Inclusion of health-related quality of life and other patient-reported outcomes in Food and Drug Administration labels

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Background

- Since the establishment of the Patient-Centered Outcomes Research Institute (PCORI) in 2010, there has been a growing emphasis on integrating outcomes significant to patients, such as health-related quality of life (HRQoL) and other patient-reported outcomes (PROs), into regulatory decisions.

Objective

- This study aimed to assess the frequency of PRO assessments and other patient-important outcomes in pivotal trials and their representation on United States Food and Drug Administration (FDA) labels for drugs approved for rheumatoid arthritis (RA), asthma, and non-small cell lung cancer (NSCLC).

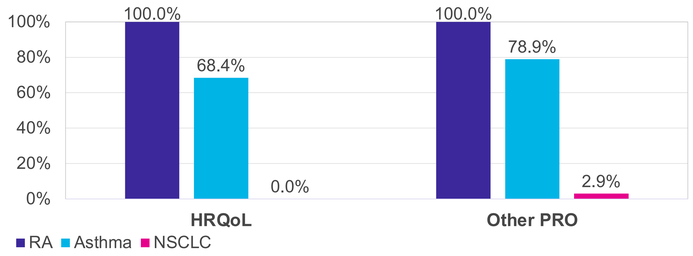
Methods

- Recent FDA labels for drugs approved for RA, asthma, and NSCLC post-2010 were identified from the FDA website and reviewed for any mention of PRO assessments, including multidimensional outcomes combining patient-reported components and clinical assessments.
- The total number of trials and the number of trials reporting on HRQoL and other PROs were collected from each label.
- Trials referenced in the labels were cross-checked on ClinicalTrials.gov to review the list of assessed outcomes for inclusion of PRO measures.

Results

- The review identified 6 approved labels for RA (23 unique trials), 19 for asthma (65 unique trials), and 34 for NSCLC (71 unique trials).
- As expected, all RA product labels cited PROs, including multidimensional outcomes like the American College of Rheumatology 20/50/70 Response Criteria and Disease Activity Score, sleep and pain assessments, and generic HRQoL measures **(Figure 1).**

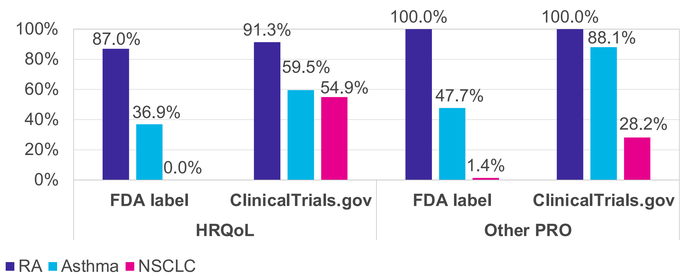
Figure 1. Percentage of FDA labels including HRQoL and other PROs



Key: FDA – Food and Drug Administration; HRQoL – health-related quality of life; NSCLC – non-small cell lung cancer; PRO – patient-reported outcome; RA – rheumatoid arthritis.

- Most asthma labels mentioned HRQoL and/or other PROs (**Figure 1**). Although HRQoL and other PROs were reported on most asthma labels, HRQoL data were collected in less than half of the trials included on the label (**Figure 2**).
- Conversely, NSCLC labels seldom included PRO assessments, with none mentioning HRQoL and 1 (3%) referencing other PROs. Although PROs were measured in the pivotal trials for NSCLC and reported on ClinicalTrials.gov, these secondary and exploratory outcomes were seldom included in the FDA labels (**Figure 2**).

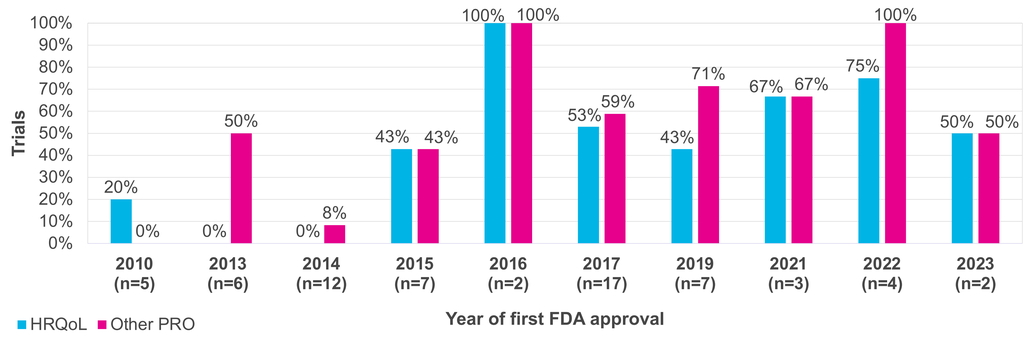
Figure 2. Percentage of trials referred to in the FDA label reporting HRQoL and other PROs on the FDA label and on ClinicalTrials.gov



Key: FDA – Food and Drug Administration; HRQoL – health-related quality of life; NSCLC – non-small cell lung cancer; PRO – patient-reported outcome; RA – rheumatoid arthritis.

