EBM e NBM

Alfredo Zuppiroli – Firenze

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19

Roma. Il figlio che ha scritto a Lorenzin: "Era malato terminale di cancro, per lui solo indifferenza"

"Urla, risate, panini mio padre moriva e il pronto soccorso era una bolgia"

MARIA NOVELLA DE LUCA

ROMA. «È morto in quello stanzone del San Camillo, tra tossicodipendenti e vagabondi, la sua agonia offerta agli occhi di tutti, mentre noi cercavamo di proteggere i suoi ultimi respiri facendo scudo con i nostri corpi. Si chiamava Marcello, aveva 74 anni, era un uomo allegro ed era mio padre: ha detto addio alla vita tra gente che urlava, medici

mato un'ambulanza».

Siete arrivati al San Camillo.

«Mio padre è entrato al Pronto Soccorso alle 5, e io sono riuscito a vederlo soltanto dopo molte ore, durante le visite delle 12.30. I medici avevano capito che era gravissimo, ma l'avevano lasciato in mezzo allo stanzone dei codici verdi e bianchi, dove finiscono i casi meno urgenti, uomini e donne insieme, da chi si rompe una gamba

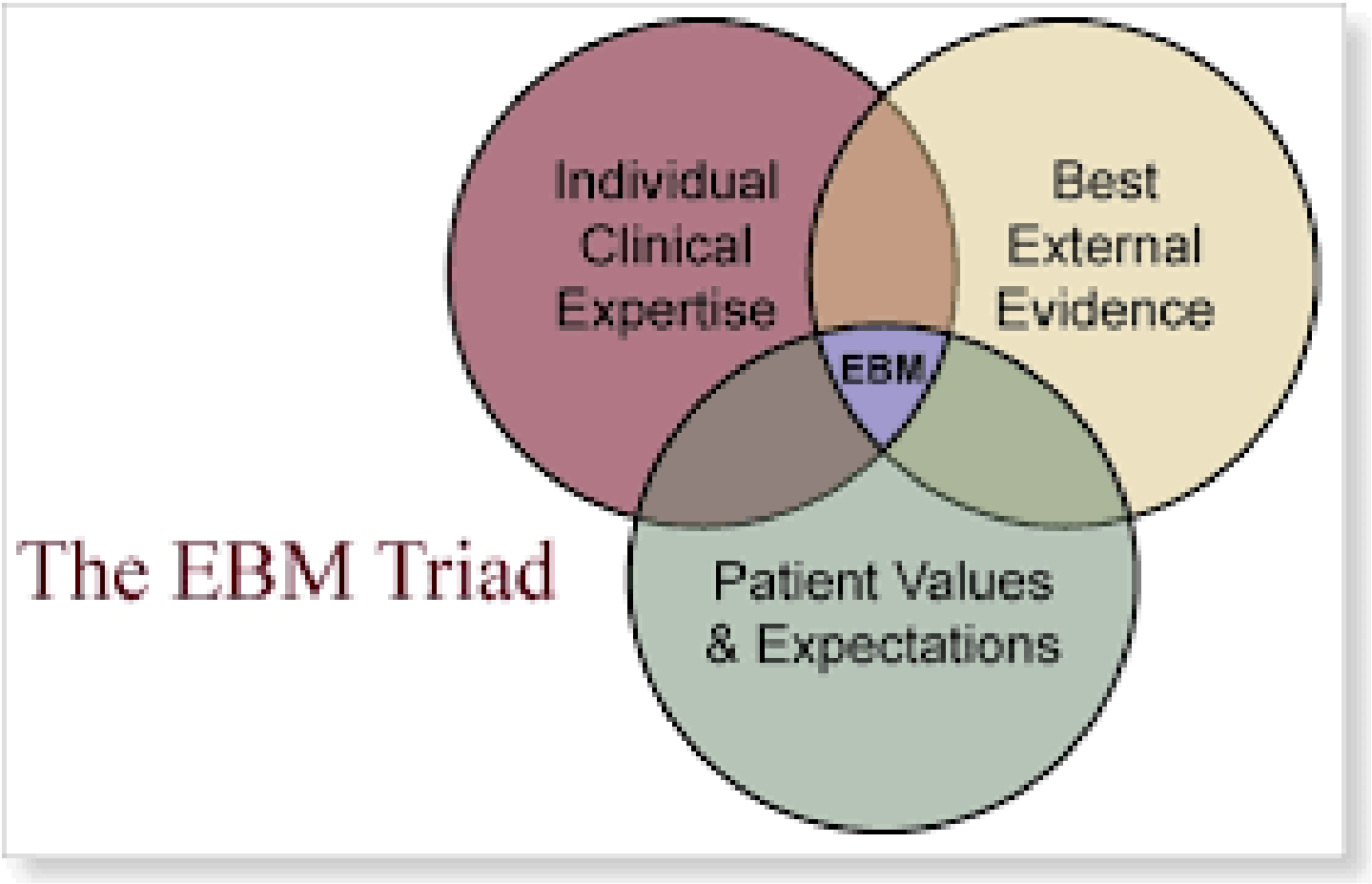
ziane, affette da Alzheimer, che continuavano a spogliarsi, e i parenti del letto accanto che ridevano. Ho vegliato mio padre seduto sul pavimento di quel girone infernale, scacciando chi si fermava a guardare la sua agonia».

