Real world data in beleidsbeslissingen in de gezondheidszorg

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The conflicting goals of healthcare policy

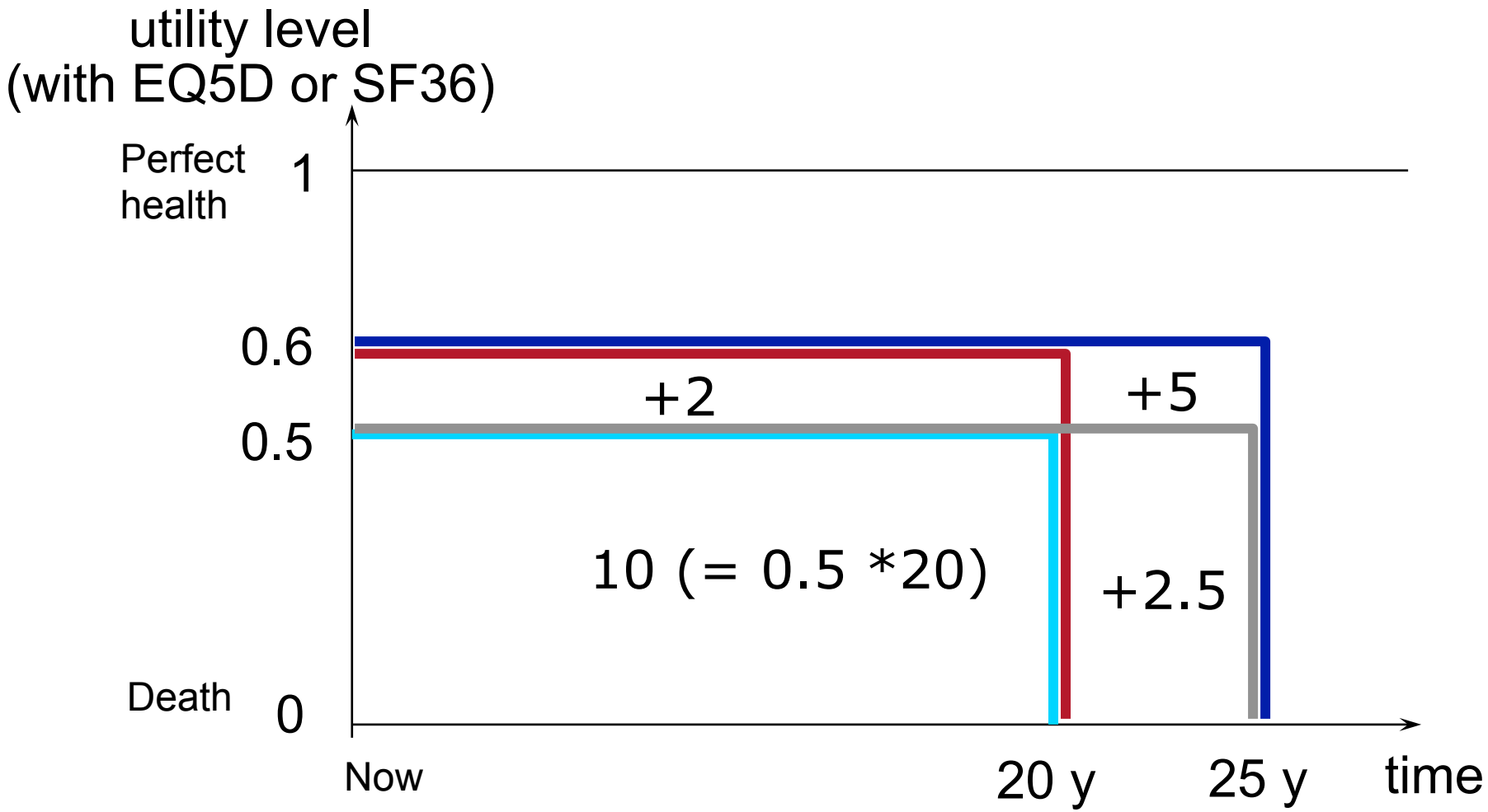
Healthcare systems need innovative treatments that:

- *Tackle important health needs*
- *Offer a benefit to patients and/or the healthcare system*
- *Are cost-effective and affordable within the healthcare budget*

TEIT

OECD 2003; Report of the Belgian EU Presidency, adopted by the EU Council of Ministers of Health in Dec 2010;
Commission February 2013; Available at: http://www.mloz.be/files/annemans_prijs_van_innovaties.pdf. Accessed J

Benefit to patients: QALY = Quality Adjusted Life Years



Annemans L, et al. Health economics for non-economists. Academia Pr

Example of utility scores

Item	Utility year 1	Utility year 2+
Acute Coronary Syndrome	0.672	0.824
Ischemic Stroke	0.327	0.524
Heart Failure	0.602	0.571

