

Sex differences in patient reported outcomes used in phase 3 trials of medicinal products

Background: The evaluation of a medicinal product's effects is often mainly based on hard clinical outcomes (except when treating experiences like pain or dyspnoea). However, attention for the effects on so-called patient-reported outcomes (PROs) is increasing. PROs are distinct from traditional endpoints in the fact that they are based entirely on the impressions of patients, without interpretation of any other person. PROs are often used in clinical studies, with >50% of the assessed medicinal products including them as outcomes, even if rarely as primary endpoint. Recent studies have shown that women report significantly more often adverse events (AEs) than men, as well as worse quality of life (QoL) outcomes in various diseases, such as thyroid disease, disease related to the digestive system, haematologic malignancies, or colorectal cancer. Currently, however, little is known about sex differences in PROs used in dossiers supporting regulatory decision-making.

Objective: This study aims to assess sex differences in PROs in phase 3 trials of human medicinal products.

Method: We will include human medicinal products that received marketing authorisation by the European Commission from 1 January 2018 for which a previous study found that a PRO was used as an endpoint in the phase 3 trial. Information regarding PROs included in phase 3 trials will be extracted from European public assessment reports (EPARs) and clinical study reports. PRO-related information extracted will include the specific PRO, the specific PRO measure (PROM), and PRO scores at baseline and end of the main study period. Differences in scores by sex (men versus women) will be examined at baseline and end of study, for active and placebo treatment. Additionally, differences will be examined with regards to change of score over time, impact of score change, and clinical relevance of differences.

Impact: The importance of including PRO data has grown in the last years, as well as the importance of including and acknowledging sex differences in treatment outcomes. In-depth knowledge regarding these topics could provide background information about sex-specific effects on PROs, contributing to more targeted approaches to improve care.