Industry survey on Patient Experience Data (PED) Survey results, proposed themes

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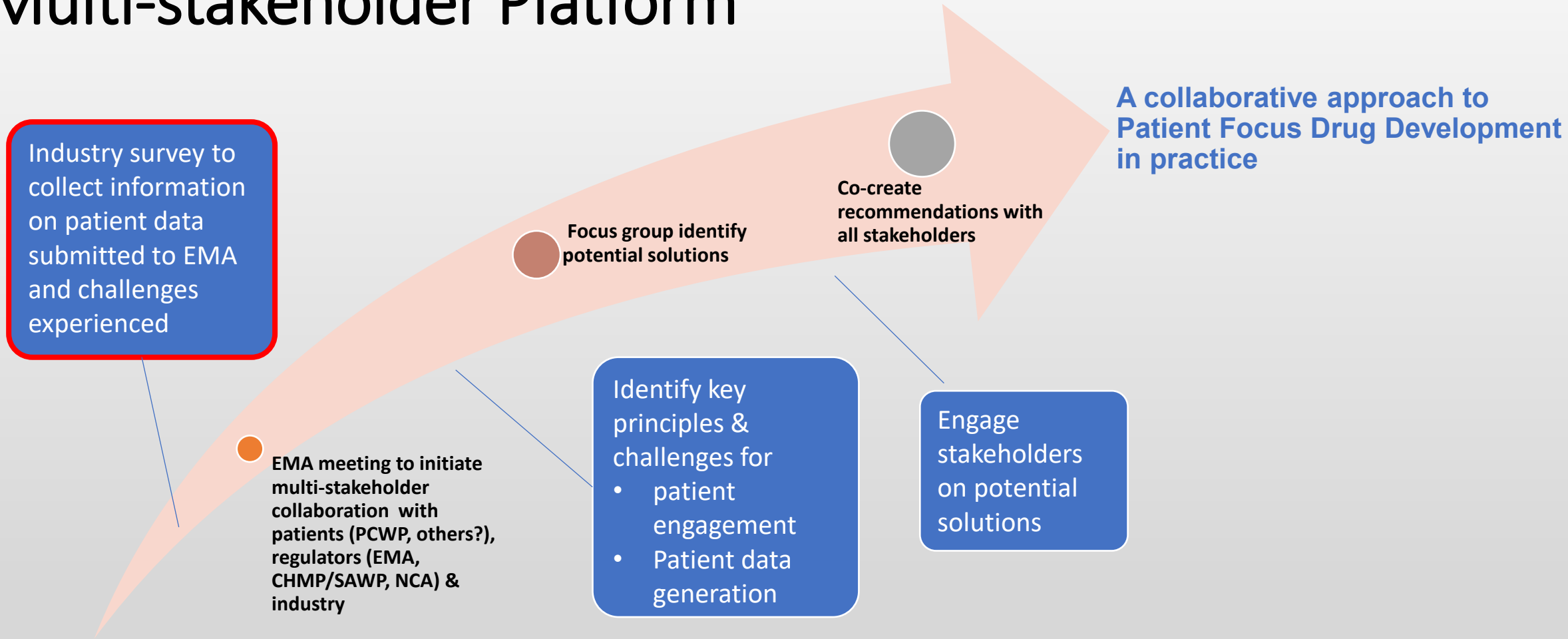
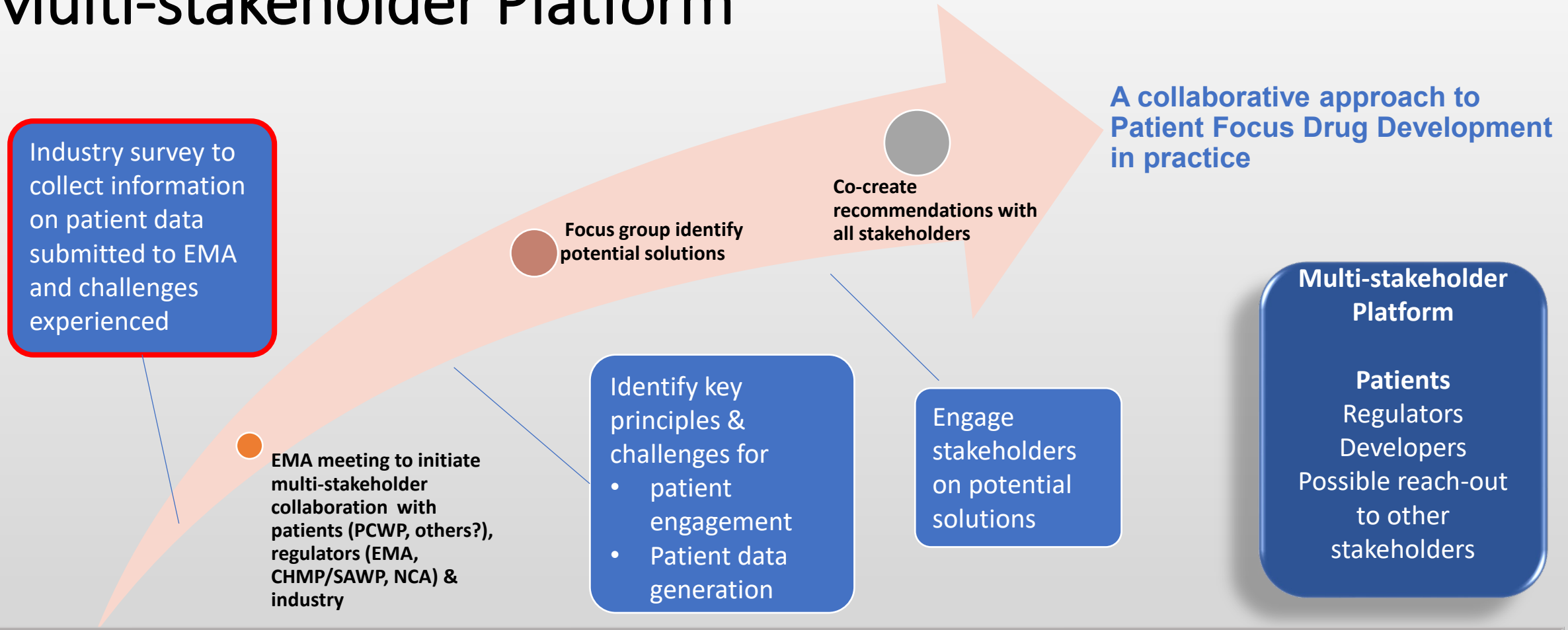
Multi-stakeholder workshop: Patient experience data in medicines development and regulatory decision-making

