

5.5 Feedback from surveys in PED priority areas

Preliminary results

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PCWP-HCPWP meeting-4 February 2026

- Introductory part
 - Objectives
 - Structure of the survey
- Overview of responses
 - Geography of responses
 - Therapeutic areas/fields selected
 - Main use of Patient Experience Data (PED)
 - Experience and impact of PED
 - Challenges with PED
 - Resources on PED
 - Therapeutic area of expertise and relevance of PED
- Concluding remarks

Objectives of the survey and overview of responses received

Objectives:

- ✓ To gain a clear view on key stakeholders' experiences with the use of PED across all therapeutic areas
- ✓ Some of the research questions we are hoping to address with the survey:
 - What are the main differences in the experience with PED between therapeutic areas?
 - Are the challenges of PED the same across the different therapeutic areas?
 - Are some Therapeutic areas more 'advanced' in terms of PED?
 - What are the most valuable aspects of PED in a given therapeutic areas? Are those aspects similar across the various Therapeutic areas?
 - How do the PED needs and expectations compare between the different stakeholders?
- ✓ To identify potential gaps and unmet needs (e.g. in terms of resources, TAs where maybe more needs to be done in terms of PED, stakeholders perceptions)
- ✓ To allow for comparative analysis between the different stakeholders' views and the different therapeutic areas
- ✓ **To further develop PED**

Objectives of the survey and overview of responses received

Participants:

Four tailored surveys for

- patient representatives and individual patients and carers,
- healthcare professionals and academic researchers,
- assessors (NCA and HTA),
- and industry.

The survey for patients and HCP was available in all EU languages.

Timelines: 22 September - 19 October 2025, extended to 2 Nov 2025

Thank
You

Some volunteers from the Patients and HCP organisations provided us with some very useful feedback when building the questionnaires!

Special thanks to Corinne Scicluna, Panagiota Tsikala, Wilma Knol, Daniela Rojas Castro and Loris Brunetta

Overview of responses received:

	111
HCP-academic researchers	
Academic researchers	39
HCP organisation representatives at EU level	9
HCP organisation representatives at national level	9
Individual healthcare professionals	54
Patients	75
Patient organisation representative at EU level	17
Patient organisation representatives at national level	20
Individual patients	36
Carers	2
Industry	26
EFPIA	17
EUCOPE	3
Other	6
NCA-HTA	21
NCA assessors	19
HTA assessors	2
TOTAL	233

Structure of the survey

Q1. What capacity - What country

Q2. Main therapeutic area or field of expertise or interest (later in the presentation will be abbreviated as "TA")

Q3. Rating:

- the range of medicines treating diseases
- the focus on patients and their experiences in the development of medicines
- the focus on patients and their experiences in the regulatory assessment of medicines
- the focus on patients and their experiences in cost-benefit analysis and reimbursement decisions of medicines

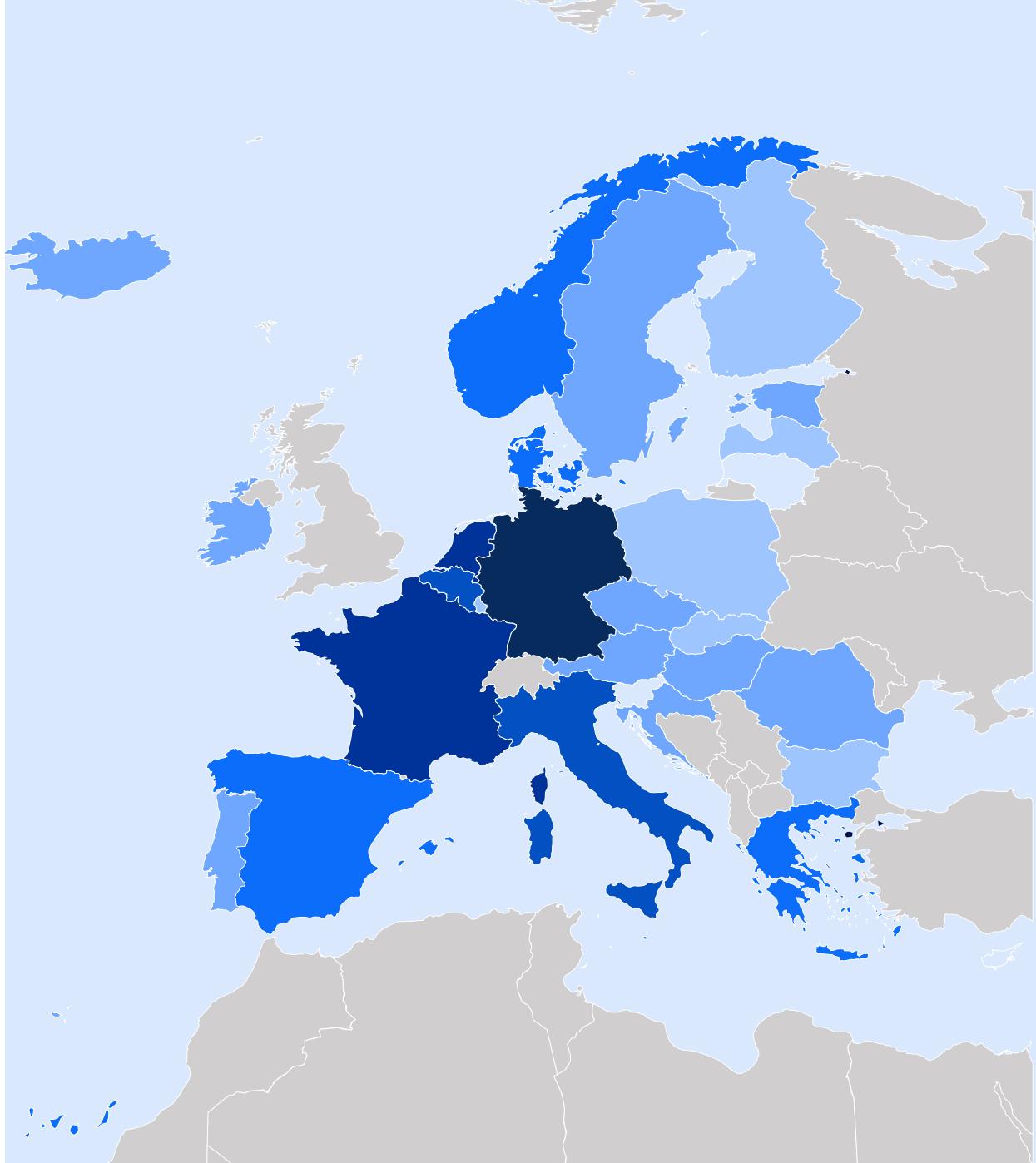
- Q4. What do you believe PED should be mainly used for in the selected therapeutic area (TA)?
- Q5. Experience with PED: What type? What impact? What challenges?
- Q6. What resources facilitate the conception/collection/review of PED
- Q7. Are you aware of data sources that are not in the HMA-EMA Catalogue of RWD sources?
- Q8. Which therapeutic area/field (TA) is the use of PED the most relevant/needed?
- Option to select a second therapeutic area ("TA2") and respond to the same questions

