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Speakers

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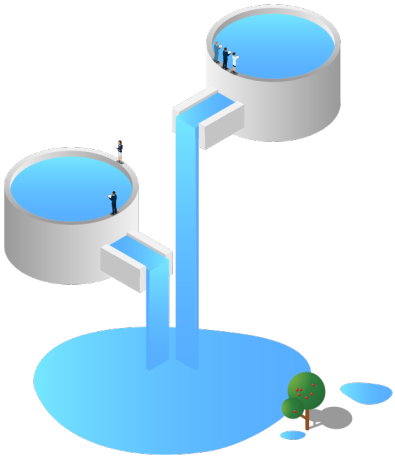
Transition from Volume to Value



Volume

Value

Source: https://healthinformatics.uic.edu/blog/shift-from-volume-based-care-to-value-based-care/

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Value of Medicines

Source: https://www.pfizer.com/files/health/Pfizer_Value_of_Medicines_Brochure_FINAL_January_2017.pdf

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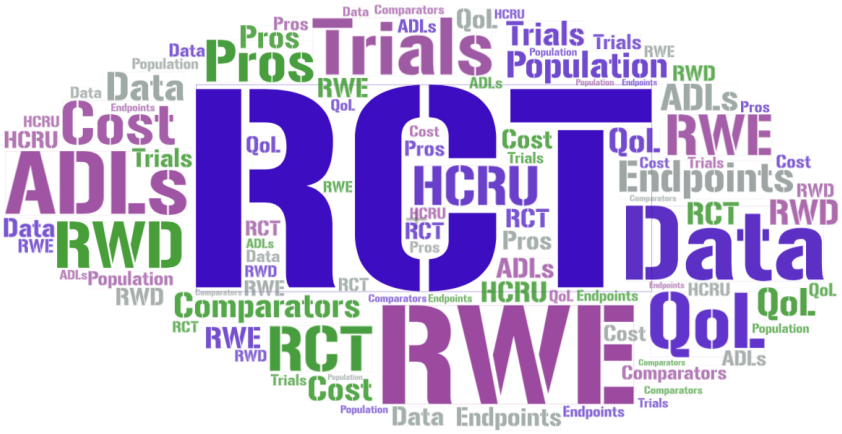
Evidence Sources and Types



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Evidence Uses

Regulators | Payers

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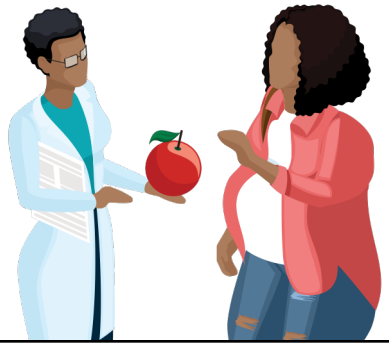
Evidence Uses

1 Quality → 2 Safety → 3 Efficacy → 4 Access → 5 Affordability

Regulators Payers



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FDA's Program for Parallel Review of Medical Devices

October 18, 2016: The Food and Drug Administration (FDA) and the Centers for Medicare & Medicaid Services (CMS) (the Agencies) are informing the public that the Parallel Review of medical devices pilot program will be fully implemented and extended indefinitely.

"…the feedback from both Agencies at the pivotal clinical trial design stage can assist manufacturers in designing pivotal trials that can answer both Agencies' evidentiary questions…"

"… concurrent review by the Agencies of clinical evidence can reduce the time from FDA premarket approval or the granting of a de novo request to an NCD."

Source: https://www.regulations.gov/document?D=FDA-2010-N-0308-0047

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What's next… in bringing payers to the table in the U.S.?

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Early Dialogue on Outcomes with Payers and HTA groups