21 September 2022, EMA, Amsterdam

Where we started from: A vision for a Roadmap to Multi-stakeholder Platform



Where we started from: A vision for a Roadmap to Multi-stakeholder Platform



Overall initiative objective: Increase **patient engagement** and systematic **patient data generation** in drug development to inform EU regulatory decision making

Stakeholder meeting objectives: Exchange on current information and initiatives from the different stakeholder groups and create an action plan; decide on best format for continuous collaboration and future exchanges

Survey results – PED collection

- Industry members of EFPIA, EUCOPE, EuropaBio surveyed on their experience in Patient Experience Data (PED). Responders expressed interest to share case examples
- Many industry developers, especially large and medium-size organisations, are systematically collecting PED and have dedicated staff focused on patient-centric activities
- Half of them are collecting PED in all development programmes; the remainder are collecting PED primarily in oncology, immunology/haematology and rare diseases
- The vast majority of responders collect PED in the EU, US,UK, Japan and Asia Pacific Region
- PED is collected from pre-clinical to post-marketing phase but especially during the clinical development phase
- Patient input is collected to inform knowledge on disease and treatment background, on clinical trial design aspects and on aspects related to the conduct of the clinical trial
- A range of qualitative and quantitative methods are used to collect PED. There is a good knowledge on methods

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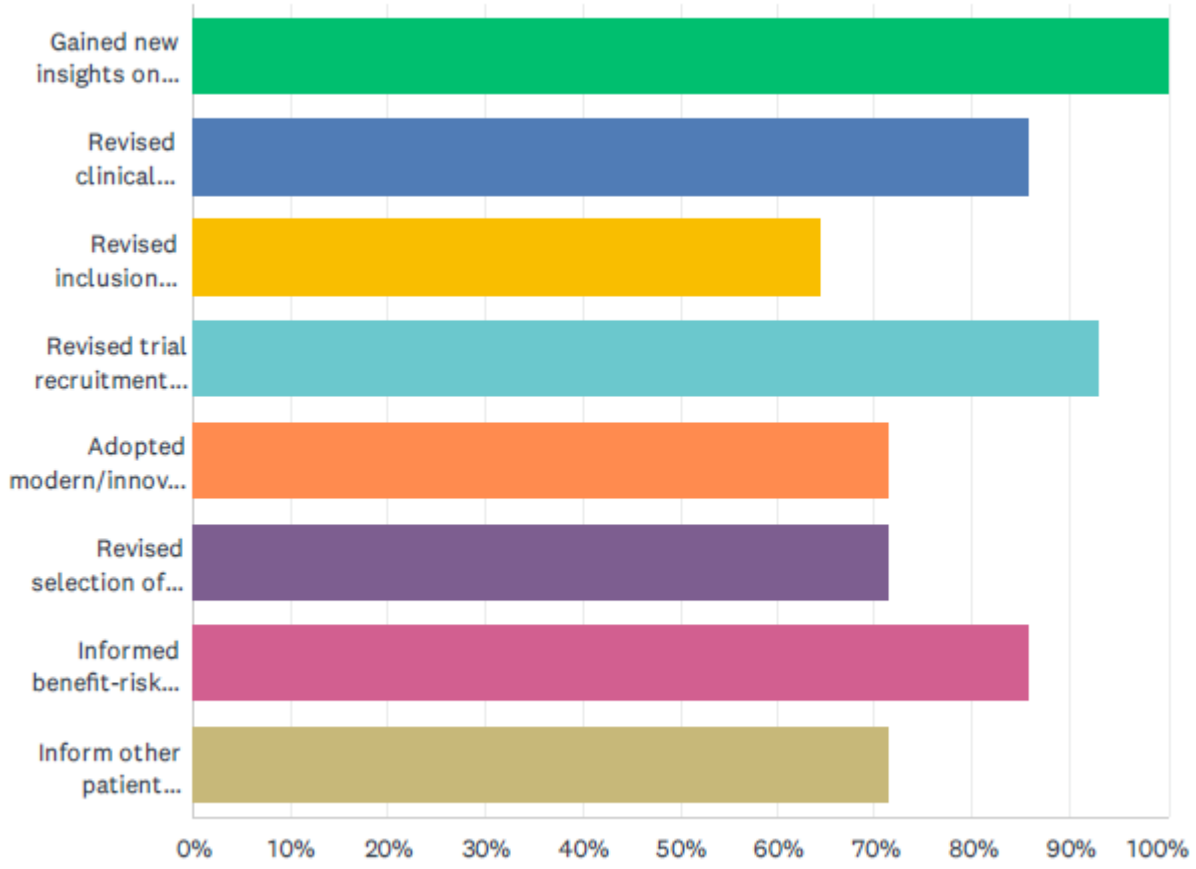
Collection and use of PED is seen by industry as high value

- All responders gained new insights on symptomology from understanding the disease and treatment burden.
- Most of responders :
  - Revised trial recruitment and/or retention strategy
  - Revised clinical protocol/clinical trial design
  - Informed benefit-risk assessment
- More than 70% of responders:
  - Adopted modern/innovative clinical trial solutions (telemedicine, digital health technology)
  - Revised selection of endpoints and/or clinical outcomes assessment strategy.
  - Inform other patient preferences regarding their treatment (e.g., preferences on dosage regime/adherence)
- Revised inclusion and/or exclusion criteria was seen as value by 64% of responders



