



Evidence Based Medicine, Health Technology Assessment, and Health Services research

Yen-Fu Chen (陳彥甫), PhD, Principal Research Fellow Y-F.Chen@Warwick.ac.uk
Warwick Centre for Applied Health Research & Delivery (W-CAHRD)
Warwick Medical School, University of Warwick, UK

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Outline of the talk

- Evidence based medicine
- Health technology assessment
- Health services research



Life before EBM


'BRAIN SALT'
OR 'NERVE AND BRAIN SALT' (EFFERVESCENT).

It is claimed for it that a small dose, used as directed, and repeated if required, will quickly remove
BRAIN FOG, NERVOUS HEADACHE, SLEEPLESSNESS,
and kindred affections.

Queen Anne Street, W. "April 1891.

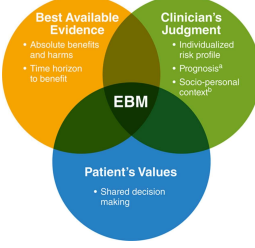
"Messrs. F. NEWBERRY & SONS.—Gentlemen.—Having been recommended to try your Brain Salt by a medical friend, I have done so, and find it invaluable in cases of NEURALGIA and HEADACHE, due to disturbances arising from insufficient mastication, or dental derangements."
"Truly truly, ——— J.D.S., R.C.S."

'BRAIN SALT' (title registered in Great Britain and America) 2s. 0d. per bottle, of all Chemists, or post free from F. NEWBERRY & SONS (112 years in St. Paul's Churchyard), King Edward Street, London, E.C.



Evidence based medicine (EBM)

► The conscientious, **explicit**, and judicious use of current **best evidence** in **making decisions** about the care of individual patients



Sackett et al. 1996
BMJ;312:71-72

Makam & Nguyen 2017;
Circulation; 135:180-195

Evidence-based medicine has been hijacked?

- Influential randomized trials are largely done by and for the benefit of the industry
- Meta-analyses and guidelines have become a factory, mostly serving self-interest
- Growth of principal investigators who excel primarily as managers absorbing more money
- Risk factor epidemiology has excelled in salami-sliced data-dredged articles with gift authorship and has become adept to dictating policy from spurious evidence

Ioannidis. J Clin Epidemiol 2016;73:82-86

Health Technology Assessment

- Moving from individual to population
- Allocation of scarce resource



Health technology assessment (HTA)

- ▶ The **systematic** evaluation of the properties and effects of a health technology, addressing the direct and intended effects of this technology, as well as its indirect and unintended consequences, and aimed mainly at informing **decision making** regarding health technologies

INAHTA Glossary

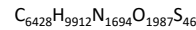
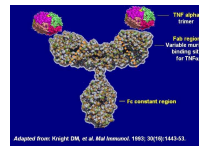


Recent issues in HTA

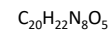
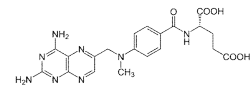
- ▶ Biosimilar
- ▶ Cancer Drugs Fund



Biosimilars



infliximab

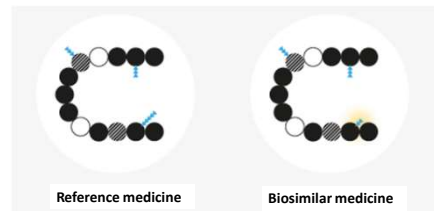


methotrexate

Biosimilar - definition

- ▶ Biosimilar means that the biological product is approved based on data demonstrating that it is highly similar to an FDA-approved biological product, known as a reference product, and that there are no clinically meaningful

Biosimilar - development



Source: European Medicines Agency and the European Commission. Biosimilars in the EU: information guide for healthcare professionals

Key issues

- ▶ Batch to batch variability
- ▶ Manufacturing change
- ▶ Biosimilarity: high similarity in terms of structure, biological activity and efficacy, safety and immunogenicity profile
- ▶ Extrapolation supported by comparability studies (quality, non-clinical and clinical)

Advantages and considerations

- ▶ Economic
- ▶ Widening access
- ▶ Further sources of supply
- ▶ Available indications and route of administration
- ▶ Suitable strength and presentation
- ▶ Administration device and product-associated support services

Switch or not?

