Introduzione al corso di insegnamento: Sviluppo del Farmaco

L'ideazione di un farmaco è una avventura, ricca di colpi di scena e personaggi sempre in viaggio.

"..... l'ideazione dei farmaci, cosa li rende efficaci ma non pericolosi, cosa è buono e cosa è cattivo, cosa è medicina e cosa è veleno, cosa è giusto e cosa è sbagliato,"

"..... non potete iniettarvi con estrema moderazione il siero di dente di vipera del Gabon, né potete ricorrere a piccole punture di scorpione giallo per rimediare alle notti insonni."

"Tutto è veleno, e nulla esiste senza veleno. Solo la dose fa in modo che il veleno non faccia effetto. È la dose che fa il veleno". - Paracelso

Un nuovo farmaco: perché si

- -rapporto costo-efficacia più vantaggioso rispetto ad una tecnologia più vecchia
- -migliore efficacia sotto il profilo tecnico
- -potenziale riduzione dei tassi di complicazioni cliniche

Un nuovo farmaco: perché no

- -Incremento dei tassi di complicazioni
- -Incremento negli effetti collaterali
- -Riduzione dell'efficacia tecnica in comparazione con soluzioni alternative
- -Peggioramento del costo-efficacia

Evidence Based Medicine



Definition of EBM

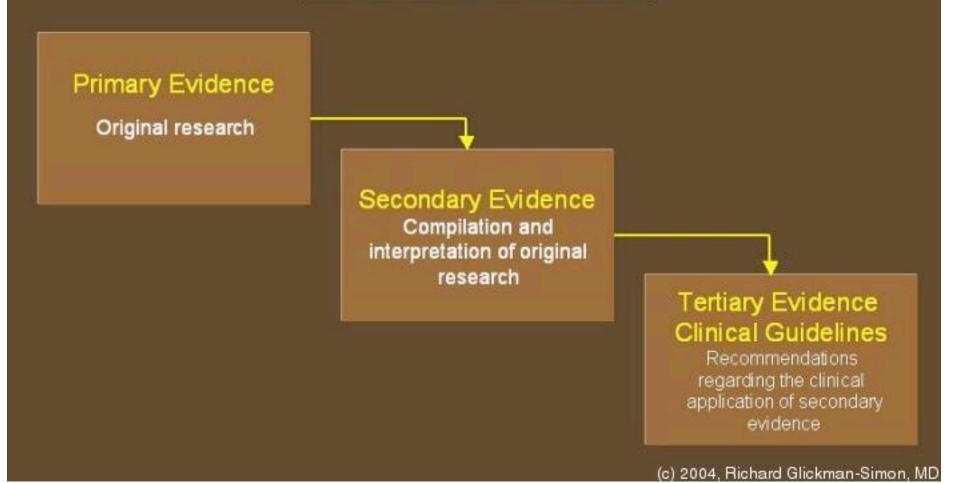
"Conscientious and judicious use of current best research evidence in combination with clinical expertise and patient values applied to the management of individual patients." Doctor (skills and clinical experience)

Patient (individual experiences and preferences)

External evidence (knowledge from studies)

What constitutes 'evidence' in evidence-based medicine?

Levels of Evidence



In the early 1990s, opinion-based medicine began to give way to evidence-based medicine

Opinion-based medicine

Physiciancentered EBM

Patientcentered EBM

- Relies on memory
- Emphasizes experience

- Popular since 1992
- Published clinical science
- Practitioner experience is important
- Acknowledges patient values

- Emphasize clinical evidence
- Focus on patient understanding
- Integrate physician experience with patient values

Formulate the question

What kind of patient or problem?

What intervention, treatment, diagnostic test, risk factor, or prognostic factor are you interested in?

What comparisons are you making (treatment A versus treatment B, treatment versus no treatment, etc.)?

Step 2

Search for answers

Does it work?

Has a systematic review been conducted (search Medline or the Cochrane Database)?

Are there RCTs that enrolled similar patients to yours?

If using guidelines, are they evidence-based or eminence-based?

Well formulated questions make it easier to locate an answer, if one exists.

Step 3

Appraise the evidence

Will it work in the "real world"?

Is it relevant to your question and your patient?

Is the statistically significant result clinically significant?

If effect size is not mentioned in the research report, is there sufficient information available to calculate the NNT for the categorical outcomes of interest?

Step 4

Apply the results

Is it worth it?

Is the intervention, treatment, diagnostic test, etc., important to you within the context of your clinical experience and important to the patient in terms of their preferences?

Step 5

Assess the outcome

Did you ask the right question?

Did you find answers?

Were the answers you found based on a highquality level of evidence?

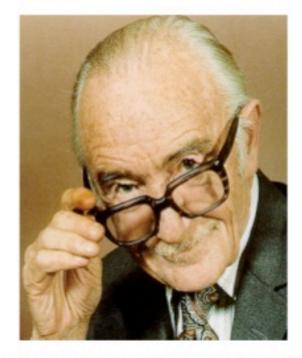
Did it make clinical sense?

Did it make a difference?

Can you quantify this?

Does the patient agree?

Archie Cochrane



"It is surely a great criticism of our profession that we have not organised a critical summary, by specialty or sub-specialty, adapted periodically, of all relevant randomized controlled trials"