ISPOR 2020 Virtual Annual Meeting

Sean Tunis, MD, MSc | 20 May 2020

CENTER FOR MEDICAL TECHNOLOGY POLICY

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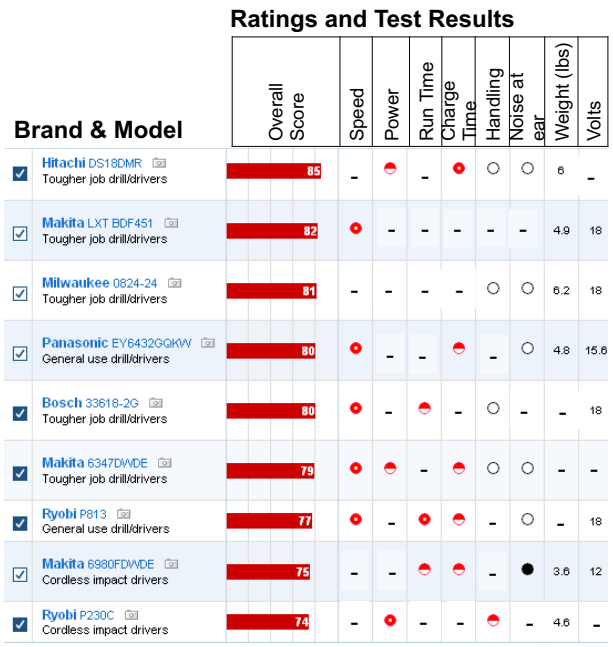
Value is Primarily About Outcomes

- Health outcomes achieved per dollar spent
  - IOM 2006
- Health outcomes are inherently condition specific and multi-dimensional
  - Michael Porter, NEJM, 2010

Ratings and Test Results

Brand & Model	Overall Score	Speed	Power	Run Time	Charge Time	Handling	Noise at ear	Weight (lbs)	Volts
Hitachi DS18DMR Tougher job drill/drivers	85	●	◓	●	●	○	○	6	18
Makita LXT BDF451 Tougher job drill/drivers	82	●	◓	◓	●	◓	○	4.9	18
Milwaukee 0824-24 Tougher job drill/drivers	81	●	◓	●	◓	○	○	6.2	18
Panasonic EY6432GQKW General use drill/drivers	80	●	○	●	◓	◓	○	4.8	15.6
Bosch 33618-2G Tougher job drill/drivers	80	●	●	◓	◓	○	○	5.9	18
Makita 6347DWDE Tougher job drill/drivers	79	●	◓	●	◓	○	○	5.4	18
Ryobi P813 General use drill/drivers	77	●	○	●	◓	○	○	4.8	18
Makita 6980FDWDE Cordless impact drivers	75	◓	●	◓	◓	●	●	3.6	12
Ryobi P230C Cordless impact drivers	74	◓	●	○	◓	◓	●	4.6	18



Core Outcome Sets

- "An agreed standardised set of outcomes that should be measured and reported, as a minimum, in all clinical research in specific areas of health or health care"
  - Definition from the COMET Initiative
  - comet-initiative.org

coreHEM

A Core Outcome Set for Gene Therapy in Hemophilia

coreHEM | Green Park Collaborative — A partnership for innovation and effectiveness | National Hemophilia Foundation — for all bleeding disorders | McMaster University

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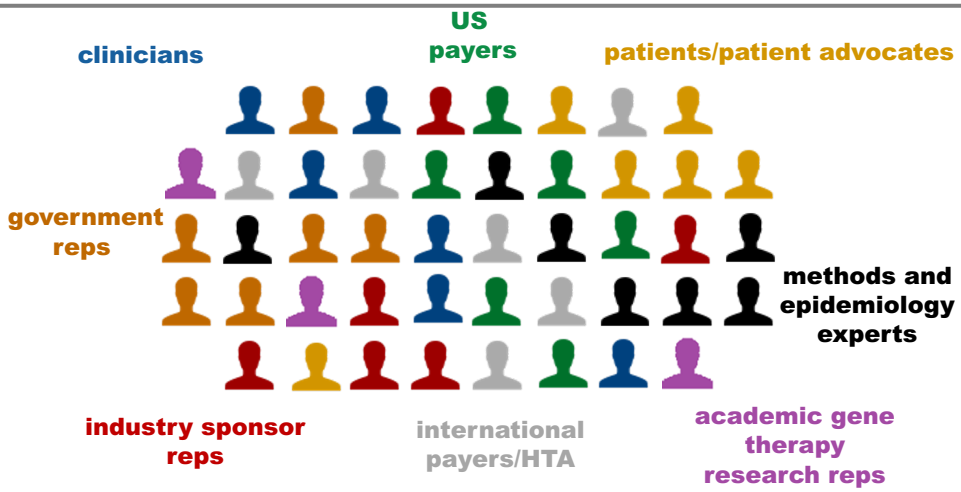
coreHEM Stakeholders



