

A background illustration showing several hands of different skin tones reaching towards the center. One hand is wearing a blue surgical glove, and another is wearing a teal surgical glove. In the center, a silver lightbulb is being held, symbolizing collaboration and innovation in drug development.

# MEETING REPORT

## CDDF Multi-Stakeholder Workshop

### Patient Access and Engagement in oncology drug development

19 - 20 September 2022 Hybrid Workshop

Prepared by Janet Fricker and CDDF Board Member Prof Mark Lawler

#### **PROGRAMME COMMITTEE**

Chair: Committee chair: Mark Lawler (Queen's University Belfast, (UK)  
Mirjam Crul (Amsterdam UMC, NL)  
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## Meeting outline

This event was organised by the Cancer Drug Development Forum (CDDF) to address the increasingly important topic of Patient Access and Involvement in Oncology Drug Development. Patient and Public Involvement and Engagement (PPIE) is becoming a critically important component of all aspects of cancer research and its translation into better care, but there is currently a dearth of opportunities for the cancer community (healthcare professionals, researchers, patients, industry, regulators, payers, policy makers and other relevant stakeholders) to learn about the latest developments and examples of best practice and to discuss and debate how patient access and involvement in oncology drug development can be enhanced to ensure a truly patient-centred approach to innovative medicines.

This multi-stakeholder event brought together experts in the field in a series of 5 sessions over two half days, delivered through keynote lectures, round tables and discussion fora. The event examined ways in which patients can be empowered to be active participants in cancer research; how we ensure that the patient voice is amplified both in the delivery of clinical oncology research and in regulatory decision making; the challenging areas of reimbursement and access to innovative oncology medicines for patients; the absolute primacy of deploying data intelligence to underpin patient-focussed oncology drug discovery and development and the highly relevant, but sometimes poorly understood, and challenging area of cross-border access for clinical trials in oncology.

## Learning objectives

- To appreciate and understand how patients and the patient voice are best integrated into cancer research, with particular emphasis on cancer drug development and its delivery for the benefit of patients
- To determine how patients can best contribute to regulatory decision making
- To understand the complexities of patient access to innovative medicines and reimbursement of innovative medicines and what constitutes best practice
- To be informed on the key role that data intelligence plays in the delivery of patient-focussed oncology medicines for the benefit of patients
- To appreciate the need and the means by which cross-border access to oncology clinical trials can enhance patient access to the latest innovative medicines

## Programme

### DAY 1 - MONDAY 19 SEPTEMBER 2022

#### **SESSION 1: EMPOWERING PATIENT INVOLVEMENT IN CANCER RESEARCH**

Session chair: Hans Scheurer (Melanoma Patient Europe, NL)

#### **Welcome note**

Francesco De Lorenzo (CDDF; European Cancer Patient Coalition, IT)

#### **Patient empowerment and co-creation in cancer research - the only way to go**

Mark Lawler (Queen's University Belfast, UK)

#### **Putting patients first - the FDA perspective**

Vishal Bhatnagar (FDA, US)

## Panel Discussion

### **SESSION 2: EMBEDDING THE PATIENT VOICE IN CLINICAL ONCOLOGY RESEARCH AND REGULATORY DECISION MAKING**

Session Chairs: Birgit Wolf (Bayer, DE); Jan Geissler (Patvocates, DE)

#### **The Added Value of Patient Engagement in Early Dialogue at EMA: Scientific Advice as a Case Study**

Maria Mavris (European Medicines Agency, NL)

#### **Evidence Based Advocacy and the impact of patient evidence on decision-making processes**

Ananda Plate (Patvocates, DE)

#### **Making scientific research understandable and accessible to the (cancer) patient and caregiver community**

Pooja Merchant (Bayer, US)

## Panel Discussion

### **SESSION 3: REIMBURSEMENT AND EARLY ACCESS TO INNOVATIVE ONCOLOGY MEDICINES FOR PATIENTS**

Session Chairs: Susan Bhatti (Merck Healthcare KGaA, NL); Mirjam Crul (Amsterdam University Medical Center, NL)

#### **Early access for patients through conditional reimbursement schemes**

Haiko Bloemendal (Radboud University Medical Center, NL)

#### **Patient Perspective on early access**

Stefan Gijssels (Patient Expert Center, BE)

