KOZMINSKI UNIVERSITY

HAS THE TIME COME TO REPLACE RANDOMIZED CONTROLLED TRIALS WITH REAL-WORLD DATA -- A CASE OF MEDICAL DEVICES?

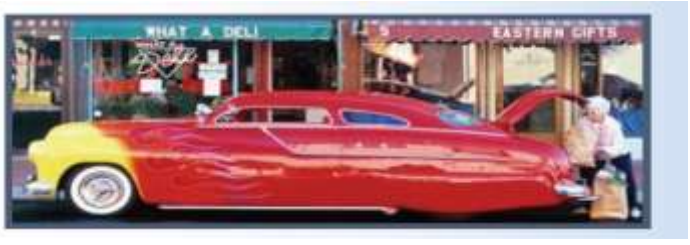
Industry perspective

Katarzyna Kolasa, PhD, Professor of Health Economics Kozminski University,

Head of Health Economics and Healthcare Management Department,

Principal Senior Consultant, Market Access HEOR Straub Medical, Wangs Switzerland

Medical devices are DIFFERENT from pharmaceuticals



Life cycle

While the time to market for a new pharmaceutical product averages at 8-12 years, the corresponding number for a MD is just 18 months.

Buxton's law

"It's always too early …. until, unfortunately, it's suddenly too late."

Source: Buxton MJ. Problems in the economic appraisal of new health technology: the evaluation of heart transplants in the UK. In: Drummond MF. Economic appraisal of health technology in the European Community. Oxford Medical Publications, 1987:103-18.

Multi dimensional outcomes

- ✓ Patient's heterogeneity
- ✓ Technical features
- ✓ Healthcare provider's infrastructure
- ✓ Coding
- ✓ Clinical experience

The volume-outcome relationship

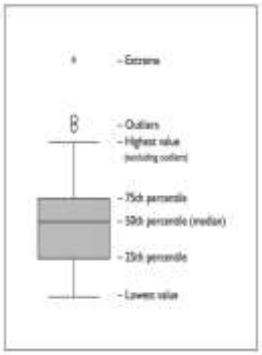
Example

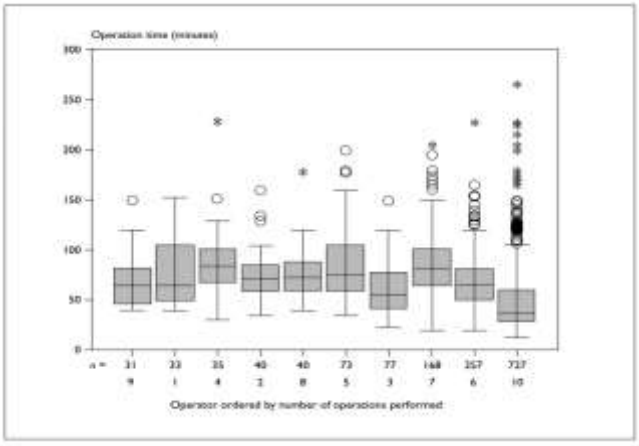
- Observational cohort study of 841 patients who underwent carotid endarterectomy (CEA) (Jan 2008 – Dec 2010)
- A low-volume surgeon was defined as a surgeon who completed 40 or fewer CEA per year
- The rate of stroke and death was 6.9% for low-volume and 2.0% for high-volume surgeons (P = .001)
- Complications were 13.4% for low-volume vs. 7.2% for high-volume surgeons (P = .008)

Maas MB et al.: Risk adjustment for case mix and the effect of surgeon volume on morbidity, JAMA Surg 2013 Jun;148(6):532-6.

Learning effect

Example





Laparoscopic cholecystectomy conducted in Aberdeen between March 1991 and March 1999 for 1481 pts by 10 surgeons

Source: Ramsay CR et al: Statistical assessment of the learning curves of health technologies Health Technology Assessment 2001;5(12):1-79.

Blinding

- Ethical issues with simulating the intervention and “standardizing” the postoperative care
- Blinding of participants, health care providers, or outcome assessors but NOT clinicians who are using the device
- A cross-sectional survey showed that 58% of orthopaedic surgeons prefer to participate in expertise-based controlled trials compared to only 17% for conventional RCTs

Source: Boutron I, Moher D, Tugwell P, Giraudeau B, Poiraudeau S, Nizard R, et al. A checklist to evaluate a report of a nonpharmacological trial (CLEAR NPT) was developed using consensus. J Clin Epidemiol. 2005;58(12):1233–40. Bednarska E, Bryant D, Devereaux PJ. Orthopaedic surgeons prefer to participate in expertise-based randomized trials.Clin Orthop Relat Res. 2008;466(7):1734–44.

RCTs for medical devices

Among 215 clinical trials conducted for 32 innovative MDs, only 15% of them were randomized controlled trials (RCTs) and more than 50% included fewer than 30 patients.

Source: Boudard A. Clinical studies of innovative medical devices: what level of evidence for hospital-based health technology assessment?J Eval Clin Pract 2013 Aug;19(4):697-702.