coreHEM

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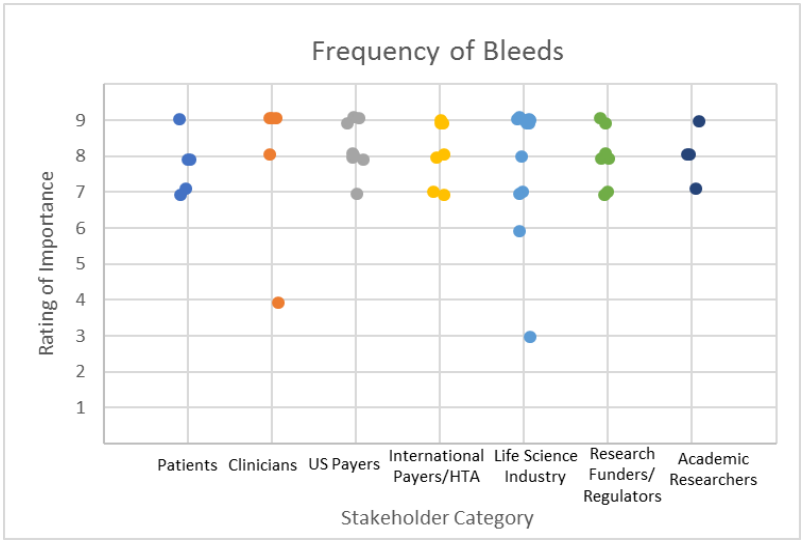
EXAMPLE OF OUTCOME WITH HIGH CONSENSUS



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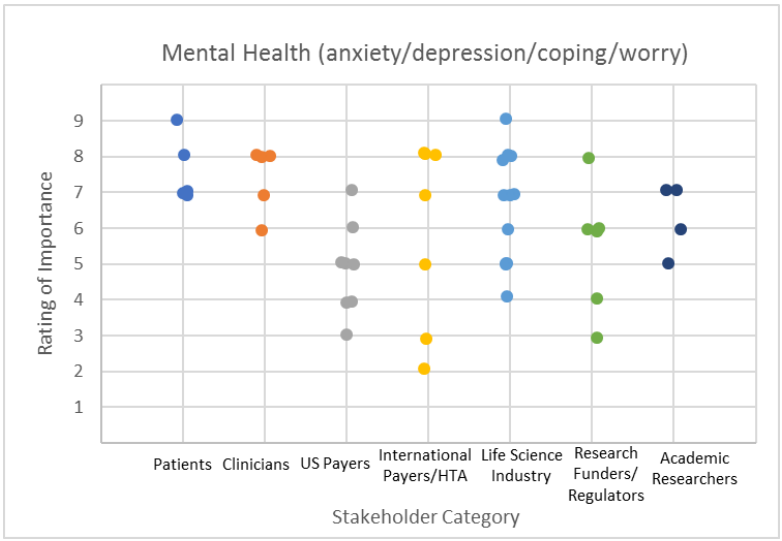
EXAMPLE OF OUTCOME RETAINED DUE TO PATIENT IMPORTANCE



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“Early Advice” in the US

- Difficult but possible to engage payers/HTA in consensus work
- Harder to engage them in early advice with single company
- Multi-stakeholder process educates all participants
- Discussion about meaningful outcomes ideally precedes availability of products
- Work is most impactful when it can influence pivotal trials

IP14: FORMAL PAYER INPUT INTO MEDICINE DEVELOPMENT IN THE US- SHOULD WE AND CAN WE BRING PAYERS TO THE TABLE?

Breakout Session 10
Virtual ISPOR 2020
20 May 2020

Michelle Mujoomdar, PhD
Director, Scientific Affairs

CADTH

Disclosure

- CADTH is funded by contributions from the Canadian federal, provincial, and territorial ministries of health, with the exception of Quebec.
- CADTH receives application fees from the pharmaceutical industry for:
  - CADTH Pharmaceutical Reviews, including Common Drug Review, pan-Canadian Oncology Drug Review, and Interim Plasma Protein Product Review
  - CADTH Scientific Advice

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Disclosure - Individual (2 years)

- Employed by CADTH
- Board of Directors (Director) – International Network of Agencies for Health Technology Assessment (INAHTA): June 2018 – present
- Engaged as an individual external expert:
  - European Commission: May 2018 – Aug 2018
  - Zorginstituut Nederland (April 2018 – May 2018)
- Advisory roles for several IMI projects
  - PREFER (travel expenses paid by University of Uppsala)
  - PARADIGM (travel expenses paid by HTAi and European Patients' Forum)
  - EHDEN (coordinated through Erasmus University)
- Other travel expenses paid by CIRS (Sept 2018) and CIHR grants on which CADTH is a knowledge user (March 2019 and May 2019)

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Formal Payer Input into Medicine Development

- Scientific Advice / Early Dialogue
  - Regulatory only
  - Multi-regulator
  - HTA only
  - Multi-HTA
  - **Regulatory-HTA**
- Other mechanisms?