## Panel Discussion

## DAY 2 - TUESDAY 20 SEPTEMBER 2022

### **SESSION 4: TURNING DATA INTO INTELLIGENCE FOR PATIENT FOCUSED ONCOLOGY DRUG DEVELOPMENT**

Session Chairs: Mark Lawler (Queen's University Belfast, UK); Kathi Apostolidis (ECPC, BE)

#### **Driving a data informed patient relevant agenda – lessons from DATA-CAN, the UK's Health Data Research Hub for Cancer**

Mark Lawler (Queen's University Belfast, UK)

#### **Data privacy by design is good for Patients and Drug development**

Gilliosa Spurrier-Bernard (MelanomeFrance; Melanoma Patient Network Europe, FR)

#### **Industry perspective**

Bartek Madej (Novartis, CH)

## Panel Discussion

### **SESSION 5: CROSS BORDER ACCESS FOR ONCOLOGY CLINICAL TRIALS**

Session chairs: Susan Bhatti (Merck Healthcare KGaA, NL); Bettina Ryll (Melanoma Patient Network Europe, SE)

#### **Accessing clinical trials abroad. The experience of the Melanoma Patient Network Europe**

Bettina Ryll (Melanoma Patient Network Europe, SE)

**Cross-Border Access to Clinical Trials in the EU: results from an exploratory study**

Teodora Lalova-Spinks (KU Leuven, BE)

**Can we improve cross-border access to clinical trials?**

Ingrid Klingmann (Chairman at Board of the European Forum for Good Clinical Practice, BE)

**Panel discussion**

## SESSION 1: Empowering Patient Involvement In Cancer Research

### Introduction

Increasingly, we are realising the importance of cancer research and its pivotal role in enhancing cancer care. There is now irrefutable evidence that those patients who are treated in research-active hospitals have better outcomes than those who are not<sup>1</sup>. Coupled with this greater focus on research and its translation, is the realisation that the role of the patient is also changing, both in terms of their care and in the delivery of more patient-centred cancer research.

Initiatives such as the European Cancer Patient's [Bill of Rights](#) and the [European Code of Cancer Practice](#) have shifted the dial; patients are transitioning from being passive recipients to becoming active participants in their care. Patients are also increasingly becoming more involved in cancer research. This is an extremely welcome development as it ensures that the challenges that patients face in the real world are given due consideration within an overarching cancer research framework. Increasingly, patients not only are empowered but feel empowered to contribute meaningfully to the co-creation and implementation of a cancer research agenda that values their unique contribution. These themes will be explored in more detail and as number of examples will be presented to emphasise the enhanced role of the patient in cancer research and its translation.

### Welcome note

**Francesco De Lorenzo (CDDF; European Cancer Patient Coalition, IT)**

Francesco De Lorenzo, President of the European Cancer Patient Coalition (ECPC), the cancer patient association providing the unified voice of cancer patients across Europe, described the role ECPC is playing in encouraging the involvement of patient advocates in the design of clinical trials.

- ECPC provides the missing link between different stakeholders, advocating for partnership models between researchers and patients, providing a mechanism for patients to contribute their unique experiences in both a cancer care and cancer research in an innovation setting.
- The Council of Ministers recently approved adoption of the '[Principles of Successful Patient Involvement in Cancer Research](#)' document, created in September 2021 by many contributors, including patient organisations and cancer researchers.
- Involvement of patient advocates in co-design of studies has become a reality, with ECPC involved in 26 EU funded [projects](#).
- ECPC is developing platforms to provide guidance and frameworks for patient advocates involved in cancer research. In partnership with the Organisation of European Cancer Institutes (OECI), ECPC has a memorandum of understanding to develop an educational module, explaining patient roles in Comprehensive Cancer Centre infrastructures.

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<sup>1</sup> [European Groundshot-addressing Europe's cancer research challenges: a Lancet Oncology Commission.](#)

## Patient empowerment and co-creation in cancer research - the only way to go

Mark Lawler (Queen's University Belfast, UK)

Mark Lawler, from Queen's University Belfast, UK, considered patient empowerment and how patients are becoming more involved in the co-creation of cancer research.

