SDA Bocconi

EMPOWERING LIVES THROUGH KNOWLEDGE AND IMAGINATION

Bocconi School of Management

MILANO I ITALY

ASSESSMENT OF MEDICAL DEVICES: KEY FEATURES AND MAJOR CHALLENGES

Rosanna Tarricone, PhD

Associate Dean, Government Health and Non Profit Division

Workshop "How can HTA methods be adapted to meet the special characteristics of medical devices?"
ISPOR Tokyo Sept 8th-13th 2018

SDA Bocconi

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*

*Drummond M, Griffin A, Tarricone R. Economic evaluation for devices and drugs: same or different? Value in Health 2009;12(4):402-406

SDA Bocconi

Challenges in assessing medical devices 1/5

- They are
  - multiple
  - diagnos

Available online at www.sciencedirect.com

ScienceDirect

Journal homepage: www.elsevier.com/locate/jval

ELSEVIER

ORIGINAL RESEARCH
Economic Evaluation

Genetic Screening for the Predisposition to Venous Thromboembolism: A Cost-Utility Analysis of Clinical Practice in the Italian Health Care System

Amelia Compagni, PhD[1,*], Alessia Melegaro, PhD[2,†], Rosanna Tarricone, PhD[1,†]

[1]Department of Policy Analysis and Public Management, [2]Centre for Research in Health and Social Care Management (CERGAS), [†]Dondena Centre for Research on Social Dynamics, Bocconi University, Milan, Italy

ABSTRACT

Objectives: In the Italian health care system, genetic tests for factor V Leiden and factor II are routinely prescribed to assess the predisposition to venous thromboembolism (VTE) of women who request oral contraception. With specific reference to two subpopulations of women already at risk (i.e., familial history or previous event of VTE), the study aimed to assess whether current screening practices in Italy are cost-effective. **Methods:** Two decision-tree models assessed costs and quality-adjusted life-years (QALY), annually from the perspective of the National Health Service. The two models were derived from a decision analysis exercise concerning testing practices and consequent prescribing behavior for oral contraception conducted with 150 Italian general-ists. Health care costs were compiled on the basis of 10-year hospital discharge records and the activities of a thrombosis center. Whenever possible, input data were based on the Italian context; otherwise, the data were taken from the international literature. **Results:** Current testing practices on women with a familial history of VTE generate an incremental cost-effectiveness ratio of €52,413/QALY, which is well above the acceptable threshold of cost-effectiveness of €40,000 to €50,000/QALY. In the case of women with a previous event of VTE, the most frequently used testing strategy is cost-ineffective and leads to an overall loss of QALY. **Conclusions:** This study represents the first attempt to conduct a cost-utility analysis of genetic screening practices for the predisposition to VTE in the Italian setting. The results indicate that there is an urgent need to better monitor the indications for which tests for factor V Leiden and factor II are prescribed.

Keywords: cost-utility analysis, genetic testing, Italy, venous thromboembolism.

Copyright © 2013, International Society for Pharmacoeconomics and Outcomes Research (ISPOR). Published by Elsevier Inc.

SDA Bocconi

Challenges in assessing medical devices 2/5

BMJ

EDITOR'S CHOICE

The scandal of medical device regulation

MEDICAL DEVICES

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 Billingsley compare the regulatory systems

MEDICAL DEVICE REGULATION

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

Deborah Cohen investigations editor

n quantity an

t assessing sa

The breast implant scandal and European Medical Device Regulation

by Maria Donzelli and Roger Gray

The recent silicone breast implant scandal in Europe has led to questions about whether or not European medical device regulations are sufficient to protect patients not only from unsafe breast implants, but unsafe medical devices in general.

What were the events that led to the discovery of substandard materials being used to manufacture the breast implants?

How are medical devices, specifically, breast implants regulated in Europe?

MEDICAL DEVICE REGULATION

Faulty hip implant shows up failings of EU regulation

Deborah Cohen describes an investigation showing how a fake hip prosthesis with dangerous design flaws stood to be approved for the EU market

Deborah Cohen investigations editor

SDA Bocconi

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)

SDA Bocconi

Challenges in assessing medical devices 4/5

Research Article

For reprint orders, please contact: reprints@futuremedicine.com

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

Journal of Comparative Effectiveness Research

Aim: 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. **Materials & 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.

Giuditta Callea*[1], Maria Caterina Cavallo[1], Rosanna Tarricone[1,2] & Aleksandra Torbica[1,2]
[1]Center for Research on Health & Social Care Management (CERGAS), Università Bocconi, Milan, Italy
[2]Department for Institutional Analysis & Public Sector Management, Università Bocconi, Milan, Italy
*Author for correspondence
Tel.: +39 02 5836 2728
Fax: +39 02 5836 2590
giuditta.callea@unibocconi.it

First draft submitted: 18 November 2016; Accepted for publication: 23 January 2017; Published online: 1 February 2017

urning curve, i.e. what

inexperience with the

tion:

f medical devices

*Spinner et al. "Do different clinical evidence bases lead to discordant health-technology assessment decisions? An in-depth case series across three jurisdictions" ClinicoEconomics and Outcomes Research 2013;5:69-85

SDA Bocconi

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

*Tarricone R, Callea G, Ogorevc M, Prevolnik Rupel V. Improving the methods for the economic evaluation of medical devices. Health Economics 2017;26(Suppl S1):70-92.