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Parallel Regulatory-HTA Advice

- European Medicines Agency (EMA) + HTA advice
  - Pilot project started in 2010 (> 100 procedures)
  - EUnetHTA (Joint Action 2: 2012-2015)
  - Shaping European Early Dialogues (2014-2015)
  - EMA-EUnetHTA Parallel Consultation
- Health Canada/HTA Parallel Scientific Advice

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Parallel Regulatory-HTA Advice

- Purpose:
  - Reducing avoidable uncertainty
  - Optimized/Efficient development plans
- Types of questions:
  - Design elements, population, comparator, endpoints
- Different remits are maintained

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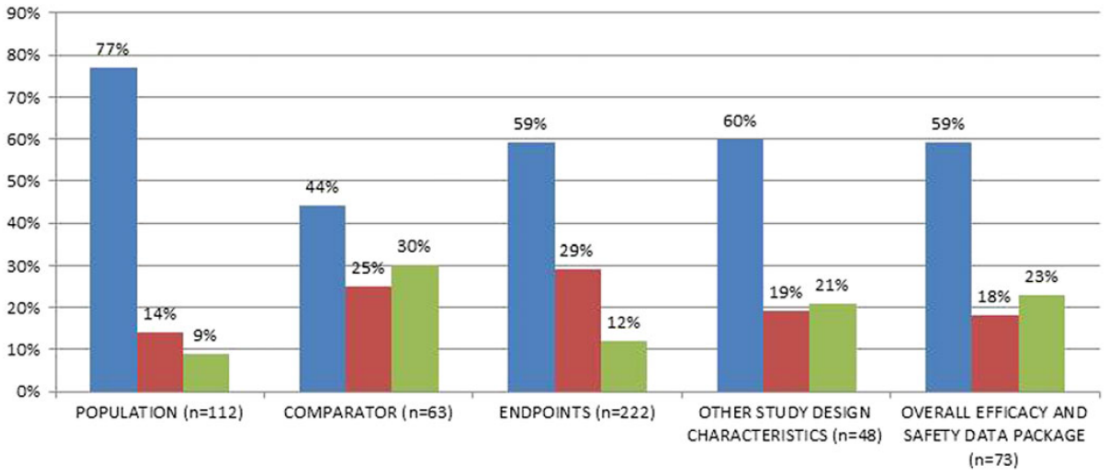
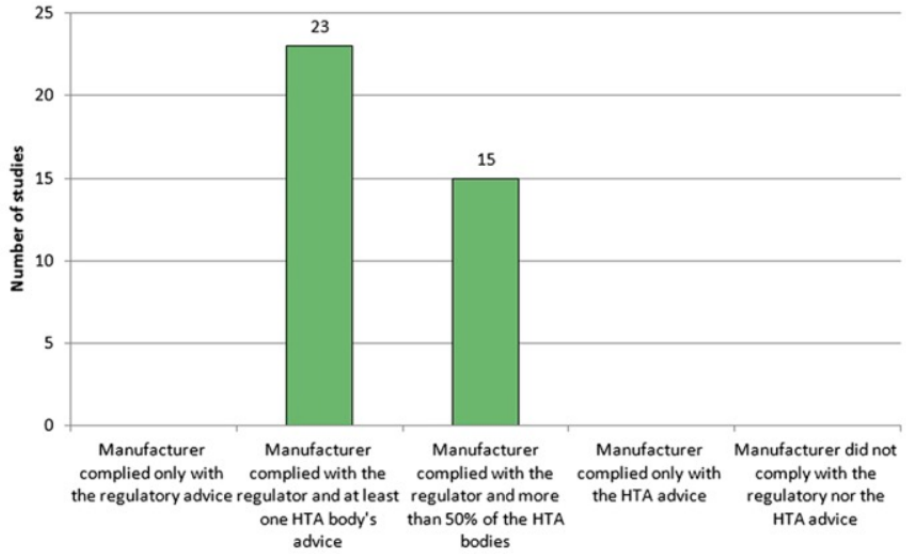
Alignment between EU regulators and HTABs



Tafuri *et al* (2016) Br J Clin Pharmacol. Oct; 82(4): 965–973.

