CDDF
Cancer Drug Development Forum
FACILITATE. DEBATE. ACTIVATE. INNOVATE.

MULTI-STAKEHOLDER WORKSHOP

Innovative oncology trial designs

18 - 19 September 2023
Amsterdam, Netherlands
Hybrid Workshop

www.cddf.org

WELCOME NOTE

On behalf of the Cancer Drug Development Forum, its partners, and my colleagues from the organizing committee, Stefan Symeonides, Rosa Giuliani, Rachel Giles, Lada Leyens, Jeanett Borregaard, we are very pleased to welcome you to the Multi-stakeholder workshop on innovative oncology trial designs.

The workshop is planned as a hybrid meeting and will give the opportunity to participants to attend both onsite and on-line. We have actively invested in video technology and refined this through previous meetings to ensure a high-quality experience for on-line participants; however we encourage our participants to join us onsite for great networking opportunities and the unique spontaneous discussions that they bring.

The program has been built to facilitate participation from many parts of the globe, although the focus is on Europe and North America. The workshop includes attendees from all relevant stakeholders (academics, regulators, patient advocates, pharmaceutical industry) in a collaborative platform, to discuss innovation in trial design and performance to help make drug development more efficient, more adaptive, more inclusive, and ultimately more informative.

The workshop will focus on a range of topics, from novel (and surrogate) endpoints, patient-reported outcomes and harnessing real-world data, to novel statistical designs and methods. Specific attention will be given to the practicalities and regulatory environment around these approaches, and to the increasingly common scenarios of rare cancer (sub)populations, combination therapies, and utilization of master protocols.

We are excited about the excellent speakers who have accepted our invitation, and incredibly thankful for the support of all the stakeholders in building the program. Many clinical researchers, pharmaceutical industry representatives, patient advocacy representatives, and regulators, will join us in undoubtedly lively discussions.

We are looking forward to the discussions with you, they are at the core of our mission to facilitate oncology drug development.

Stefan Symeonides and Rosa Giuliani
Programme co-chairs

EVENT OUTLINE

We are in the midst of a rapid evolution in clinical trial design, aiming to develop treatments that best meet the needs of all patients. Trials that are more efficient, more adaptive, more inclusive, and ultimately more informative. This has led to a need for a multi-stakeholder debate to discuss the wide range of key topics that underpin future trial design and performance. This 1.5-day hybrid workshop is a neutral and collaborative platform for the evaluation and discussion of innovation in these areas. It will include attendees from all relevant stakeholders (academics, regulators, patient advocates, pharmaceutical industry).

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Informative lectures and panel discussions will be open to all attendees, to formulate how novel techniques can be best implemented in drug development-related trials, based on insights from all relevant stakeholders. The workshop outcome will be published by CDDF and is expected to provide a valuable resource for the clinical trial industry.

LEARNING OBJECTIVES

- How to use novel endpoints and include them in novel trial designs.
- How to use integrate real-world data or more pragmatic trial designs.
- How to best describe patient-reported evidence generation.
- To familiarize with the new statistical methods and designs now in use.
- How to address the practicalities of running trials in small (sub)populations.

PROGRAMME COMMITTEE

- Committee chair: Stefan Symeonides (CDDF, UK)
- Committee chair: Rosa Giuliani (Guy's and St Thomas' NHS Foundation, UK)
- Rachel Giles (International Kidney Cancer Coalition, NL)
- Lada Leyens (Roche, CH)
- Jeanett Borregaard (Genmab, DK)

TARGET AUDIENCE

The target is a multidisciplinary audience of academia representatives, EU and US regulatory bodies (EMA, FDA, National Agencies), pharmaceutical Industry, HTAs and patient advocate.

WORKSHOP VENUE

Van der Valk Amsterdam Amstel
Joan Muyskenweg 20, 1096 CJ Amsterdam, Netherlands

HYBRID WORKSHOP

The workshop will be held in Amsterdam. However, participation online via the Brella event platform will also be possible. Only approved participants will receive the link and log-in details to access the virtual platform.