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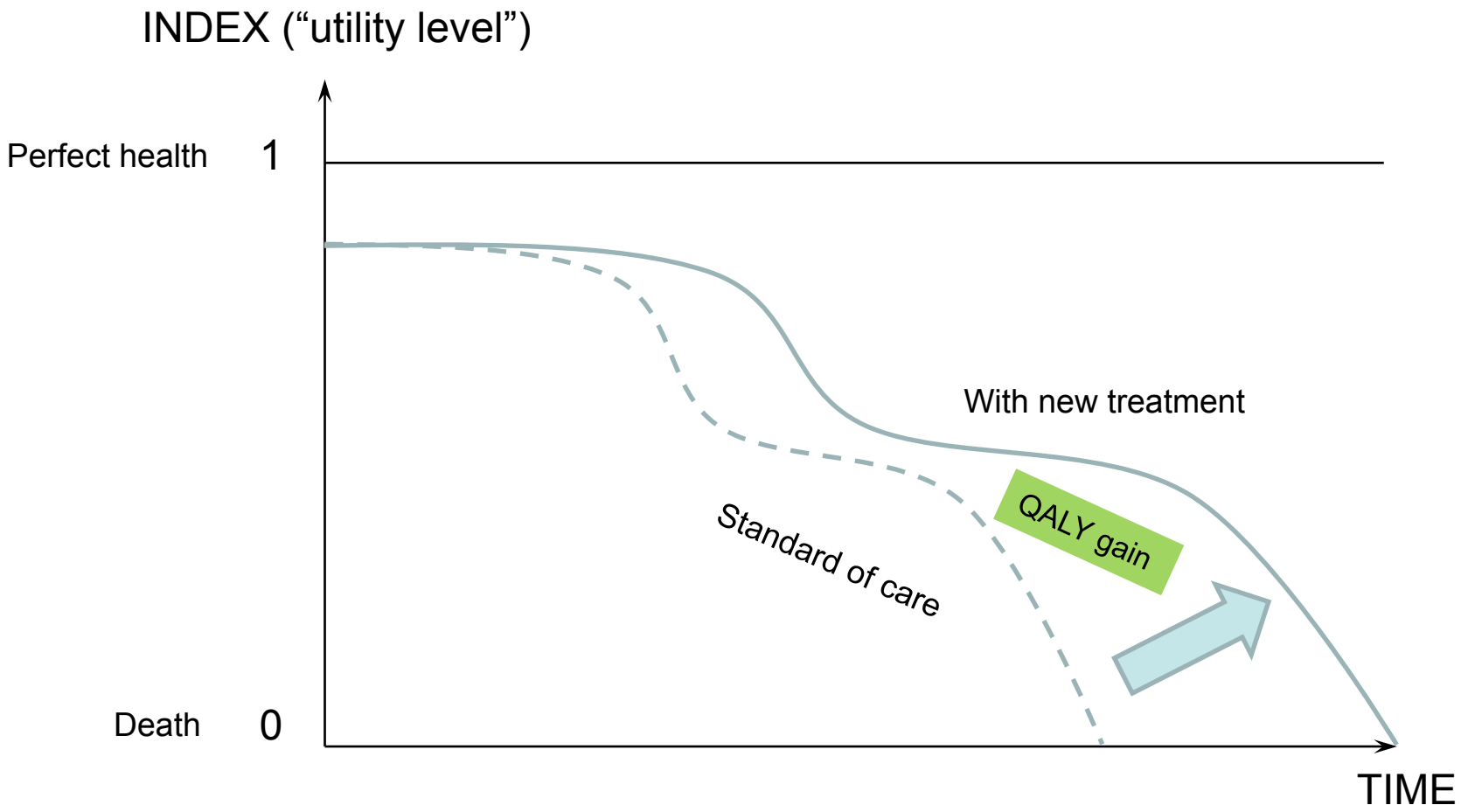
Villa G, et al. *Clin Ther* 2017;39:7

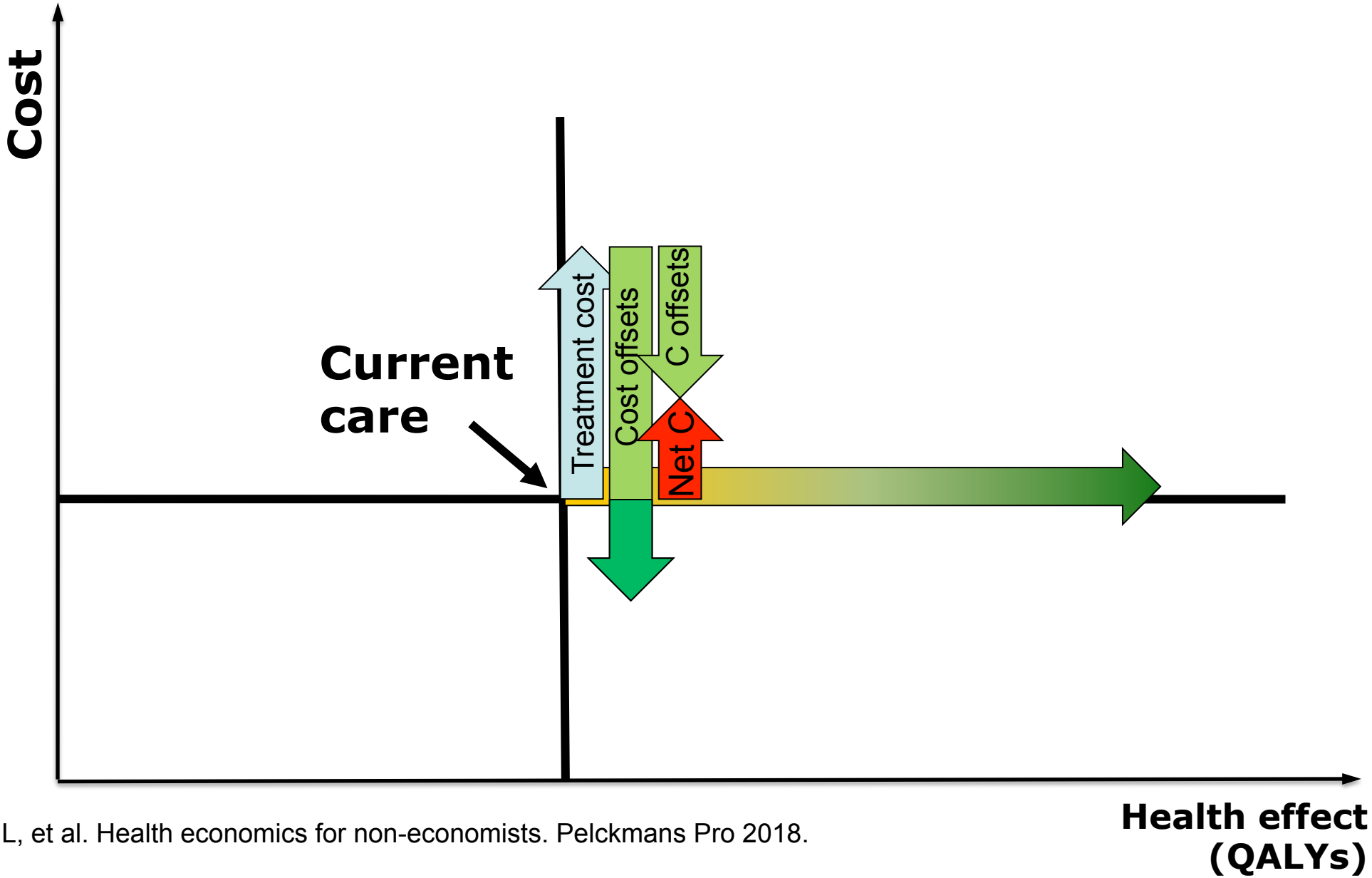
Gain in QALY by avoiding/delaying disease progression



Annemans L, et al. Health economics for non-economists. Pelckmans Pro 2018.