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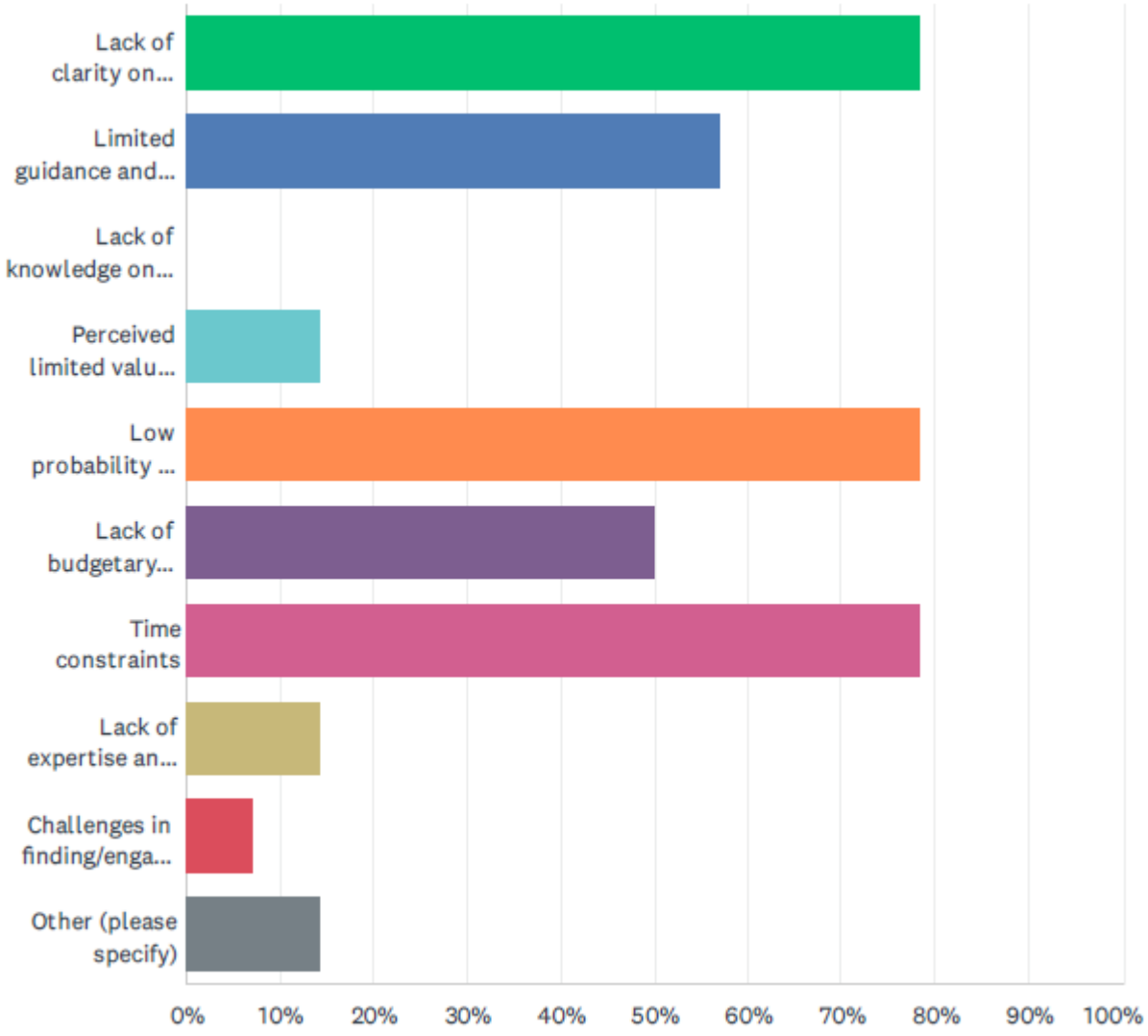
Main challenges in collecting/using PED

- Majority (78%) of responders perceive as challenges in collecting/using PED:
  - Lack of clarity on regulatory acceptance or use in benefit risk decision making
  - Low probability of inclusion in the label
  - Time constraints
- More than 50% identify as challenges:
  - Limited guidance and recommendations
  - Lack of budgetary commitment
- Other perceived challenges (<15%):
  - Perceived limited value to development program
  - Lack of expertise and resources
  - Challenges in finding/engaging patients to collect patient centric data
- Lack of knowledge on various types and methods is not identified as a challenge



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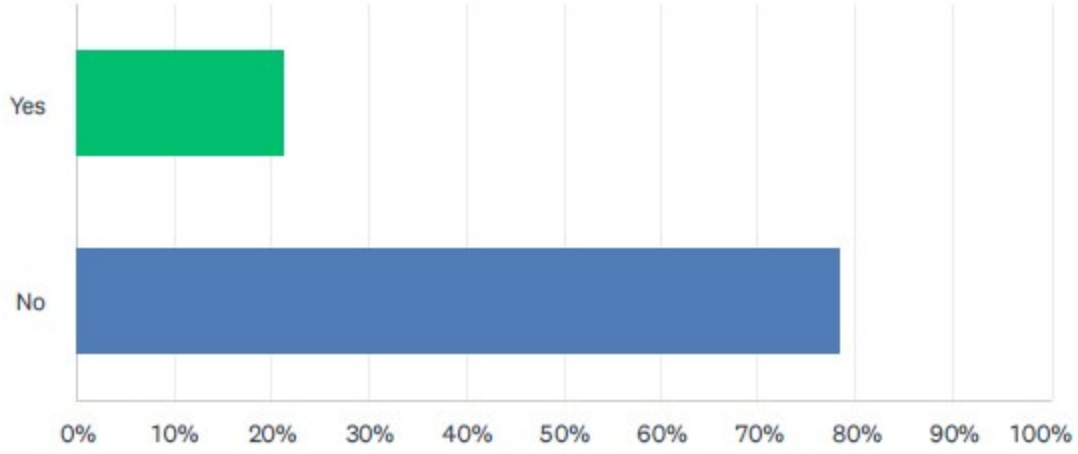
Survey results – PED in regulatory decision making

The majority (78%) of responders reported that Health Authorities (HA) accepted PED submitted in MAA.

However, a range of different feedback from HA is reported; there are examples of traditional PED being accepted (e.g. pertaining to COAs) but there is less acceptance of broader types of PED e.g. preference data.

