



*The future of cancer therapy*

# MULTI-STAKEHOLDER WORKSHOP

Innovation and Access in  
Rare Cancers

**23-24 September 2024**  
**Amsterdam, Netherlands**  
**Hybrid Workshop**



[www.cddf.org](http://www.cddf.org)



[www.eortc.org](http://www.eortc.org)

## EVENT OUTLINE

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This workshop will evaluate the current state of play in terms of trials and regulatory perspectives to improve access to innovative therapies in patients with rare cancers. The workshop is being jointly organised by CDDF and EORTC, bringing together their respective expertise in the treatment, clinical trials and regulatory perspectives, and will include representatives from industry and academia as well as patient groups to explore these issues.

At this multi-stakeholder workshop, meeting delegates will have the opportunity to discuss the regulatory landscape for orphan drug indications, innovative trial designs and international programmes to help identify patients for trials and share data to build cohorts. The 1.5-day meeting consists of 3 plenary sessions and adequate discussion and networking time. Each session will also include various perspectives of academics, HTAs, regulatory agencies, patient advocates and industry and discuss challenges of improving treatment for patients with rare cancers.

## LEARNING OBJECTIVES

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From this interactive workshop, participants will achieve the following outcomes:

- To understand the current landscape of patient identification and profiling in rare cancers
- To explore regulatory aspects, challenges and plans for formal registration of novel agents in rare indications
- To learn about the innovative trial designs available and international efforts to improve data collection and outcomes for these patients

## PROGRAMME COMMITTEE

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- Committee chairs: Ruth Plummer (CDDF); Winette Van der Graaf (EORTC)
- Nina Heiss (Merck Healthcare KGaA, DE)
- Carolyn Hynes (AZ, UK)
- Simone Keller (BMS, CH)
- Ariane Weinman (EURORDIS, FR)

## TARGET AUDIENCE

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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

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Novotel Amsterdam Schiphol Airport  
Taurusavenue 12, 2132 LS Hoofddorp, Netherlands

## HYBRID WORKSHOP

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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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Cancer Drug Development Forum (CDDF)  
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# PROGRAMME

Day 1 : Monday, 23 September 2024

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## SESSION 1: CHALLENGES, COLLABORATION AND NEEDS

Session Chairs: Ruth Plummer (CDDF, UK); Carolyn Hynes (AstraZeneca, UK)

12:00 - 12:50	Lunch
12:50 - 13:00	Welcome note Programme chair
13:00 - 13:25	Keynote lecture Francesco Pignatti (EMA, NL)
13:25 - 13:45	Navigating the evolving regulatory landscape of orphan drugs in Europe Pedro Franco (Merck Healthcare KGaA, UK)
13:45 - 14:00	The EU Clinical Trial Information System public portal Francesca Scotti (EMA, NL)
14:00 - 14:20	Rare cancers: challenges for new treatment developments in the precision medicine era Jean-Yves Blay (Centre Léon Bérard; UNICANCER, FR)
14:20 - 15:05	Panel Discussion
15:05 - 15:35	Coffee Break

## SESSION 2: INNOVATIVE TRIAL DESIGNS

Session Chairs: Winette Van der Graaf (EORTC, NL); Simone Keller (BMS, CH)

15:35 - 15:40	Session opening
15:40 - 16:00	EORTC TRACE platform Denis Lacombe (EORTC, BE)
16:00 - 16:20	Precision oncology: a revolution for rare cancers David Thomas (OMICO, AU)
16:20 - 16:40	Drug development in adolescent and young adults with cancer Nathalie Gaspar (Gustave Roussy , FR)
16:40 - 17:00	EU HTA - HTA perspective Beate Wieseler (Institute for Quality and Efficiency in Health Care (IQWiG), DE)
17:00 - 17:45	Panel Discussion  <b>Guest panelists:</b> <ul style="list-style-type: none"> <li>Salem Abi Nehme (BMS, CH)</li> <li>Roger Wilson (Sarcoma Patients Advocacy Global Network, UK)</li> </ul>
18:00 - 19:00	CDDF Leadership and Industry Members meeting (internal meeting)
19:00 - 22:00	Onsite dinner (B House - ground floor)  Dinner speech by <b>Deborah Binner</b> (author of "Yet Here I am: how to survive life after death") - starting time: 19:15



## Day 2 : Tuesday, 24 September 2024

### SESSION 3: INNOVATIVE SOLUTIONS TO IMPROVE ACCESS

Session Chairs: Nina Heiss (Merck Healthcare KGaA, DE);  
Gauthier Bouche (Anticancer Fund, BE)

08:30 - 10:00	CDDF Board of Directors meeting (internal meeting)
10:15 - 10:20	Session opening
10:20 - 10:30	Testimony on the path for access (patient perspective) Max Williamson (Queen Elizabeth University Hospital Glasgow, UK)
10:30 - 10:40	Testimony on the path for access (medical professional perspective) Winette Van der Graaf (EORTC, NL)
10:40 - 11:00	Regulatory perspective (FDA) Timil Patel (FDA, US)
11:00 - 11:20	Opportunities and Challenges of Managing Early Access in Rare Cancers: An Industry Perspective Philipp Schlatter (Roche, CH)
11:20 - 11:40	Patient access & data collection/ownership Rachel Giles (VHL Europa, NL)
11:40 - 12:00	DRUP - across countries studies Sahar Van Waalwijk van Doorn-Khosrovani (CZ Health Insurance, NL)
12:00 - 12:40	Panel Discussion  <b>Guest Panelist:</b> <ul style="list-style-type: none"> <li>Paul Lacante (BMS, NL)</li> </ul>

## SESSION 4: WRAP UP AND NEXT STEPS

Session Chairs: Ruth Plummer (CDDF, UK), Winette Van der Graaf (EORTC, NL)

12:40 - 13:05

Wrap up panel discussion

**Panelists:**

- Ruth Plummer (CDDF)
- Winette Van der Graaf (EORTC)
- Nina Heiss (Merck Healthcare KGaA, DE)
- Carolyn Hynes (AZ, UK)
- Simone Keller (BMS, CH)
- Ariane Weinman (EURORDIS, FR)

13:05 - 13:10

Farewell message

13:10 - 13:40

Takeaway lunch