Can we quantify the financial benefit of Patient-Reported Outcomes (PROs) on the value of oncology products?

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INTRODUCTION

Regulatory bodies, HTA agencies, payers, clinicians and patient advocates increasingly emphasize the importance of patient experience evidence including PROs in decision making in oncology.[1, 2, 3] Previous studies have supported the importance of PROs in oncology drug development, [4, 5, 6] but there have been limited research on quantifying financial impact and ROI of PRO evidence in recent years.

PURPOSE

This work aims to report the framework and preliminary results to quantify the financial benefit and estimating the return on investment (ROI) of patient reported outcomes (PRO) evidence in five major European markets.

STUDY DESIGN

A conceptual framework to assess ROI of PRO evidence in oncology was developed and variables to quantify impact on revenue and cost identified (Fig 1). To assess the HTA impact, quantitative analysis of 302 oncology launches with randomized control trial (RCT) data (93 novel oncology products) was conducted over the past decade (2011-24) to assess how PRO in pivotal trials relate to regulatory labelling claims and HTA outcomes (recommendations, benefit ratings). New indication approvals by EMA and FDA between 2011 and 2024 in oncology were identified using IQVIA's Market Access Insights platform. The pivotal study design, endpoints, PRO regulatory labelling claim and HTA/reimbursement outcomes in selected countries (England, Germany, France, Italy, and Spain) were extracted. To assess the HTA impact, quantitative analysis of 300 oncology launches with randomized control trial (RCT) data (93 novel oncology products) was conducted, to assess how PRO results in pivotal trials relate to regulatory labelling claims and HTA outcomes (recommendations, benefit ratings).

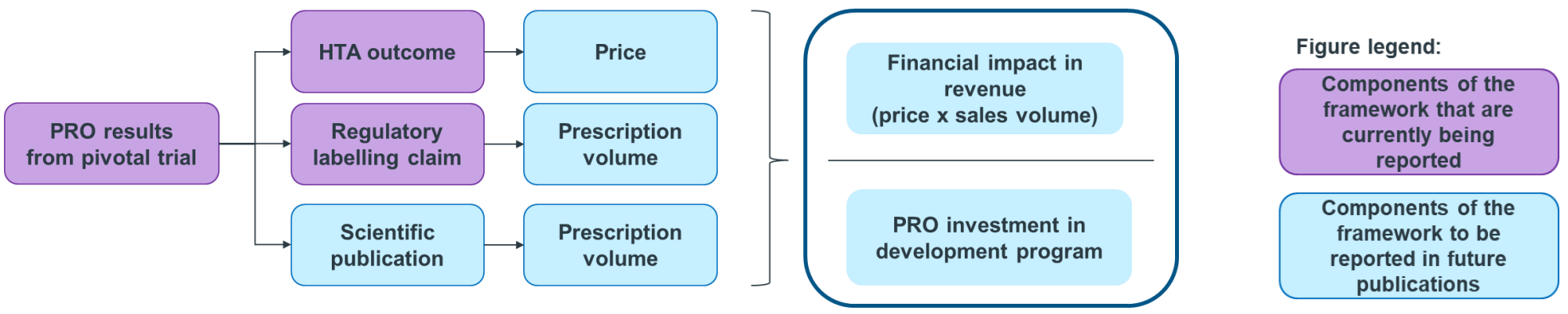
RESULTS

Overall, 4% of the indications approved in oncology since 2011 have PRO claims in the FDA label and 18% have PRO claims in the EMA SmPC (Fig. 2). Products with positive PRO data showed better added benefit ratings in Germany (Fig. 3) regardless of overall survival (OS) benefit (Fig.4). Indications with a recognized PRO benefit in Germany might be more likely to be reimbursed in UK, France, Italy and Spain.

