Research Briefing

A Conceptual Framework for understanding effectiveness and its assessment before launch (D2.1)

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Rationale
The main objectives of WP2 were (1) to better understand the gap between efficacy and effectiveness and (2) to provide tools to address it during the drug development process. To this end, a conceptual framework was built, defining effectiveness and providing operational solutions to explore and identify drivers of effectiveness.

Contextual Framework
Effectiveness is the result of interactions of different contextual factors, on the efficacy of a drug – interaction being used in the broad sense of “interaction” and “effect modification”. Effectiveness may be measured when all interactions are at play. Three levels of interaction are considered (Figure):

- The patient/disease-related characteristics
- The real/actual use of drug
- The healthcare system-related characteristics

Figure. Contextual factors related to the actual use of drug, patients and disease as well as the healthcare system interact with the efficacy of drugs
Drivers of effectiveness

Drivers of effectiveness are the contextual factors related to the patients/disease, the actual use of drug or the healthcare system which interact with the drug’s pharmacological effect.

If drivers of effectiveness are not correctly taken into account during the drug development research (e.g., in randomized-controlled trials), the effectiveness of the drug measured in routine clinical practice may differ from what was measured during the drug development hence an “efficacy-effectiveness gap” (EEG).

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