Alla fine il paravento lo avete conquistato...

«Sì, grazie ad una infermiera, molto professionale ma anche umana, che ce ne ha rimediato

che a tutti sembrasse normale che un uomo potesse agonizzare in una stanza piena di gente. E ancora una volta, soltanto grazie a quella infermiera che vorrei ringraziare, abbiamo ottenuto un posto un po' più appartato. E in quella bolgia l'unica persona che ci ha dato un po' di conforto è stata la mamma di una ragazza gravemente disabile. Ricordo il dolore dei suoi occhi».

Perché non avete trasferito


Individual
Clinical
Expertise
Best
External
Evidence
EBM
Patient Values
& Expectations
The EBM Triad

Parachute use to prevent death and major trauma related to gravitational challenge: systematic review of randomised controlled trials

Gordon C S Smith, Jill P Pell

Abstract

Objectives To determine whether parachutes are effective in preventing major trauma related to gravitational challenge.

Design Systematic review of randomised controlled trials.

Data sources: Medline, Web of Science, Embase, and the Cochrane Library databases; appropriate internet sites and citation lists.

Study selection: Studies showing the effects of using a parachute during free fall.

Main outcome measure Death or major trauma, defined as an injury severity score > 15.

Results We were unable to identify any randomised controlled trials of parachute intervention.

Conclusions As with many interventions intended to prevent ill health, the effectiveness of parachutes has not been subjected to rigorous evaluation by using randomised controlled trials. Advocates of evidence based medicine have criticised the adoption of interventions evaluated by using only observational data. We think that everyone might benefit if the most radical protagonists of evidence based medicine organised and participated in a double blind, randomised, placebo controlled, crossover trial of the parachute.

accepted intervention was a fabric device, secured by strings to a harness worn by the participant and released (either automatically or manually) during free fall with the purpose of limiting the rate of descent. We excluded studies that had no control group.

Definition of outcomes

The major outcomes studied were death or major trauma, defined as an injury severity score greater than 15.[9]

Meta-analysis

Our statistical apprach was to assess outcomes in parachute and control groups by odds ratios and quantified the precision of estimates by 95% confidence intervals. We chose the Mantel-Haenszel test to assess heterogeneity, and sensitivity and subgroup analyses and fixed effects weighted regression techniques to explore causes of heterogeneity. We selected a funnel plot to assess publication bias visually and Egger's and Begg's tests to test it quantitatively. Stata software, version 7.0, was the tool for all statistical analyses.

Results

Our search strategy did not find any randomised controlled trials of the parachute.

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BMJ 2003;327:1459–61

Patient need versus evidence: a balancing act

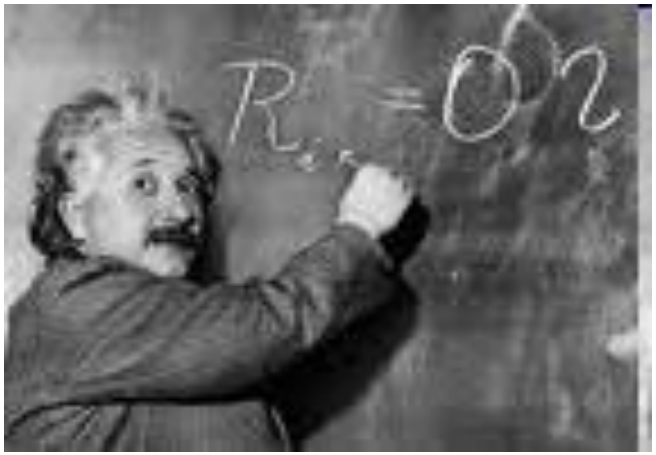
For the FDA Summary Review see http://www.accessdata.fda.gov/drugsatfda_docs/nda/2016/206488_summaryRO...review_Redacted.pdf