Geography of responses

Country	TOTAL	Academic researchers	HCPs	Patients/ carers	Assessors	Industry
Germany	45	5	3	27	4	6
France	16	1	6	2	1	6
Netherlands	16	1	5	7	1	2
Italy	12	2	7	2	0	1
Belgium	11	3	3	2	3	
Spain	9	1	8	0	0	
Denmark	8	2	1	1	3	
Greece	7	3	4	0	0	
Norway	5	2	3	0	0	
Estonia	4	1	2	1	0	
Hungary	4		2	0	2	
Portugal	4	2	1	1	0	
Romania	4	1	2	1	0	
Austria	3		0	0	3	
Croatia	3		1	0	2	
Czechia	3	1	0	0	2	
Ireland	3		1	2	0	
Sweden	3		2	1	0	
Bulgaria	2		0	2	0	
Finland	2	1	1	0	0	
Latvia	1		0	1	0	
Luxembourg	1		1	0	0	
Poland	1	1	0	0	0	
Slovakia	1		0	1	0	
Outside EU/EEA	39	12	10	7	0	10
EU representation	26		9	17	0	
	233	39	72	75	21	26

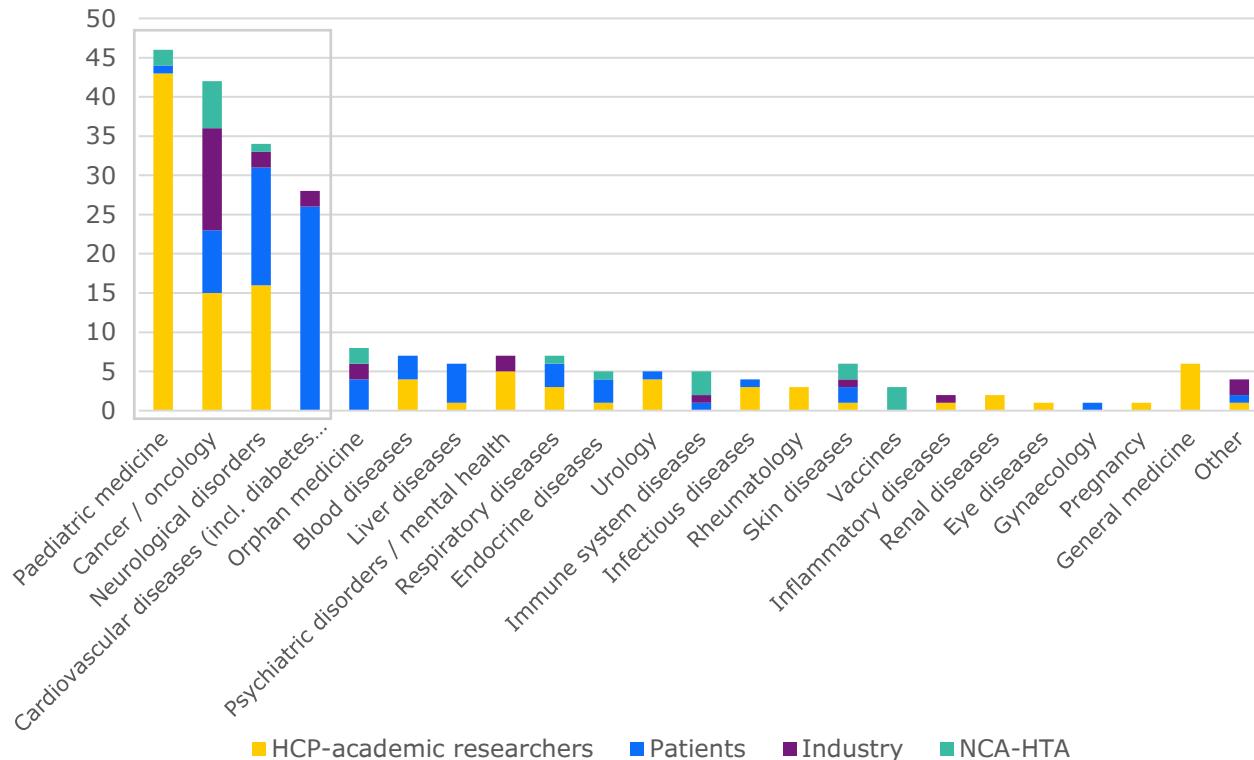
Of note:

- Representatives of an EU organisation were not asked to select a country but are recorded under “EU representation” (~11% of respondents)
- Respondents at national level selected 24 different EEA nationalities, Germany being significantly higher (selected by more than 1/3 of patients)
- Almost 40% of Industry declared being from outside the EU



Therapeutic areas/fields of interest or expertise (1/2)

First therapeutic area (TA1) selected
(with manual corrections*) per stakeholder group

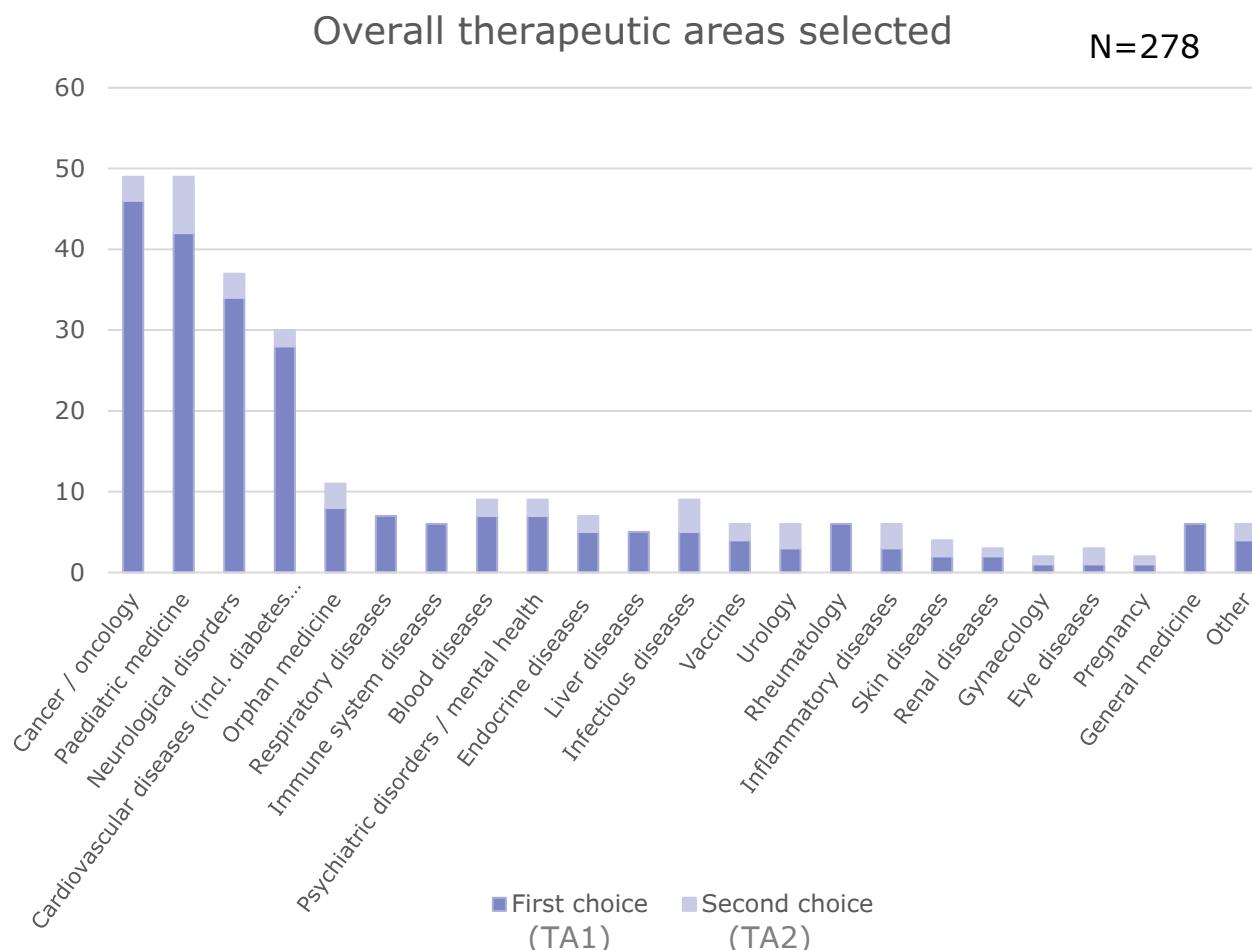


***Some respondents selected "other" but when giving more details they indicated a category that was among the proposed choices, so we manually corrected and also created a category for "general medicine" that few people mentioned in "other". A total of 19 such corrections was done.**

Of note:

- 4 therapeutic areas have been selected significantly more than the other: paediatric, oncology, neurology and CVS
- From our proposed list, only two therapeutic areas have not been selected by any stakeholders: "Anaesthesiology" and "Geriatric Medicine"
- Although these therapeutic areas/fields rank among the most frequently selected overall, no academic researchers have chosen "Cardiovascular diseases" or "orphan medicine"
- 1/3 of HCP/academic researchers have selected "paediatric medicine" as their main field of interest/expertise
- 1/3 of the patients (individual + org) have selected "Cardiovascular diseases (incl. diabetes and obesity)" and 1/6 "neurological disorders" as their main field of interest/expertise
- Almost 1/2 of industry and 30% of assessors have selected "Cancer / oncology" as their main field of interest/expertise
- As the response rate for Industry and assessors was low, many therapeutic areas have not been selected by them

Therapeutic areas/fields of interest or expertise (2/2)

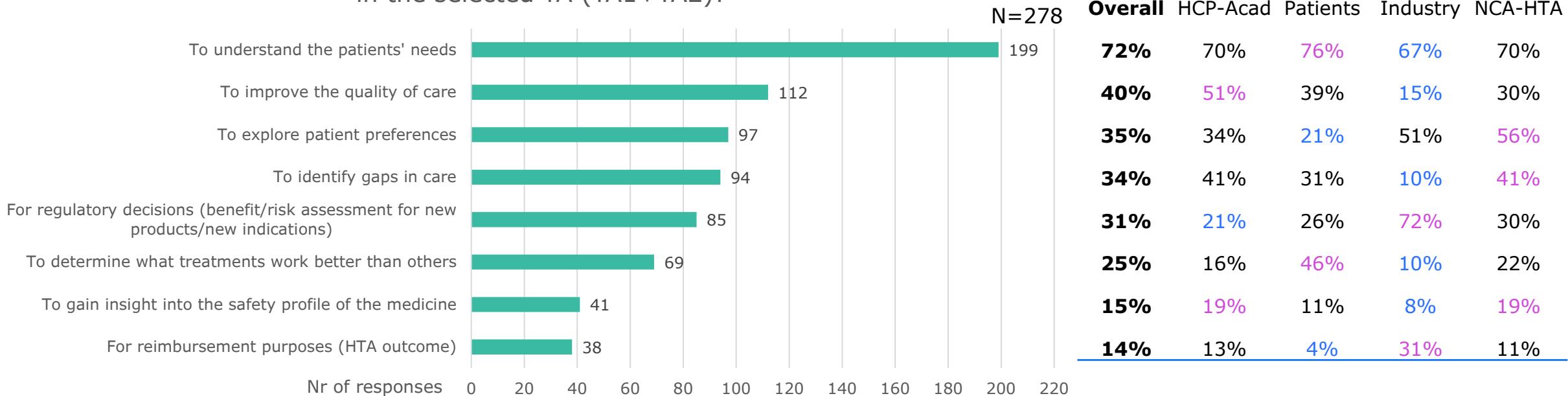


Of note:

- 45 respondents selected a second therapeutic areas, so a total of $233+45 = 278$ responses are available for the analysis
- When combining the first and second choices of therapeutic areas/fields, the most frequently selected fields are:
 - Oncology (17.6% of all responses)
 - Paediatric medicine (17.6%)
 - Neurological disorder (13.3%)
 - Cardiovascular diseases – including diabetes and obesity (10.8%)

Main use of PED

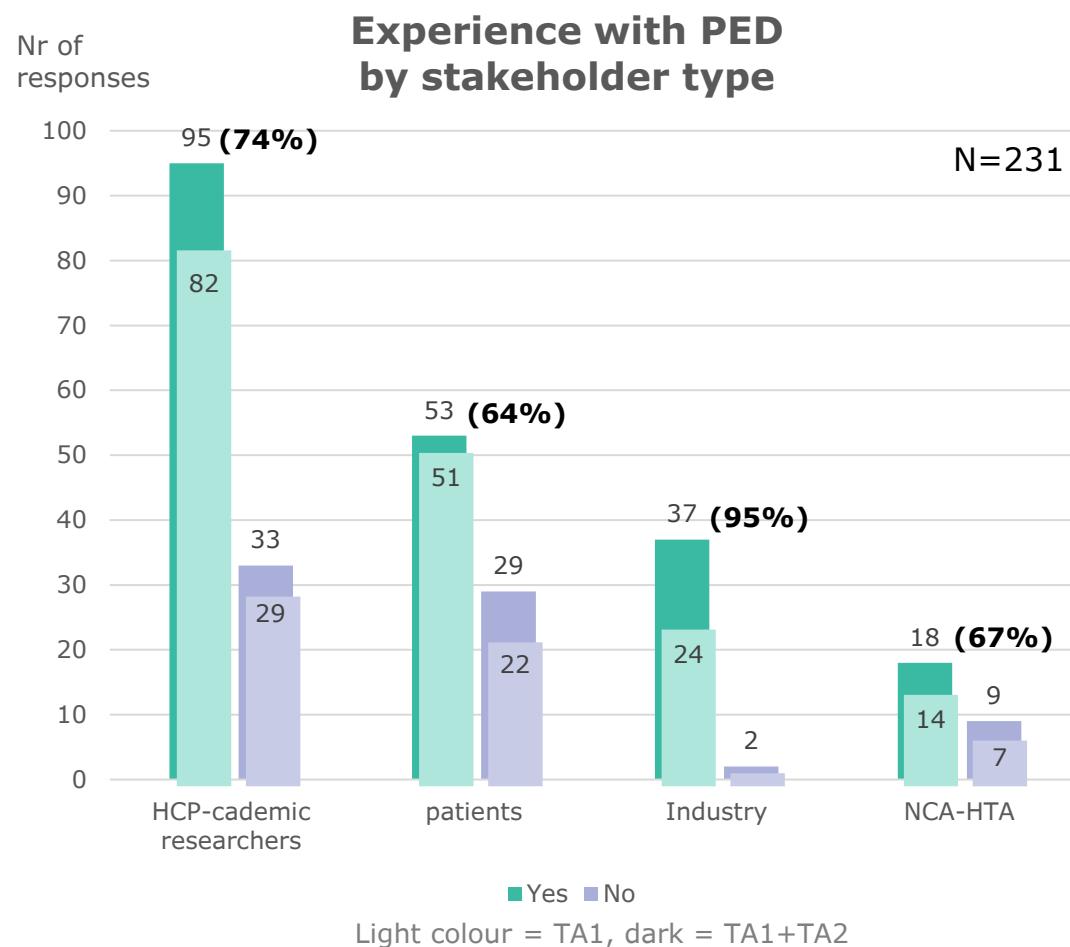
What do you believe PED should be mainly used for in the selected TA (TA1+TA2)?