- ▶ At the discretion of the individual prescriber in partnership with the patient
- ▶ With appropriate monitoring
- ▶ Automatic substitution at pharmacy level is not permitted
- ▶ Prescribe using brand name rather than generic name

Pharmacovigilance

- ▶ Additional monitoring mandated by EU pharmacovigilance legislation (as for a new active substance and all biological medicines), indicated by black triangle ▼
- ▶ Story so far: no difference in the nature, severity or frequency of adverse effects

Cancer Drugs Fund

- ▶ Set up in 2011 as one of the election pledges to reduce delay and improve access
- ▶ Increased from £50 million to £200 m (2013/4), £280 m (£2014/5) and £340/466 m (£2015/6)
- ▶ 47 indications (29 drugs): only 18 (38%) reported statistically significant overall survival; median 3.1 months (1.4 to 15.7 months)
- ▶ From July 2016 under NICE appraisal

Aggarwal et al. Ann Oncol 2017;28: 1738–50

Cancer Drugs Fund

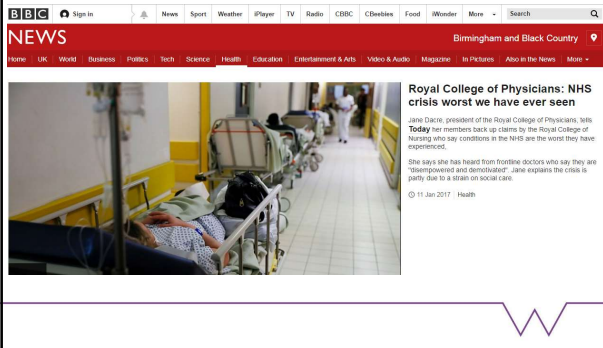
Five things £1.27bn can pay for in the NHS (over five years)

- ▶ 10,000 nurses
- ▶ 2,500 hospital consultants
- ▶ One of England's 10 regional ambulance services
- ▶ A one-off pay rise of 2.5% for every member of NHS staff
- ▶ An extra 200 GP surgeries



<http://www.bbc.co.uk/news/health-39711137>

Beyond HTA



Moving beyond HTA

- ▶ Beyond individual patients – health service delivery
- ▶ Beyond “curative” health care – public health
- ▶ Beyond health care – social care & public policy

Health services & delivery research (HSDR)

Health services research (HSR); applied health research
 Research to produce **evidence** on the quality, accessibility and organisation of health services. This includes **evaluations** of how the NHS might improve delivery of services. The **audience** for this research is the public, service users, clinicians and managers

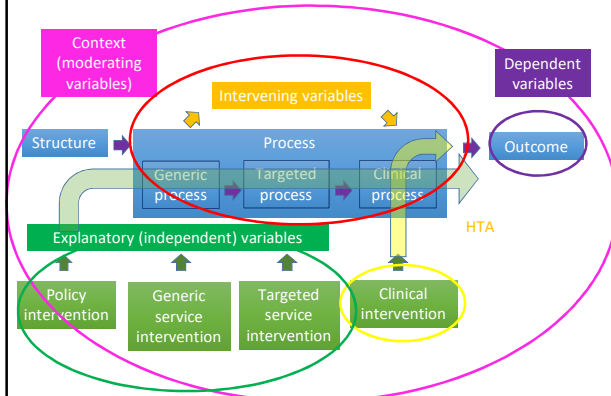
UK NIHR HS&DR Programme

EBM, HTA & HSDR

- ▶ Inform decision making
- ▶ Use of best available evidence
- ▶ Take into account stakeholders'
- ▶ Different in scope



Challenges in improving health services



Characteristics of HSR

- ▶ Broader scope
- ▶ Complex intervention
- ▶ Complex system

Evaluating service delivery interventions

Strategies to change organizational culture to improve healthcare performance

- Cochrane EPOC criteria: considered randomised controlled trials (RCTs) quasi-experimental studies, controlled clinical trials (CCTs), controlled before and after studies (CBAs) and interrupted time series analyses (ITS)
- Searched 15 databases & screened 4239 records
- No study met the quality criteria
- "It is not possible to draw any conclusions about the effectiveness of strategies..."



Parmelli et al. 2011. CDRS;1: CD008315

Research methods used in HSDR

- ▶ Evidence for intervention effectiveness
- ▶ Randomised controlled trials
 - Cluster RCTs
 - Stepped wedge trials
- ▶ Quasi-experimental studies
 - Difference-in-difference method
 - Synthetic controls
 - Instrumental variables
 - Propensity scores

Research methods commonly used in HSDR

- ▶ Questions about how, why, when, who
- ▶ Qualitative & mixed methods research
- ▶ Case studies
- ▶ Operational research
- ▶ Policy analysis

Pharmacy and health services research

- ▶ Strengthen the services they provide
- ▶ Build the evidence base for developing and commissioning new services
- ▶ Improve patient care
- ▶ Contribute to the knowledge base within health service research more widely
- ▶ Gain both professionally and personally in the process

Roberts R and Kennington E, Pharm J 2010, 11 Mar

Evidence-based pharmacy practice

- ▶ Bhattacharya et al. The feasibility of determining the effectiveness and cost-effectiveness of medication organization devices compared with usual care for older people in a community setting: systematic review, stakeholder focus groups and feasibility randomised controlled trial

Health Tech Assess 2016 ;20(50).

Summary

- ▶ EBM has revolutionised medical care
- ▶ HTA has become a necessity
- ▶ Evidence-based approach is spreading across fields & disciplines
- ▶ Moving beyond decision making for individuals to wider systems
- ▶ Diverse and evolving health services research methods and opportunities, which pharmacists are well placed to get involved