Quali sono le *priorità* della Farmacoepidemiologia per supportare la politica del farmaco e dei vaccini?

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RESEARCH

Availability of evidence of benefits on overall survival and quality of life of cancer drugs approved by European Medicines Agency: retrospective cohort study of drug approvals 2009-13

Courtney Davis,[1] Huseyin Naci,[2] Evrim Gurpinar,[2] Elita Poplavska,[3] Ashlyn Pinto,[2] Ajay Aggarwal[4,5]

thebmj | *BMJ* 2017;359:j4530 | doi: 10.1136/bmj.j4530

5/10/2017

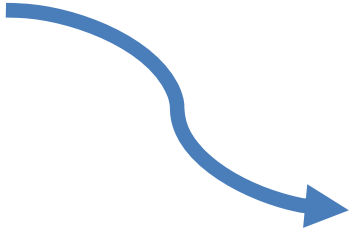
Cosa Dice

Farmaci approvati 2009-2013
43 farmaci per 68 indicazioni

Qualità delle evidenze
8 indicazioni (12%) approvati sulla base di *single arm studies*

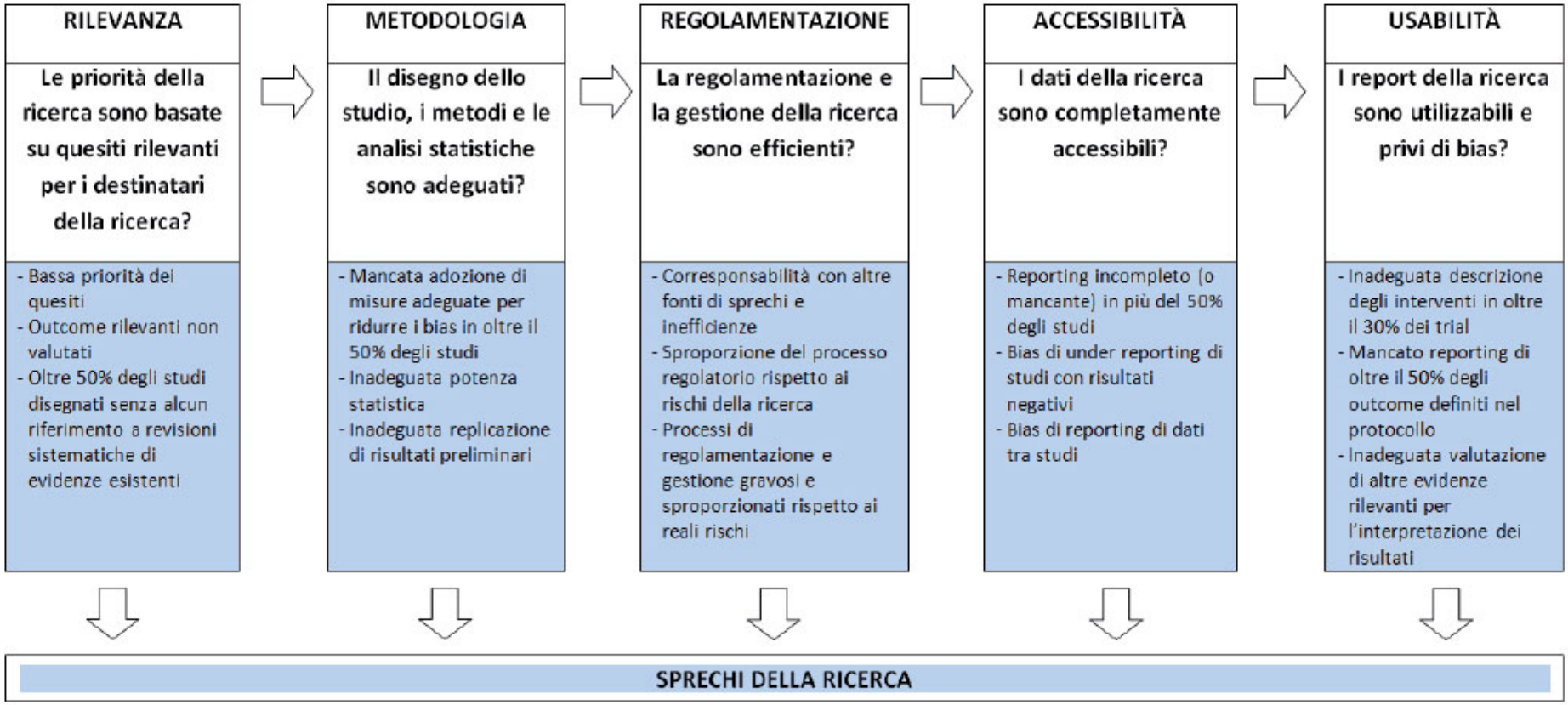
Qualità degli outcomes
❖24 su 68 (35%) aumento di sopravvivenza *at time of approval*
❖Benefit mediano OS 2.7 mesi (1,0 – 5,8)

Qualità della vita
7 su 68 (10%) dimostrato un miglioramento di qualità della vita *at time approval*



"At a minimum of 3.3 years after market entry, there was still no conclusive evidence that these drugs either extended or improved life for most cancer indications."

La ricerca ed il metodo scientifico nello sviluppo di un farmaco: criticità



Solidità/dati preliminari delle evidenze su cui si fondano RCTs

SPERIMENTAZIONE CLINICA E PERCORSO REGOLATORIO DEI FARMACI IN ONCOLOGIA : CRITICITA'

- Disegno sperimentale su format US e non EU: spesso standard of care non perfettamente sovrapponibili e quindi limiti nel disegno dello studio clinico

- Approvazione in fast track da fasi 1 e 2 con studi di espansione (pochi pazienti): manca spesso approvazione confermatoria da studi fase 3

- Studi clinici con 3 o più biologici/immunoterapici: non abbiamo piano di risk assessment e risk managment

- **Scarsa cultura di farmacovigilanza e di farmacovigilanza attiva**

BMJ 2017;359:j4528 doi: 10.1136/bmj.j4528 (Published 2017 October 05)

EDITORIALS

Do cancer drugs improve survival or quality of life?

You don't need to know, according to our broken regulatory system

Vinay Prasad *assistant professor of medicine*

Oregon Health and Science University, Portland, Oregon, USA

Real-world Data for Clinical Evidence Generation in Oncology.

J Natl Cancer Inst. 2017 Nov 1;109(11)
Khozin S[1], Blumenthal GM[1], Pazdur R[1].
Oncology Center of Excellence, Food and Drug Administration, Silver Spring, MD.

- Conventional cancer clinical trials can have limited external validity
- Difficult for patients to participate in.
- The use of real-world data (RWD) to improve current methods of clinical evidence generation.
- Sources of RWD outside of conventional clinical trials are important
- Original intent of data collected at the point of care can distinguish RWD from conventional clinical trial data

- Prospective collection of RWD can enable evidence generation based on pragmatic clinical trials (PCTs) that support randomized study designs and expand clinical research to the point of care.

- PCTs may help address the growing demands for access to experimental therapies while increasing patient participation in cancer clinical trials.

- RWD can support active pharmacovigilance, insights into the natural history of disease, and the development of external control arms

- Conducting valid real-world studies **requires data quality assurance** through auditable data abstraction methods and new incentives to drive electronic capture of clinically relevant data at the point of care.