- Scale of challenge: every minute three European citizens die from cancer, and ~ 20 million people are living beyond a cancer diagnosis, with issues resulting from surviving cancer.
- The [European Cancer Patient's Bill of Rights](#), launched in 2014, challenges the inequalities faced by European cancer patients on a daily basis. Building on the European Cancer Patient's Bill of Rights, the aim of the [70:35 vision](#) is to achieve an average of 70% long-term survival for people in Europe affected by cancer by 2035, underpinned by progress on cancer control, better survivor experiences and improved quality of life. These initiatives have resonated across Europe, providing a catalyst for change with empowerment tools for people with cancer.
- The [European Code of Cancer Practice](#), launched in September 2020, is a citizen and patient-centred manifesto signposting what Europeans with cancer should expect from health care systems, empowering them to be active participants rather than passive recipients in their care. Co-produced by patient advocates and cancer health professionals under the guidance of the [European Cancer Organisation](#), the code (translated into 31 European languages) defines 10 key overarching rights, linked to three questions patients can ask their doctor/health professionals.
- Addressing survivorship, the [European Academy of Cancer Sciences](#) (EACS) proposes [embedding](#) a European Cancer Survivorship Research and Innovation Plan within the EU Cancer Mission (including three distinct pillars: medical, socioeconomic and politico-legal).
- [DATA-CAN](#), the UK's Health Data Research Hub for Cancer, included patients in stakeholder groups, harnessing the power of data for citizen and patient benefit. The aim is to enable genuine research co-creation involving patients [at all levels of decision-making](#).

## Putting patients first - the FDA perspective

Vishal Bhatnagar (FDA, US)

Vishal Bhatnagar, Associate Director for Patient Outcomes in the Oncology Center of Excellence (OCE) at the FDA, reviewed ways in which the FDA are engaging with patients.

- Public panel discussions. 'Oncology Conversations on Cancer' hosted wide ranging sessions including topics on clinical trials, health access for underserved communities, and effects of toxicity on patient experience.
- Funding external collaborators to perform research on improving collection, analysis, interpretation and reporting of data in oncology trials e.g., a qualitative interview study determining how people with cancer interpret and respond to physical functioning questions.
- Hosting conferences with people with cancer involved in panel discussions. This year OCE hosted its seventh annual ['Clinical outcomes assessment in clinical trials](#)



[workshop](#) to discuss collection, analysis and interpretation of patient reported outcomes (PROs) in open label trials.

- Guidance for Industry. '[Core Patient-Reported Outcomes in Cancer Clinical Trials Guidance for Industry](#)' draft guidance, published June 2021, encourages sponsors to focus on core (Patient Reported Outcomes Measures (PROMs) in early and late phase clinical trials.
- [Project Patient Voice](#), launched in June 2020, is a website intended to provide a complementary source of information on tolerability of medicines for patients, providing a tool for framing patient/ provider discussions.

## Panel Discussion

- Training empowers patient advocates to become equal partners in cancer research. Training budget provision needs to include honorarium and expenses to ensure patients are not 'out of pocket'. In addition to patient advocates bringing lived experiences to discussions, they can also offer professional skills, e.g., backgrounds in data have contributed greatly to the work of DATA-CAN
- Patient advocates should represent all demographics, including underserved communities. Materials need to be accessible to all, patient advocates have a role in ensuring that a number of important issue, such as informed consent are presented in 'patient friendly' understandable language.
- Patient input can increase understanding around treatment tolerability issues, such as additional toxicity generated by combination regimens. Therapies will not be effective if they are intolerable for patients.
- Patients need to be involved from the earliest stages of study design, including grant writing. In DATA-CAN, involvement of patients helped refine research questions and provide ideas for better ways to collect information/data.
- The health research and care community should be open to learn from other professional sectors. Inter and multidisciplinary team working are the essence of 21<sup>st</sup> century cancer research and care
- Greater investment is needed in survivorship research, e.g. looking at ways to improve quality of life.
- It is important to ensure the involvement of representatives of different patient groups rather than just individual patients and avoid tokenism. Lobbying can be more effective if organisations take a 'partnership' approach, bringing different stakeholders together with a uniform voice.
- Inequalities exist in a number of areas including different tumour types; those cancers with a poor prognosis often lack long-term survivors to act as patient advocates. There is a need to ensure those with poor survival and rare diseases are not underserved/underfunded.