For the **Lancet Neurology** Policy View see Lancet Neurol 2016; 15: 882–90

On Sept 19, the US Food and Drug Administration (FDA) granted accelerated approval for eteplirsen, a new drug for the treatment of Duchenne muscular dystrophy. The decision goes against the recommendation of the FDA's advisory committee—which earlier voted not to approve the drug, citing concerns about the quality of the evidence—and follows months of internal wrangling among FDA officials. The approval was applauded by parents and advocacy groups, who had been vigorously lobbying the FDA, but led to accusations from drug policy experts that the agency was setting a dangerous precedent by approving a drug on such limited evidence and ignoring the advice of its expert panel.

Eteplirsen restores the readability of a damaged part of the gene encoding dystrophin—absence of which causes muscular dystrophy—resulting in formation of a truncated but functional form of dystrophin. Approval was largely based on the surrogate endpoint of increased dystrophin expression in muscle biopsies from just 12 boys after 48 weeks of eteplirsen treatment. However, increases in dystrophin were modest and whether such gains are sufficient to slow functional decline remains to be seen. Sarepta Therapeutics, the drug's manufacturer, must now undertake a 2-year randomised controlled trial to assess the clinical benefit of eteplirsen. If efficacy is not confirmed, the FDA could withdraw approval.

With few treatment options available for muscular dystrophy, parents are understandably desperate. The FDA clearly states that the functional effects of eteplirsen are not proven. Balancing patients' needs and expectations against the weak evidence base is a difficult task. But raising hope, perhaps unrealistically, by approving drugs on such uncertain evidence is not the answer, and could even be counterproductive by jeopardising the ability to undertake placebo-controlled trials. Well designed and funded studies of the functional efficacy and safety of eteplirsen should be the way forward, and a recent Policy View highlighted the power of a collaborative effort between patients, scientists, and regulators to help develop drugs for muscular dystrophy. Patients with muscular dystrophy deserve an effective treatment—only time will tell whether the FDA's decision was the correct one. ■ *The Lancet*



Non tutto ciò che può essere contato

conta

e non tutto ciò che conta

può essere contato.

Picasso - Scienza e carità - 1897

The NEW ENGLAND JOURNAL of MEDICINE

December 25, 2008

PERSPECTIVE

The I Patient…

Culture Shock — Patient as Icon, Icon as Patient

Abraham Verghese, M.D.

On my first day as an attending physician in a new hospital, I found my house staff and students in the team room, a snug bunker filled with glowing monitors. Instead of sitting down to hear about the patients, I suggested we head out to see them. My team came willingly, though they probably felt that everything I would need to get up to speed on our patients — the necessary images, the laboratory results — was right there in the team room. From my perspective, the most crucial element wasn't.

For the next few weeks, I ensured that we spent as little time as possible in the bunker. These were excellent residents who cared enormously about patients' welfare. They enjoyed being shown common findings — white nails of liver disease, an accessory nipple, Dupuytren's contracture, parotid enlargement, spider angiomas, café au lait spots, the paradoxical splitting of the second heart sound in left bundle-branch block, signs of pseudo-

PATIENT-CENTERED CARE

Sachin H. Jain, Harvard Medical School

VIEWPOINT

The McDonaldization of Medicine

E. Ray Dorsey, MD, MBA
Department of Neurology, University of Rochester Medical Center, Rochester, New York.

George Ritzer, PhD, MBA
Department of Sociology, University of Maryland, College Park.

As put forth in *The McDonaldization of Society*, "the principles of the fast-food restaurant are coming to dominate more and more sectors of American society,"[1] including medicine (Table). While designed to produce a rational system, the 4 basic principles of McDonaldization—efficiency, calculability, predictability, and control—often lead to adverse consequences. Without measures to counter McDonaldization, medicine's most cherished and defining values including care for the individual and meaningful patient-physician relationships will be threatened.

length of patient visits can result in equal care that does not address individual needs.

