Exploring the Landscape of Patient-reported Outcomes in PCOS clinical trials

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Background

- Polycystic ovarian syndrome (PCOS) affects approximately 8-13%[1] of women of reproductive age and is a leading cause of infertility.
- While PCOS is known for affecting reproductive health, it is also associated with other health issues that affect patients' physical health (e.g., acne/skin issues and excessive hair growth) and emotional health (e.g., anxiety and depression).
- Despite their prevalence, PCOS symptoms or experiences expressed by patients (cramps, bodily pain (not related to menstruation), heavy bleeding, and bloating) may not be routinely assessed by clinicians who treat PCOS[2]. Therefore, integrating PROs into clinical practice can help capture treatment benefit/experiences that were not clinically expected.
- There is currently no treatment that has been approved to treat PCOS – medications have been used to treat individual symptoms of PCOS such as Eflornithine for hirsutism[3] and Ethinyl estradiol (for menstrual disturbances)[3], and both have included PROs in their labels.
- This review aimed to evaluate the landscape of patient reported outcomes (PROs) and concepts measured in PCOS clinical trials.

Methods

Figure 1: Overview of methodology

Performed structured review of ClinicalTrials.gov (including terms "Polycystic Ovary Syndrome" and "PCOS") to identify Phase 2-4 active (not enrolling) or completed interventional trials.

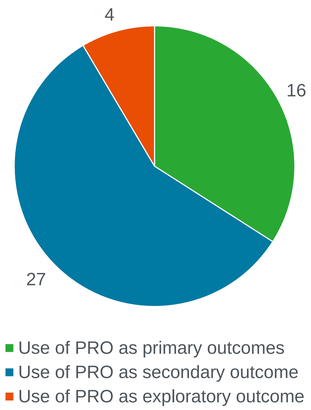
Data on (i) primary, secondary, and exploratory endpoints and measures, (ii) study phase, and (iii) start date were extracted from the resulting studies.

Studies including PROs were identified and trends on frequency, types of PRO measures, domains measured, phase distribution, and study start date were analyzed.

Results

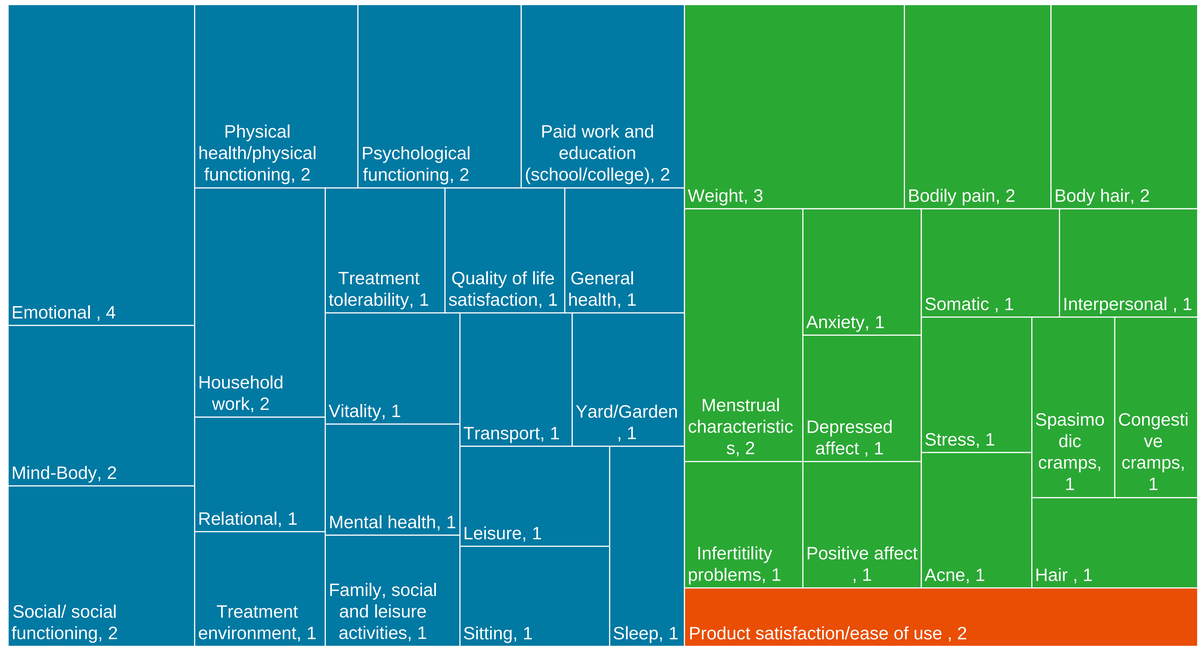
- 172 total studies were identified in the review, of which only 43 included PRO measures.
- Most endpoints were supported by clinical markers of reproductive health (pregnancy/ovulation rate or hormone levels) or BMI/weight.
- Most PROs supported primary and/or secondary endpoints (n=16 and n=27 respectively) (Figure 2).
- The most common type of PRO included were symptoms/symptom burden measures (n=31), of which almost half (n=14) were Phase 4 trials (Figure 4).
- The most frequently utilized PRO instruments were unspecified/general menstruation questionnaires (n=30). This was followed by the PCOS-QOL (n=6); other studies referenced quality of life (QoL) instruments, such as SF-36 (n=2), ChiQoL (n=1), FertiQoL (n=1), QoL VAS (n=1), or mentioned QoL PROs but were not specific (n=3).
- Among QoL questionnaires (n=10), most contained items measuring emotional function (n=4) (Figure 3). Other PROs identified assessed symptom/symptom burden measured areas of weight/BMI (n=3), menstrual characteristics (n=2), and bodily pain (n=2).