There is not sufficient clarity on how PED is used to inform regulatory decision making

- For the majority (78%) of responders there is not clarity on how PED is used to inform regulatory decision making



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A collaborative approach to Patient Focus Drug Development in practice: themes and example of proposals for a multi-stakeholder platform

Continue to support patient engagement

Advance Science of Patient Input*
Development of methodological/regulatory guidelines

Establish EMA-sponsor dialogue platform, informed by patient engagement, dedicated to discussing collection and use of patient input in drug development

Need and Benefits of Transparency in Decision Making

*The *"science of patient input"* refers to systematic collection and incorporation of methodologically-sound, robust, fit-for-purpose patient input/patient experience data throughout the drug development lifecycle

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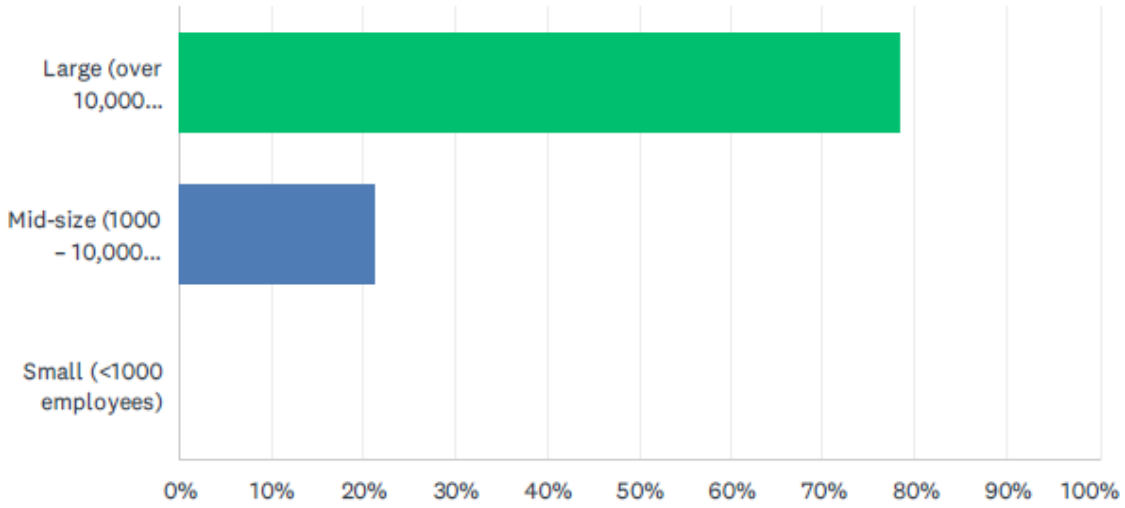
Thank you!

back-up slides

Q1 – what size company do you represent?

- Majority responders Large industry (> 10000 employees)
- Mid-size industry represented



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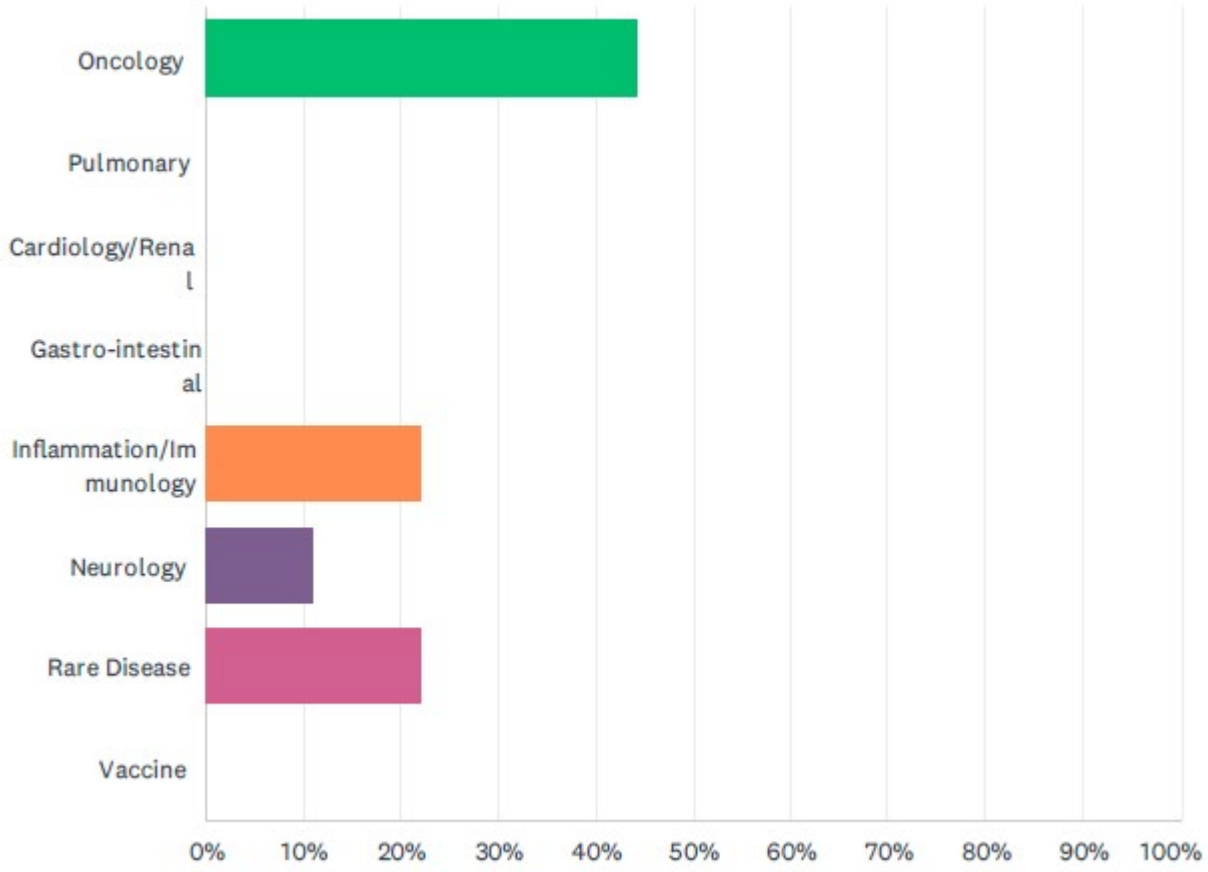
Q2 – In which therapeutic areas is your company mainly collecting PED as part of the development programme?