The final dimension of McDonaldization is control of humans by nonhuman technology,[1] which is increasingly applied to both physicians and patients. In fast-food restaurants, machines, not workers, control cooking. In medicine, resident physicians now spend far more time with computers (40%) than with patients (12%).[4] Billing codes and policies, which specify the length and content of visits, dictate the care that patients receive, influence clinicians, lead to unnecessary procedures, and

“E’ molto più importante sapere

che tipo di paziente ha quella malattia,

piuttosto che sapere

che tipo di malattia ha quel paziente”

William Osler - Remark on specialism. Boston Med Surg J 1892;126:457-9

CentroNazionaleMalattieRare
CNMR

Consensus Conference

"Linee di indirizzo per l'utilizzo della medicina narrativa

in ambito clinico-assistenziale, per le malattie rare e cronico-degenerative"

11-12-13 giugno 2014

Aula Rossi
Istituto Superiore di Sanità
Via Giano della Bella, 34 - Roma

Con il termine di Medicina Narrativa (mutuato dall'inglese *Narrative Medicine*) si intende una metodologia d'intervento clinico-assistenziale basata su una specifica competenza comunicativa. La narrazione è lo strumento fondamentale per acquisire, comprendere e integrare i diversi punti di vista di quanti intervengono nella malattia e nel processo di cura. Il fine è la costruzione condivisa di un percorso di cura personalizzato (storia di cura).

La Medicina Narrativa (NBM) si integra con la Medicina Basata sulle Evidenze (EBM) e, tenendo conto della pluralità delle prospettive, rende le decisioni clinico-assistenziali più complete, personalizzate, efficaci e appropriate.

La narrazione del paziente e di chi se ne prende cura è un elemento imprescindibile della medicina contemporanea, fondata sulla partecipazione attiva dei soggetti coinvolti nelle scelte. Le persone, attraverso le loro storie, diventano protagoniste del processo di cura.

Oliver Sacks - L'uomo che scambiò sua moglie per un cappello, 2001

"Ognuno di noi ha una storia del proprio vissuto,

un racconto interiore, la cui continuità, il cui senso è la nostra vita …

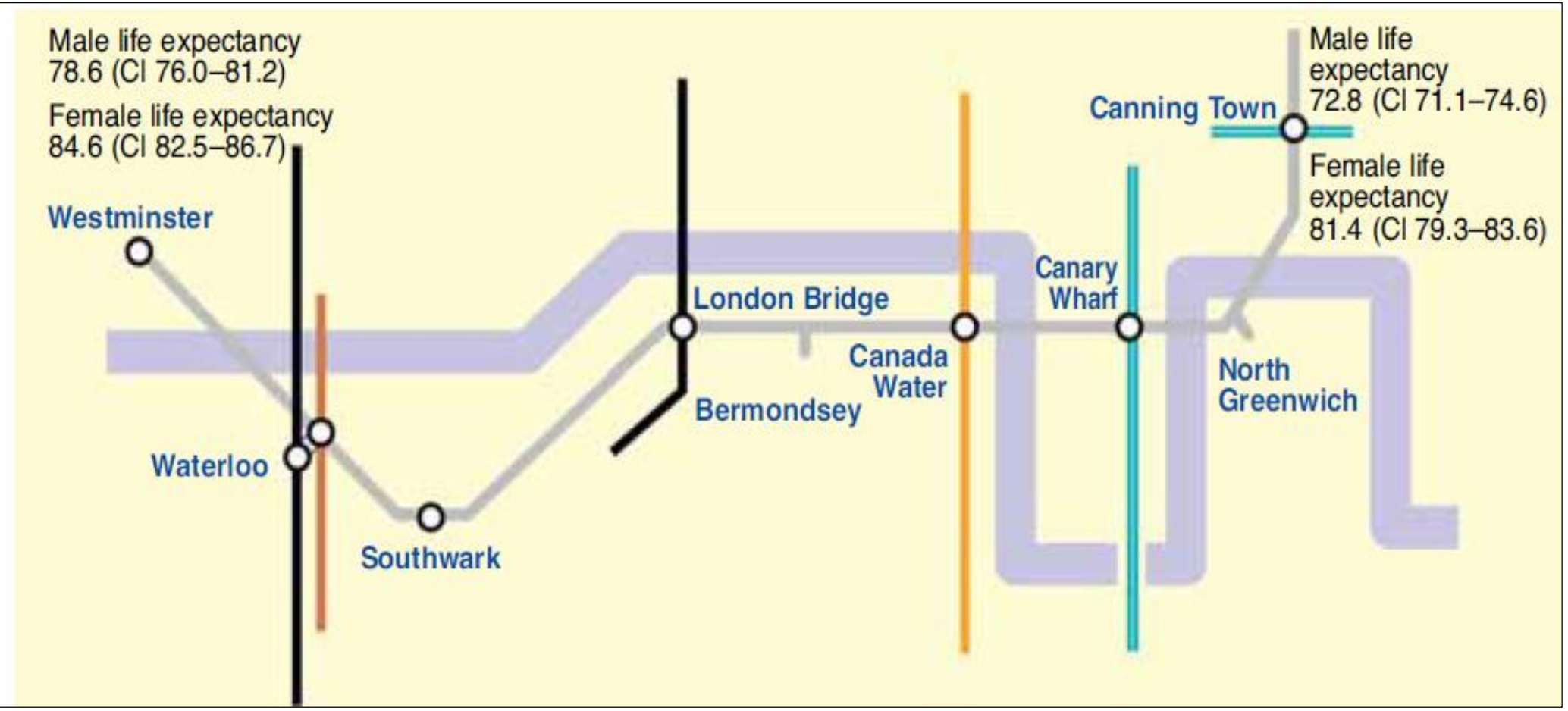
ciascuno di noi è una biografia, una storia.