Figure 2: Distribution of PRO measures in used as primary, secondary, and exploratory outcomes in PCOS trials*



*Counts are not mutually exclusive

Figure 3: Domains measured by PRO measures included in PCOS trials*



*The size of and numerical value in each box corresponds to the frequency of the specific domain being measured in PRO measures.

Figure 4: Distribution of trials including PRO measures stratified by type of PRO and trial phase

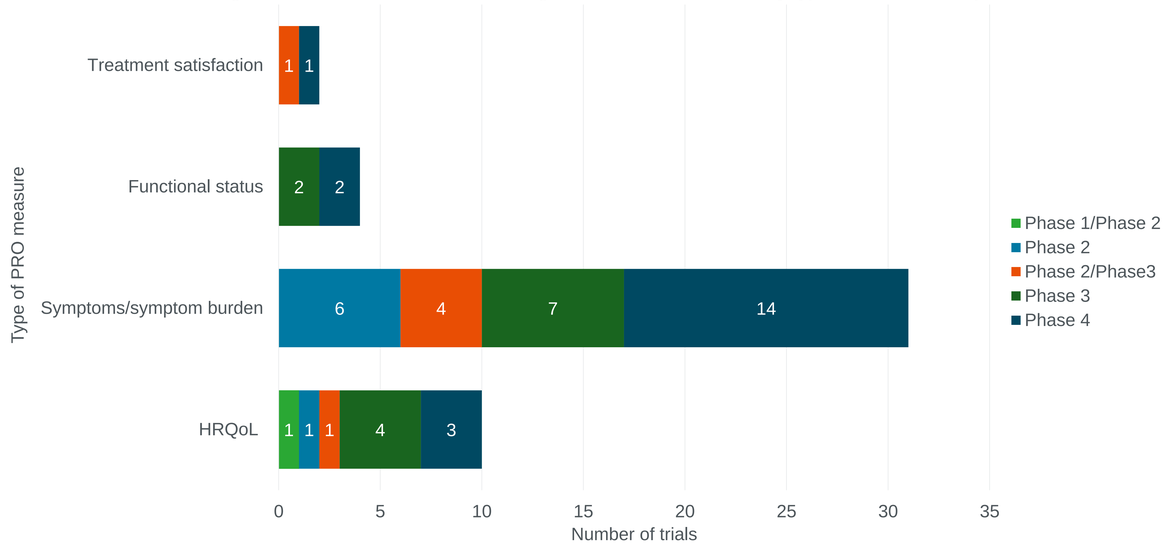


Figure 5: Frequency of commonly included PRO measures (n≥2)

- Unspecified menstruation questionnaire (n=29)
- PCOS-QOL/PCOS-Q/PCOS Questionnaire (n=6)
- Quality of Life questionnaire (n=3)
- Sleeping questionnaire (n=3)
- Structured questionnaire to assess perceived stress questionnaire (n=2)
- Zung SAS questionnaire (n=2)
- Zung SDS questionnaire (n=2)
- SF-36 (n=2)
- ChiQOL (n=2)

Figure 6: Comparison of trials including PRO measures before and after the 2009 FDA PRO Guidance*

*1 trial did not include a study start date

Conclusions

- Less than half of PCOS trials included PROs, among which the majority focus solely on menstrual characteristics. There was either a lack of clarity or consistency among the PROs that were incorporated.
- There is growing interest from the FDA to address the gap in treatment/care in this area as evidenced by collaboration with advocacy organizations (PCOS Challenge)[4] where patients shared their experiences with the agency and advocated for aligning endpoints with patients' priorities.
- As treatment and management of PCOS extends beyond just reproductive health, further efforts need to be made to incorporate a comprehensive assessment of the patient experience, for the benefit of comprehensive clinical care, and opportunities to better measure potential treatment benefits of PCOS therapies.
- Additional research can help identify existing PROs developed for PCOS and identify those that capture the relevant concepts to patients' lives and therefore, allow for that specificity in selection of PROs in this indication.

References: