

# Patient-Driven Outcome Selection (PDOS): a patient-focused approach for selecting outcomes using patient-generated data

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## INTRODUCTION

- Patient-Reported Outcomes (PROs) are increasingly used to support the development and the evaluation of health products and services. Due to the wide variety of instruments available, identifying the most suitable PRO to use in a specific context might be complex.
- There is **no consensus on a standardized method to choose PROs** considering the scientific context and the regulatory recommendations.
- This research aimed at developing a patient-centric, structured, replicable and generalizable method for PROs selection and development using patient-generated data.

## PATIENT-CENTRIC APPROACH (PATIENT-DRIVEN OUTCOME SELECTION - PDOS) TO OPTIMIZE PRO SELECTION

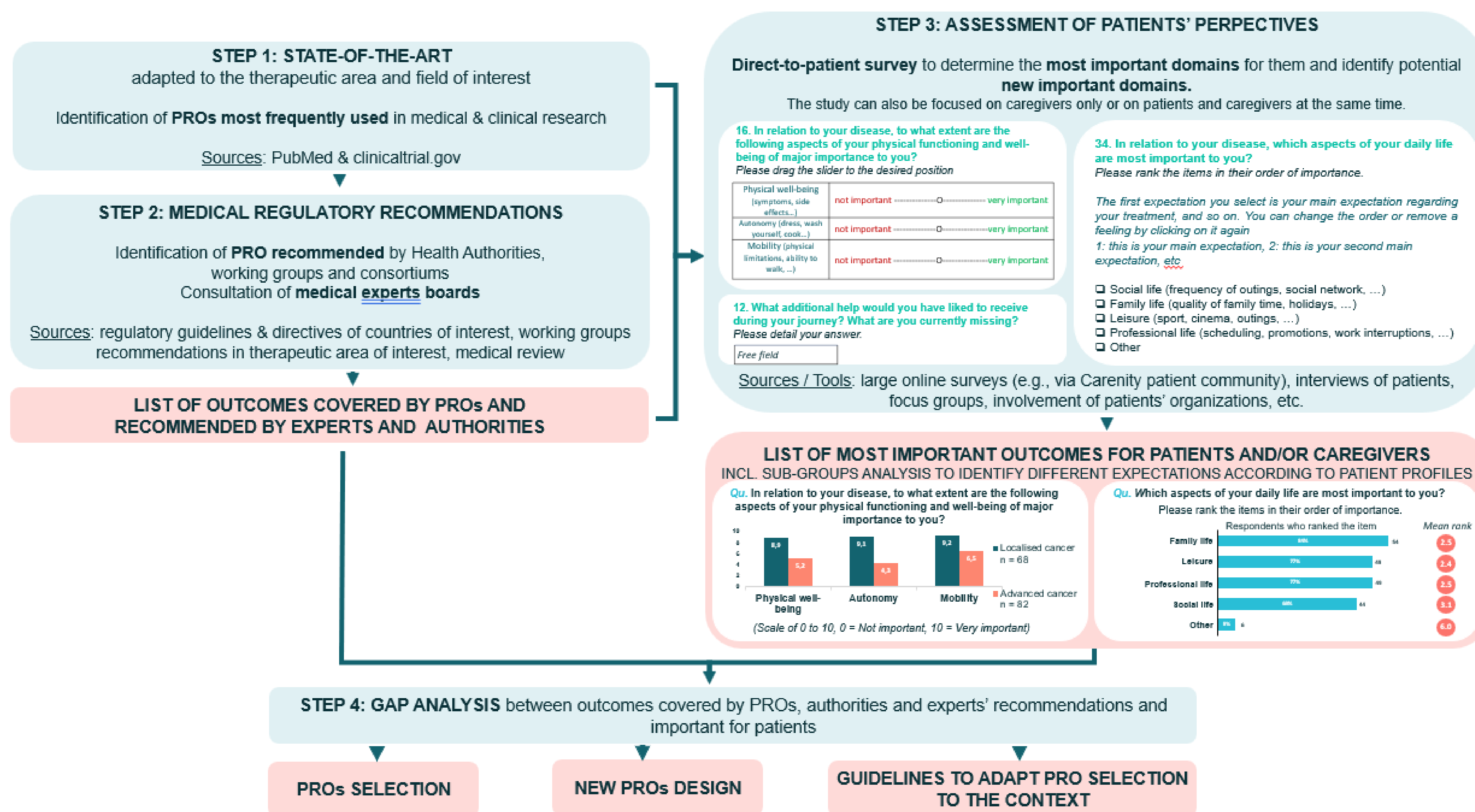


Figure 1: Presentation of the method PDOS (Patient-Driven Outcome Selection)

## PRACTICAL APPLICATIONS OF PDOS IN DIABETES, BREAST CANCER AND LUNG CANCER

PDOS method has been applied in the **diabetes** (n=249 respondents) and **breast cancer** (n=146 respondents) areas and respectively showed that patient's perspectives can differ for a treatment than for a digital health solutions (figure 2) and can depend on the profile of the patients (e.g, age). For instance, in the application to breast cancer, impact on quality of life was ranked among the 3 most important domains by 62% of patients younger than 60 years but only by 31% of patients older than 60 years old.

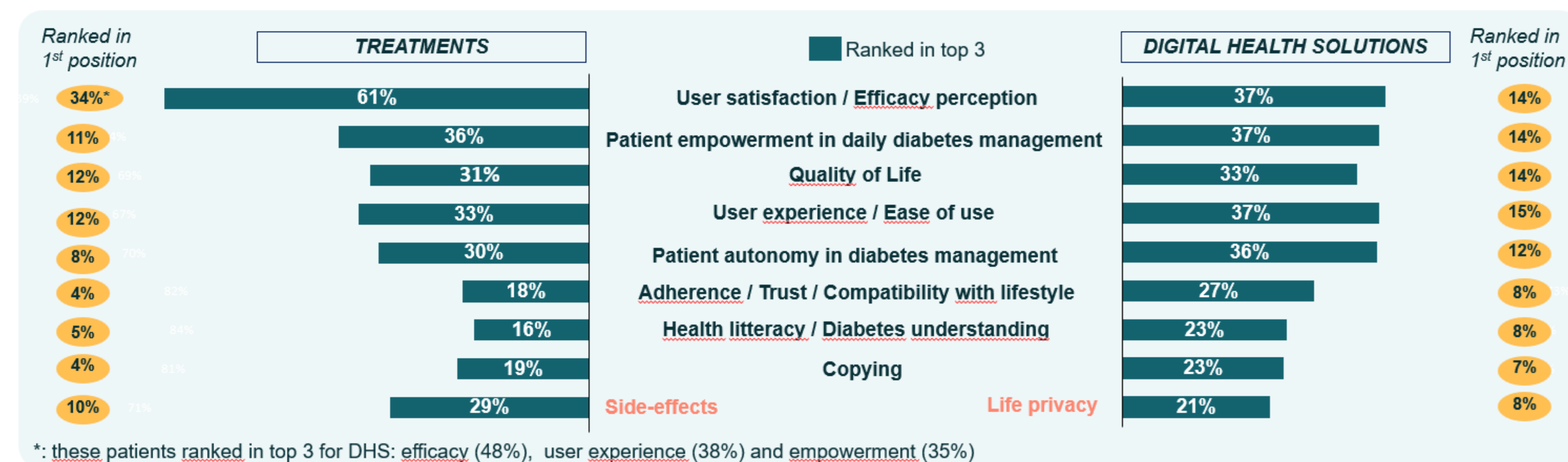


Figure 2: Most important domains for patients with diabetes for the evaluation of treatments and digital health solutions (n= 249 respondents)

Evaluation of the method in **breast and lung cancer** have also shown that domains valued by medical and HEOR experts and those valued by patients in existing PROs could differ (table 1 and 2).

## CONCLUSIONS

- The increasing importance of Health-Related Quality of Life (HR-QoL) in healthcare decision-making reinforces the need to choose relevant PRO measures.
- This new patient-centric approach presents the advantage of considering patients' expectations in the choice of instruments. This approach also contributes to more in-depth discussions, streamlines the study process, and enhances its overall credibility by backing up the measured outcomes.
- The methodology developed in this pilot showed promising results tending to apply to several conditions (breast cancer, lung cancer or diabetes). Further investigations could be implemented in order to test this methodology in other therapeutic areas.

## REFERENCES - COMMUNICATION

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- 3- Varriale P., Müller B., Katz G., Dallas L., Aguaron A., Azoulai M., Girard N. Patient Perspectives on Value Dimensions of Lung Cancer Care: Cross-sectional Web-Based Survey. JMIR Form Res. 2023; 7: e37190. <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC9912155/>

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Table 1: Comparison of domains covered by PROs and valued by patients with breast cancer (n=146 respondents)

| DOMAIN   | Current PROs | Patients' perspectives |
|--|--------------|------------------------|
| Quality of Life (personal, professional, social, physical and emotional well-being)  | Major Domain | Major Domain           |
| Survival vs QoL considerations (efficacy, side effect)   | Major Domain | Major Domain           |
| Satisfaction with cancer healthcare pathway  | Major Domain | Major Domain           |
| Coping strategy (attitudes and behaviors used to maintain emotional well-being and to adjust to the stresses caused by cancer) | Major Domain | Major Domain           |
| Satisfaction and confidence in decision-making   | Major Domain | Major Domain           |
| Burden of the disease (lack of energy, ability to feel like a woman, pain, weight loss)  | Major Domain | Major Domain           |
| Information provided on treatment efficacy, side effects, administration...  | Major Domain | Major Domain           |
| Use of complementary therapies to reduce the side effect and the impact on QoL   | Major Domain | Major Domain           |

Table 2: Comparison of domains covered by PROs and valued by patients with lung cancer

| ITEM                                | PROs (FACT-L, EQ5D-5L, QLQ-C30, QLQ-LC13, ICHOM guidelines) | Medical & HEOR experts (n= 4) | Patients & caregivers Online questionnaire (n=150) |
|-------------------------------------|---|-------------------------------|--|
| Daily life                          | Major Domain  | Major Domain                  | Major Domain                                       |
| Professional life                   | Major Domain  | Major Domain                  | Major Domain                                       |
| Physical functioning and well-being | Major Domain  | Major Domain                  | Major Domain                                       |
| Emotional well-being                | Major Domain  | Major Domain                  | Major Domain                                       |
| Medical care                        | Major Domain  | Major Domain                  | Major Domain                                       |
| Treatment care                      | Major Domain  | Major Domain                  | Major Domain                                       |
| End-of-life care                    | Major Domain  | Major Domain                  | Major Domain                                       |
| Economic burden                     | Major Domain  | Major Domain                  | Major Domain                                       |
| Relationship with HCPs              | Major Domain  | Major Domain                  | Major Domain                                       |
| Caregivers' perspectives            | Major Domain  | Major Domain                  | Major Domain                                       |