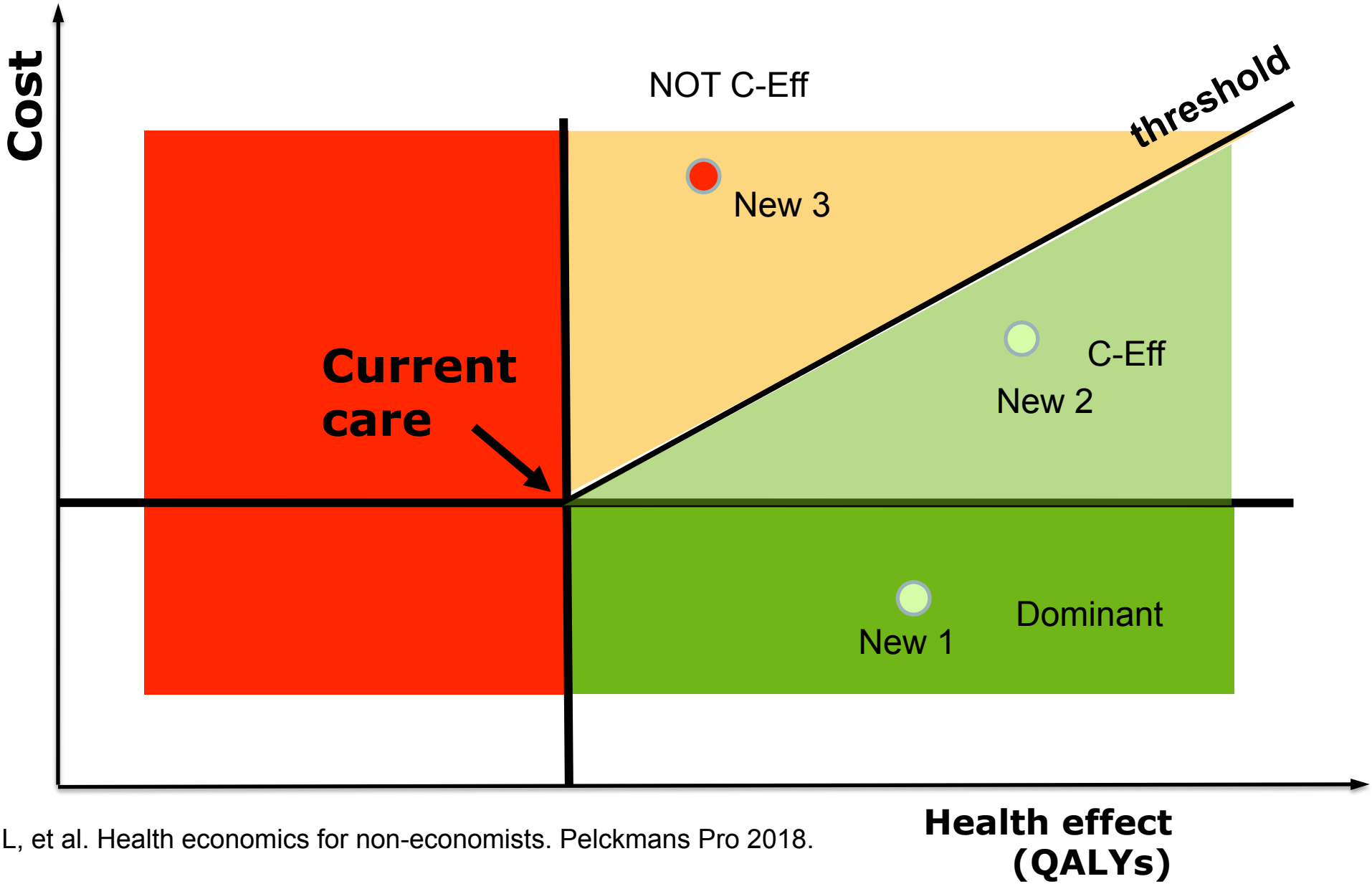
Great, but at what cost??

Combining costs and effects



Annemans L, et al. Health economics for non-economists. Pelckmans Pro 2018.

Cost-effectiveness



Annemans L, et al. Health economics for non-economists. Pelckmans Pro 2018.

Where is the threshold?

BENCHMARKING

e.g. cost-effectiveness of caring for a dialysis patient

historically **50,000 $ per QALY (+/- 200,000$ for 4 QALYs)**

(note: now +/- 100,000$ per QALY)

WHO - CHOICE **Highly cost-effective:** < GDP per capita);
(→ Belgium = +/- €37000)

(but recently challenged by the WHO)

At the discretion of the decision maker (England: 30,000£ per QALY)

(but recently challenged)

ncremental Cost Effectiveness Ratios (ICER) of selected interventions (Belgium)

Treatment	Cost per QALY gained (€)
Intensive cardiovascular rehabilitation in CHD	dominant
Procoralan Chronic Heart Failure	6,000
Total Hip Replacement	10,000
Brillique Acute Coronary Syndrome	14,000
Prezista HIV	16,000
Sovaldi HCV	18,000
Velcade multiple myeloma	30,000
Tysabri MS	47,000
Annual mammography for women aged 60-70yr	70,000
Annual CT for 60 year-old heavy smokers	130,000

CTG/CRM (RIZIV) (at official prices)