SDA Bocconi

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

*Tarricone R. Use of Real-world Evidence to Shape Health Policies for Medical Devices. ISPOR Boston, 2017.

SDA Bocconi

Real World Data's major advantages


The NEW ENGLAND JOURNAL of MEDICINE

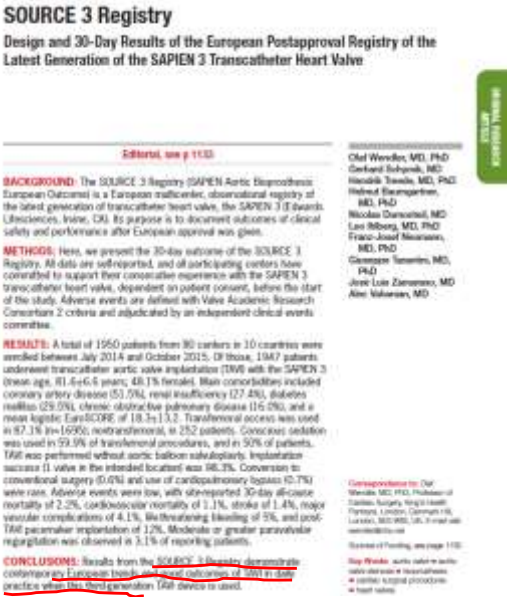
JUNE 5, 2011

Transcatheter versus Surgical Aortic-Valve Replacement in High-Risk Patients

The NEW ENGLAND JOURNAL of MEDICINE

APRIL 28, 2016

Transcatheter or Surgical Aortic-Valve Replacement in Intermediate-Risk Patients


SOURCE 3 Registry

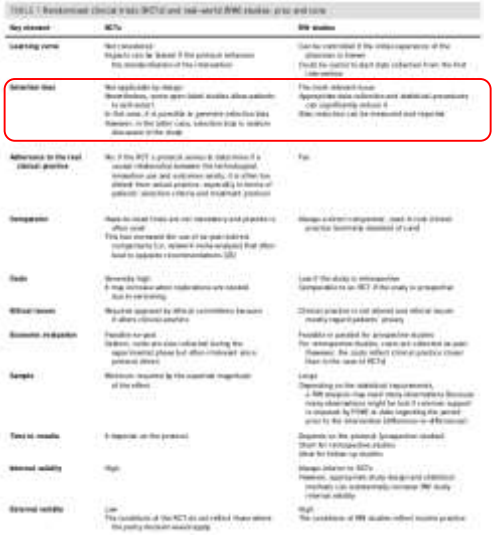
Design and 30-Day Results of the European Postapproval Registry of the Latest Generation of the SAPIEN 3 Transcatheter Heart Valve

SDA Bocconi

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



*Berger ML et al., Good Practices for Real-World Data Studies of Treatment and/or Comparative Effectiveness: Recommendations from the Joint ISPOR-ISPE Special Task Force on Real-World Evidence in Health Care Decision Making. Value in Health 2017;20:1003-1008.

*Tarricone R., Boscolo P.R., Armeni P. What type of clinical evidence is needed to assess health technologies? European Respiratory Review, 2016;25:259-265.

SDA Bocconi

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*

*Berger ML et al., Good Practices for Real-World Data Studies of Treatment and/or Comparative Effectiveness: Recommendations from the Joint ISPOR-ISPE Special Task Force on Real-World Evidence in Health Care Decision Making. Value in Health 2017;20:1003-1008.
**Tarricone R, Torbica A, Drummond M. (for the MedtecHTA project group) Key Recommendations from the MedtecHTA Project. Health Economics 2017;26(Suppl S1):145-152

SDA Bocconi

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

on medical devices, amending Directive 2001/83/EC, Regulation (EC) No 178/2002 and Regulation (EC) No 1223/2009 and repealing Council Directives 90/385/EEC and 93/42/EEC

(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

SDA Bocconi

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

Document issued on August 31, 2017.

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 301-796-5967 or CDRHRealEvidence@fda.hhs.gov. For questions about this document regarding CBER-regulated devices, contact the Office of Communication, Outreach, and Development (OCOD) at 1-800-835-4709 or 240-402-8010.

U.S. FOOD & DRUG ADMINISTRATION

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Devices and Radiological Health
Center for Biologics Evaluation and Research

13

SDA Bocconi

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

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)

{SWD(2018) 41 final} - {SWD(2018) 42 final}

- 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

14

SDA Bocconi

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

*Tarricone R, Torbica A, Drummond M. (for the MedtecHTA project group) Key Recommendations from the MedtecHTA Project. Health Economics 2017;26(Suppl S1):145-152