

# SUPPLEMENTING PUBLISHED EVIDENCE WITH STRUCTURED OBSERVATION OF CASES FROM A CLINICAL AUDIT REGISTRY: A PILOT

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## Introduction

Developing high-quality guidelines for rare diseases has been proven challenging. Sound guideline methodology can significantly contribute to the quality of clinical guidelines and provides structure to the development process. Most methodological strategies for clinical guidelines rely on the availability of high quality evidence. As evidence is often lacking in rare diseases, many guidelines are build off experts opinions and lack structured methodology and sound justification for their recommendations.

*Exploration of innovative methodological strategies for rare disease guidelines is therefore imperative.*

## Innovative methodological strategy: Evidence Supplement

Presentation of raw, uncorrected case data from the *European Pediatric Surgery Audit (EPSA)*, specified to the guidelines PICO questions. Experts rate their perceived effectiveness per outcome in a structured observation form as suggested by Pai et al.(1), see figure 1. We chose to pilot the use of structured observation forms for scarce registry data, as opposed to self collected patient data by guideline panel members. Structured observation forms were considered as supplement to the published literature (if available). The ERNICA guideline on omphalocele was chosen as the pilot project. Omphalocele is a rare congenital abdominal wall defect with high practice variability within our network.

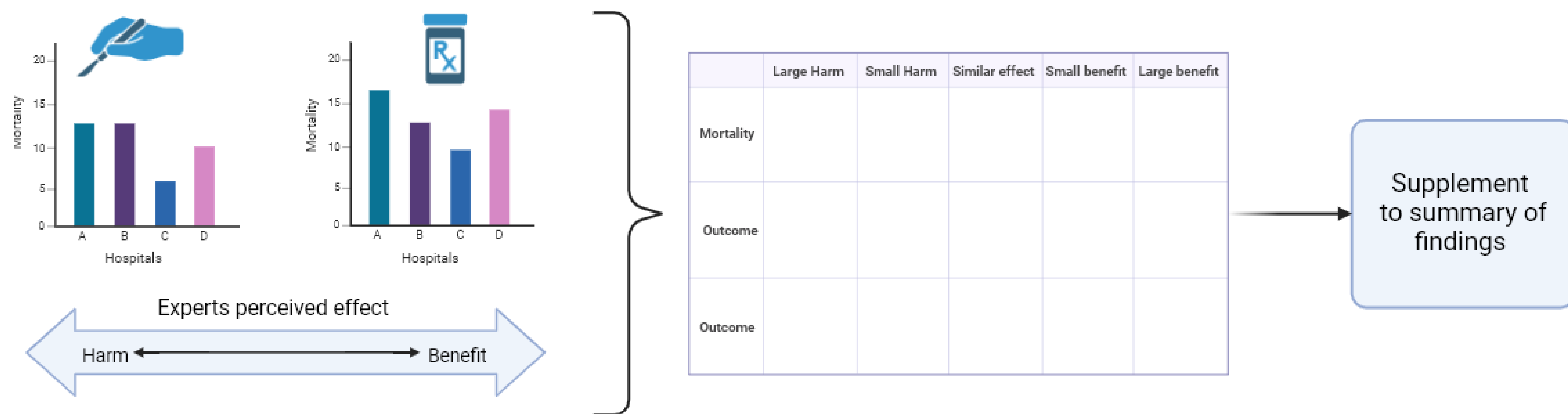


Figure 1. Use of EPSA data as supplementary evidence

## Results

The EPSA provided supplementary data for 7 out of 12 prioritized PICO questions. In total, data on 225 patients with omphalocele was entered in the registry. Due to the specificity of the PICO questions, the remaining samples for most questions were small. Structured observation forms were completed by 21 out of 26 panel members. Overall, panel members were quite homogeneous in their perceived effect but noticed difficulties with interpretation because of the small samples and amount of missing values. Mortality was selected as an important outcome by panel members, but when data was presented, most panel members indicated their perceived effect as 'Don't know' because data from the EPSA did not provide a cause of death. During a face to face meeting where recommendations were formed according to the Evidence to Decision Framework (2), *the EPSA data significantly contributed to the recommendation in 4 PICO's*. Evaluation of results versus added value of the evidence supplement and additional costs is ongoing.