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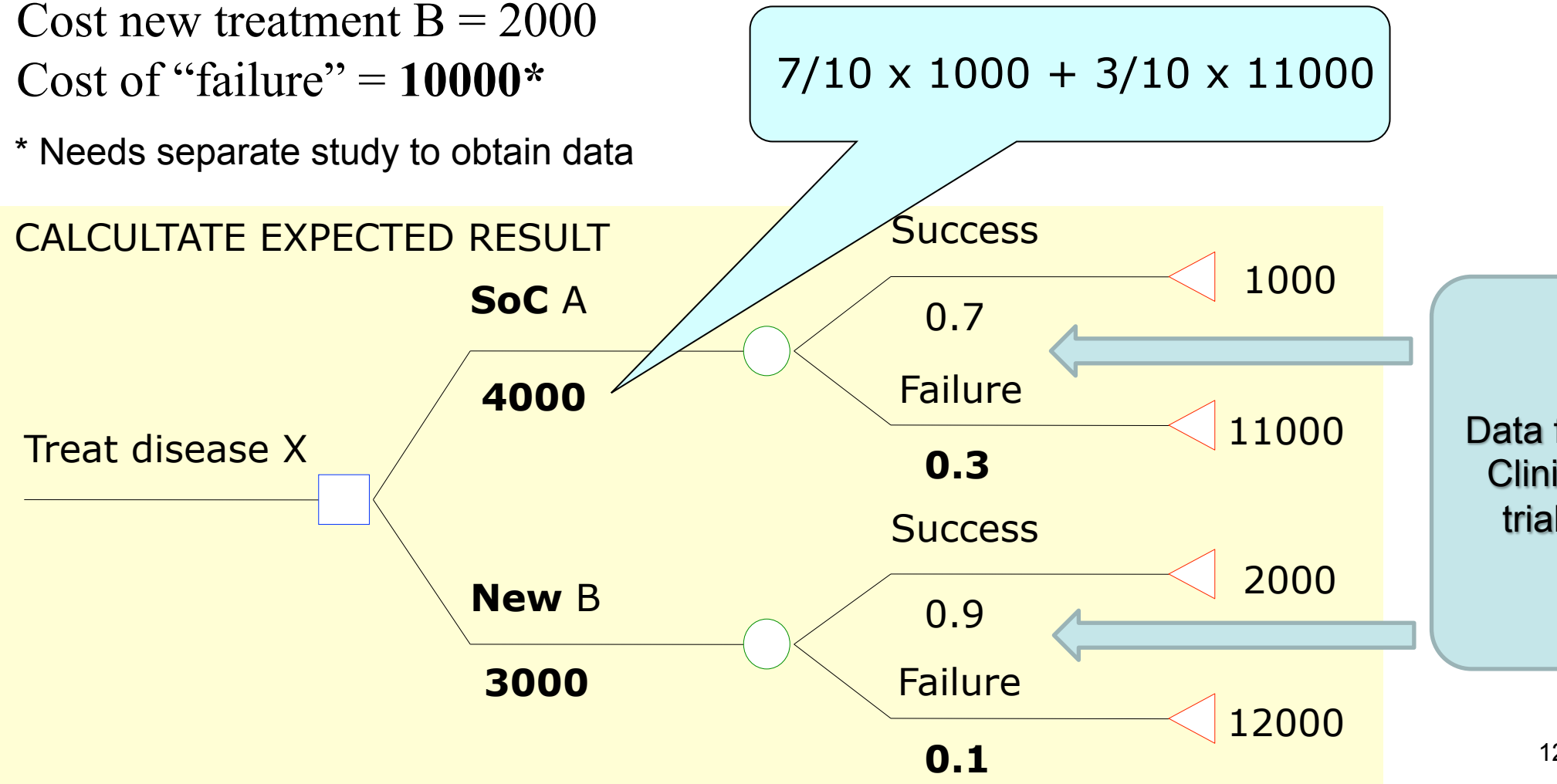
Available at: http://www.mloz.be/files/annemans_prijs_van_innovaties.pdf. J

Method = decision tree ; example

Cost treatment A (Standard of Care) = 1000
Cost new treatment B = 2000
Cost of "failure" = **10000***

* Needs separate study to obtain data



PROBLEM : Uncertainty

Why clinical trials are not doing the job

- Artificial patient population
- Artificial medical management
- Too short
- Participation Bias (Hawthorne)

Artificial population

This Issue Views 2,159 | Citations 5 | Altmetric 257

Viewpoint

July 2017

Overall Survival in Cancer Drug Trials as a New Surrogate End Point for Overall Survival in the Real World

Sham Mailankody, MB, BS[1]; Vinay Prasad, MD, MPH[2,3]

Author Affiliations

JAMA Oncol. 2017;3(7):889-890. doi:10.1001/jamaoncol.2016.5296

"Younger"
"Whiter"
"Less comorbidities"
"More motivated"
"Better socio-economic status"

Artificial medical management

Journal of Diabetes and Its Complications 31 (2017) 1188–1196

Contents lists available at ScienceDirect

Journal of Diabetes and Its Complications

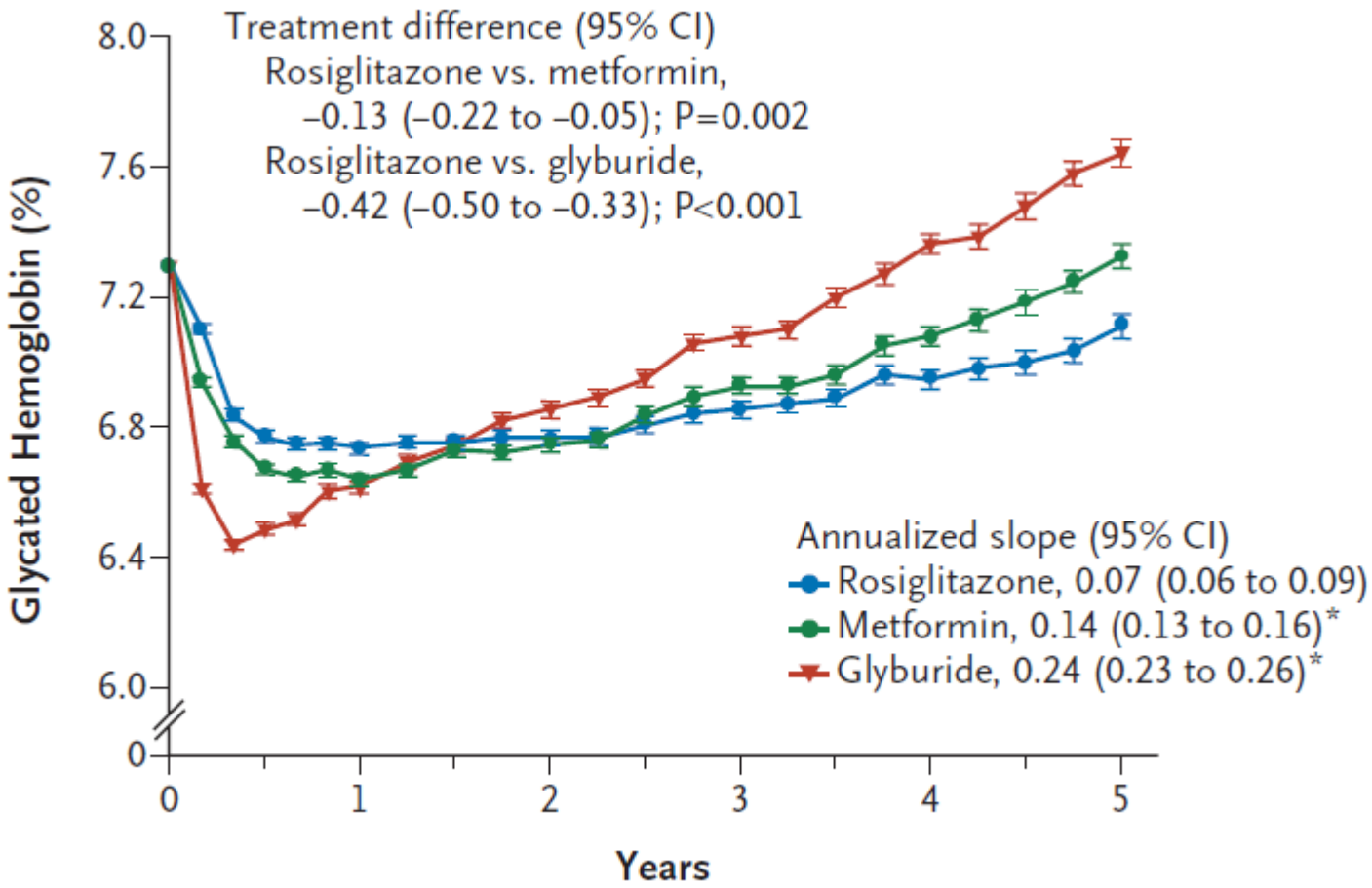
journal homepage: WWW.JDCJOURNAL.COM

Towards an improved global understanding of treatment and outcomes in people with type 2 diabetes: Rationale and methods of the DISCOVER observational study program

