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CDDF Multi-Stakeholder Workshop

Involving patients in oncology drug development

Reflection on Meeting Outcomes & Next Steps

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Patient Involvement in Oncology Drug Development

Examples include:

- Optimising patient /patient advocates representation in discussions on Medical Research, CT design & endpoints, B/R assessment, reimbursement
- Optimising the assessment of PROs and patient preferences in drug development
- Using digital health applications to increase CT access (e.g., decentralized trials), decrease completion burden (home completion); generate additional evidence of treatment experience

Challenges include:

- International drug development & regional differences in clinical practices and patient's input
- Regulatory, payers frameworks, & legislation differ

(Selected) Perspectives

Patients

- Are the most important partners in the fight against cancer; they want to be involved from the very beginning of the drug development process.
- Distinction between ‘expert’ and ‘naïve’ patients seems counterproductive

Authorities

- Uncertainties on how to consider patient preferences and input in decision making.
- Publication of best practices / case studies & engaging with the scientific / patient community.

Science of Patient Input

- Patient voice is not uniform
- Reflects a multitude of subjective experiences (determined by the patient’s health literacy, socio-economic status)
- All stakeholders on a steep learning curve.

Industry

- needs clarity on regulators /health authorities’ expectations regarding patient experience information/patient involvement format in drug development.

Highlights of the Discussion

Case studies where patients input really moved the needle, e.g.

- Research: quantify the value of patient-relevant attributes of Multiple Myeloma treatment; understand how preferences may vary according to disease and patient characteristics.
- Lenalidomide in Multiple Myeloma: patient & thalidomide victims letter & involvement in the RMP discussions at EMA
- US Crizotinib PI: example how descriptive PRO data can complement clinical safety findings
- HTA decision-making process: using the PICO methodology to collect patient feedback on the relevant Population, Intervention, Comparators, and Outcomes
- Patient preferences for mode of administration (Rituxan Hycela)
- Patient advisory board meetings as mechanism to solicit input in CT design

Meeting Outcomes - Consensus

The **mindset change** required to drive patient involvement has already happened.

Further research is necessary to define the best methodologies for patient preference studies.

Need to raise awareness of the benefits of CT in general and facilitation of patient access to CT (huge educational gaps).

Stakeholders are encouraged to develop policies on patient involvement.

Industry perspective: important to seek early parallel scientific advice (regulators/HTA) on PRO strategies, especially in areas of uncertainty.

Take Home Messages

- **Multi-stakeholder discussions drive change.**
- **Patient advocacy perspective:** political will for change is important to establish the right framework for PFDD at EU level.
- **Standardized outcome assessments** can improve informed decision making and the quality of care
- **Regulator perspective:** significant progress has been made. Current priorities are advancement and standardization of PRO and patient experience data collection, analysis, and communication.
- **Time pressure for industry to move fast** - may not always allow for patient involvement in a meaningful way.

Next Steps

Use the multi-stakeholder network within CDDF to periodically share information and perspectives to drive progress.

Monitoring of the global regulatory environment on patient involvement in (oncology) drug development

Potential follow-up workshop in 2021?

Proposed ICH Guideline Work to Advance PFDD

- Draft reflection paper by the FDA and the EU Commission: **not yet published for public consultation:** identifies a series of drug development and regulatory decision-relevant questions along drug "life cycle"
  - **PROPOSES New ICH guideline** addressing what to measure in a clinical trial, including refining the set (list) of important impacts and concepts from patients, to select or develop clinical outcome assessments (COAs) that can demonstrate change, defining endpoints, and meaningful change.
  - **PROPOSES New ICH guideline** addressing methods for elicitation or collection of qualitative or quantitative assessments of the relative desirability or acceptability to patients of specified alternatives or choices among outcomes or other attributes that differ among the alternatives.
- Work on the proposed ICH topics should wait **until IMI PREFER is completed in Sept 2021.**
- **Challenge:** membership of patient organizations not allowed in current ICH structure.

Back-up

FDA PFDD Draft Guidances: progress update

PFDD Guidance 1: Collecting Comprehensive and Representative Input

- Workshop held on Dec 18, 2017
- Issued draft guidance in June 2018; available here
  - Defining Research Questions and Objectives
  - Defining Target Populations
  - Selecting Methods and Study Design
  - Operational Aspects
- For more background information click here on FDA website

PFDD Guidance 2: Methods to Identify What is Important to Patients

- Workshop held on Oct 15-16, 2018
- Issued draft guidance in October 2019; available here
  - What do you ask, and why?
  - How do you ask non-leading questions that are well understood by a wide range of patients and others?

FDA PFDD Draft Guidances - progress update

PFDD Guidance 3: Select, Develop or Modify Fit-for-Purpose Clinical Outcome Assessments

- Workshop held on Oct 15-16, 2018
- Issued discussion document available here;
  - How do you decide what to measure in clinical trials and select or develop fit-for-purpose COA (clinical outcome assessments)?

PFDD Guidance 4: Methods and Technologies for Clinical Outcome Assessments

- Workshop held on December 6, 2019
- Issued draft guidance in October 2019; available here
  - Technologies that may be used for collection, capture, storage and analysis of PPI
  - Methods to better incorporate COAs into endpoints robust enough for regulatory decision making