

Examples of Proof of Concept studies in clinical development

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Background: Proof of Concept (POC) studies have been used in clinical drug development and this approach has gained a lot of interest in the recent past because of its potential to increase efficiency in early stages of clinical drug development and in general, to reduce risk in drug development.

Objectives: The purpose of the presentation will be to review several examples of POC studies that were performed at a large pharmaceutical company so the objectives, general principles and issues around POC studies are understood.

Results: Pharmacological POC studies are used in early clinical development (2 examples of studies will be presented), whereas clinical POC studies in patients take place later, typically right after the studies in healthy subjects (2 examples). For New Chemical Entities (NCE) that belong to a new pharmacologic class/new target, POC studies are usually performed to provide evidence for efficacy (2 examples). For NCE's that are not first in their pharmacological class, the main purpose is to provide evidence for differentiation (2 examples). A wide range of tools and methods (e.g., Positron Emission Tomography, Modelling and Simulation) are used during the conduct or evaluation of these POC studies.

Conclusion: Proof of Concept studies are essential for early clinical validation and identifying promising targets and projects. They should provide an active reduction in uncertainty on key development risks and should be designed to allow decision-making.