CDDF
Cancer Drug Development Forum

EORTC
European Organisation for Research and Treatment of Cancer

The future of cancer therapy

CDDF-EORTC Joint Workshop

INNOVATION AND ACCESS IN RARE CANCERS

EXECUTIVE SUMMARY

23-24 SEPTEMBER 2024
HYBRID, AMSTERDAM (NL)

CDDF-EORTC Joint Workshop

Innovation and Access in Rare Cancers

23-24 September 2024 , Amsterdam (NL)

Cancer Drug Development Forum (CDDF) Multi-Stakeholder Workshops are neutral, non-competitive meetings that address topical issues and recent innovations in oncology drug development with the aim of improving cancer treatment. The workshops facilitate multi-stakeholder discussion and collaboration, bringing together leading voices from academia, the healthcare industry, regulatory authorities, and patient advocacy groups.

The CDDF-EORTC joint workshop on "**Innovation and Access in Rare Cancers**" took place on 23-24 September in Amsterdam (NL). This collaborative effort between CDDF and EORTC focused on the current state of play in clinical trials and exchanged multi-stakeholder perspectives in order to improve access to innovative therapies for patients with rare cancers. A multi-stakeholder group of experts provided insights on important topics in rare cancers such as the exisiting challenges, ongoing collaborations, unmet needs, innovative clinical trial designs and potential solutions to improve patient access.

This interactive meeting generated fruitful dialogue and the following take-home messages that emphasize collaborative efforts among all stakeholders:

SESSION 1: CHALLENGES, COLLABORATION AND NEEDS

KEY TAKEAWAYS

- Regulators are open to considering **the totality of data**, **including Real-World Data (RWD)**, when making approval decisions; especially when investigational medicinal products for rare cancers show high activity.

- **Having incentives** for drug development for rare diseases is very important; as is collaboration with patient advocacy groups.

- Data shows that **treatment in Reference Centres is key** to improving patient outcomes, especially for rare cancers. Collaboration to share data around the EU to build data cohorts is also vital.

NEXT STEPS

- To overcome the challenges described in this session, **improved collaboration and willingness** for this to happen between industry, regulators, academia and patients will be key.

SESSION 2: INNOVATIVE TRIAL DESIGNS

KEY TAKEAWAYS

- **Drug development in children and adolescent and young adult** (AYA) patients (15-24 years) is much delayed versus adults. The ACCELERATE FAIR trial is a multistakeholder solution to enable early drug trials in children and AYAs. More collaboration between the pediatric and adult oncology is necessary.

- There are **different aims for histology agnostic trials**: learn, conclude, submit for regulators or implement in healthcare systems; the trial design should be related to the specific aims.

- There are **many challenges for comparative effectiveness evaluation** in rare cancers. Single arm studies form such challenges and maybe used too often. **Randomizing**, even in phase 2, generates more meaningful data, particularly when **prospective Real World Data** are collected simultaneously.

- While Overall Survival benefit is still what regulators/HTA are looking for, there may be **other valuable endpoints for patients**.

SESSION 3: INNOVATIVE SOLUTIONS TO IMPROVE ACCESS

KEY TAKEAWAYS

- A challenge for rare cancer patients is that they often do not match the **clinical trial eligibility criteria** which prevents trial access. For young cancer patients in particular, age limits participation on clinical trials. Thus, regulatory flexibility would be welcomed. Compassionate use can provide access to a medicine outside of a clinical trial in certain cases.

- There is a need to **modernize clinical trials** and **evidence generation**. Examples of progress discussed were DCTs and DRUPs.

- A common theme to facilitate access is the need to **collect data more systematically** and **maximize use of real-world data**. Integrating the patient voice early on and the compliant sharing and protection of data through leveraging patient-owned data is key.

- For patients and families, there is a **need to raise more awareness** on clinical trials (e.g. social media campaigns). More transparency is needed on offering diagnostics, and on having a clear pathway for the evaluation and reimbursement of diagnostics and off-label use of a drug.

SESSION 3: INNOVATIVE SOLUTIONS TO IMPROVE ACCESS

NEXT STEPS

- As science and technology advances, **advanced collaboration with all stakeholders** is more crucial than ever (national, international, HTA bodies, patients, pharma, payors, funders, multi-institutional collaboration).

- **Harmonization at EU level of regulatory pathways** that allow patients to gain access (compassionate use, 'good' off-label use).

- As food for thought: Going forward in the era of big data, **AI solutions** may help address some of the challenges of designing, running clinical trials with small patient populations and facilitating patient access.