## Session 2: Embedding The Patient Voice In Clinical Oncology Research And Regulatory Decision Making

### Introduction

This session looked at patient engagement in cancer drug development throughout a medicine's lifecycle. It looked at the added-value of early patient input for example at regulatory scientific advice procedures, use of patient preferences studies and evidence-based patient advocacy. Including patients in communicating oncology clinical research illustrates the benefit to have patients also as important contributors at this step of the lifecycle.

### The Added Value of Patient Engagement in Early Dialogue at EMA: Scientific Advice as a Case Study

Maria Mavris (European Medicines Agency, NL)

Maria Mavris, who works in the Public and Stakeholder Engagement department at the European Medicines Agency (EMA), presented a survey exploring patient input in clinical trials. She described the 'added-value' that can be achieved by involving patients in early dialogue.

- Since 2008, the EMA has involved patients in providing scientific advice, starting in rare diseases and then in 2013 extending to all diseases.
- In 2022, the EMA published research in '[Frontiers in Medicine](#)', analysing patient input in scientific advice procedures over a four-year period. Altogether, 371 survey responses were received for the 478 patients (78%) involved from 20 different scientific officers who responded to the survey.
- Results showed that 1 in 5 scientific advice requests submitted to EMA included clinical questions in the dossier. Where the questions were appropriate, patients were involved. Reasons for requesting patient involvement included questions around study populations (77%), endpoints (74%), study feasibility of conducting a trial (52%), and quality of life (48%). Patient input resulted in further discussion by the Scientific Advice Working Party in 52% of cases, with patient input on population inclusion/ exclusion criteria (49%), endpoints (48%), quality of life (37%) and feasibility of study (29%).
- Patient input complemented scientific and medical contributions to specific questions bringing real life experiences (71%), offering different perspectives (42%) and raising issues not previously considered (14%). Based on patient contributions, recommendations made to the developers of the studies were modified in 20% of cases where patients were involved.
- A patient survey (n=125) showed that > 80% understood what was expected from them, > 80% felt they were able to provide input on issues discussed, 75% felt their comments were taken into account, and 80% felt positive about the overall experience. The main barriers were complex information (lack of resources to adapt scientific information into lay formats) and short regulatory deadlines.
- Examples of added-value from patient input include a patient survey identifying the most appropriate comparator medicine and multiple myeloma patients saying they would trade toxicity for additional therapeutic options<sup>2</sup>.

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<sup>2</sup> [2016-annual-report-european-medicines-agency\\_en.pdf \(europa.eu\)](#)

- Challenges included finding suitable experts based on availability and language, ensuring comprehensive tailored training to facilitate and enhance participation, managing potential conflicts of interest, achieving representativeness and measuring value/ impact of patients
- EMA patient activities are supported by their [Engagement Framework](#) with a network of European patient and consumer organisations. The EMA have just completed a successful pilot for orphan medicines by including patient organisations earlier in the evaluation of marketing authorisation applications with plans to expand the approach to other medicines. A report on the pilot was published shortly after the workshop: [Pilot outcome report - CHMP early dialogue with patient organisations](#)
- The EMA held a multi-stake holder workshop '[Patient experience data in medicines development and regulatory decision-making](#)' on 21 September 2022.

## Evidence Based Advocacy and the impact of patient evidence on decision-making processes

Ananda Plate (Patvocates, DE)

Ananda Plate, Executive Director at Patvocates, a consultancy specialising in patient advocacy, addressed the need for evidence-based patient advocacy.

