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.”


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 ($P = .008$)


Learning effect

Example

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

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


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.

The current use of RWE for medical devices

prior approval
- generation of hypotheses
- as historical control

after approval
- to expand the labeling
- post-market surveillance

Payers wants RWE

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?

Payers wants RWE

Beyond price: considering total cost of care delivery

- Stockholms län lansförsäkring
- 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


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


The future use of RWE for medical devices

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.