholders doing all they can to avoid delay by reducing unnecessary duplication, repetition and wastage?



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## Q&A



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Mobile App

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The screenshot shows the mobile app interface with the following elements:

- Navigation Bar:** Back, Schedule, My Schedule, My Favorites, Notifications, Search, and Menu (highlighted with a red box).
- Menu List:** Exhibitors, Sponsors, Favorites, Schedule, Contacts, Notifications, Technical Support, Attendees, User Gateway, and Live Polling (highlighted with a red box).
- Step 1:** A yellow box labeled "Step 1" with an arrow pointing to the Menu icon.
- Step 2:** A yellow box labeled "Step 2" with an arrow pointing to the Live Polling item.
- Step 3:** A yellow box labeled "Step 3 (Select the appropriate session)" with an arrow pointing to a specific poll question.
- Session Details:**
  - Monday, November 4**
  - ISPOR AUSTRIA CHAPTER: USING DIGITAL DATA TRANSFORMATION FOR STRATEGIC PATIENT EMPOWERMENT** (7:00am - 8:15am)
  - PL1 - HEALTHCARE DIGITALIZATION- INSTANT, ON DEMAND, AND ALWAYS CONNECTED** (8:30am - 10:30am)
  - Evaluating Digital Health Technologies: Why, What And How?** (11:00am - 12:00pm)
  - Is Delayed Access Due To Issues Of Hta Capacity A Necessary Reality Or An Unacceptable Problem?** (11:00am - 12:00pm) (highlighted with a red box)

## Have Your Say!

1. Do HTA capacity issues contribute to delayed access for patients?
  - Yes
  - No

### *Live Content Slide*

*When playing as a slideshow, this slide will display live content*

**Poll: 1. Do HTA capacity issues contribute to delayed access for patients?**

## Have Your Say!

2. Are these delays inevitable given the need for robust, locally-relevant appraisal?
  - Yes
  - No

*Live Content Slide*

*When playing as a slideshow, this slide will display live content*

**Poll: 2. Are these delays inevitable given the need for robust, locally-relevant appraisal?**

## Have Your Say!

### 3. Who is most responsible for contributing to HTA capacity issues?

- HTA bodies
- Industry
- Regulators (e.g. EMA)
- Policy makers
- All equally responsible

#### *Live Content Slide*

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**Poll: 3. Who is most responsible for contributing to HTA capacity issues?**

# Is Delayed Access Due to Issues of HTA Capacity a Necessary Reality or an Unacceptable Problem?

The Patient Perspective  
Eric Low

## Key questions

**Question 1:**

Are HTA capacity issues and the consequent risks of delayed access inevitable given the need for robust, locally-relevant appraisal?

**Answer:**

No

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**Question 2:**

Are all stakeholders doing all they can to avoid delay by reducing unnecessary duplication, repetition and wastage?

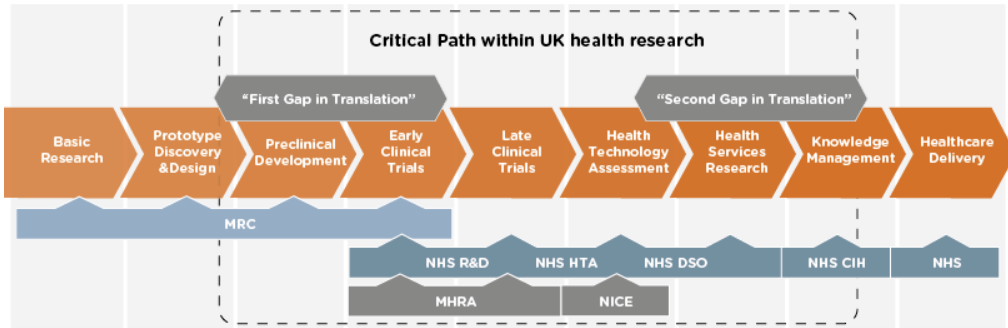
**Answer:**

No





## Potential for system delays



## Impact on patients

- Hope (?)
- Quantity and quality of life (?)
- Anger and frustration (?)
- Depression (?)
- Suicidal thoughts (?)
- Impact on family, carers etc. (?)
- Unwillingness to engage in or with system (?)



