ASSESSMENT OF MEDICAL DEVICES: KEY FEATURES AND MAJOR CHALLENGES

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Workshop “How can HTA methods be adapted to meet the special characteristics of medical devices?”
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Premise

➢ Health Technology Assessment (HTA) is undoubtedly the most widespread approach to set priorities and help supporting the allocation of scarce resources in the health care sector
➢ If it originally responded to an ever increasing squeezed healthcare systems’ budgets, HTA has now found a better place in the new value-based healthcare paradigm
➢ Health Technology Assessment has diffused having pharmaceuticals in mind
➢ Assessment of medical devices are more challenging than drugs in several respects*

Challenges in assessing medical devices 1/5

- They are usually needed to support the decision to move from the technology (VTE) of interest into regular use.
- They are based on information from a broad variety of sources, including clinical trials and postmarketing surveillance. However, sometimes this information is not available, and assessment of devices starts a few years after their licensing.
- Generally, VTEs are used to summarize safety and performance data.

Challenges in assessing medical devices 2/5


BMJ

EDITOR’S CHOICE

The scandal of medical device regulation

Europeans are left to their own devices

When it comes to medical devices, Europeans seem to get a worse deal than US patients. Deborah Cohen and Matthew Bingley compare the regulatory systems.

How a fake hip showed up failings in European device regulation

Deborah Cohen investigates how EU authorities would be prepared to allow a fake hip prosthesis with dangerous design flaws onto the market.

The breast implant scandal and European Medical Device Regulation

Deborah Cohen describes an investigation showing how a fake hip prosthesis with dangerous design flaws should not have been approved for the EU market.
Challenges in assessing medical devices 3/5

- Clinical evidence is often poor in quantity and quality:
  - Current regulatory systems aim at assessing safety, performance and – sometimes – efficacy of medical devices

- What clinical evidence?
  - Large Randomized Controlled Studies (RCTs) represent the standard to look for causal relationships between outcomes and interventions, however…
  - MDs' features often make RCTs unethical, inapplicable or very difficult and too costly (e.g. proven effectiveness, learning curve, incremental innovation)

Challenges in assessing medical devices 4/5

Learning effect and diffusion of innovative medical devices: the case of transcatheter aortic valve implantation in Italy

Aims: We investigated the diffusion of transcatheter aortic valve implantation (TAVI) since its introduction into the Italian market aimed at identifying the potential drivers of uptake and diffusion at hospital and regional levels. Methods & methods: We estimated the determinants of TAVI diffusion in Italy from 2007 to 2015 with a regression analysis based on registry data. Results: Since 2007, TAVI has shown significant diffusion rates in Italy. The diffusion is positively correlated with implanting centers' experience and with the presence of key opinion leaders. Regional recommendations on the use of TAVI negatively influence the diffusion. Reimbursement policies do not exert a relevant impact. Conclusion: Learning effect seems to be the major driver of TAVI diffusion in Italy.

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Challenges in assessing medical devices 5/5

- **Timing** of assessment (i.e. Buxton Law’s “It is always too early until, unfortunately, it’s suddenly too late”):
  - Shall we wait until the use of the innovative MD becomes as experienced as the standard of care or shall we assess the innovative device at an early stage so to allow patients to access better care if cost-effective?
  - Challenge to assess long-term benefits and/or spillovers vs. upfront costs
- **Medical devices have wider economic implications** (e.g. organisational impact): rarely assessed*
  - It’s important to widen the perspective (i.e. NO silos-mentality and silos-budgeting)
- **Pricing** strategies also depend upon country-based procurement policies: instability of ICERs across jurisdictions and over time

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The Increasing Role of Real World Evidence

- Clinical evidence for MDs is often generated in clinical practice and often precedes (if any) RCTs:
  - E.g. 40% of high risk implantable MDs accessed the Italian market with no RCTs*
- Under certain conditions, real-world data, defined as data obtained outside the context of RCTs, can become relevant to decision makers, even in absence of RCTs
  - To be not only a complementary source of evidence but also a low-cost, rapid and valuable substitute especially for technologies whose diffusion process has already started

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*Tarricone R. Use of Real-world Evidence to Shape Health Policies for Medical Devices. ISPOR Boston, 2017.
Real World Data’s major advantages

- Learning curve
- Adherence to the real clinical practice:
  - Comparator (SoC)
  - Costs
- Time to results
- External validity

Real World Data are not problem-free (1/2)

Major concerns about RWD refer to*:

- Selection bias
- Internal validity
- Inaccurate recording of health events
- Missing data
- Opaque reporting of conduct and results
- Selective publications


Real World Data are not problem-free (2/2)

Major steps have been done to address methodological and procedural concerns:

- several techniques have been applied to reduce the impact of selection bias like multivariate regression or nonparametric techniques based on the propensity score
- methodological standards have been issued by ISPOR, ISPE, the US FDA, the European Network for Health Technology Assessment - EUnetHTA and MedtecHTA**
- good procedural practices as policies about the planning, execution, and dissemination of RWD studies have been developed to assure the public of the integrity of the research process and enhance confidence in the RWE produced from RWD studies*

Is this a favorable season for Real World Evidence in regulation?

REGULATION (EU) 2017/745 OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL
of 5 April 2017
(Text with EEA relevance)

Better clinical evidence for high risk and implantable medical devices based upon technologies’ characteristics and previous consultation of experts leaves room for RWE
Is this a favorable season for Real World Evidence in regulation?

Use of Real-World Evidence to Support Regulatory Decision-Making for Medical Devices

Guidance for Industry and Food and Drug Administration Staff


The draft of this document was issued on July 27, 2016

For questions about this document regarding CDRH-regulated devices, contact the Office of Surveillance and Biometrics (OSB) at 301-596-3807 or OSB.ODD@fda.hhs.gov. For questions about this document regarding EU-regulated devices, contact the Office of Communication, Outreach, and Development (ECCO) at 1-888-877-4350 or 202-402-3030.

the European Commission has proposed a regulation aimed at a better functioning of the internal market and of health protection through Joint Clinical Assessments (also) based on RWD for medical devices.

Is this a favorable season for Real World Evidence in policy making?

U.S. Food and Drug Administration

Center for Devices and Radiological Health

Center for Biologics Evaluation and Research

EUROPEAN COMMISSION

Brussels, 31.1.2018
COM(2018) 51 final
2018/0018 (COD)

Proposal for a
REGULATION OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL
on health technology assessment and amending Directive 2011/24/EU

(Text with EEA relevance)

Concluding remarks

• HTA is an unavoidable fact of life and is now located in the value-based paradigm, i.e. HTA is here to stay

• Medical devices are technologies different from those traditionally assessed by regulators and HTA bodies, i.e. pharmaceuticals:
  – These characteristics are seldom recognised by decision-makers, i.e. this is part of the challenge

• Much work has been done to improve methods to assess MDs*

• Part of this work has started influencing policy-making:
  – Regulatory and HTA bodies consider 1) the possibility to gather real-world evidence to complement the lack of RCTs and 2) to proceed with «early dialogues» aimed at advising manufacturers on key points, e.g. type of study, comparator(s), target population

• Other work is on its way and will certainly keep improving policy-making and patients’ access to modern care