- 50% responders systematically collect PED in all therapeutic area programmes
- Collecting PED in* Oncology, Inflammation/Immunology, rare diseases and neurology development programme



*Primary therapeutic area indicated in the response

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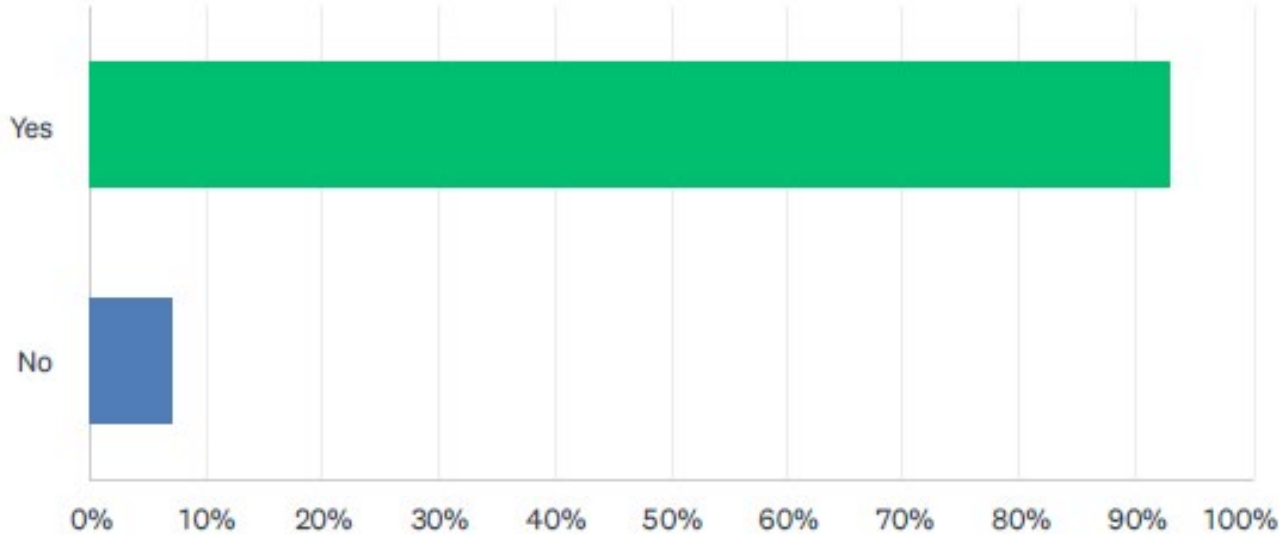
Q3 – Does your company have dedicated staff focused on patient centric activities?

- The majority of responders have dedicated staff focused on patient centric activities



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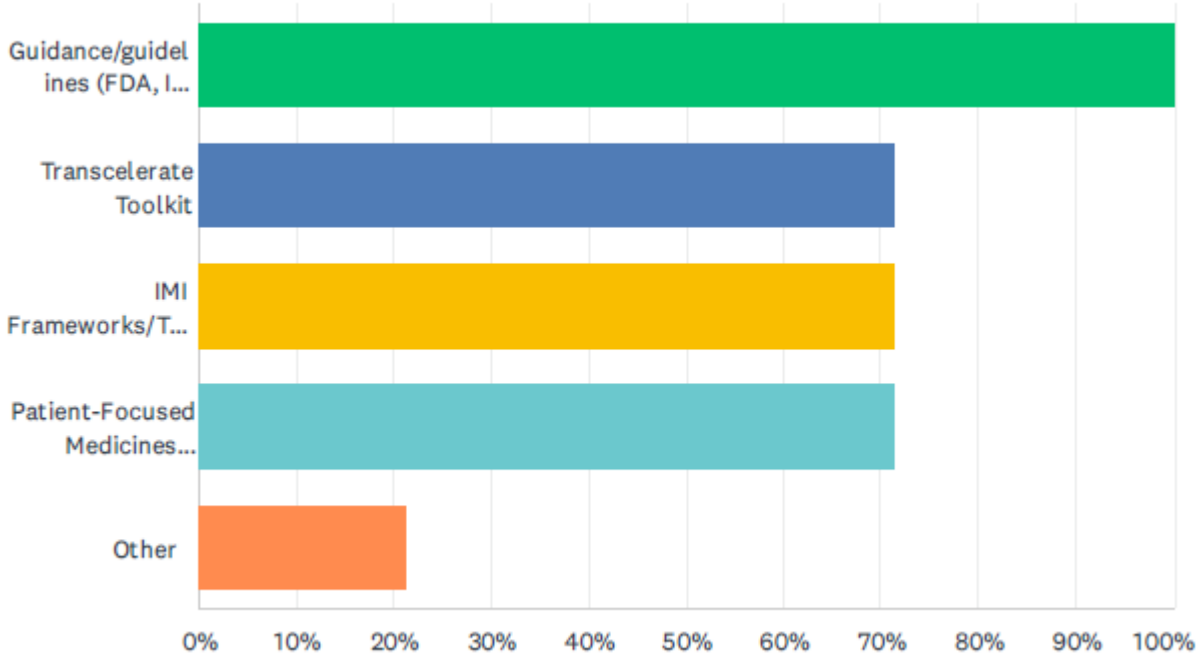
Q4 – What resources have you used to help guide your patient centric work (e.g., guidance docs, TransCelerate Toolkit, etc.)?

- Regulatory guidelines are used by all responders
- Other tools like Transcelerate toolkit, IMI Frameworks/Tools (PARADIGM, PREFER) and other Patient-Focused Medicines Development frameworks are used by 75% of responders