Uptake of Advice – Primary Endpoint



Tafuri *et al*. Br J Clin Pharmacol. 2018 May; 84(5): 1013–1019

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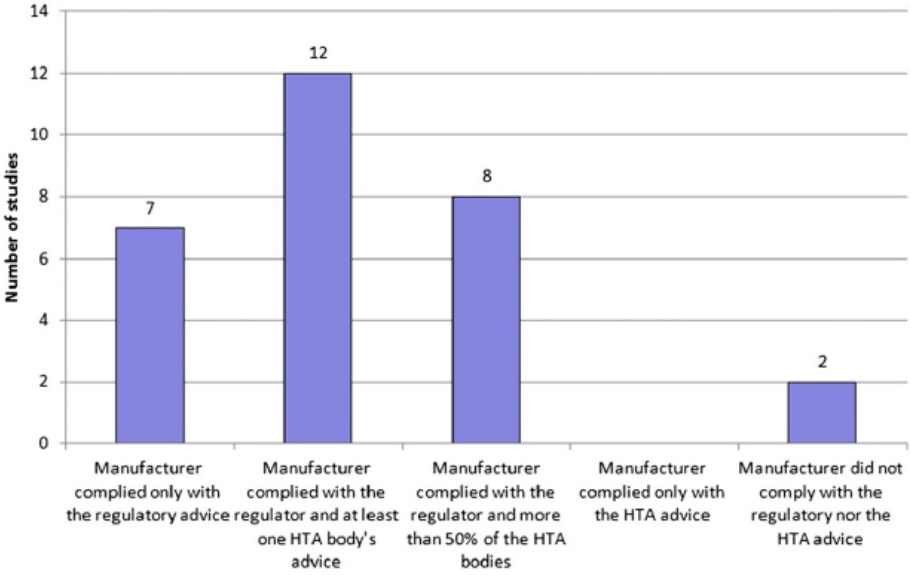
Uptake of Advice - Comparator



Tafuri *et al*. Br J Clin Pharmacol. 2018 May; 84(5): 1013–1019

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Reflections

- Pre-authorization advice is one opportunity for collaboration between regulators
- Post-licensing/Post-launch advice
- Non-product specific discussions
- Multi-stakeholder platforms
  - Core outcome sets
  - Emerging areas
- Collaboration on guidance documents

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CADTH Evidence Driven.

ACMTS Preuves à l'appui.

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Challenges to Access: Bringing Payers to the Table

Cristina Masseria, PhD
Vice President, PHI Vaccines
Pfizer

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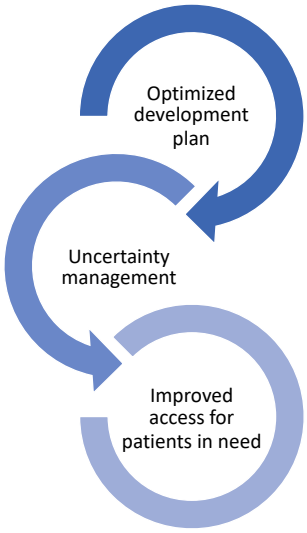
The information and opinions presented in these materials are the express opinion of the author and may not represent the opinions or views of Pfizer Inc. and Pfizer Limited.

Regulators-Payers Parallel scientific Advice – Benefits

- Increased opportunities for **mutual understanding** and **problem-solving ability** between regulators and relevant reimbursement bodies via a structured platform
- Clearly, this facilitates optimal and robust evidence generation for different stakeholders bringing benefits for patient access and public health …..