Dal punto di vista biologico, fisiologico,

noi non differiamo molto l'uno dall'altro;

storicamente, come racconti, ognuno di noi è unico"

The Jubilee Line



Source: Analysis by London Health Observatory using Office for National Statistics data revised for 2002–6. Diagram produced by the Department of Health. (Reproduced under the terms of the Click-Use Licence.)

BMJ 2016;352:i563 doi: 10.1136/bmj.i563 (Published 10 February 2016)

ANALYSIS

An open letter to *The BMJ* editors on qualitative research

Seventy six senior academics from 11 countries invite The BMJ 's editors to reconsider their policy of rejecting qualitative research on the grounds of low priority. They challenge the journal to develop a proactive, scholarly, and pluralist approach to research that aligns with its stated mission

Trisha Greenhalgh *professor of primary care health sciences, Nuffield Department of Primary Care Health Sciences, University of Oxford, UK*, Ellen Annandale *professor, Sociology, University of York, UK*, Richard Ashcroft *professor of bioethics, Queen Mary University London, UK*, James Barlow *professor of technology and innovation management–healthcare, Imperial College Business School, UK*, Nick Black *professor of health services research, London School of Hygiene and*

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Some clinical and policy questions are best answered by the results of randomised controlled trials or other quantitative approaches, but other decisions and outcomes are more usefully informed by qualitative studies. Qualitative studies help us understand why promising clinical interventions do not always work in the real world, how patients experience care, and how practitioners think. They also explore and explain the complex relations between the healthcare system and the outside world, such as the sociopolitical context in which healthcare is regulated, funded, and provided, and the ways in which clinicians and regulators interact with industry.