Of note:

- Respondents had up to 3 choices among the proposed list.
- All stakeholders selected in majority that the main use of PED was to understand the patient needs, except Industry which selected "For regulatory decisions (benefit/risk assessment for new products/new indications)" in majority (followed by the understanding of patient needs)
- The table with the % of selected responses shows many discrepancies per stakeholder group, in particular between Industry and the other groups

Respondents' experience with PED



Question asked:

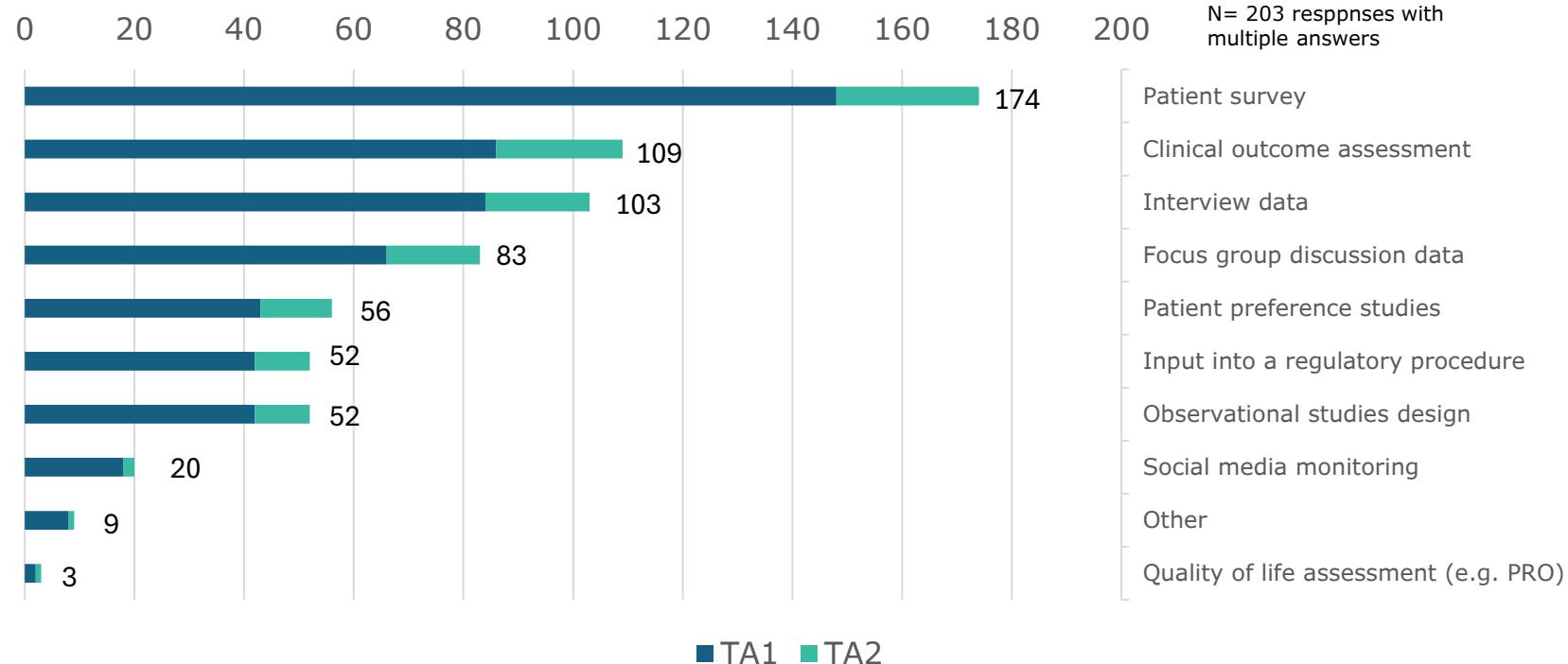
- **To HCPs, HCP representatives and clinical researchers:** have you ever collected, consulted or shared patient experiences that would qualify as PED within your therapeutic area/field (TA)?
- **To industry representatives:** have you integrated PED within your product development?
- **To assessors:** have you ever assessed PED within your TA?
- **To patient organisations:** has your organisation ever collected and shared data that would qualify as PED within the selected TA?
- **To individual patients:** have you ever shared your experience so that it would qualify as PED within the selected TA?