Linong Ji [a,*], Fabrice Bonnet [b], Bernard Charbonnel [c], Marilia B. Gomes [d], Mikhail Kosiborod [e,f], Kamlesh Khunti [g], Antonio Nicolucci [h], Stuart Pocock [i], Wolfgang Rathmann [j], Marina V. Shestakova [k,l], Iichiro Shimomura [m], Hirotaka Watada [n], Peter Fenici [o], Niklas Hammar [p,q], Kiyoshi Hashigami [r], Greg Macaraeg [s], Filip Surmont [s], Jesús Medina [t]

The key strength of our study is the naturalistic design of our study, whereby the chances of protocol-induced findings and protocol-induced healthcare resource use are minimized

Too short → need for longer studies



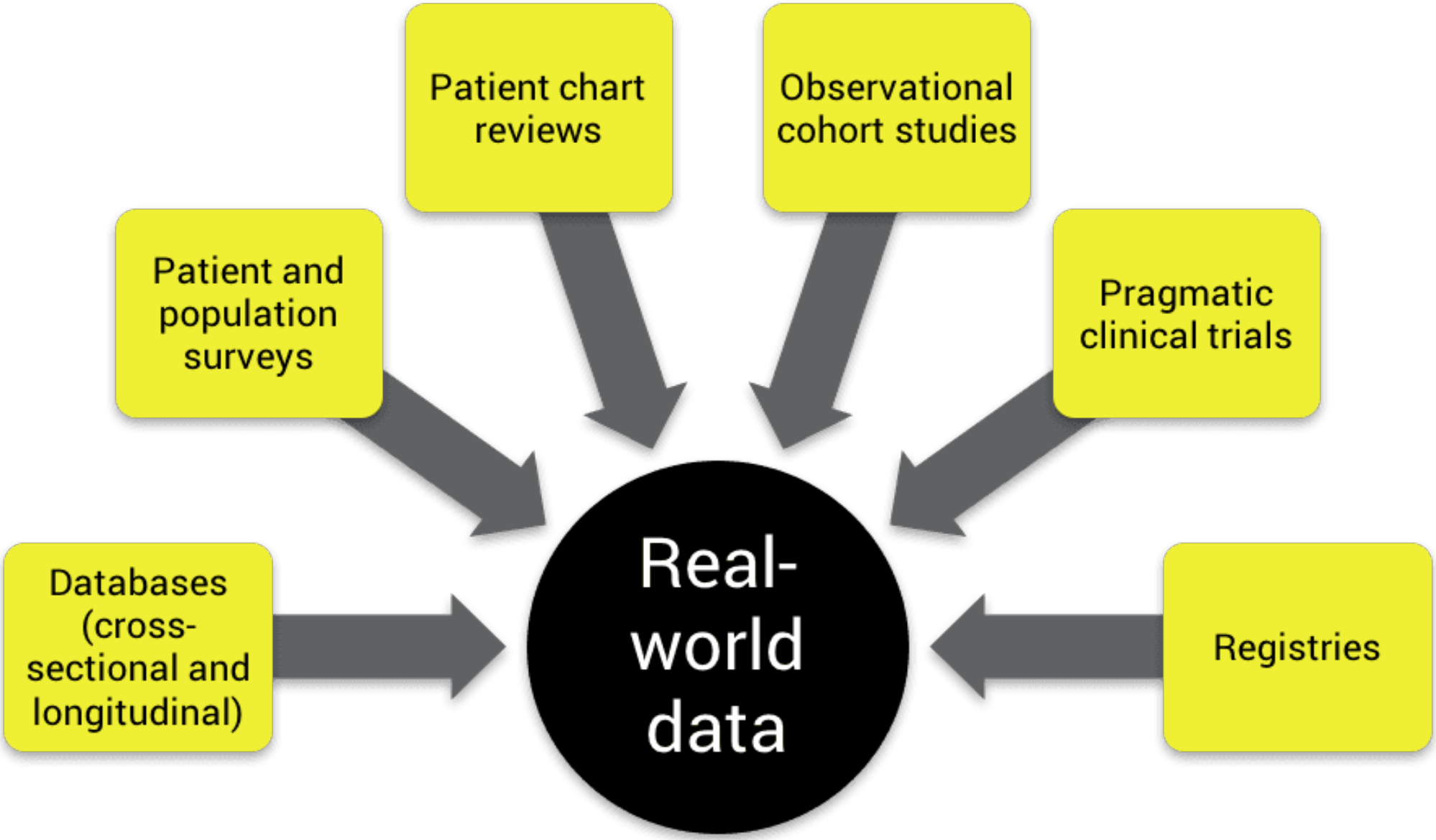
DOPT study, NEJM, 2006

The Hawthorne effect

- First published in 1933 based on experiments in the Hawthorne factory of the Western Electric Company.
- Looked at the effects of working conditions on the functioning and productivity of employees
- Whathever was done (e.g. increasing/ decreasing lights at the workfloor), the production increased
- The only explanation: workers started to feel “special”