The current use of RWE for medical devices

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

prior approval

- generation of hypotheses
- as historical control

after approval

- to expand the labeling
- post-market surveillance

Payers wants RWE

This document is intended to provide clear, factual, and balanced information that may facilitate detailed understanding of the Micra TPS CED Study when used in accordance with FDA-approved labeling. This document addresses the Micra TPS CED Study specifically, and unless otherwise noted does not apply to the Micra TPS Post-Approval Study (PAS).

The study of claims data to address the following research questions:

1. What are the peri-procedural and post-procedural complications?
2. What are the long-term outcomes?
3. What are the effects of patient characteristics (age, gender, comorbidities)?
4. What are the device-related issues?
5. How are operators and facility characteristics related to complications and long-term outcomes?

Source: https://www.medtronic.com/content/dam/medtronic-com/products/cardiac-rhythm/coding-coverage-reimbursement/documents/20180846aenp1-micra-ced-provider-faq-august-2017.pdf

Payers wants RWE

Beyond price: considering total cost of care delivery

- Stockholm County Council tendered for **wound-care products**
- Instead of pure price, a cost **model** incl. **care delivery costs** was used
- Suppliers had to demonstrate **total costs** for 3 different **fictive patients**
- Bidder with highest price won (ConvaTec): lowest overall cost and strong evidence to support their claim

Outcomes support: collaboration on measuring outcomes

KAROLINSKA

PHILIPS

- Karolinska university hospital tendered for **imaging equipment**
- Used innovative **competitive dialogue** format
- Tender contained **conditions** around **contributing to outcomes**
- Philips won thanks to support on **outcomes measurement**

Source: Wahttp://imsta.ie/wp-content/uploads/2016/02/VALUE-BASED-HEALTHCARE-A-NEW-FRAMEWORK-FOR-SMARTER-PROCUREMENT-Gotz-Gerecke-The-Boston-Consulting-Group-AG.pdf

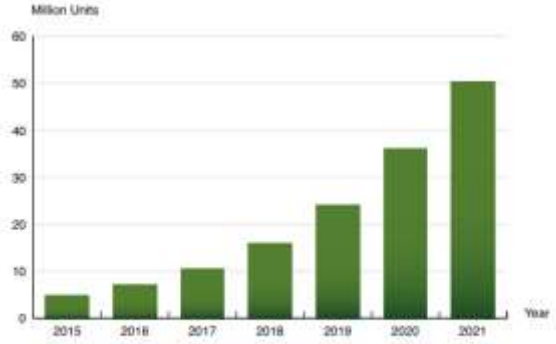
A new wave of RWD- the Internet of Medical Things (IoMT)

- Vitals-Tracking Wearables
- Medication Adherence Tools
- Virtual Home Assistants
- Portable Diagnostics Devices/Disability Assistance Tools
- Personal Emergency Response Systems
- Smart Implants
- Smart Senior Homes
- Family Caregiver Remote Monitoring Tools

Source: https://www.forbes.com/sites/reenitadas/2017/05/22/10-ways-internet-of-medical-things-is-revolutionizing-senior-care/2/#4023fc7f8c27

A new wave of RWD- integrated healthcare models

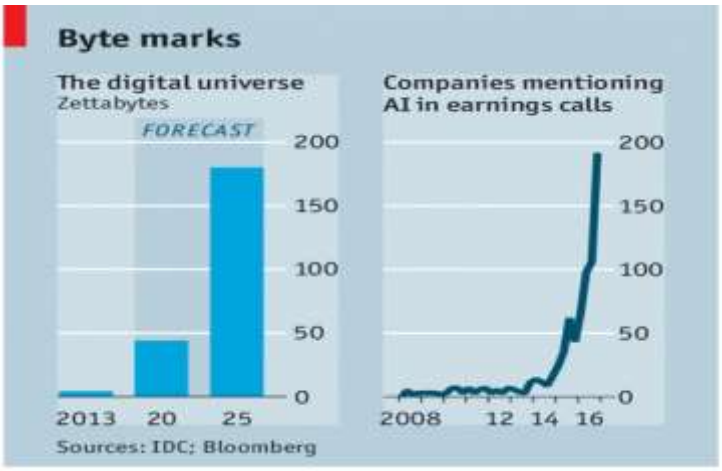
There are 7 mln patients being monitored remotely today and it is projected to exceed 50 million by 2020



Connected home medical monitoring devices, million units (World 2015–2021)

Source: http://www.berginsight.com/ReportPDF/ProductSheet/bi-mhealth8-ps.pdf

The future use of RWE for medical devices



Source: https://www.economist.com/news/briefing/21721634-how-it-shaping-up-data-giving-rise-new-economy

Conclusions

- To define the incremental value of medical devices requires more efforts compared to pharmaceuticals due to challenges with randomised clinical trials (RCTs)
- There are number of specific features of medical devices that needs to be accounted for in the value assessment such as learning curves and lack of head to head data
- The future growing amount of RWD will require holistic approach and search for the value assessment of integrated healthcare models.