HPR34

# Emerging Role of Patient-Reported Outcomes in Drug Approval in Europe

**Chatterjee M**[a], Pal S[a], Rosario V. M.[a], Sreedharan S[a], Ghosh S[a],

[a]IQVIA India

**Patient-reported outcomes (PROs) have paved the way for a paradigm shift in European drug approval processes owing to the standardisation of disease-specific PROs capable of capturing information related to patient preferences concerning treatment decision-making.**

## Background

- Marketing of all drugs in the European Union (EU) requires authorisation from the scientific committee of the European Medicine Agency (EMA), which performs a comprehensive examination of clinical trial data.[1]
- In addition to clinical trial data, the EMA and other regulatory authorities are also considering patient preferences in decision-making.[1, 2]
- Patients' perspectives of their health status, prior to and post-treatment – measured using patient-reported outcome (PRO) tools – have found an integral role in clinical trials.[3,4]
- The use of PROs during treatment follow-up will enable the gathering of empirical data, which may support or enable regulatory authorities and healthcare professionals (HCPs) to understand patient perspectives and integrate the same into regulatory and treatment decision-making.

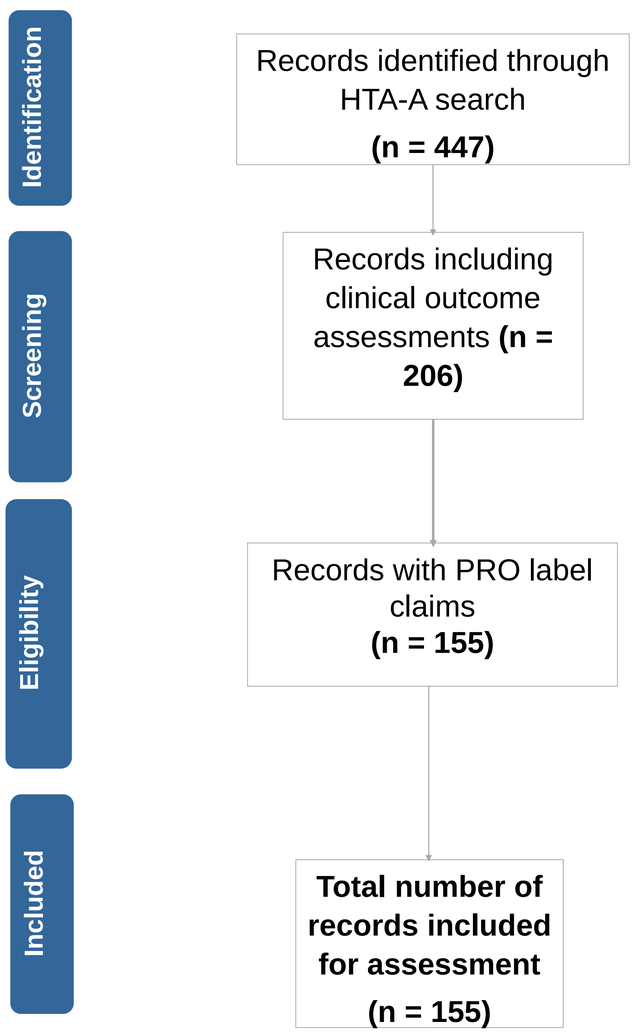
## Objectives

- The aims of this study were to evaluate the influence of PRO endpoints in drug approval decision-making processes of the EMA between 2020 and 2022 and to identify a decade-long trend in the role of PROs in EMA drug approval processes.