## Solutions

- Better horizon scanning
- Early engagement
- Scientific Advice
- Better evidence inputs into HTA
- Redefining patient organisation advocacy efforts
- New methods/processes – less engineered/quicker
- More funding from government – capacity creation
- Charging for appraisals



# Striking a Balance Between Timely and Robust Health Technology Assessments

Michael Drummond  
Centre for Health Economics  
University of York

## Outline of Presentation

- What do we mean by 'timely' and 'robust'?
- Does HTA lead to delays in patient access?
- What could be done to speed up HTA?
- What should *not* be cut in the interests of timeliness?
- Where can we find more resources for HTA?

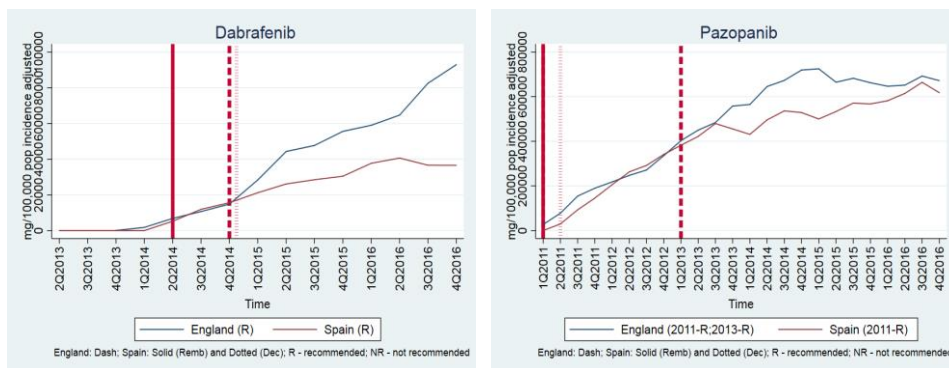
## What do We Mean by 'Timely' and 'Robust'?

- *Timely:*
  - the process of conducting the HTA should not delay patient access to clinically and cost-effective therapies
- *Robust:*
  - the HTA should produce reliable and relevant analyses that are useful to decision-makers and other stakeholders

# Does HTA Lead to Delays to Patient Access?

- No denying that HTA, if performed thoroughly, can take a substantial amount of time:
  - NICE Technology Appraisals in England can take between 290 and 350 days, depending on whether an Appraisal Consultative Document is issued as a preliminary stage or not
  - if an appeal is made, the process can take longer
- However, the extent of any delay depends on:
  - whether the HTA is formally linked to decisions on reimbursement and price
  - whether patients are allowed access to the drug, pending conclusion of the HTA

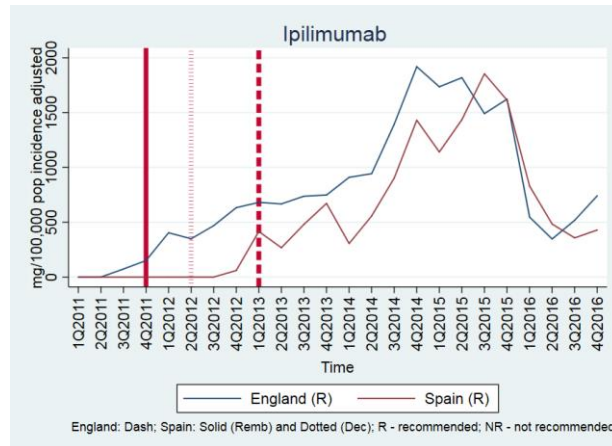
## Medicines Utilization Before and After Positive HTA Recommendations (1)



### Legend

- Vertical solid red line: date of Spanish national assessment ( for price and reimbursement)
- Vertical hashed line: date of NIICE recommendation (England)
- Vertical dotted line: date of Genesis recommendation(Spain)