The paradox of the clinical trial is that

it is the best way to assess whether an intervention works,

but is arguably the worst way to assess who will benefit from it

Mant D. Evidence and Primary Care

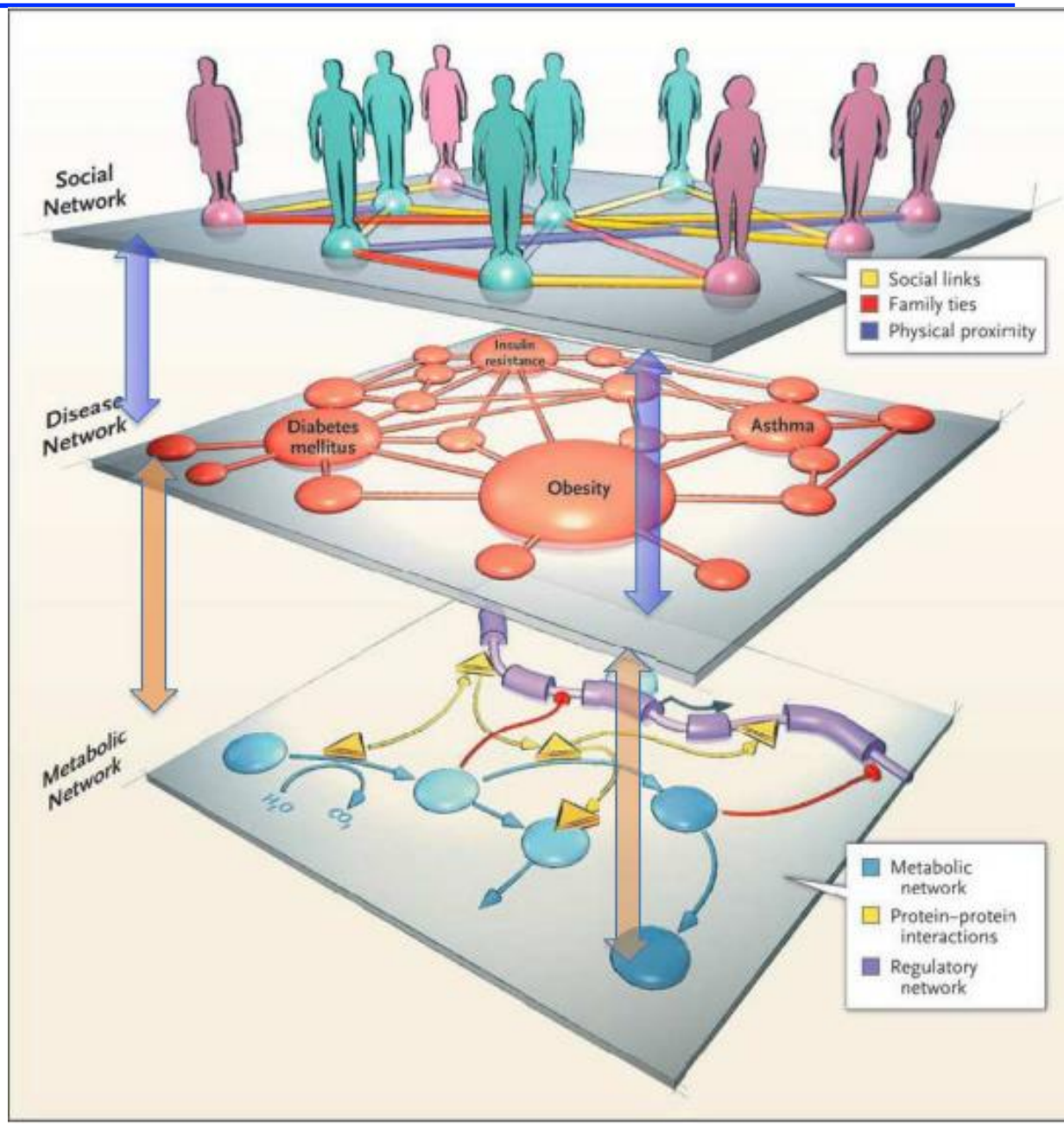
Can randomised trials inform clinical decisions about individual patients?

Lancet 1999; 353: 743-6.

Fenotipi complessi di malattia come rete di relazioni intra ed inter dimensionali

Nel modello classico
le manifestazioni di malattia
sono considerate
entità separate tra di loro,
eventualmente coesistenti
come comorbilità.

In realtà tali manifestazioni finali
sono spesso espressione
di complessi processi comuni,
iterativi, influenzanti
e perturbanti a vicenda



Network Medicine — From Obesity to the "Diseasome"

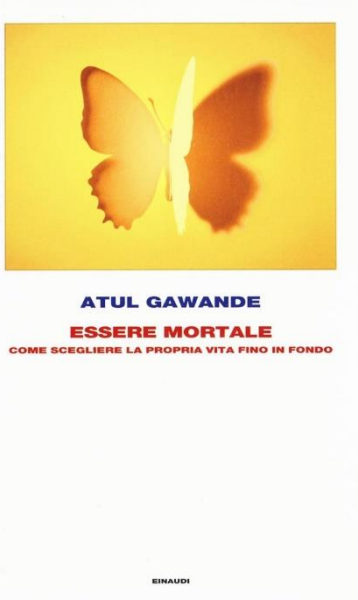
Albert-László Barabási
N Engl J Med 2007;357:404-7

ATUL GAWANDE

ESSERE MORTALE

COME SCEGLIERE LA PROPRIA VITA FINO IN FONDO

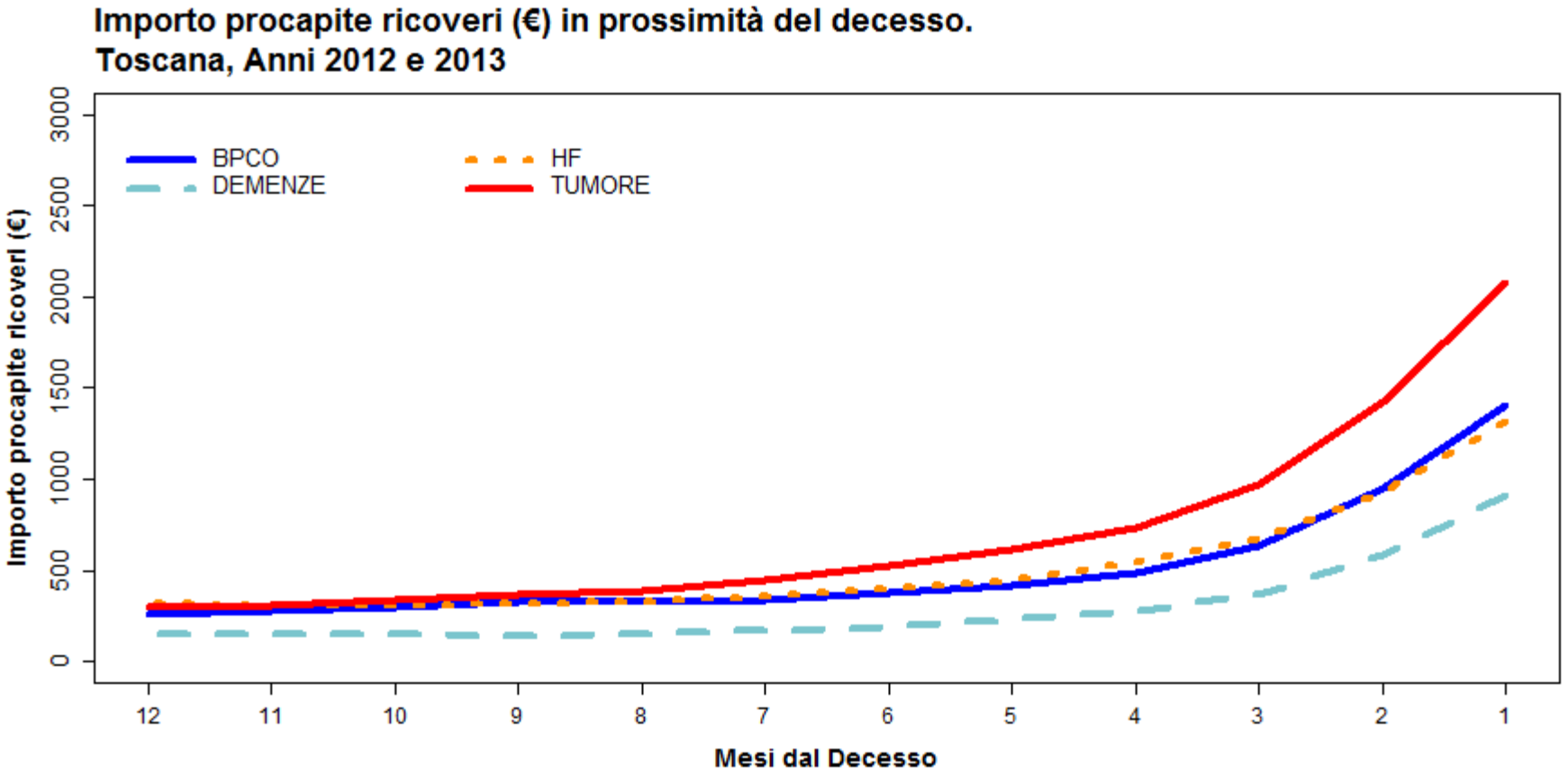
EINAUDI



Non occorre passare molto tempo con anziani o malati terminali per rendersi conto di quanto spesso la medicina abbandoni le persone che sarebbe tenuta ad aiutare. Gli ultimi giorni delle nostre vite restano affidati a cure che stordiscono la mente e minano il corpo in cambio di minime probabilità di beneficio. Li passiamo in istituzioni – case di riposo e reparti di terapia intensiva – dove un sistema regolamentato di procedure anonime ci isola da tutto ciò che c'importa in questa vita…abbiamo permesso che i nostri destini fossero controllati dagli imperativi della medicina, della tecnologia e di persone estranee.

