PCR37
THE IMPACT OF OUT-OF-POCKET COSTS ON ADHERENCE AMONGST US ONCOLOGY PATIENTS ON ORAL ANTICANCER MEDICATIONS

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Objectives: To synthesize the evidence published over the last ten years on the relationship between out-of-pocket (OOP) costs and adherence to oral anticancer (OAM) amongst adult cancer patients in the United States. ***Methods:*** We conducted a targeted literature review of PubMed for observational studies published from January 2014 to August 2024. We included the search terms "oral," "cancer," "adherence," and "out of pocket." We excluded studies involving intravenous medications, review articles, studies evaluating adherence during clinical trials, and patients without active cancer. We extracted the following data from the studies: study period, number of patients, cancer type, insurance type, results, OOP amounts, and drugs studied. ***Results:*** We identified 740 articles using our search term. We excluded 713 articles based on the title or abstract because the population was not of interest (n=451), outcomes were not related to the objective (n=173), or incorrect study design (n=89). We included 17 studies after a full-text review of the remainder. The studies evaluated claims data from 2002 to 2018, and the sample sizes ranged from 105 to 38,111 patients. We found studies that evaluated breast cancer (n=9), chronic myeloid leukemia (n=5), multiple myeloma (n=2), metastatic renal cell carcinoma (n=2), non-small cell lung cancer (n=2), and other cancers (n=2). We found studies that used commercial insurance claims (n=7), Medicare claims (n=5), and both (n=5). We found significant effects on adherence when OOP costs were studied in monthly increases of $1 to $2,000. We found studies thatsupporteda negative relationship (aOR up to 3.91) between higher OOP costs and adherence (n=12), and studies that had mixed results (n=3) or non-significant results (n=2). ***Conclusions:*** We found that higher OOP costs for patients with 11 different cancers had a significant, negative impact on adherence to OAM in increases of $1 to $2,000 per month for Medicare and commercial insurance populations.

PCR39
PATIENT-CENTERED RESEARCH (PATIENT-REPORTED OUTCOMES AND QUALITY OF LIFE OUTCOMES)

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Objectives: Geographic atrophy (GA) is a chronic, progressive degeneration of the macula that affects >5 million people worldwide. While there are FDA-approved treatments for GA, there is no patient-reported outcome measure designated as fit-for-purpose to measure quality of life in GA patients. The purpose of this qualitative study was to explore the content validity of the National Eye Institute Visual Functioning-25 item questionnaire (NEI-VFQ-25) among patients with subfoveal (SF) and non-subfoveal (NSF) GA and to understand if the measure is fit-for-purpose for use as a clinical trial (CT) endpoint in GA. ***Methods:*** Fifteen US-based participants (8 SF, 7 NSF) participated in cognitive debriefing interviews as part of a larger qualitative interview study. ***Results:*** The sample was mostly female, White, and non-Hispanic. All participants demonstrated a good understanding of measure instructions and appropriateness of response options. Inadequate understanding of items from 6 of 11 domains was demonstrated by 20% to <50% of study participants, including 11 overlapping items and 6 items unique to each subtype. Items were generally relevant across groups, although there were some group-level differences. Items in the general vision, driving, and near and distance activities domains were rated as most relevant across groups. Dependency domain items and social functioning domain items were rated as least relevant to the NSF and SF groups, respectively. Participants did not report any missing concepts in the measure. ***Conclusions:*** This study indicated content validity for the NEI-VFQ-25 in a GA population. Given the differences in item relevance and interpretation across groups, revisions to the measure, including potential removal of specific domains, could be explored to increase content clarity and relevance to each GA subtype. Debriefing with a larger sample and psychometric evaluation of the measure within a CT context could be meaningful.