# Medicines Utilization Before and After Positive HTA Recommendations (2)



## Key Steps in HTA

- Setting priorities and scoping assessments
- Collecting data and performing analyses
- Formulating recommendations and implementing decisions

*Q: What can be done at each stage to speed up the process without compromising the robustness of the assessments?*

## Setting Priorities and Scoping Assessments

- Good 'horizon scanning' means the HTA scoping process can start early (eg when the manufacturer files with the EMA)
- Setting priorities for HTA can ensure that the key HTAs are expedited
- Not all new medicines need a full HTA; a decision-making pathway can determine those medicines that can be fast-tracked, or subjected to limited HTA

## Collecting Data and Performing Analyses

- Much depends on whether the HTA agency is performing its own assessments or critically appraising company submissions
- Some HTA agencies outsource one or both of these activities
- Major resource savings would be possible if HTA agencies could use a common systematic review/network meta-analysis, or a standard, publically available, decision-analytic model for the disease in question
- Much can also be learned from earlier HTAs conducted elsewhere (eg ICER in the US)

# Formulating Recommendations and Implementing Decisions

- Although all HTAs formulate recommendations, the link to reimbursement and pricing decisions differs by jurisdiction
- Why have two committees (ie reimbursement/listing, followed by pricing) when you can have one?
- Why have multiple submissions/decisions for the same drug/indication when you can have one?
- The implications (for patient access) of HTA recommendations should be made as clear as possible (eg full implementation of positive recommendations within 3 months)
- There should be more monitoring of the implementation of HTA recommendations
- Outcomes-based managed entry agreements could be used to allow patient access in situations where there is uncertainty surrounding cost-effectiveness (eg Cancer Drugs Fund in the UK)

## What Would I *Not* Cut in the Interests of Timeliness?

- The time to undertake thorough economic evaluations:
  - the major potential resource savings are in the systematic review/ NMA, through more inter-agency collaboration (eg EU initiatives)
- The time to allow adequate stakeholder involvement;
  - patient organizations need the time and resource to make an adequate input to the process
- The involvement of independent expert committees
  - these committees add more credibility and trust to the process

# Where Would I Look for More Resources?

- Charging fees for HTA
  - has its problems, but ensures that the level of resourcing keeps pace with the expanded workload
- More Use of Volunteer Labour
  - some HTA agencies already rely heavily on the time of unpaid academic experts
- More outsourcing of tasks
  - this offers more flexibility when there are spikes in the workload; can add to credibility also

*ISPOR Copenhagen, 4<sup>th</sup> November 2019*



## The HTA Agency Perspective

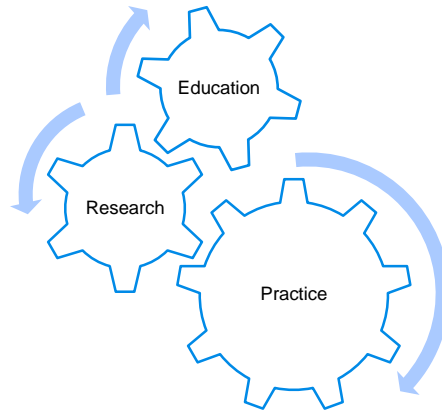
Lesley Tilson BSc (Pharm), Dip Stat, PhD  
National Centre for Pharmacoeconomics



National Centre for  
Pharmacoeconomics  
NCPE Ireland

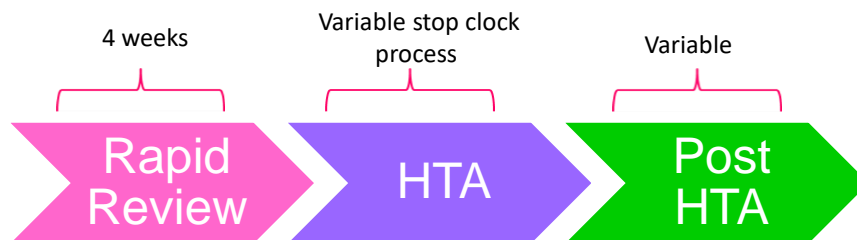
# The National Centre for Pharmacoeconomics in Ireland

The NCPE is an autonomous, independent body whose primary activity is to conduct Health Technology Assessment of all new drugs and also existing drugs for the Irish Health Service within the national Framework Agreement on the Supply and Pricing of Medicines 2016.