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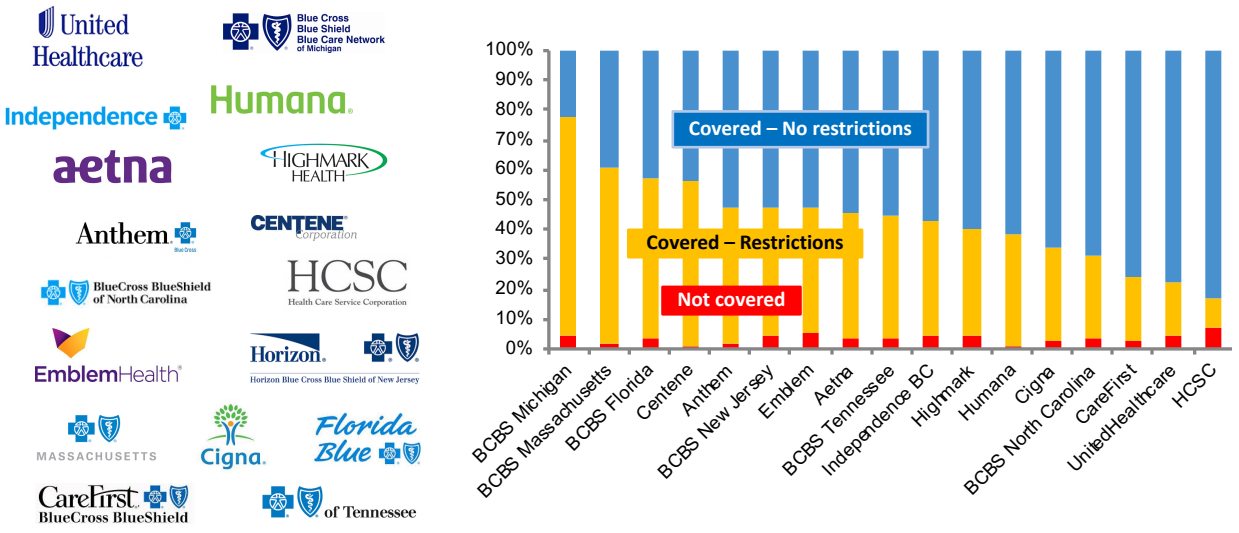
Variation in coverage of specialty drugs among commercial insurers (SPEC database)



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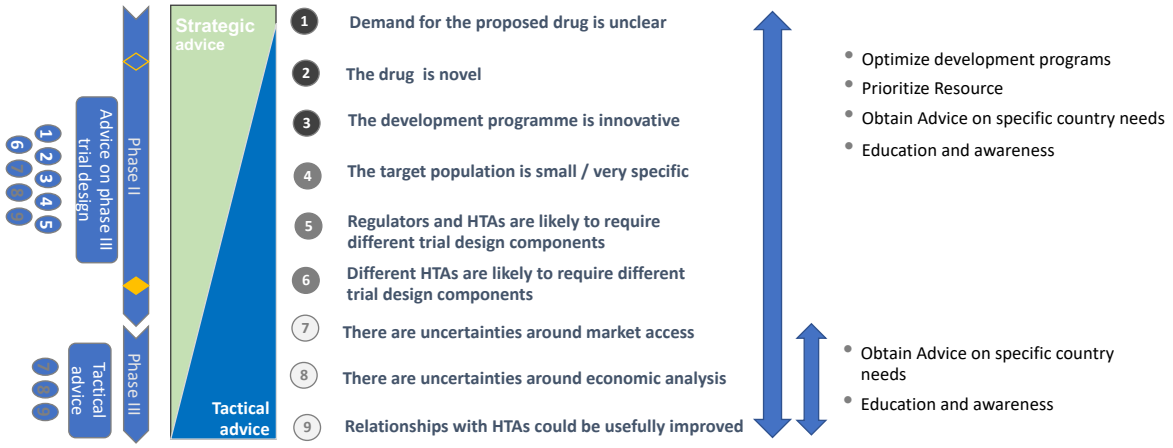
Regulator-Payers Parallel Scientific Advice – Timing

Early dialogue with HTAs should be considered if the access hurdles identified can be resolved through the clinical development programme or evidence development pre-launch



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Elements of Clinical Development Plan Relevant for both Regulators and Payers

