CDDF MULTI-STAKEHOLDER WORKSHOP on INVOLVING PATIENTS IN ONCOLOGY DRUG DEVELOPMENT

[Amsterdam, Netherlands]

18-19 June 2019

Organizing association

(Cancer Drug Development Forum)

Key Contact

Cancer Drug Development Forum (info@cddf.org)

EVENT OUTLINE

Patient involvement in development, review and approval of drugs is an increasing focus for health authorities and other stakeholders.
Patients, as true research partners, are increasingly involved in defining the research agenda to address unmet medical needs, advising on clinical trial designs and study implementation and fostering generation and review of patient-relevant evidence for regulatory decision and market access. Patients, regulators, HTA bodies, payers, and health-care providers are increasingly demanding patients' experience and/or preference data to comprehensively ascertain the benefit-risk or value of a given medicinal product or to support treatment decisions in the increasingly complex clinical treatment options.

New guidelines are being developed to inform generation of patient relevant experience (e.g., FDA PFDD guidances), and inclusion of such evidence as part of the benefit-risk assessment (ASCO and ESMO clinical benefit models, patients involvement in scientific advices in Europe). Overall, however, there is not enough transparency on how best to involve patients in clinical research and which patient-relevant evidence could be used to inform stakeholders decision making.

This session will therefore look at the opportunities and challenges of incorporating the patient-relevant evidence into oncology drug development and approval process from the perspective of patients, health care providers, health authorities, HTA bodies, payers and industry and will address the following topics:

- Examine the different type of patient-relevant evidence for patient-focused drug development
- Exemplary models for interactions between patients' communities, sponsors and decision-makers
- Examples of qualitative and quantitative patient-experience data and its contribution to development, review, approval and access to new drugs

PROGRAMME COMMITTEE

- **Academia:** Axel Glasmacher (CDDF Board)
- **Industry representatives :** Claudia Hey (Merck), Elisabeth Piault-Louis (Genentech), Marloes Van Bruggen (Roche)
- **Patient Advocates**: Francesco De Lorenzo (ECPC, Italy)
- **Regulatory Authorities**: Ralf Herold (EMA)

TARGET AUDIENCE

The target is a multidisciplinary audience of Patient Associations, Academia Representatives, EU and US Regulatory Bodies (EMA, FDA, National Agencies), Pharmaceutical Industry, and HTA representatives.

WORKSHOP VENUE & & HQ HOTEL

Park Hotel, Amsterdam
Stadhouderskade 25
1071 ZD Amsterdam
The Netherlands

DRAFT PROGRAMME

DAY 1

13:00 Introduction and evolving landscape

This workshop programme addresses the increasing focus of the patient's perspective on products in development, assessment and evaluation of benefit/risk and makes also reference to recent developments in the regulatory landscape, e.g. FDA draft guidelines on patient focused drug development released since 2018.

13:15 Involving Patients in oncology drug development: overall views and expectations (30')

Panel with short statements (5 min) from stakeholders to kick-off the meeting. What does patient focused drug development mean for the stakeholders, why are they interested in the meeting and what are their expectations.

[Addressing also proposals for envisaged concrete proposals e.g. white paper/publication to be re-addressed in the final panel discussion.]

13:45 Involving Patients in oncology drug development: when and how? (60')

Industry and patient representative usually generating the data give relevant examples on their experiences on involving patients in oncology drug development. (same case study from different perspective- call for industry experience selecting 2 different cases; other examples as posters?

Presentation on 2 industry cases & patient perspective (2x 20min); Panel & participants discussion

14:45 COFFEE BREAK (30')

15:15 Involving Patients in oncology drug development: impact on decision-making (60')

Stakeholders using patient experience data for their decision-making exchange their experience by referring to critical factors (e.g. how the data will be used to evaluate a product, impact of such data). Recent ICH proposal in the context of PRO and harmonisation of standards in general.

3 x 15 min presentations followed by panel & participants discussion; Panel & participants discussion

16:15 Fostering collaboration between patient community, sponsor and decision-makers (45')

Stakeholders will address obstacles to overcome (e.g. weak health literacy) and address initiatives/enablers to facilitate platforms discussing in depth multi-stakeholder collaboration on involving patients in oncology drug development.

17:00 Introduction into break-out sessions

The chairs of the break-out sessions in the next morning will briefly introduce topics and goals of the break-out sessions.

17:15 End of Day 1

19:30 Networking Dinner

DAY 2

09:00 Three break-out sessions – Roadblocks and solutions (60')

The break-out sessions will allow for an in depth discussion on obstacles and opportunities in the different stages of product development and approval. The groups will analyse challenges and propose potential solutions to overcome the existing challenges.

1. Patient involvement in protocol and ICF development

Discuss scope and timing of patients' involvement in protocol development and review of ICF: e.g., Transcelerate initiative focussing on standardization of protocol and ICF across the industry and in documenting patients' experience participating in clinical trials. Outcome selection.

2. Patient involvement during product assessment

Discuss collection of patient-relevant evidence (e.g., qualitative data to inform unmet medical need, Patient-reported outcomes to inform clinical benefit assessment, other) during the drug lifecycle and opportunities to submit such evidence to inform regulatory decision making (e.g., in briefing package for Advisory Committee, in CTD for approval decision). Discuss Regulators approach to involve patients in regulatory decision making. How to systematically include patient evidence into the regulatory approval process; where in the label - recent US examples. Let us know how we can improve. Involvement in B/R assessment; reference to IMI PREFER project.

3. Patient input into HTA assessment

Patient-relevant post approval activities (PASS, Patient registries, RWE)

10:00 COFFEE BREAK (30')

10:30 Feedback presentations from the break-out sessions (45')

One presenter from each break-out group will report back from the discussion and summarize potential solutions identified by the group.

11:15 Summary and next steps (60')

Panel participants and meeting chairs will summarize the main pain points and potential solutions and give an outlook on the meeting report on recommendations from the workshop (i.e. what are the panelists going to do different in future).

12:15 END OF THE MEETING