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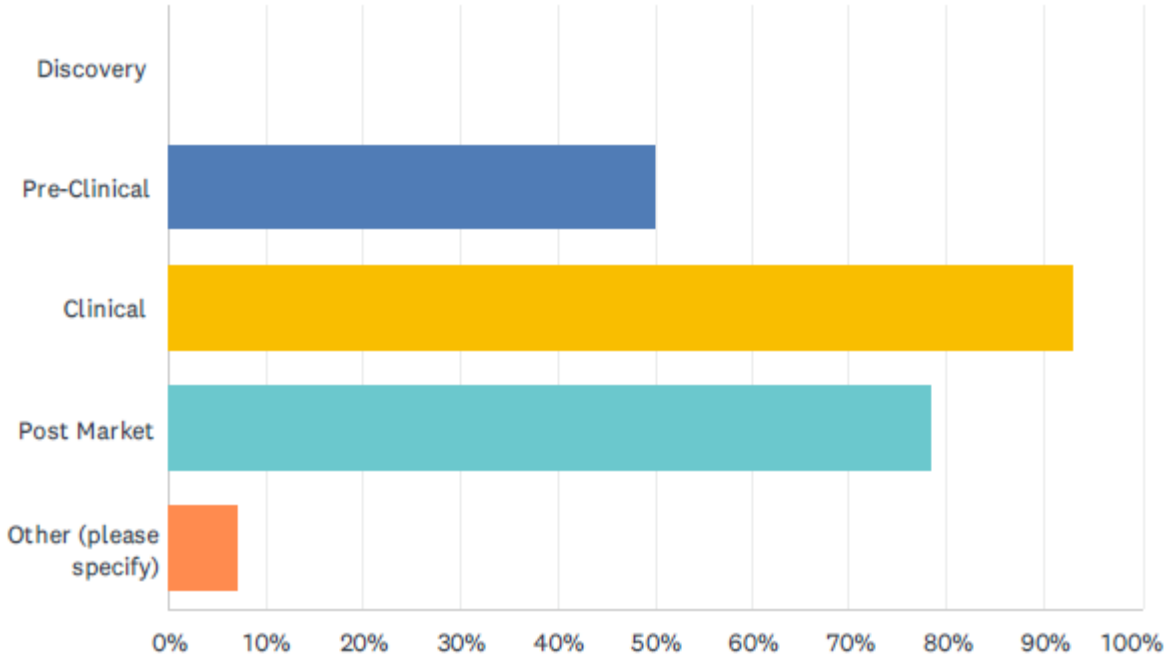
Q6 – When is patient experience data collected in the medical product lifecycle for your program?

- PED is collected from pre-clinical phase to post-marketing phase but especially during the clinical development phase (93% responders)



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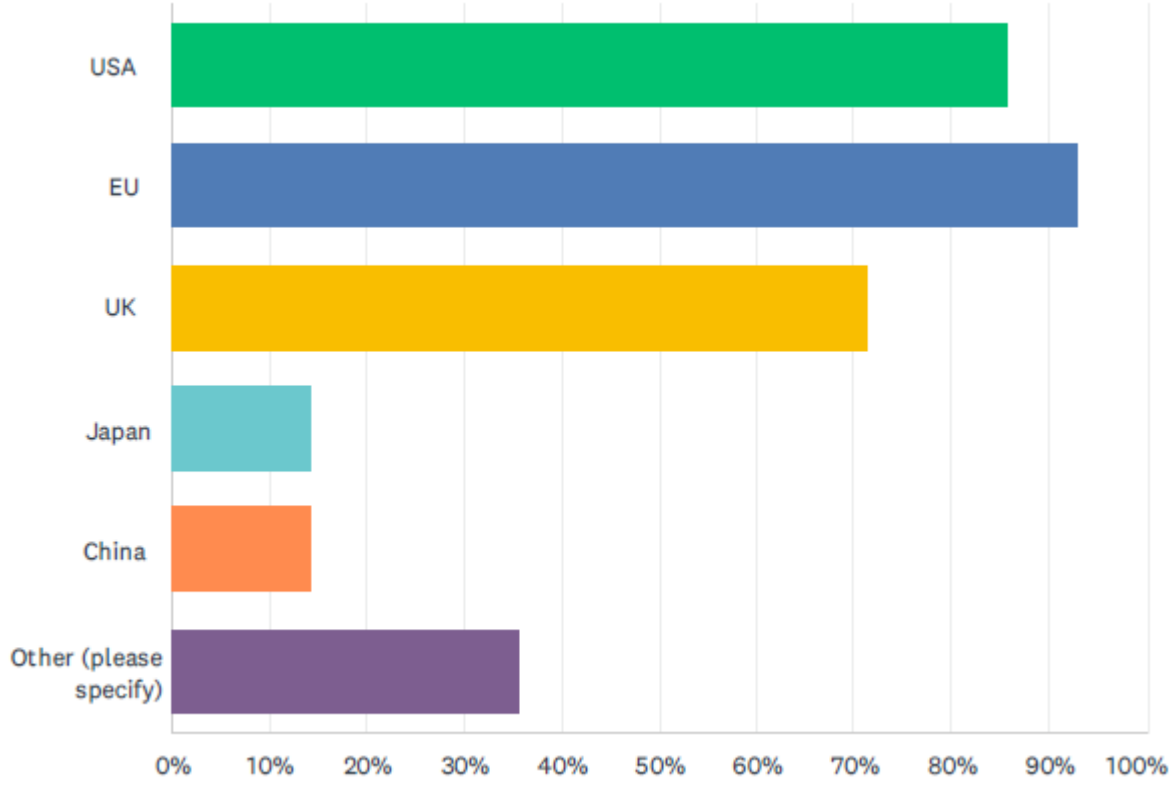
Q7 – In which countries/regions is your company mainly collecting patient experience data?

- PED is collected by the majority of responders in the EU (93%) ,USA (86%) and the UK (71%)
- Japan, China and other JAPAC countries are sites where PED are collected (cumulative 30%)
- Few responders collect data globally depending on the location of the trial site