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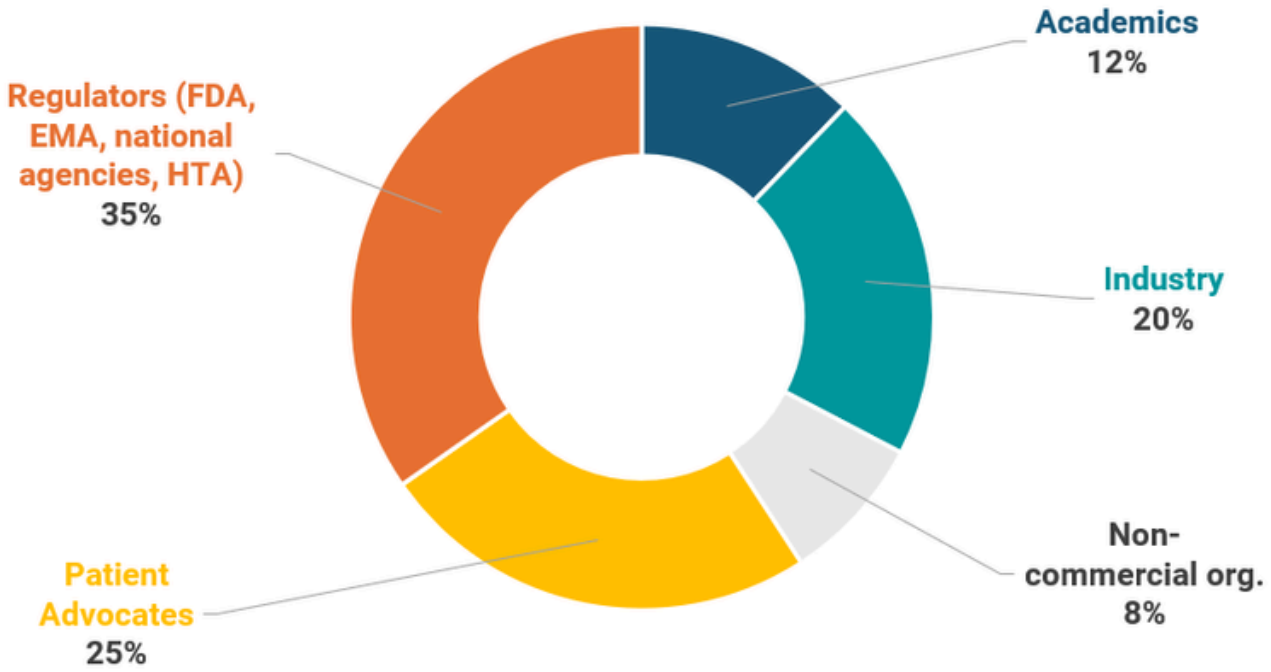
Collaboration and **open dialogue** among **all stakeholders** is **key** to accelerating and improving oncology drug development for patients

AUDIENCE AT THE CDDF WORKSHOP

The CDDF's meetings present **a wide range of prespectives** from **various stakeholders** who are involved in the development of oncology drugs. Our **multi-stakeholder, collaborative approach** facilitates a productive dialogue in a neutral, non-competitive space in order to **accelerate effective cancer drug development.**

Onsite Participants & Speakers



The graph illustrates the distribution of online and onsite speakers/chairpersons/panelists alongside onsite attendees.

57
IN-PERSON ATTENDEES

54
ONLINE ATTENDEES

WHAT PARTICIPANTS SAY ABOUT CDDF'S DISCUSSION?

"This was one of the best and most open debates about rare cancer clinical trials I have been involved with. It was down to earth, truly expert speakers from all stakeholder groups giving real, relevant and clear opinions based on strong experience and expressing important visions."

Roger Wilson
Sarcoma Patients Advocacy Global Network, UK

"Participating to these kinds of workshops is extremely valuable for us as regulators, since we have the possibility to interact and listen to experiences and questions of such a wide variety of stakeholders. We not only receive important feedback on the work done, but also receive valuable inputs and suggestions on what we can do better."

Francesca Scotti
European Medicines Agency (EMA), NL

"For participants from big companies, We often focus on our part of drug development process. We can lose the entirety of the perspective of the drugs we are working on, access and reaching patients. I think CDDF workshops really bring in all of the key stakeholders. So the equality of the voices we are hearing here makes CDDF workshops unique. "

Carolyn Hynes
AstraZeneca, UK

"The fantastic thing about this meeting is brining focus to patients with rare cancers. There are populations, which have been invisible traditionally. It is time they have started to become the focus of policy development, research and ultimately implementing a new model of healthcare."

David Thomas
OMICO, AU

"The small size of the meeting gave me the opportunity to really be able to make network and connection with people who are here to have discussion. Also, I like how CDDF is introducing challenges, also presenting some possible solutions."

Sabrina Hanna
Cancer Collaborative, CA

The views expressed in this page are the personal views of the participants and may not be understood as being made on behalf of or reflecting the position of the regulatory agency/agencies or organisations with which the participants are employed/affiliated.

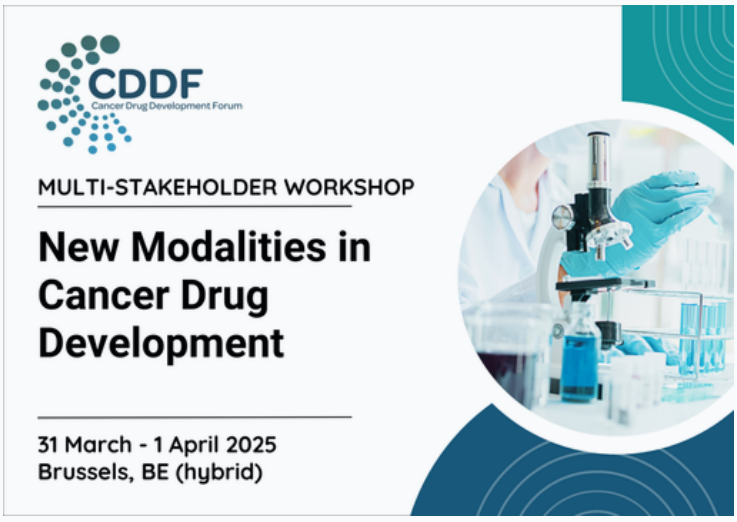
CDDF'S UPCOMING MEETINGS & DISCUSSION

MULTI-STAKEHOLDER MEETINGS

Session Topics

1. CDDF-AAADV joint session on diversity in global drug development
2. Endpoints in Drug Development: Progress and Controversies
3. Accelerate with clinical trial innovation
4. Controversial topics in diagnostics
5. EU HTA Regulations

Programme | Registration



Programme | Registration

Programme | Registration

CDDF'S UPCOMING MEETINGS & DISCUSSION

CDDF LIVE WEBINARS

Webinar Outline | Registration

Webinar Outline | Registration

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ACKNOWLEDGEMENTS

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Cancer Drug Development Forum (CDDF)

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