Element	Regulator	Payers
POPULATION	• A homogeneous, defined population • With sufficient, quantifiable baseline disease to allow demonstration of a meaningful state but also a meaningful improvement from baseline • With sufficiently stable disease	• Is the patient group appropriate (i.e. reflects the population in whom the intervention is likely to be used in clinical practice? • Are there any subgroups to consider? Are all relevant subgroups prospectively identified or retrospectively identified and analysed?
ENDPOINTS	• Is the primary endpoint clinically meaningful?	• What is the relationship between the primary endpoint and longer term outcomes? • Are relevant endpoints required to adequately profile the expected fluctuations in health-related quality of life included • Is the clinical development plan capturing the most relevant patient journey?
DOSE	• Are appropriate doses being studies adequately to allow determination of the of a marketed dose with most favourable benefit:risk	• Dose regimens that will be allowed by the licence, and how these are anticipated to be used in future clinical practice.
COMPARATOR	• In a therapeutic indication where placebo is deemed ethical, a placebo control would be expected. The need for an active control must be agreed on a case-by-case particularly if important for estimated benefits and risks to be contextualised through comparison to active control or if treatment with placebo is unethical. • Normally the expectation would be for use of gold-standard, EU-licensed, product for the appropriate indication.	• Place in therapy- anticipated positioning of the drug in the treatment pathway and the relevant comparators for each of the anticipated positions. • These may be the gold-standard licenced drug or other drugs (even if not within their labelled indication) if it is used as part of practice norm/treatment guidelines.
TRIAL DESIGN	• Duration of trials should be sufficient for demonstration of long term efficacy and safety and the development plan should adequately support an evaluation of benefit:risk.	• Appropriate trial duration to reduce uncertainty on clinical outcomes for reimbursement decision • Role of RWE

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Challenges

- How to translate the ex-US experience to the US fragmented health care system
- Will regulators and reimbursement bodies give aligned advise or parallel?
  - Can regulators and payers align on the most appropriate use of RWE and PRO?
- Legal implications?

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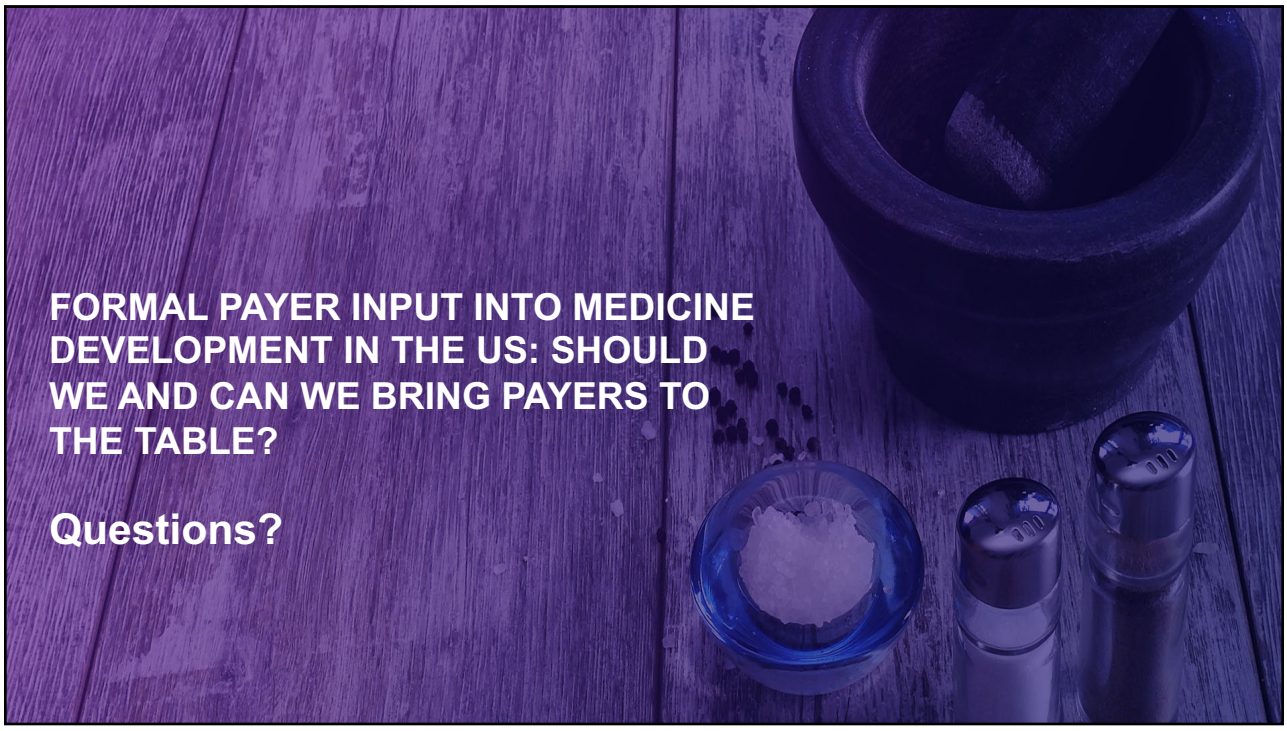

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