CONTACT

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PROGRAMME

Day 1 : Monday, 18 September 2023

SESSION 1: ENDPOINTS FOR INNOVATIVE CLINICAL TRIALS

Session Chairs: Rachel Giles (International Kidney Cancer Coalition, NL); Jeanett Borregaard (Genmab, DK)

12:00 - 12:50	Lunch
12:50 - 13:00	Welcome note Stefan Symeonides (CDDF, UK)
13:00 - 13:15	Endpoints for clinical trials in oncology Elisabeth de Vries (UMC Groningen, NL)
13:15 - 13:25	Regulatory perspective (EU) Aarón Sosa Mejia (DKMA, DK)
13:25 - 13:35	Regulatory perspective (US) Nicole Gormley (FDA, US)
13:35 - 13:45	HTA perspective Anja Schiel (Norwegian Medicines Agency, NO)
13:45 - 14:25	Panel Discussion
14:25 - 14:40	Coffee Break

SESSION 2: INNOVATIVE TRIAL DESIGNS AND ESTIMANDS

Session Chairs: Lada Leyens (Roche, CH); Peter van de Ven (Julius Center, Utrecht UMC, NL)

14:40 - 14:45	Session opening and polls
14:45 - 15:00	Innovation in early phase trials Christina Yap (Institute of Cancer Research, UK)

15:00 - 15:15	Innovation in late-phase trials Jan Bogaerts (EORTC, BE)
15:15 - 15:30	Estimands framework (regulatory perspective) Theodor Framke (EMA, NL)
15:30 - 15:45	Estimands ICH E9(R1) addendum - an industry perspective Kaspar Rufibach (Roche, CH)
15:45 - 16:25	Panel Discussion
16:25 - 16:45	Coffee Break

SESSION 3: TOWARDS PATIENT-CENTRIC EVIDENCE GENERATION

Session Chairs: Rosa Giuliani (Guy's and St Thomas' NHS Foundation, UK); Ana Amariutei (WECAN, DE)

16:45 - 16:50	Session opening
16:50 - 17:05	A patient perspective on evidence generation Ana Amariutei (European Patient Advocacy Institute , DE)
17:05 - 17:20	Synthetic and external control arm Laurence Collette (GSK, BE)
17:20 - 17:35	Project Pragmatica Donna Rivera (FDA, US)
17:35 - 18:15	Panel Discussion **Guest panelists:** • Nafsika Kronidou-Horst (Roche, CH) • John Smyth (University of Edinburgh, UK)
18:30 - 19:30	CDDF Leadership and Industry Panel Meeting (internal meeting)
19:30 - 22:00	Onsite dinner

Day 2 : Tuesday, 19 September 2023

SESSION 4: PRACTICALITIES ON INNOVATIVE CLINICAL TRIALS

Session Chairs: Jeanette Doorduijn (Erasmus Medical Center, NL); Stefan Symeonides (CDDF, UK)

08:30 - 10:00	CDDF Board of Directors meeting (internal meeting)
10:30 - 10:35	Session opening
10:35 - 10:50	Combinations Alessandro Crotta (BMS, CH)
10:50 - 11:05	Studies on rare populations Paolo Casali (EURACAN, IT)
11:05 - 11:20	Master protocol practicalities (Industry perspective) Brian Simmons (Roche, CH)
11:20 - 11:35	ICH guidance Fergus Sweeney (Retired Clinical Trial Expert, IE)
11:35 - 11:55	Panel Discussion

SESSION 5: WORKSHOP WRAP-UP AND NEXT STEPS

Session Chairs: Stefan Symeonides (CDDF, UK); Rosa Giuliani (Guy's and St Thomas' NHS Foundation, UK)

11:55 - 12:40	Wrap up panel discussion **Panelists:** • Peter van de Ven (Julius Center, Utrecht UMC, NL) • Laurence Collette (GSK, BE) • Paolo Casali (EURACAN, IT) • Aaron Sosa Mejia (Danish Medicines Agency, DK)
12:40 - 12:50	Farewell message
12:50 - 13:20	Takeaway lunch