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## ERNICA guideline on omphalocele

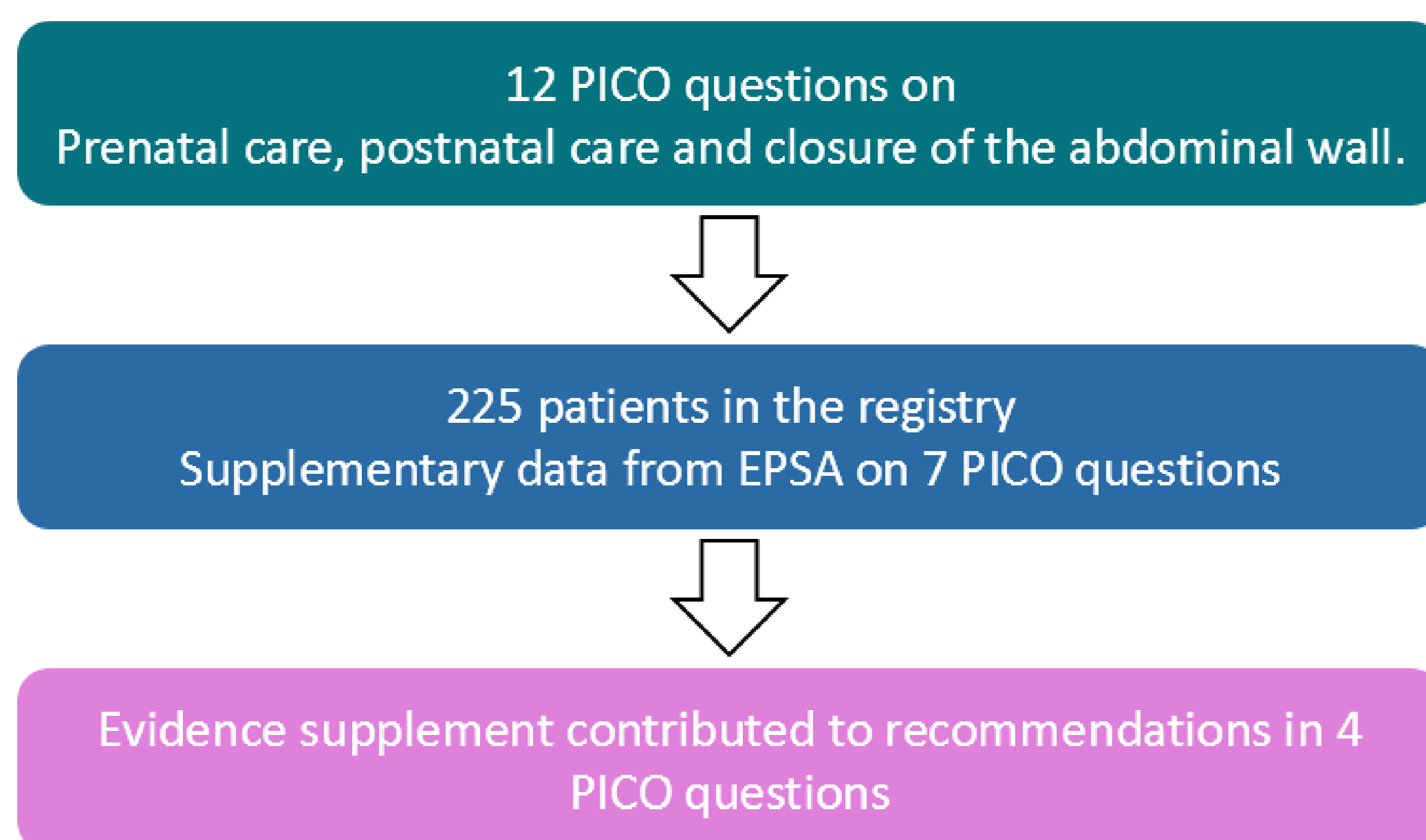


Figure 2. Pilot results

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