Reference: Framework Agreement on the Supply and Pricing of Medicines 2016. Available at: <http://www.hse.ie/eng/about/Who/cpu/IPHAAgreement2016.pdf>

## NCPE Assessment Process



- ALL drugs are subject to an initial rapid review
- The process can start at CHMP positive opinion
- Approximately 50% of drugs subject to a full HTA
- Reimbursement decision and pricing are separate to HTA process
- Post HTA: Assessment of impact of confidential price discount or new clinical evidence.

# Rapid Review and HTA Timelines 2015-2017



(Mean Number of days)

- Time period from MA to RR submission contributed substantially to timeline.
- 15% of companies submitted a RR prior to MA being granted.
- 51% of RR submissions did not require a full HTA.
- One third of time to complete HTAs was spent with the company.

**Abbreviations:** MA: Marketing Authorisation, RR: Rapid Review, HTA: Health Technology Assessment

**Reference:** Connolly E, O'Donnell H, Lamrock F et al. Health Technology Assessment of Drugs in Ireland: An Analysis of Timelines. Pharmacoeconomics Open 2019. <https://rdcu.be/bRkZw>.

# What Key Factors Contribute to HTA Timelines?

Factors	Key Issues
<b>Timing of Submissions</b>	Variable time from MA to HTA submission. At what stage are submissions accepted?
<b>Volume of Submissions</b>	Predictability around submission dates? Prioritisation – Are all new drugs assessed?
<b>Complexity of Submissions</b>	Early regulatory approval; limited evidence at time of assessment Discrepancy between regulatory and HTA evidence requirements Complex methodology required to handle this
<b>Limited resources</b>	Capacity / staffing Required expertise



# What steps can HTA bodies take to improve timelines?

1. Streamline processes
  - Prioritise and standardise
2. Build capacity
  - Highly trained staff required
  - Research, teaching, training
3. Collaborate
  - Exchange knowledge
  - Reduce duplication

**How can we do this?  
What are the benefits and risks?**

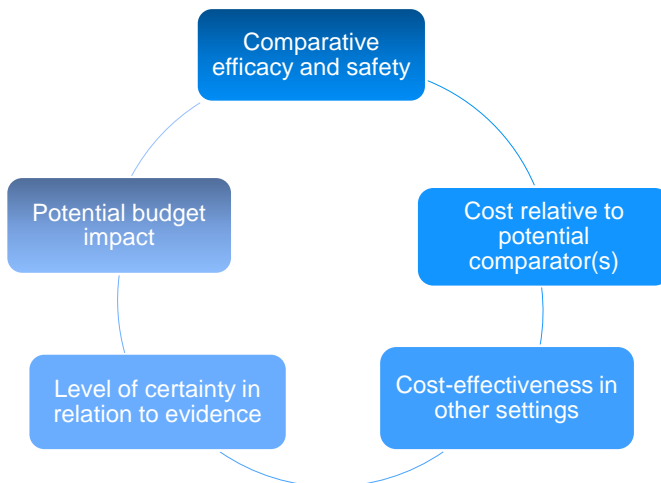


## 1. Streamline Processes

### **Example: NCPE Rapid Review Process**

- Established in 2009.
- All new drugs undergo an initial rapid review.
- Prioritise drugs where a full HTA is needed.
- Approximately 50% of drugs do not require full HTA.

## NCPE Rapid Review Criteria



## Standard Processes for HTAs

- Patient Organisation Submission Process
- Pre-submission meeting with companies
- Standard submission and appraisal templates
- Standard budget impact model
- Continued NCPE-led communications with companies during the evaluation process
- Regular pro-active communication with stakeholders
  - Operational meetings with industry
  - Annual symposium

## 2. Build Capacity

- Broad skill mix required
- Partnership with academia
  - Funded PhD programme
  - Subject matter experts to support staff
  - Promote culture of research and learning
- Charge fee for assessments?
- Outsource / contract staff ?