Of note:

- 26% of all respondents reported no direct experience with PED in the therapeutic areas selected as their first or second choice, including 1/3 of assessors and 1/3 of patients.
- In contrast, 95% of industry respondents have had experience with PED

Type of PED experience

What type of PED?



Examples among “other”:

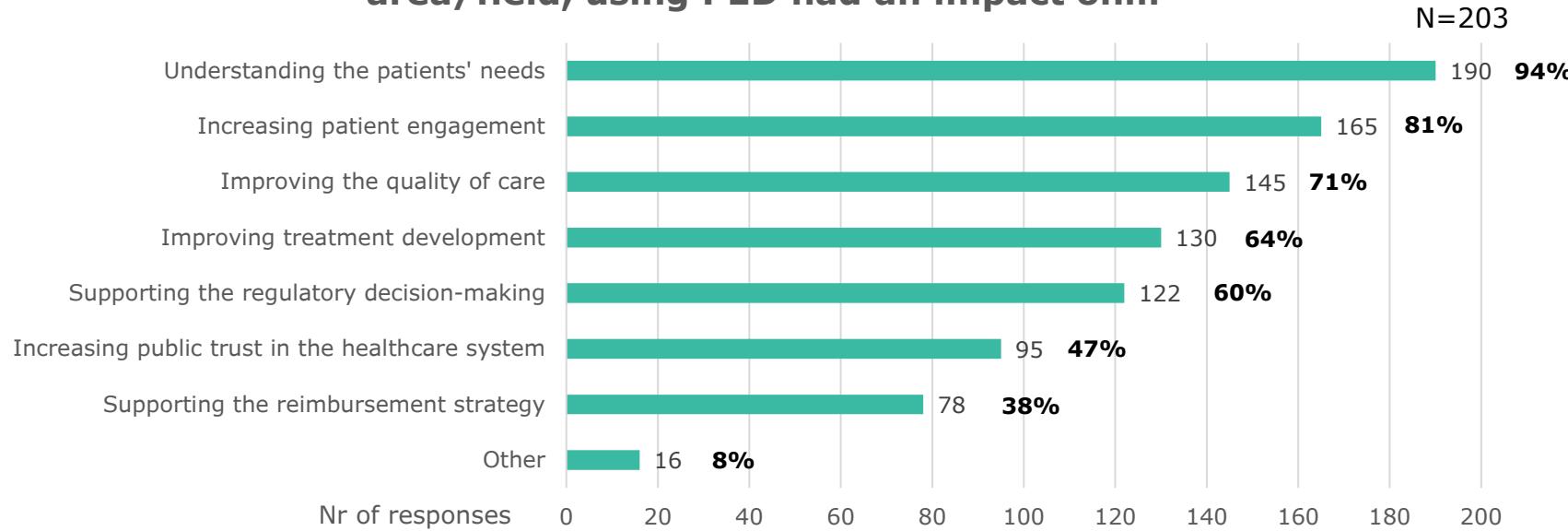
- "Speaking to my doctor about the personal experience, discussing other options, also speaking to my pharmacist and discussing lower dose options"
- E-registry of patients own collected longitudinal medical data

Of note:

- As only 45 respondents across all four surveys selected a second therapeutic area the data for TA2 is smaller
- Across all four surveys, respondents with experience in patient experience data most commonly reported using **patient surveys or questionnaires (84%)**.
- Among healthcare professionals/academic researchers, industry respondents, and NCA-HTA representatives, **clinical outcome assessments were the second most frequently used instrument**, whereas **patients most often cited participation in focus group discussions**.

Impact of PED

Do you believe that, within your selected therapeutic area/field, using PED had an impact on...



Of note:

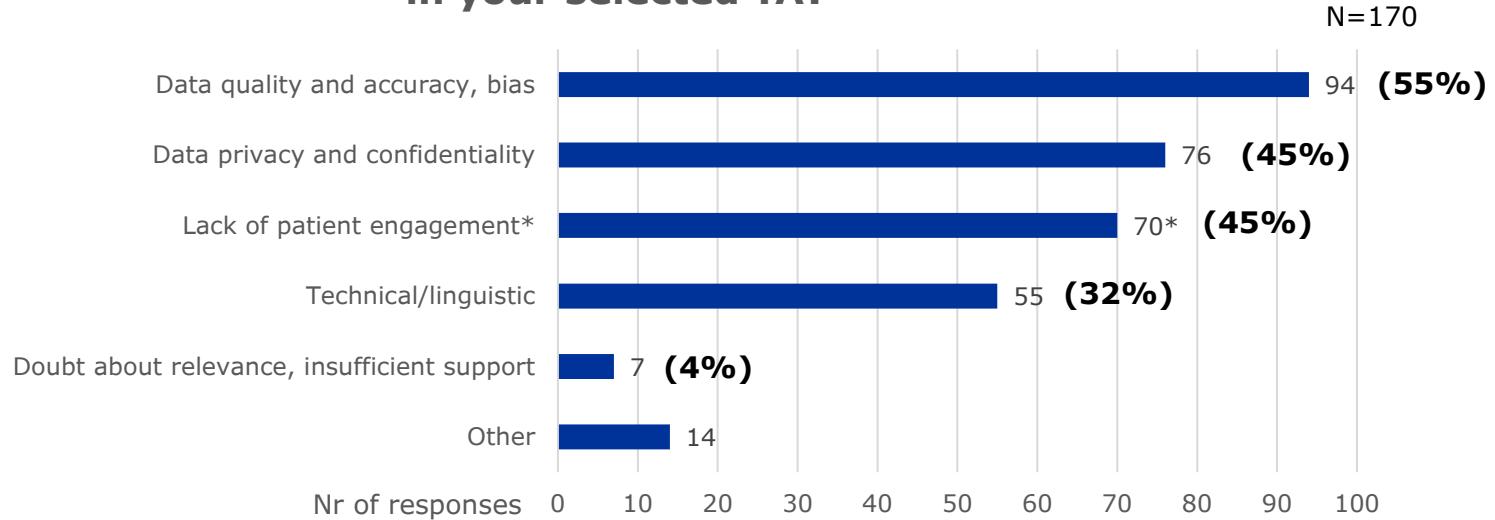
- Out of the 203 responses where experience with PED was confirmed (171 as TA1 and 32 as TA2), the main impacts identified are
 - understanding the patients' needs: selected by 94%,
 - increasing patient engagement: selected by 81%,
 - improving quality of care: 71%