- In the time immediately following the establishment of PCORI, generally less than half of the trials described on FDA labels for asthma treatments reported PROs (**Figure 3**). An increasing trend has been observed since 2016.

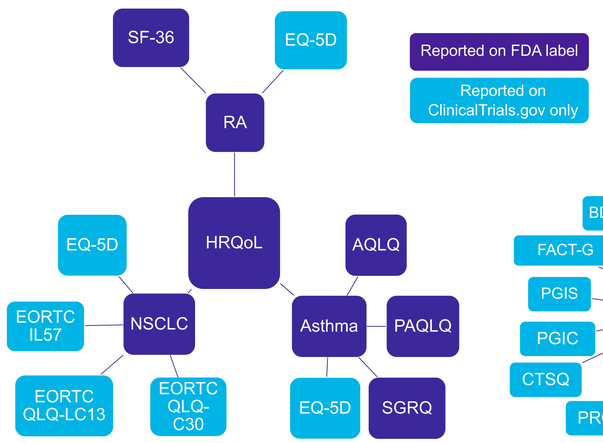
Figure 3. Percentage of asthma trials including HRQoL and other PROs by year, as reported on FDA label



Note: No relevant FDA labels were identified for years that do not appear in the chart (2011, 2012, 2018, 2020, and 2024).
Key: FDA – Food and Drug Administration; HRQoL – health-related quality of life; PRO – patient-reported outcome.

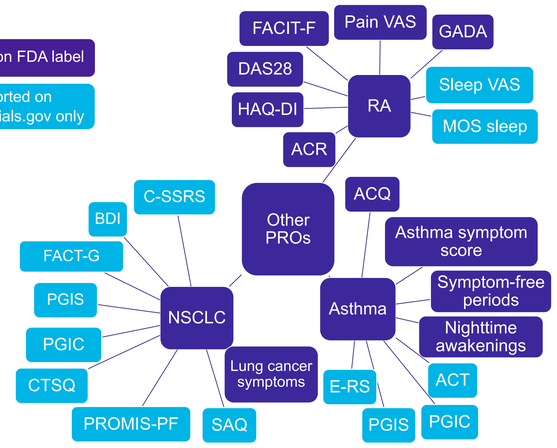
- All 20 RA trials reporting HRQoL outcomes used the SF-36, while 24 asthma trials used multiple questionnaires (**Figure 4**).
- Other PROs collected for RA included pain, fatigue, and several composite measures of disease activity, while PROs in the asthma trials focused largely on control of asthma symptoms **(Figure 5).**

Figure 4. Tools used to collect HRQoL in trials on FDA labels



Key: AQLQ – Asthma Quality of Life Questionnaire; EORTC – European Organization for Research and Treatment of Cancer; FDA – Food and Drug Administration; HRQoL – health-related quality of life; IL57 – Item Library 57; NSCLC – non-small cell lung cancer; PAQLQ – Pediatric Asthma Quality of Life Questionnaire; QLQ-C30 – Quality of Life Questionnaire Core 30; QLQ-LC13 – Quality of Life Questionnaire Lung Cancer 13; RA – rheumatoid arthritis; SGRQ – St. George's Respiratory Questionnaire.

Figure 5. Tools used to collect other PROs in trials on FDA labels



Key: ACQ – Asthma Control Questionnaire; ACR – American College of Rheumatology Response Criteria; ACT – Asthma Control Test; BDI – Beck Depression Inventory; C-SSRS – Columbia Suicide Severity Rating Scale; CTSQ – Cancer Therapy Satisfaction Questionnaire; DAS28 – Disease Activity Score 28; E-RS – Evaluating Respiratory Symptoms; FACIT-F – Functional Assessment of Chronic Illness Therapy-Fatigue; FACT-G – Functional Assessment of Cancer Therapy-General; FDA – Food and Drug Administration; GADA – global assessment of disease activity; HAQ-DI – Health Assessment Questionnaire Disability Index; MOS – Medical Outcomes Study; NSCLC – non-small cell lung cancer; PGIC – Patient Global Impression of Change; PGIS – Patient Global Impression of Severity; PRO – patient-reported outcome; PROMIS-PF – Patient-Reported Outcomes Measurement Information System-Physical Function; RA – rheumatoid arthritis; SAQ – Symptom Assessment Questionnaire; VAS – visual analog scale.

Conclusions

- Among the evaluated therapeutic areas, RA labels most commonly included PROs, aligning with the subjective nature of most clinical symptoms in this condition.
- Asthma labels included some PROs capturing symptom control but mainly focused on assessment of objective outcomes.
- In contrast, NSCLC labels seldom highlighted PROs despite their assessment in pivotal trials.
- As patient-centered care gains prominence, PROs offer valuable insights into condition impact and treatment effects, warranting consideration in the evaluation of new medicinal products by regulatory bodies.

Limitations

- This assessment was limited to 3 disease areas with expected differences. Additional research on other conditions, particularly other indications in oncology, would provide valuable insights.
- Trial registry numbers were not reported for all clinical trials referenced on the FDA labels, so it was not possible to identify the relevant record on ClinicalTrials.gov. This limited the ability for comparison. It also has the potential to introduce bias if registered trials are systematically different than those that are not.

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