Research Briefing

The “Efficacy-Effectiveness Gap”: historical background and current conceptualization (D2.1)


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Context

The concept of the “efficacy-effectiveness gap” (EEG) has started to challenge confidence in decisions made for drugs, when based on Randomized Controlled Trials (RCTs) alone. Launched by the Innovative Medicines Initiative, the GetReal project aims to improve understanding of how to reconcile evidence to support efficacy and effectiveness and at proposing operational solutions.

Objectives

The objectives of the present narrative review were:

(1) to understand the historical background in which the concept of the EEG has emerged;
(2) to describe the conceptualization of EEG

Methods

A focused literature review was conducted across the grey literature and articles published in English reporting insights on the EEG concept. The identification of different “paradigms” was performed by simple inductive analysis of the documents’ content.

Findings

The literature on the EEG falls into 3 major paradigms, where EEG is related to: (a) real-life characteristics of the healthcare system; (b) the method used to measure the drug’s effect; and (c) a complex interaction between the drug’s biological effect and contextual factors.

Conclusions

The third paradigm provides the opportunity to look beyond any dichotomy between “standardized” vs “real-life” characteristics of the healthcare system and study designs. Namely, future research will determine if the identification of these contextual factors can help to best design RCTs which provide better estimates of drug effectiveness.
Reference