PCR41
EXPLORING THE LANDSCAPE OF PATIENT-REPORTED OUTCOMES IN PCOS CLINICAL TRIALS

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Objectives: Polycystic ovarian syndrome (PCOS) affects approximately 8-13% 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 (such as acne/skin issues and excessive hair growth) and emotional health (such as anxiety and depression). This review aimed to evaluate the landscape of patient reported outcomes (PROs) and concepts measured in PCOS clinical trials. ***Methods:*** The terms "Polycystic Ovary Syndrome" and "PCOS" were searched in ClinicalTrials.gov to identify Phase 2-4 active or completed interventional trials. Endpoints from resulting studies were extracted and each study's primary, secondary, and exploratory outcome data was reviewed. Studies including PROs were identified and trends in PRO use were analyzed. ***Results:*** 172 total studies were included in the review, of which only 43 included PROs. Most endpoints measured clinical markers of reproductive health (pregnancy/ovulation rate or hormone levels) or BMI/weight. The most frequently utilized PRO in trials was unspecified menstruation questionnaires (n=30), 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, most contained items measuring emotional function (n=4). Other PROs identified assessed signs/symptoms (n=7), especially weight/BMI (n=3), menstrual characteristics (n=2), bodily pain (n=2), and treatment satisfaction (n=3). Most PROssupportedprimary and/or secondary endpoints (n=16 and n=27 respectively). ***Conclusions:*** Less than half of the 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. 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.

PCR42
MEASURING PATIENT- AND CARER-REPORTED OUTCOMES FOLLOWING GENOME SEQUENCING FOR RARE DISEASE DIAGNOSIS: A PSYCHOMETRIC ASSESSMENT OF OUTCOME MEASUREMENT INSTRUMENTS

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Objectives: Rare diseases (RDs) collectively affect 6-8% of the general population, yet diagnoses can take years and require many costly investigations. Genome sequencing is increasing RD diagnostic yield and speed. However, the information uncovered is complex and can impact patients and carers across clinical, emotional, cognitive, behavioural, and social outcome domains. This study aims to determine the generic multi-attribute utility instruments (MAUIs) best suited to measuring outcomes from genome sequencing for RD diagnosis. ***Methods:*** Systematic literature review (SLR) and critical appraisal: Primary studies measuring patient- and carer-reported outcomes from genome sequencing unrestricted by context and published by 16-March-23 were included. Instruments were critically appraised using COnsensus-based Standards for the selection of health Measurement INstruments (COSMIN) methodology. Cohort study: Adults undergoing genome sequencing for RD diagnosis and their carers, and carers of children undergoing sequencing, will be recruited from three UK National Health Service Genomic Medicine Services from December-2024 to February-2025. Six-hundred participants will complete quantitative surveys at baseline, and six- and 12-months post-baseline, and a sub-set will be qualitatively interviewed. Content validity, construct validity, responsiveness, and feasibility will be assessed. ***Results:*** SLR and critical appraisal: Twenty-nine studies using 58 eligible instruments were included. Twelve studies reported psychometric results but only four aimed to develop or validate an instrument. Four generic MAUIs (EQ-5D-5L, EQ-HWB-S, 15D, AQoL-6D), one generic psychological instrument, one generic carer health-related quality of life (HRQoL) instrument, and three genome sequencing-specific instruments were selected for inclusion in the cohort study. Cohort study: Baseline results for all participants will be presented, including Likert scale assessments of instrument relevance. ***Conclusions:*** Generic MAUIs may not be sufficiently relevant or comprehensive for measuring the impact of genome sequencing for RD diagnosis on patients and informal carers. Overlooking important outcomes from genome sequencing in health technology assessments could result in inefficientfundingdecisions.

PCR43
CHALLENGES AND OPPORTUNITIES FOR THE USE OF PATIENT EXPERIENCE DATA IN GENE THERAPY TRIALS

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Objectives: Gene therapies (GTs) offer the potential to substantially improve the lives of patients; however, evaluating their benefit from the patient perspective in clinical trials presents unique challenges. Although patient experience data is commonly suggested by regulators in approval applications, its inclusion in GT applications is limited. This research aims to summarize key challenges for GT trials and opportunities