SAME OCCURS IN PROSPECTIVE TRIALS

The reality is different

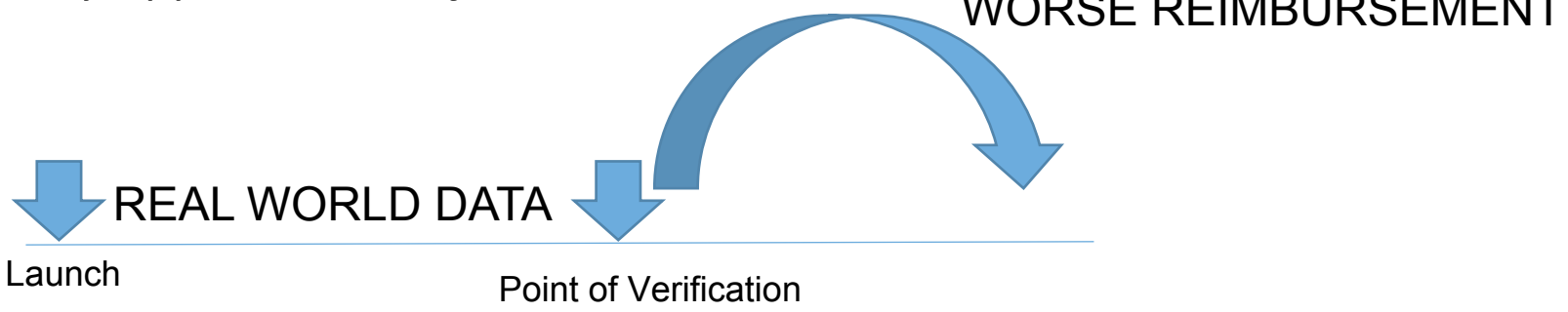

Patient chart reviews
Observational cohort studies
Patient and population surveys
Pragmatic clinical trials
Databases (cross-sectional and longitudinal)
Real-world data
Registries

Benefits of really real world data

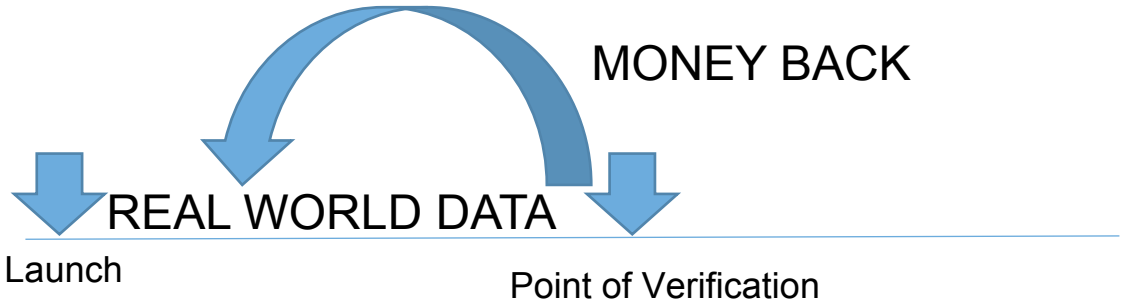
- Confrim or not the benefits of treatment
- As input into contracts with pharma
- To develop and improve clinical decision tools

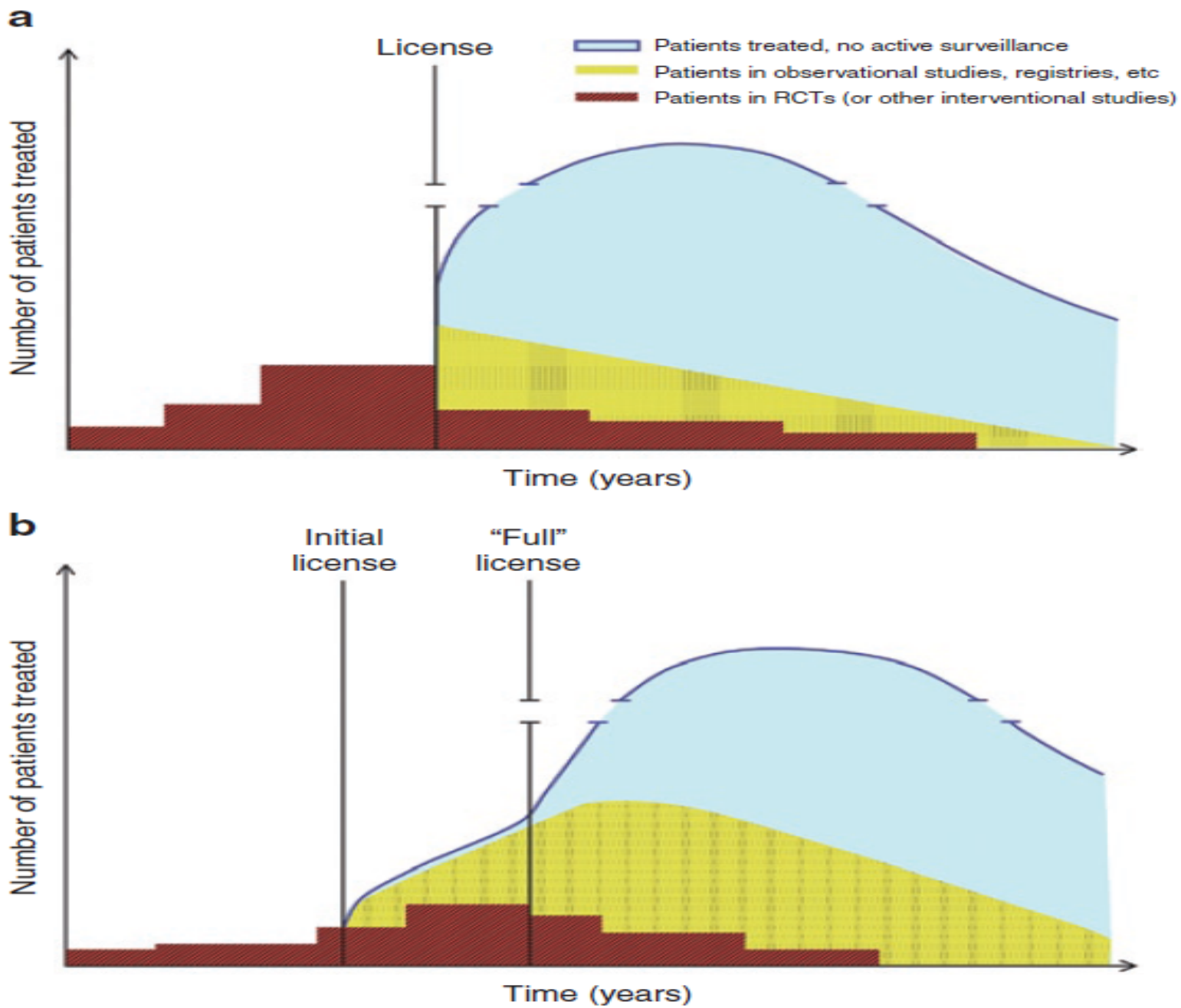
A new trend: outcomes based contracts!