Examples among “other”:

- Adaptation of treatment options to patient need, personalised medicine.
- Design better clinical practice, clinical guidelines, patient support programs.
- Guiding research priorities: Highlighting unmet needs or patient-reported outcomes that influence future clinical studies.
- Identifying gaps in treatment
- Improvement in targeted therapies that meet the needs of intended population.
- Increasing public trust in pharma industry drug development

Challenges with PED

What were the challenges with PED in your selected TA?



Among "other":

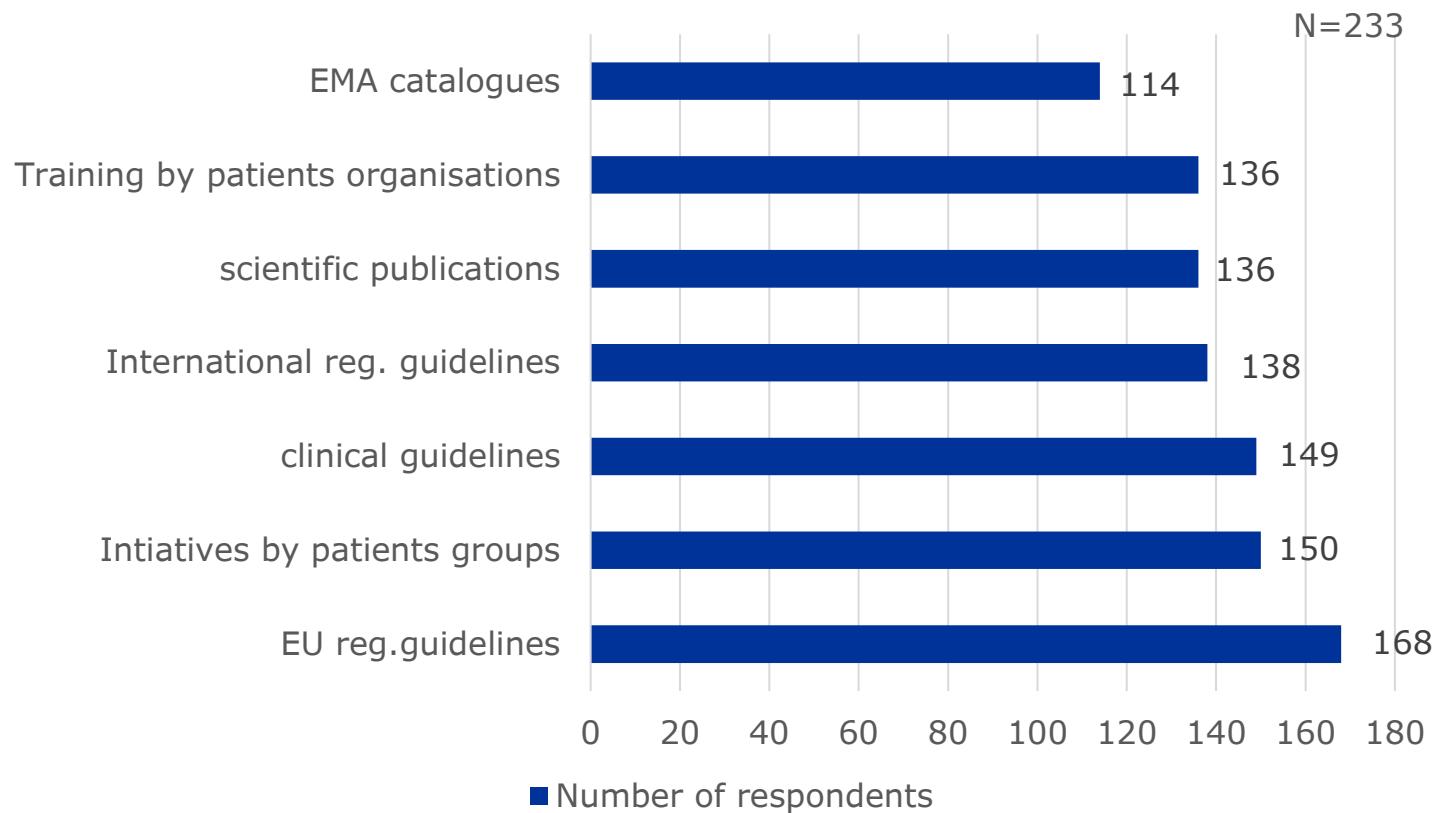
- High standard of regulatory requirement
- Lack of clear framework and easier process
- Lack of clear understanding of how the regulatory authorities are using the data
- Fear of misunderstandings
- Identification of patients can be difficult, especially in rare conditions
- Recruitment and ethical issues
- Lack of internal capacity within the patient organisations
- Non harmonisation of the rules from country to country (e.g., regarding compensation)