- There has been a long tradition of patient involvement in R&D and regulatory processes, starting in 1992 with the FDA through the HIV movement to EUPATI's PFMD's trainings and methodologies. Essential ingredients (shared purpose and collaborative spirit, political and institutional will, engagement frameworks and capacity to engage by patient organisations) are in place to allow patient involvement. However, ingredients are not used systemically, with issues including 'cherry picking' of individuals by decision makers rather than letting patient communities decide, involving patients after decisions have been made, and opacity of expectations regarding evidence generation.
- Evidence-based patient advocacy involves advocating in a targeted evidence-based, well-educated and professional manner, and measuring the impact and outcomes of what is done. Over the past years, [WECAN](#) has been driving the adoption of evidence-based advocacy in the cancer patient community<sup>3</sup>.
- Types of community-generated evidence include disease mapping (involving patient experience, quality of life, burden of disease, unmet patient needs and adherence), patient preferences (benefit/risk, and value of outcomes) and inequality mapping (worst outcomes, lowest access to diagnostics or treatment and vulnerable groups).
- The true impact of patient evidence in decision-making cannot be achieved while key elements (lack of transparency and guidance on the establishment and use of patient evidence and methodologies, and involvement of cherry-picked individuals) are disregarded.
- Potential solutions include producing guidelines on methodology (collection, integration and impact of patient generated evidence in regulatory decision making), clear and communicated timelines and deadlines for R&D and regulatory processes, patient involvement in early advice, generation of patient preference data independent of specific product characteristics, and systematic involvement of patient organisations in choosing individuals for specific interactions.

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<sup>3</sup> [wecanadvocate.eu/patients-in-publications/](https://wecanadvocate.eu/patients-in-publications/)

## Making scientific research understandable and accessible to the (cancer) patient and caregiver community

Pooja Merchant (Bayer, US)

Pooja Merchant, Global Head Patient Partnerships & Engagement, Bayer Oncology, explained how Bayer is involving patient advocates in co-design and co-authorship of studies.

- At Bayer Oncology, patient advisors, but also patients and caregivers, provide input across the lifecycle of studies (concept development, planning, start-up, conduct and close-out).
- In a recent phase III prostate cancer study, Bayer deployed a Patient Engagement Council to gather insights about a new imaging method and whether people would want to participate in the study.
- The Covid-19 pandemic highlighted that poor health literacy is an underestimated public health problem, making health disparities worse.
- A survey 'Optimizing Readability and Format of Plain Language Summaries for Medical Research Articles' of patients and patient advocates, conducted in December 2020, found the highest preference was patient infographics, followed (in this order) by animated videos, plain language summaries of publications and text-only descriptions.
- As a result, Bayer introduced infographics, podcasts, Instagram, PLS text, poster videos and animation videos.
- To take patient involvement further, Bayer invited two patients to co-author a [publication](#) with investigators about a phase III study of darolutamide in nonmetastatic, castration-resistant prostate cancer.
- The publication, translated into eight languages, provides patient perspectives on what the study means for the cancer community. Health professionals have downloaded the study in significant numbers, demonstrating they appreciate its simplified scientific content.
- Challenges for co-authorship include finding patient advocates sufficiently informed, educated and willing to take part, and journals understanding the concept of patient-friendly publications and the importance of open access.
- Involving patients in writing publications reflects 'The nothing about us without us' concept<sup>4</sup>.

### Panel Discussion

- The patient voice is integrated into FDA regulatory decision making, with patient advocates represented on the Oncology Drug Advisory Committee, which explores risk benefit analysis. Patients also inform the Agency 's research protocols, i.e. review study consent and help draft information on research objectives from patient perspective also write study consent information explaining why studies are important
- The [EMA's Committee for Human Medicinal Products \(CHMP\)](#), which provides opinions to the European Commission on the marketing of drugs, is considering whether to permit patient and health professional membership. Methodologies are in place, such as surveys and membership advisory boards, to ensure that the patient voice is captured in committee deliberations.
- Patient advocates took issue with the sentiment that patients were 'just another voice at the table,' stressing they were the ultimate beneficiaries of medicines and if side

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<sup>4</sup> <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC8996208/>

effects were unacceptable to patients, they would not take the drugs that were being investigated. Patients bring to the table an awareness of all aspects that are relevant to patients.