1. Coverage upon evidence development
   - *Temporary approval, then final decision*



2. Performance Linked Reimbursement (outcomes guarantee)
   - *Not as good as promised → industry pays back*





Adaptive pathways → dynami process

et al. Clin Pharmacol Ther. 2012 Mar

Problems with Real world data

"pragmatic trial" → not really real life

Registries & observational cohort studies → expensive, administrative burden, still Hawthorne

Patient and population surveys: they tell you what they think they do but not what they do, plus selection bias

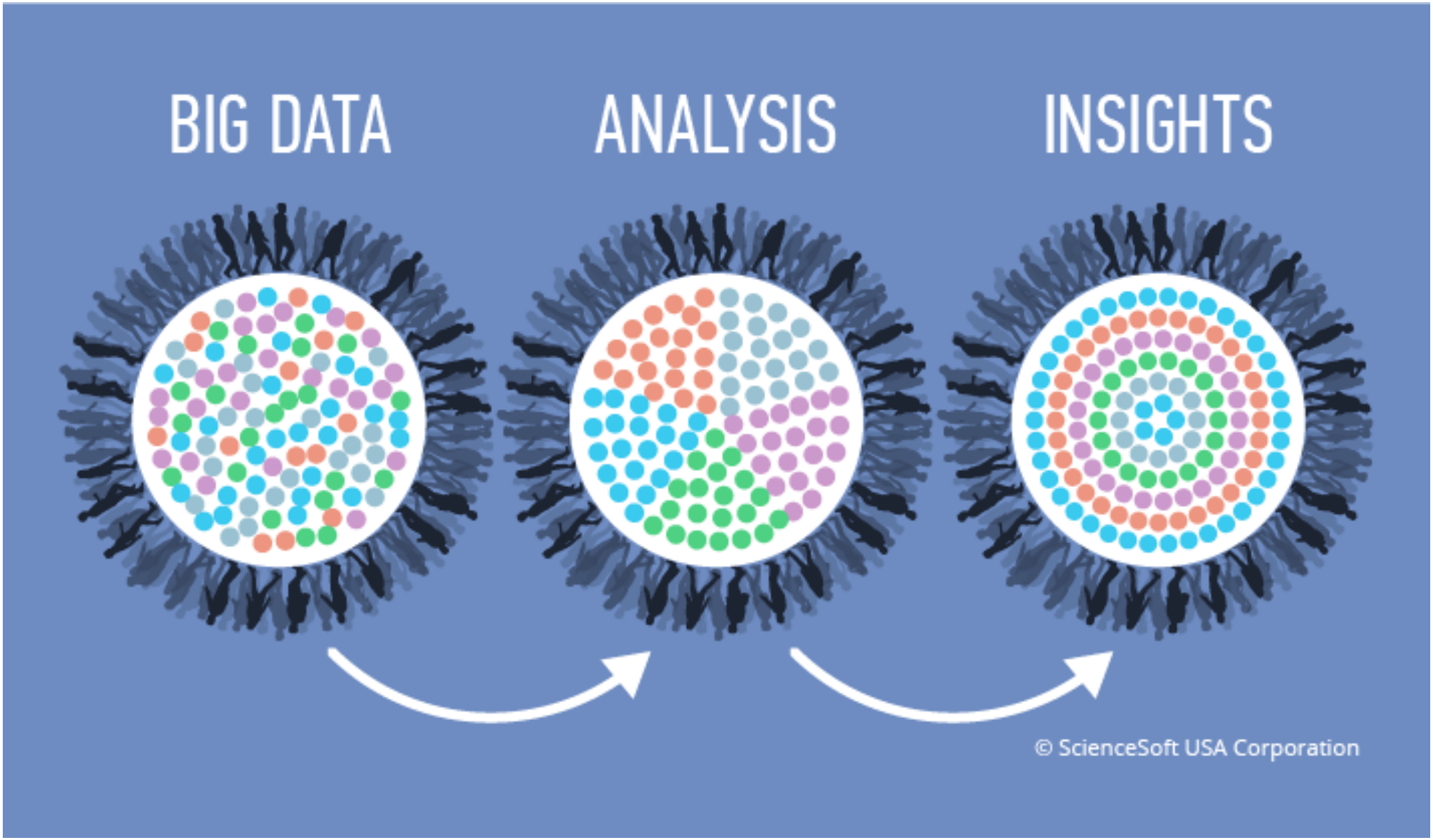
Patient chart review → really real life, but very resource intensive

Database of medical records: really real life, but possible data gaps

“ If you do not have
all information for all the patients , all the time
you are wasting your money ”