Of note:

- Out of the 203 responses where experience with PED was confirmed (171 as TA1 and 32 as TA2), 33 (16%) declared they did not encounter any challenges
- 170 (84%) responses confirmed challenges out of which more than 50% selected "data quality and accuracy"
- 44% (70 out of 157) of patient and HCP organisations, Industry, assessors declared that one of their main challenges was the "lack of patient engagement"

Resources on PED

Question: What resources do you believe can facilitate the conception/collection/review and assessment of PED in your selected TA?



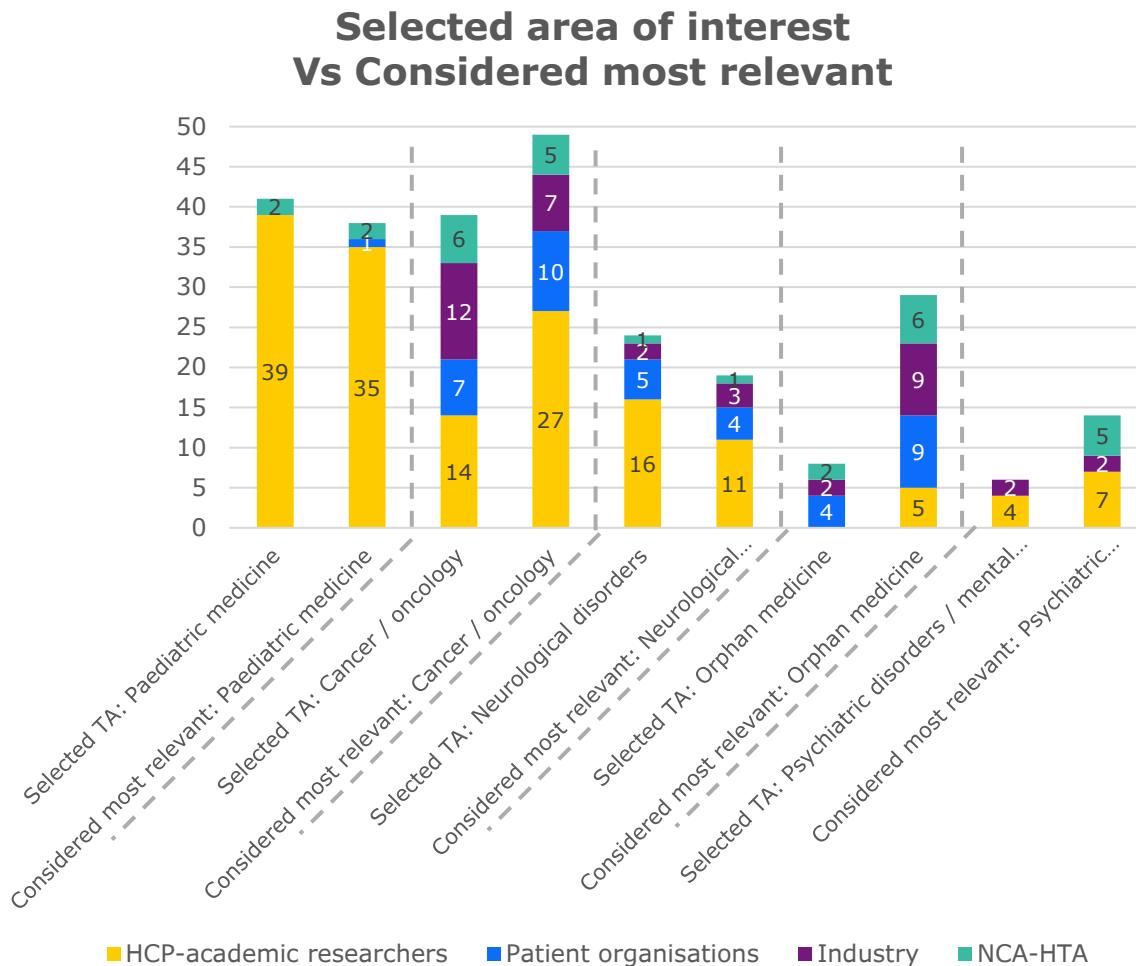
Of note:

- Respondents had up to 3 choices among the proposed list.
- All types of resources have been considered needed
- “EU regulatory guidelines” comes first
- “Initiatives by patient groups” comes second across all stakeholders and TAs

Free text field:

- Disease specific and horizontal guidelines needed
- More guidance on PPD
- More resources for rare diseases
- Network of experts publicly available on EMA website
- High expectation from PED reflexion paper

Therapeutic area/field most relevant for PED



Of note:

- Stakeholders did not always select the area they consider most relevant for PED as their own area of expertise or interest.
- Notably, orphan medicine was rated as highly relevant, despite being chosen as an expertise area by only a small number of stakeholders — and not by any HCPs or academic researchers.



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