## Methods

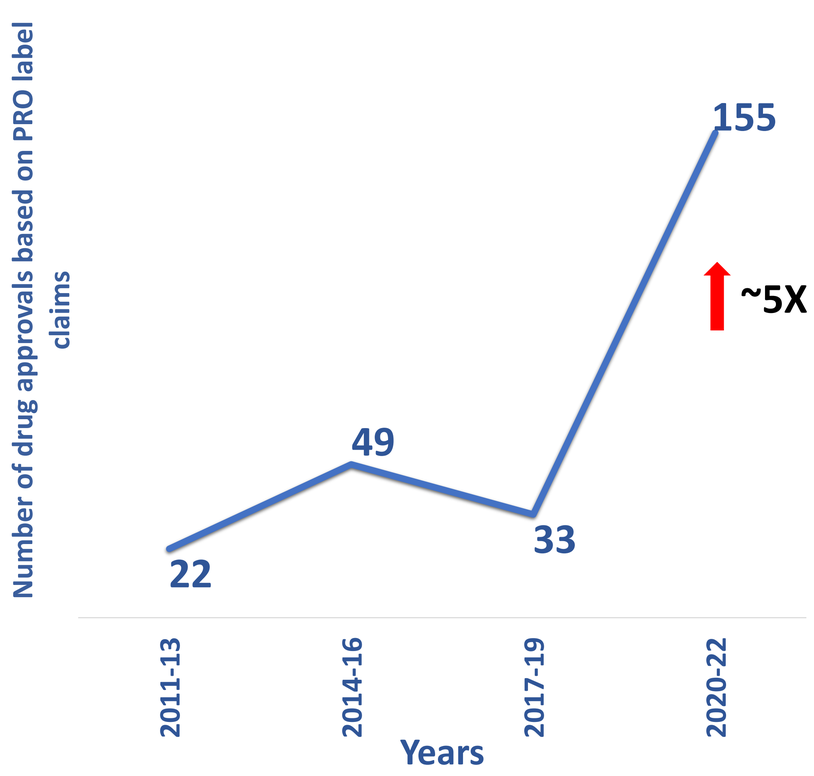
- HTA accelerator (HTA-A) was used to extract information on the EMA authorisation status of drug approvals granted between 2020 and 2022 (Figure 1).[5]

**Figure 1: Flow diagram for identification of records from HTA database between 2020 and 2022 (to date)**




- EPAR product labels were reviewed to check for PRO claims as one of the clinical outcome measures.
- A similar analysis was performed to identify emerging trends of PROs in drug approval processes from 2011 to 2022 using a three-year time interval range.
- The disease categories of the approved drugs, as well as the PRO instruments used for clinical evaluation, were assessed specifically for the time period of 2020–22.

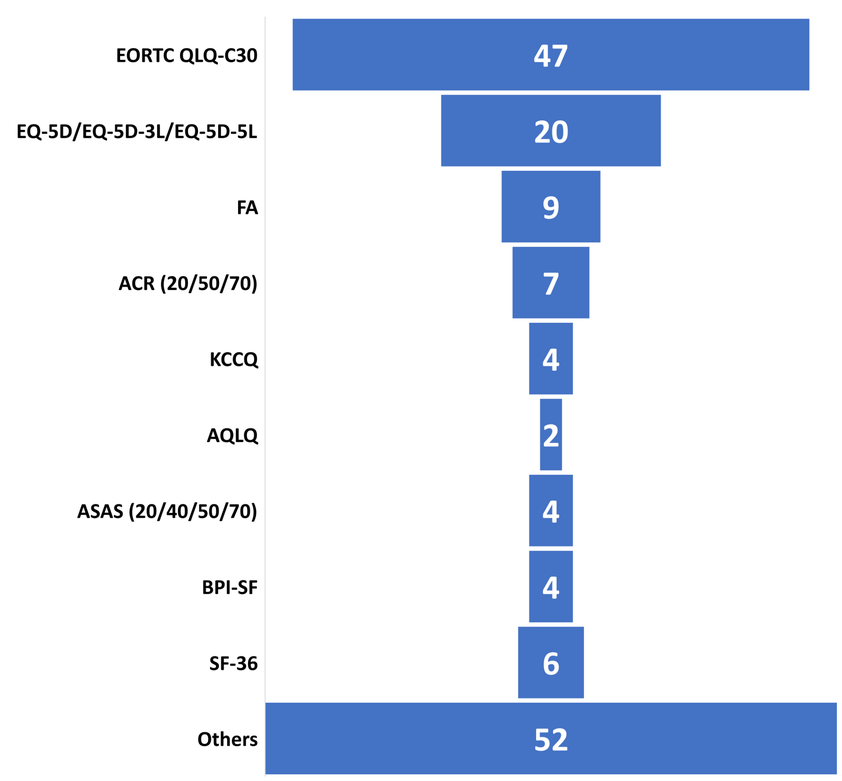
**Figure 2: Trend analysis of last 10 years for usage of PRO label claims for EMA drug approvals**