CONCLUSION

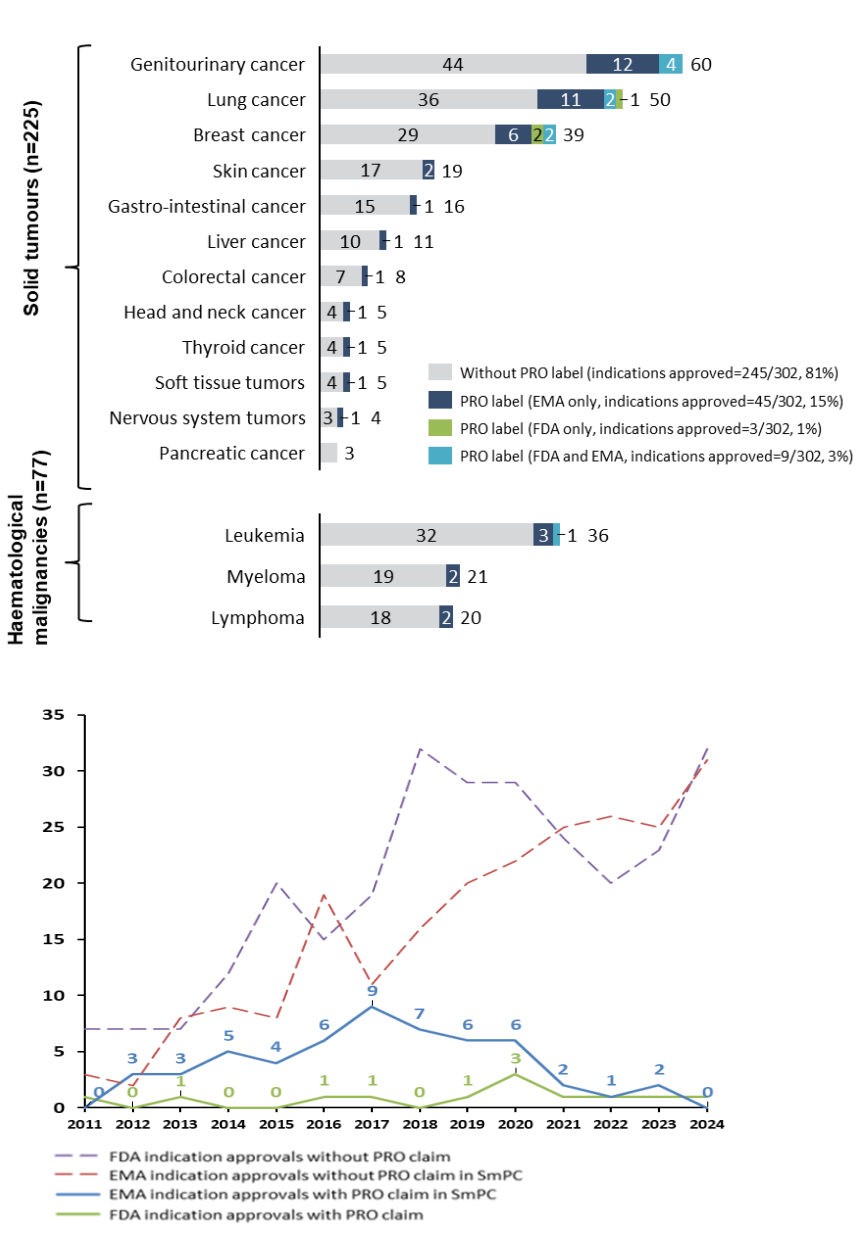
Preliminary results from this work suggest that well-designed investment in a PRO strategy might increase the probability of demonstrating treatment benefit with PROs, positively impacting reimbursement and HTA outcomes. Further analyses may provide additional details of the drivers behind RPO for PRO data.

1. Framework used to assess ROI of PRO development programs



2. Indications approved in oncology by FDA or/and EMA since 2011 with or without PRO claims in the FDA label or EMA SmPC

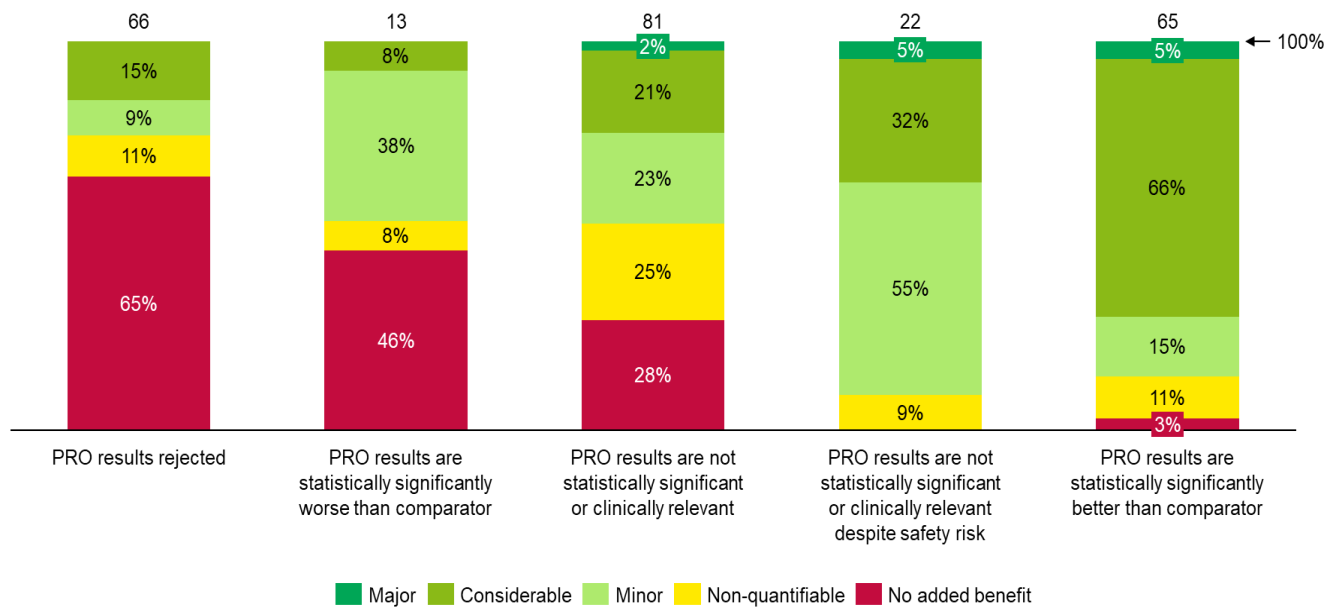
Indications approved with FDA and/or EMA labelling claim for PROs (n=302)*