George Halvorson,
CEO,Kaiser Permanente

One step further…



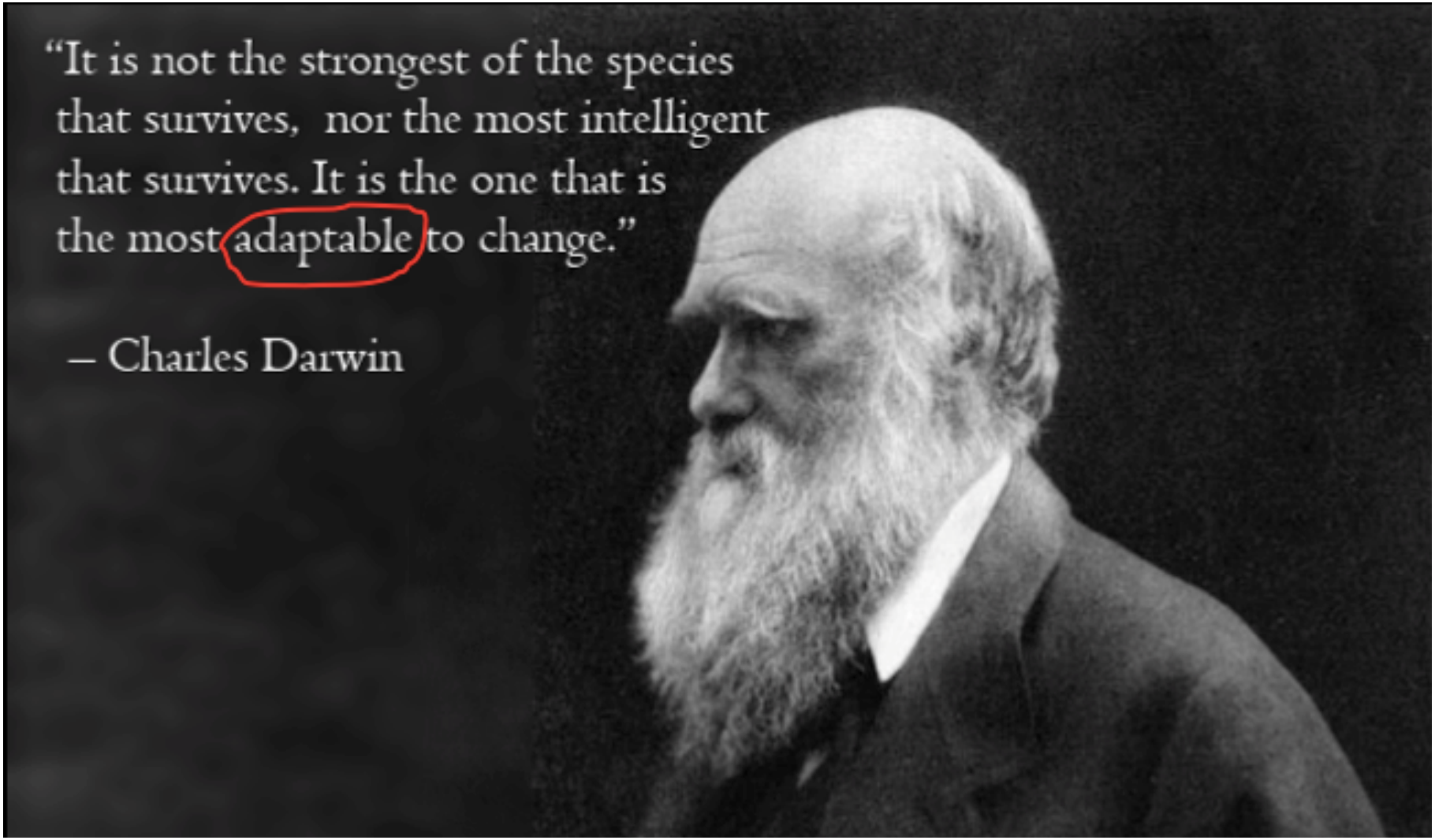
Current Osteoporosis Reports
https://doi.org/10.1007/s11914-018-0445-9

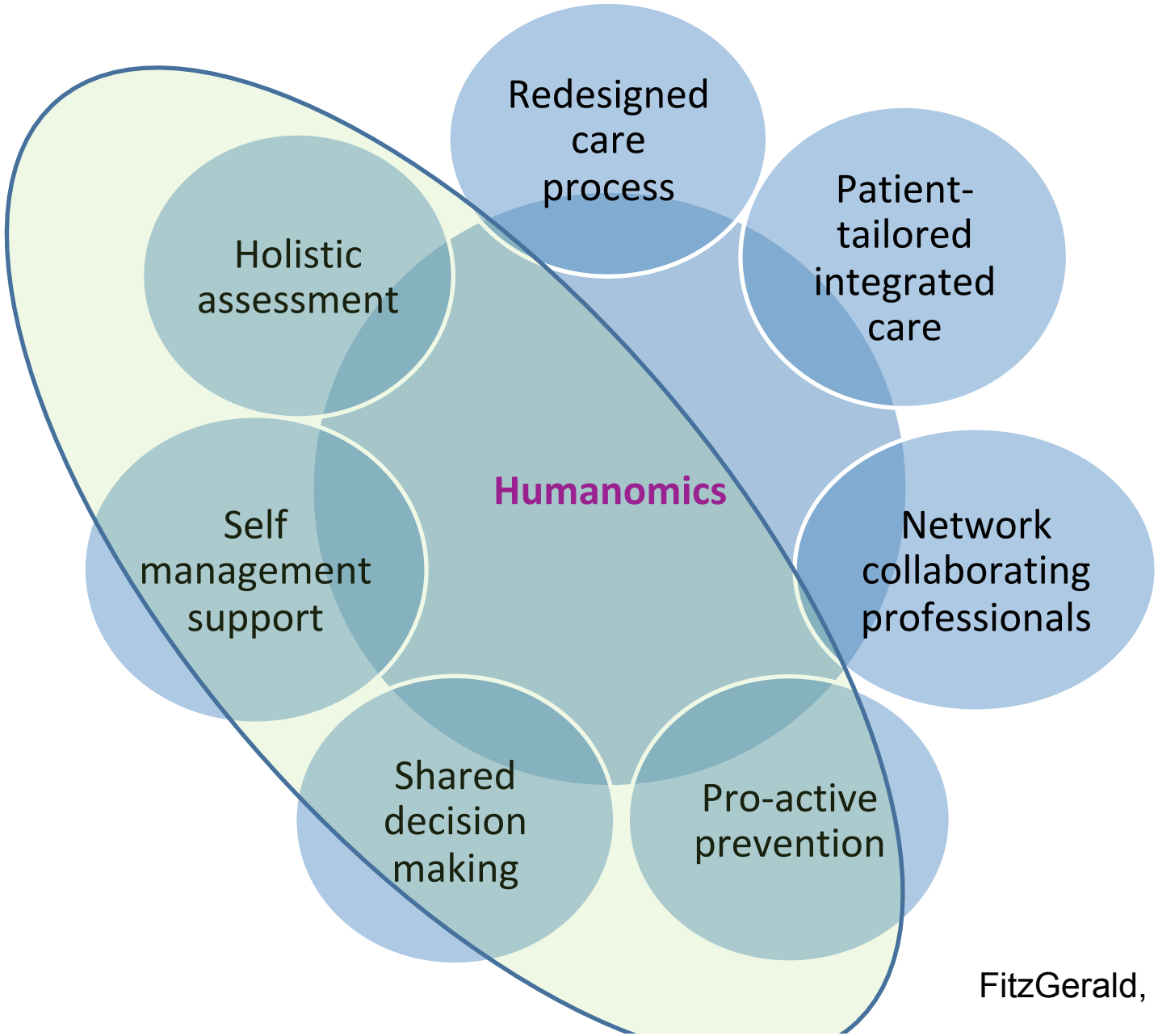
BONE AND DIABETES (A SCHWARTZ AND P VESTERGAARD, SECTION EDITORS)

The New Possibilities from "Big Data" to Overlooked Associations Between Diabetes, Biochemical Parameters, Glucose Control, and Osteoporosis

Christian Kruse [1,2,3]

What does it mean for us?


"It is not the strongest of the species
that survives, nor the most intelligent
that survives. It is the one that is
the most adaptable to change."
– Charles Darwin



FitzGerald, Chest 2014;146(1):10-

AAR
TEIT

EVERYTHING WILL
BE OK IN THE END.
IF IT'S NOT OK,
IT'S NOT THE END

John Lennon

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