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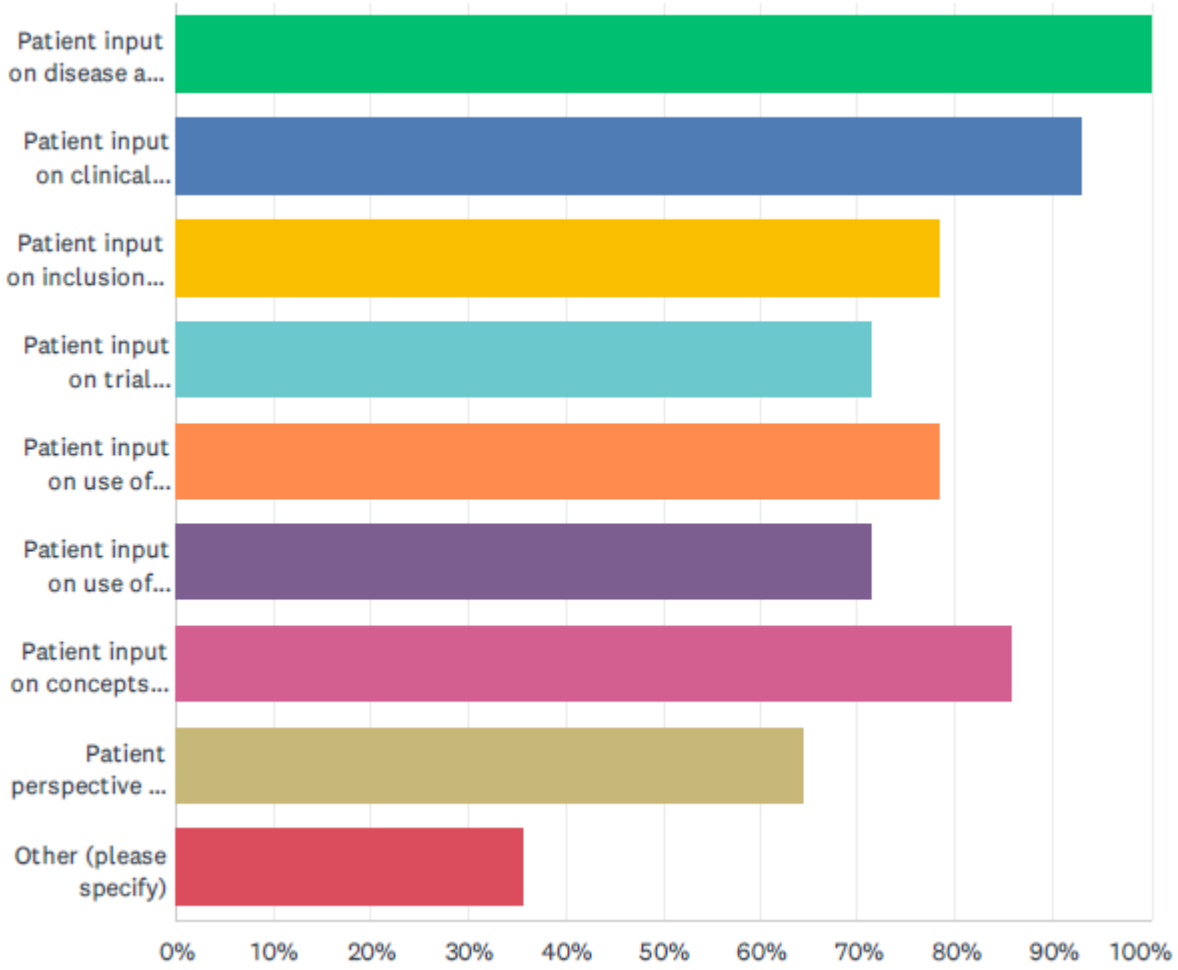
Patient input is collected to inform knowledge on disease and treatment background, on clinical trial design aspects and on aspects related to the conduct of the clinical trial

- Patient input on disease and treatment background (symptoms, impact, treatment burden) is collected by all responders
- The vast majority of responders collect :
  - Patient input on clinical protocol/clinical trial design
  - Patient input on concepts that matter most to them to inform COA/PRO development/selection
  - Patient input on inclusion and/or exclusion criteria
  - Patient input on use of wearables or other digital health tools to collect patient input in a trial
- More than 60% collect
  - Patient input on trial recruitment, enrolment, and/or retention strategy
  - Patient preference information



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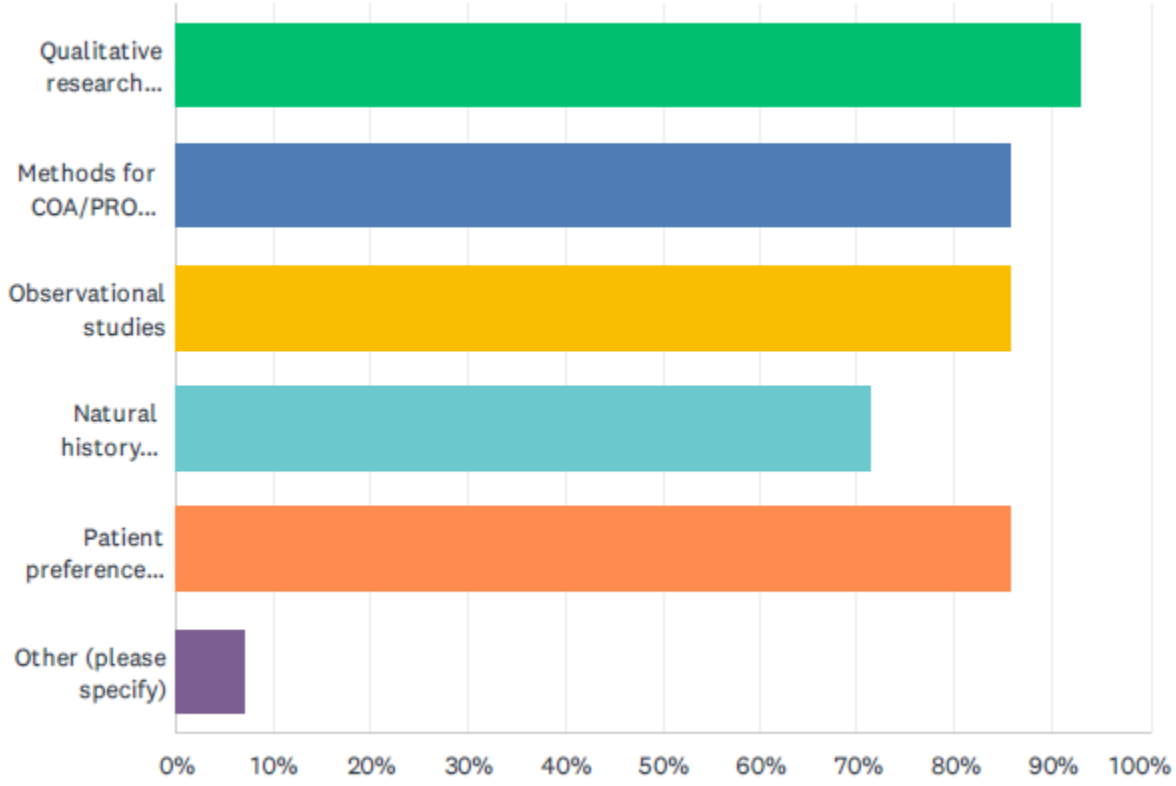
A range of qualitative and quantitative methods are used to collect PED. There is a good knowledge on methods