## Results

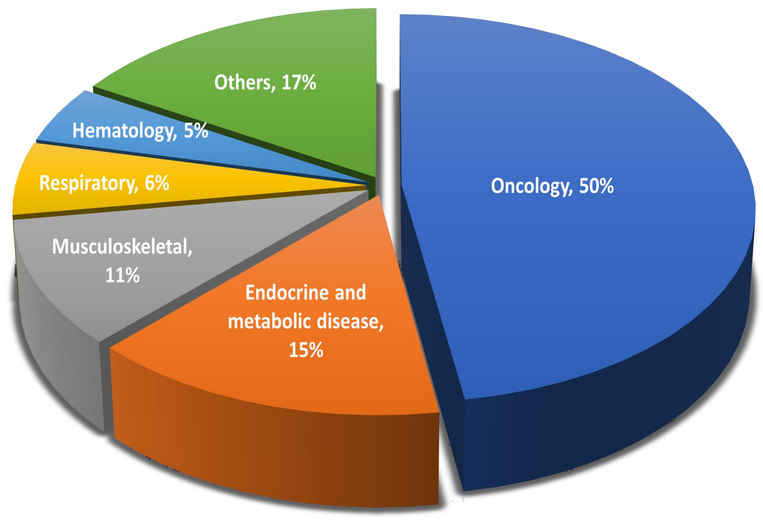
- Between 2020 and 2022 (to date), 447 drugs received EMA approval, of which 155 (35%) drug labels indicated PROs in their label claims.
- A decade-long trend analysis of the role of PRO label claims in drug approval indicated an exponential increase in the last two years. There was an approximately 4.7-fold increase in the number of drug approvals based on PRO label claims in the time period of 2020–22 versus 2017-19 (Figure 2).

**Figure 3: PROs used for EMA drug approval decision-making between 2020 and 2022**




- Around 28 unique PROs, designed for 14 different therapeutic areas, were reported in the assessed PRO label claims. Out of these, EORTC-QoL and EuroQoL were the most frequently used PRO tools (Figure 3).

**Figure 4: Distribution of EMA-approved drugs in different disease areas**




- 50% of these approved drugs using PRO label claims were targeted towards the oncology disease area, followed by endocrine and metabolic diseases, and musculoskeletal diseases (Figure 4).

## Conclusion

- The current study highlights an exponential increase in the inclusion of PRO label claims, with EORTC-QoL and EuroQoL being the most predominant PRO tools reported in EMA drug approval processes, possibly because these two are the most validated and customisable tools.
- A higher number of drug approvals were reported for the oncology therapeutic area, which might have been influenced primarily by the higher acceptance of PRO tools and the importance accorded to patients' perspectives in treatment decision-making by all stakeholders.[6]
- The inclusion of PROs in drug approval necessitates the customisation of disease-specific frameworks and guidelines to ensure the effective use of PRO data.
- In conclusion, we anticipate an increasing number of PRO-based approvals in the future, taking patients' perspectives into consideration for regulatory decision-making across diverse therapy areas.

## References

1. European Medicine Agency. How are new medicines approved by EMA? . Updated 15 August 2018. Accessed 12 October 2022, https://www.ema.europa.eu/en/news/how-are-new-medicines-approved-ema
2. Food and Drug Administration. Focus Area: Patient-Reported Outcomes and other Clinical Outcome Assessments. Updated 09 June 2022. Accessed 12 October 2022, https://cacmap.fda.gov/science-research/focus-areas-regulatory-science-report/focus-area-patient-reported-outcomes-and-other-clinical-outcome-assessments
3. Valsangkar N, Fernandez F, Khullar O. Patient reported outcomes: integration into clinical practices. *J Thorac Dis*. Nov 2020;12(11):6940-6946. doi:10.21037/jtd.2020.03.91
4. LionBridge. Patient-Reported Outcomes and Clinical Outcome Assessments. Updated 21 November 2021. Accessed 12 October 2022, https://www.lionbridge.com/blog/life-sciences/outcomes-and-assessments-in-drug-development-and-clinical-trials/
5. HTA.Quintiles. Regulatory search result_HTA-accelerator. https://hta.quintiles.com/
6. Getu MA, Chen C, Wang P, Kantelhardt EJ, Addissie A. Quality of life and its influencing factors among breast cancer patients at Tikur Anbessa specialised hospital, Addis Ababa, Ethiopia. *BMC Cancer*. Aug 17 2022;22(1):897. doi:10.1186/s12885-022-09921-6



**Acknowledgement:** We would like to acknowledge Tulika Paul from IQVIA, for providing useful insights for this research work and Shlok Kumar from IQVIA for editorial assistance.