## 3. Collaborate

- Academic partnerships
- Knowledge exchange with other HTA bodies
- International collaborations
  - Potential to reduce duplication
  - Joint Relative Effectiveness Assessments
  - Joint economic models



## Benefits and Risks

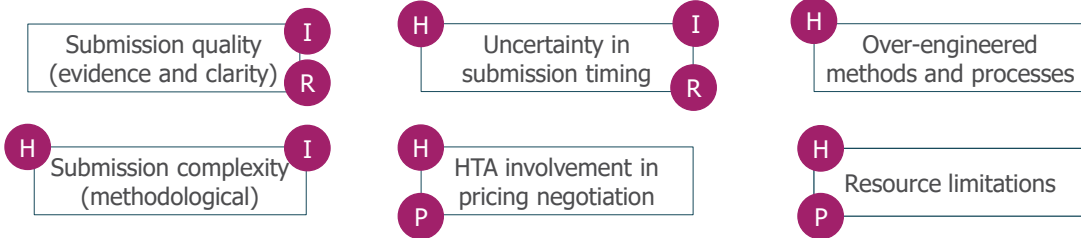
	Benefits	Risks
<b>Streamline processes:</b> Early application process	Timely assessment	Limited data at time of assessment
Standard templates	Consistency and efficiency	Less flexibility
<b>Build capacity:</b> Academic partnership	Attracts high calibre staff Research, teaching, training	Limited time to focus on research component
PhD programme	Promotes staff development and retention Therapy area experts	Long term solution; does not address immediate requirements
Outsourcing / contracting	Provides flexibility	Governance/ quality of outputs
Charging fees for HTAs	Additional resource for outsourcing/contract work	Maintenance of independence
<b>Collaborate:</b>	Knowledge exchange Reduce duplication	Limited resource/time Rigour/ quality

## Summary & Discussion

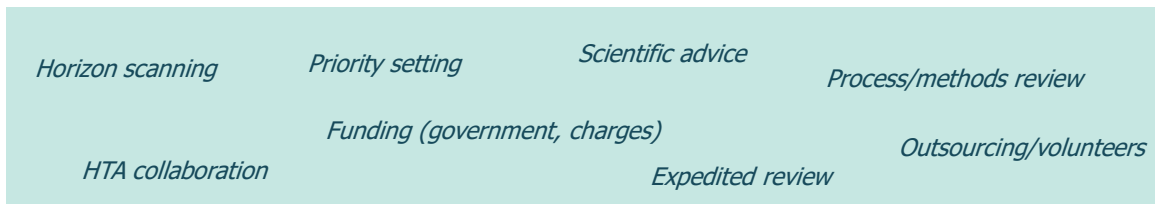
# Summary



## Pressures on HTA Capacity

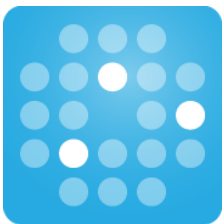


## Solutions?



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## Q&A



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### *Live Content Slide*

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## Social Q&A

### Have Your Say!

4. Do HTA capacity issues contribute to delayed access for patients?
  - Yes
  - No

*Live Content Slide*

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**Poll: 4. Do HTA capacity issues contribute to delayed access for patients?**

**Have Your Say!**

5. Are these delays inevitable given the need for robust, locally-relevant appraisal?
- Yes
  - No

*Live Content Slide*

*When playing as a slideshow, this slide will display live content*

**Poll: 5. Are these delays inevitable given the need for robust, locally-relevant appraisal?**

**Have Your Say!**

**6. Who is most responsible for contributing to HTA capacity issues?**

- HTA bodies
- Industry
- Regulators (e.g. EMA)
- Policy makers
- All equally responsible

*Live Content Slide*

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**Poll: 6. Who is most responsible for contributing to HTA capacity issues?**

Thank you!