- To enable true partnership, patient representatives need to be able to communicate in a language researchers understand and vice versa.
- The need for 'evidence based' advocacy was recognised, with patient organisations, academia and industry requiring guidance about the type of evidence they should generate for inclusion in regulatory decisions. The FDA representative said it was helpful for research to address specific questions rather than taking too broad a scope. For the first time, Covid-19 had led to the development of large surveys capable of capturing different patient perspectives.
- There was debate around the issue of how 'representative' individual patients would be when involved in committees and panels. Patient advocates felt that involved patients should reflect the heterogeneity of the patient population they represent, and should, wherever possible, base their interventions on evidence from their community. Surveys, patient preference studies and other social science methodologies should be used to capture variance in patient populations (e.g., age, gender and cultural differences). Regulators said they need to make population-based decisions regarding whether drugs should be marketed, making it helpful to have broader representative feedback on patient experiences and preferences as opposed to individual patient feedback. However, they acknowledged opinions and patient preferences and priorities may differ, e.g. in relation to quality of life, toxicity and overall survival. Patient representation was considered challenging in multiregional trials, where US, European and Asian populations were likely to have different perspectives and viewpoints.
- Developing lists of organisations that industry, regulators, and HTAs can approach for patient representation was considered valuable to avoid 'cherry picking' individuals. Potential conflicts of interest among patient representatives should be addressed.
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## Session 3: Reimbursement And Early Access To Innovative Oncology Medicines For Patients

### Introduction

Innovative medicinal products for oncology, including gene therapies and cell therapies, can offer promising treatment options for patients. However, the costs of these innovative treatments are often high, and not always attainable for lower-income regions in Europe. Therefore, inequality in access presents a realistic scenario for these types of treatments. In this session, we investigated novel reimbursement strategies and other mitigating factors that can be taken to ensure all patients eligible for a novel treatment modality will be able to receive it in a timely fashion. The session offered the views of prescribers, patients and payers.

### Early access for patients through conditional reimbursement schemes

**Haiko Bloemendal (Radboud University Medical Center, NL)**

Haiko Bloemendal, a medical oncologist from Radboud University Medical Center, Netherlands, described an innovative initiative in the Netherlands where access for a patient

to a new drug is initially funded for 4 months by the marketing authorisation holder (after receiving EMA approval) and only further funded by the insurance company (for the next 2 years) when the patient shows signs of improvement (either stable disease, partial response, or full response).

- The increasing number of innovative anticancer drugs can lead to increased financial pressure on health systems and the subsequent evaluation by national health technology bodies (after EMA approval) is prolonging the time needed to agree on reimbursement. For instance, in the Netherlands it takes 6 to 12 months for new medications to be reimbursed, while in other European countries (particularly in Eastern Europe) the time is often longer.
- To tackle this problem, there is a pilot project ongoing in the Netherlands to collect real world evidence for new drugs that are approved by EMA. To be eligible for the initiative, the product has to address an unmet medical need and meet defined overall response criteria. Furthermore, drugs need to be under EMA review or have positive Committee for Medicinal Products for Human use (CHMP) opinions or formal EMA registration for the indication
- First, potential drug candidates register by email with the Drug Access Desk and are discussed with the Dutch Health Insurers and Drug Access advisory committee.
- Next, participating hospitals are selected. Patients are treated according to the Summary of Product Characteristics (SmPC) of the pharmaceutical company under a drug access protocol.
- Real world data on anti-tumour activity are then collected under a Drug Access Protocol (DAP) and the first four months of access are funded by the manufacturer. If positive impact on the tumour is found, further treatment funding is then covered by insurance companies for the next 2 years. After two years, the product will be assessed by the Dutch Medical Oncology Society and Health insurance companies, and will be upgraded for full reimbursement if it meets the criteria.
- Since 2021, 138 patients have been included in the initiative. Drugs being considered for inclusion in the programme include cemiplimab, Larotrectinib, selpercatinib, capmatinib, tepotinib, entrectinib and amivantamab.

## Patient Perspective on early access

### Stefan Gijssels (Patient Expert Center, BE)

Stefan Gijssels, cofounder of the Patient Expert Center (PEC) in Belgium, an initiative to educate patients to become patient experts, talked about the patient perspective.

- Prices of drugs changes significantly over time, e.g., in 2008 the Folfox chemotherapeutic regimen in colorectal cancer cost more than 20 times standard of care, but by 2017 it was the least expensive treatment (just €56 per month of progression free survival). The value of innovation and future drop in prices of new drugs after the period of patent and data protection should be considered when discussing access to new cancer treatments.
- When considering the price of drugs, people often fail to take into consideration benefits generated, e.g., a Belgium breast cancer analysis showed the cost saving from the reduction in morbidity and mortality of patients more than off-set the direct healthcare costs of the treatment. Despite the increase of direct healthcare costs for the treatment

of breast cancer, the overall gain for society increased by €3,896 per patient per year, thanks to reduced mortality and morbidity<sup>5</sup>

