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

[Amsterdam, Netherlands]

18-19 June 2019

Organizing association

(Cancer Drug Development Forum)

CDDF
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, DE)
- **Industry representatives :** Claudia Hey (Merck Healthcare KGaA, DE), Elisabeth Piault-Louis (Genentech), Marloes Van Bruggen (Roche, BE)
- **Patient Advocates**: Francesco De Lorenzo (ECPC, IT)
- **Regulatory Authorities**: Ralf Herold (EMA, NL), Bellinda King-Kallimanis (FDA, US)
- **HTA bodies:** Margarida Oliveira (INFARMED, PT), Giovanni Tafuri (EUnetHTA, NL)

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 HTAs.

WORKSHOP VENUE & & HQ HOTEL

Park Hotel Amsterdam
Stadhouderskade 25
1071 ZD Amsterdam
The Netherlands

DRAFT PROGRAMME

DAY 1 - Tuesday 18 June 2019

Meeting Chairs:	Axel Glasmacher (CDDF, DE), Ralf Herold (European Medicines Agency, NL) & Paul Kluetz (FDA, USA)
Panelists:	Sigrid Klar (Swedish Medical Products Agency, SE) Bellinda King-Kallimanis (FDA, USA) Francesco de Lorenzo (ECPC) or Katie Apostolidis (ECPC) → to be confirmed Claudia Hey (Merck Healthcare KGaA, DE) or Elisabeth Piault-Louis (Genentech) Margarida Oliveira (INFARMED, PT) Academic: to be determined

13:00 **Introduction and Evolving Landscape**
Axel Glasmacher (CDDF, DE), Peter Mol (Medicines Evaluation Board, NL) & Paul Kluetz (FDA, USA)

Session 1: Involving Patients in Oncology Drug Development: Overall Views and Expectations

Session Chair: Axel Glasmacher (CDDF, DE)

13:15 **Regulator (EU):** Sigrid Klar (MPA, Sweden)
13:25 **Regulator (US):** Paul Kluetz (FDA, USA)
13:35 **Patient advocate (EU):** Francesco de Lorenzo (ECPC, IT)
13:45 **Industry:** Claudia Hey (Merck Healthcare KGaA, DE) or Elisabeth Piault-Louis (Genentech)
13:55 **HTA:** Margarida Oliveira (INFARMED, PT)
14:05 **Academic:** To be determined

Session 2: Involving Patients in Oncology Drug Development: When and How?

Session Chair: Claudia Hey (Merck Healthcare KGaA, DE)

14:15 **FDA Secondary Analyses of Submitted PRO Assessment Strategies and Data**
Bellinda King-Kallimanis (FDA, USA)
14:30 **CASE STUDY: Pancreas Carcinoma**
Natalija Frank (Medical University of Vienna, AT)
14:45 **CASE STUDY: Patients Feedback into Oncology Drug Development through Patient Advisory Board Meetings**
Tanka Keiper (Merck Healthcare KGaA, DE)
15:00 **CASE STUDY: Title to be submitted**
Elisabeth Piault-Louis (Genentech, FR)

15:15 Panel & & Participants Discussion
15:30 **Coffee Break**

Session 3: Involving Patients in Oncology Drug Development: Impact on Decision-making

Session Chair: Ralf Herold (EMA, NL)

16:00 **How FDA is Using Patient Experience Data in the Determination of Risk and Benefit**
Bellinda King-Kallimanis (FDA, USA)
16:15 **Patient Involvement in Benefit/Risk Discussions at EMA**
Sigrid Klar (Swedish Medical Products Agency, SE) & Nathalie Bere (EMA, NL)

16:30 **Patient Involvement in HTA in Portugal – a Part of the INCLUIR Project**
Margarida Oliveira (INFARMED, PT)

16:45 **Panel & Participants Discussion**

Session 4: Fostering Collaboration between Patient Community, Sponsor and Decision-makers

Session Chair: Axel Glasmacher (CDDF, DE)

17:00 **From Experience through Collaboration to Partnership - Patients' Scientific Institute as a Novel Programme for Cancer Medicines Development**
Rafal Swierzewski (European Cancer Patient Coalition, PL)

17:15 **Presentation on patient perspectives: title to be confirmed**
Rosanne Janssens (EMA, NL) & Patient advocate (Myeloma Patients Europe)

17:30 **Panel & Participants Discussion**

Session 5: Patient Preference Studies

17:45 **Title to be submitted (30')**
Vikas Soekhai (Erasmus University Rotterdam, NL) & Samare Huls (Erasmus University Rotterdam, NL)

18:15 **Introduction into Break-out Sessions on Day 2**

18:30 END OF DAY 1

19:30 **Networking Event** (New York Room, Park Hotel Amsterdam)

DAY 2 - Wednesday 19 June 2019

Co-chairs: Axel Glasmacher(CDDF, DE) & Ralf Herold (European Medicines Agency, NL)

Session 6: Break-out Sessions - Roadblocks and Solutions

09:00 **BO1: Patient Involvement in Research and Development**
Chairs: Rafal Swiezewski (ECPC, PL), Natalija Frank (Medical University of Vienna, AT) & Claudia Hey (Merck Healthcare KGaA, DE)
Discussion on the scope and timing of patients' involvement including experiences and challenges in e.g. involvement in protocol and ICF development, safety review, beyond individual trials, advisory boards

BO2: Patient Involvement in Assessment and Use of Medicinal Products
Chairs: Sigrid Klar (MPA, SE), Belinda King-Kallimanis (FDA, USA), Giovanni Tafuri (EUnetHTA, NL), Marloes van Bruggen (Roche, BE)
Discussion on what type of data is important, why, and for whom when it comes to patient involvement in regulatory/HTA decision making and case example presentation e.g. authorisation, reimbursement, treatment guidelines, treatment optimisation and post-authorisation studies.

10:00 **Coffee Break**

10:30 **Feedback Presentation from the Break-out Session 1**
Speaker to be determined during the breakout session

10:45 **Feedback Presentation from the Break-out Sessions 2**
Speaker to be determined during the breakout session

Session 7: Next Steps

Session Chair : Axel Glasmacher (CDDF) & Ralf Herold (EMA)

11:00 **Panel & Participants Discussion**

Panel participants and meeting chairs will summarize the main pain points and potential solutions (aspirations and recommendations i.e. what are the panelists going to do different in future).

12:00 **End of the Workshop & Lunch at Park Hotel Amsterdam**