Note: *'Indications approved ' includes all products with unique indication by FDA/EMA, often linked to a single RCT; PRO label claims at both agencies may not refer to the same label claim at both agencies

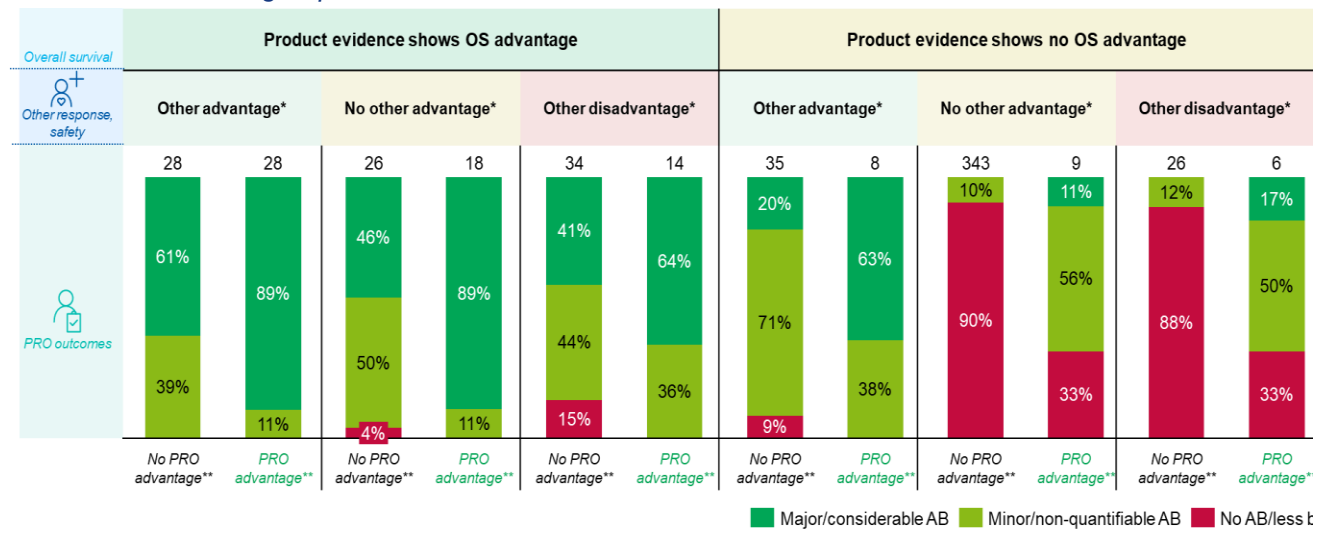
3. Added benefit ratings with positive PRO data in Germany

Benefit rating awarded by G-BA in the first assessment of an approved indication by PRO benefit type (247 assessments)



4. Positive impact of PRO data with or without OS in Germany

Data shown at subgroup level



*Other advantages refer to statistically significant differences in cure failure, perceivable response, or safety endpoints, whereas other disadvantages are always in safety endpoints
**PRO advantage = Statistically significant advantage vs appropriate trial comparator in symptom(s) and/or Health-related Quality of Life (sub)scales ("no PRO advantage" includes 23 subgroups with a statistically significant disadvantage vs the trial comparator)

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