- More than 86% of responders use the following methods to collect PED:
  - Qualitative research methods (1:1 Interview, Focus group, Patient Advisory board, Surveys)
  - Methods for COA/PRO development/implementation (Concept elicitation, cognitive debrief, etc.)
  - Observational studies
  - Patient preference studies
- Natural history studies/Patient registry are used to collect PED by 71% of responders
- Social media listening, natural language processing was flagged by one responder



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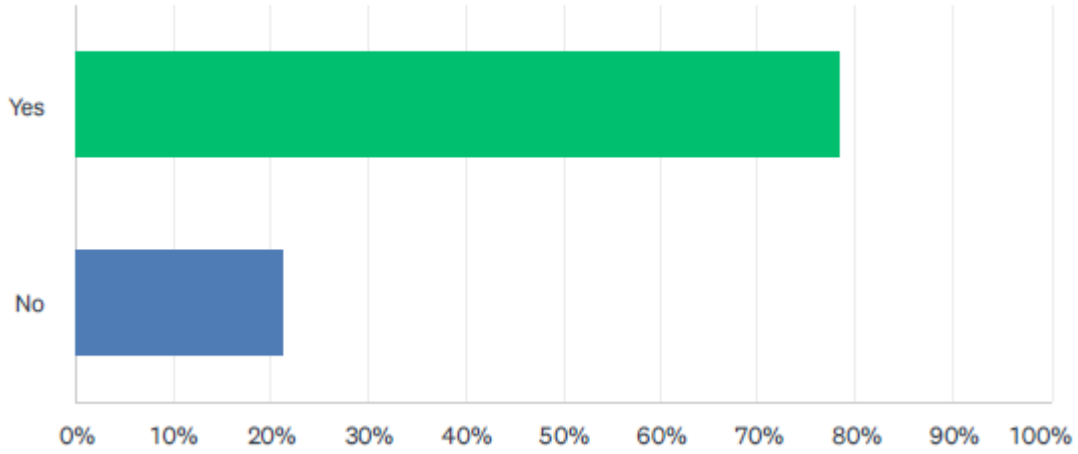
Health authorities accepting the patient experience data submitted as part of marketing authorization application

- The majority (78%) of responders reported that HA accepted PED submitted in MAA
- Range of different feedback from HA is reported: examples of traditional PED being accepted (e.g. pertaining to COAs), apparent less appetite for broader types of PED e.g. preference data.



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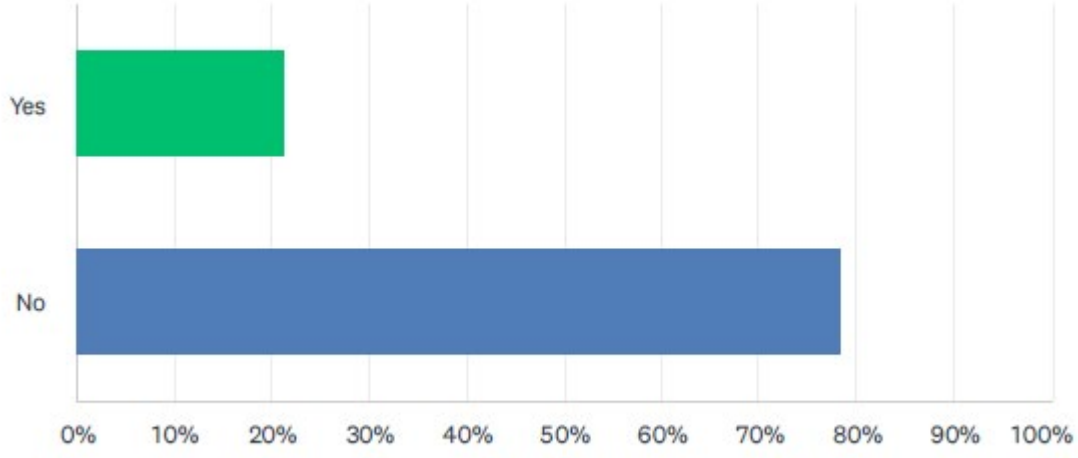
Need for clarity on how the submitted patient experience data is used to inform regulatory decision-making

- For the majority (78%) of responders there is not clarity on how PED is used to inform regulatory decision making



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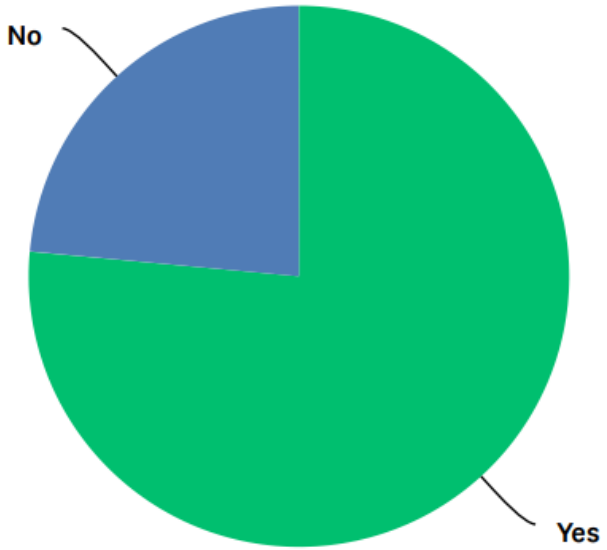
Clarity needed on how PED is used to inform regulatory decision making

The majority (76%) of responders:

- have experience, as part of discussions of a development programme, interaction by regulators with patients or PED and
- have received advice on PED collection in scientific advice, protocol assistance or PRIME kick-off meeting



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Several case studies collected on a range of themes/topics

- Developing patient relevant endpoints (the what)
  - Developing patient relevant Clinical and Patient reported Outcomes
  - Patient relevant endpoint selection
- Patient consultation during development (the how)
  - Patient consultation during pre-clinical development
  - Patient input during protocol development
- Patient input during scientific advice
  - Patient input during scientific advice
  - Patient preferences in scientific advice with HTA
- Patient experience data and patient voice in benefit risk and decision making
  - Patient experience data to inform benefit risk (the what)
  - Patient input and use during regulatory assessment and decision making (the how)
- Transparency

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