Abbiamo costruito il sistema sanitario e la cultura medica attorno alla coda lunga delle curve di sopravvivenza, a quella lunga ma esigua coda di pazienti che non si comportano come la media, ma presentano sopravvivenze anche lunghe. Che c'è di male a cercare questa coda di possibilità? Niente, a meno che questo non significhi non aver preparato il paziente all'esito più probabile. Abbiamo costruito un apparato da molti miliari di dollari per dispensare l'equivalente sanitario dei biglietti della lotteria, mentre disponiamo soltanto di sistemi rudimentali per preparare i pazienti al fatto quasi certo che quei biglietti non verranno estratti.

Ricoveri H - costo pro capite



Cure palliative

L‘ insieme degli interventi terapeutici, diagnostici e assistenziali,

rivolti sia alla persona malata sia al suo nucleo familiare,

finalizzati alla cura attiva e totale

dei pazienti la cui malattia di base,

caratterizzata da un'inarrestabile evoluzione

e da una prognosi infausta,

non risponde più a trattamenti specifici

American College of Emergency Physicians®
ADVANCING EMERGENCY CARE

Emergency Department Palliative Care

Information Paper

Developed by Members of the
Emergency Medicine Practice Committee

June 2012

American College of Emergency Physicians
ADVANCING EMERGENCY CARE

Emergency Department Palliative Care
Information Paper

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June 2012

Patients with advanced and end-stage disease in need of symptom management and pain relief often present to the ED

Research focusing on patients who were at the end of life found that these patients often did not receive the care they anticipated

Once in the acute care setting the patient's objectives and goals may be in direct contrast to the ED strategies of life-prolonging treatment

The need for palliative care and end-of-life care in the ED becomes apparent when considering that these medically complex patients present to EDs every day

Providers of emergency care have a unique opportunity to support palliative care interventions early in a patients disease trajectory, promoting quality of life as well as reducing cost associated with treatments

The ED offers a solution to the large gap in outpatient services for these patients by providing access to multidisciplinary teams for assessment, planning and needed interventions 24 hours a day, seven days a week

American College of Emergency Physicians®
ADVANCING EMERGENCY CARE

Emergency Department Palliative Care
Information Paper

Developed by Members of the Emergency Medicine Practice Committee

June 2012

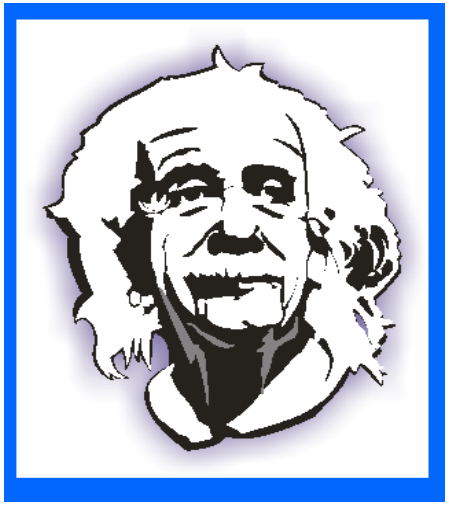
Recent literature suggests that palliative intervention in the ED provides numerous benefits:

- **timely provision of care**
- **improved outcomes**
- **direct referrals to hospice**
- **reduced hospital length of stay**
- **improved patient and family satisfaction**
- **less utilization of intensive care**
- **cost savings**

“We cannot solve our problems

with the same thinking

we used when we created them”



L’attuale paradigma scientifico riduzionistico

è incapace di riconoscere la «persona»,

la cui complessità bio-psico-psico-socio-ambientale

richiede un approccio scientifico nuovo,

basato su un radicale cambiamento epistemologico di tipo sistemico.