- The European Code of Cancer Practice states patients have a right to “Equal access to affordable and optimal cancer care, including the right to a second opinion”. Early access schemes run by the FDA and EMA can fail to take into consideration that common cancers, as well as rare diseases, have unmet needs.
- The regulatory environment fails to take into consideration the way patient data could be captured in a more systematic and robust way. Data on pharmacovigilance, quality of life, patient reported outcomes could all be reported by smart phones for example, adding to the evidence base to help inform decision making.
- Although > 200 cancer drugs have been licensed since 1999, the biggest challenge is access. Time frames between formal EMA approval and patient access in member states is unacceptable
- The total costs of cancer drugs per capita per year in Europe, according to a 2020 study in [EJC](#), range from €4 in Estonia and Greece to €108 in Austria, with an average cost in Europe of ~ €50 per capita per year.
- Between 2017 and 2020, according to the [EFPIA WAIT Indicator survey](#), 41 oncology drugs were approved by the EMA, with only Germany providing access to all drugs on 1<sup>st</sup> January 2022. At the lower end, Bosnia provided access to 4 drugs and Albania to 2 drugs in the timeframe assessed.
- The time to availability between marketing authorisation and date of reimbursement (for 2017-2020), [according to the EFPIA WAIT Indicator survey](#), ranges from 100 days in Germany to 960 days in Estonia. In most European countries, time between EMA approval and availability is too long, taking an average of 500 days before drugs reach the market.
- Treatment costs should be covered by public health insurance by ‘solidarity mechanisms. Affordability should be a public issue not a private issue, with no patients denied access because drugs are too expensive.
- Most health care systems are badly organised and need greater focus on screening. For example, early detection from colorectal cancer screening leads to 90% survival versus 10% when diagnosed at stage IV disease, where treatment costs are 10x more expensive.
- A recent report, ‘[Every Day Counts: Improving Time to Patient Access to Innovative Oncology Therapies in Europe](#)’, identified 10 barriers to fast oncology drug market access in the EU, and included six core recommendations for all stake holders.
- Patient organisations need to play a greater role in decision-making. They understand what it means to live with the disease and can provide useful data and intelligence.

## Panel Discussion

- Many European patient organisations do not receive financial support from the EC or individual governments, instead relying on fundraising activities. Allowing patient organisations to play a role in managing data (e.g., disease registries, as happens with the Belgium cystic fibrosis charity), would help provide greater influence and benefit.
- To improve funding, patient organisations need to demonstrate value and show they complement health care systems. Identifying potential services that patient

<sup>5</sup> [https://pharma.be/sites/default/files/2021-08/value-of-medicines\\_0.pdf](https://pharma.be/sites/default/files/2021-08/value-of-medicines_0.pdf)

organisations might offer hospitals and patients would allow value of services to be determined.

- Instead of each country undertaking its own HTA assessment, Europe could harmonise the process through joint assessments. Member states would have access to joint data, but make decisions according to country-specific priorities.
- An alternative view was rather than taking a pan-European approach, pilots (like the one in the Netherlands) could explore ways of getting innovative drugs to patients quickly. Such approaches demonstrate whether benefits continue over time.
- Stakeholders need incentives to provide patients with early access. One suggestion was for patient organisations to take a leadership role by creating dashboards for every type of cancer (rating services from diagnosis to end of life) to demonstrate performance differences between hospitals. Opportunities to make comparisons would ultimately result in better patient outcomes and quality of life.
- Remote patient monitoring systems enable evidence to be managed in real time. However, privacy issues are cited as reasons not to undertake such projects. A survey by the European Patients' Forum demonstrated three-quarters of respondents were willing to share their data if it benefited the entire community.
- Determining drug reimbursement according to effectiveness data would encourage health services to feel they were achieving value for money.

## Session 4: Turning Data Into Intelligence For Patient Focussed Oncology Drug Development

### Introduction

If there is one thing that the Covid-19 pandemic has taught us, it is the absolute centrality of data. Turning that data into intelligence underpinned many aspects of our response to the pandemic, allowing us to understand the dynamics of the virus and rapidly design novel vaccines to attenuate its impact. A digital health revolution is now upon us and our challenge is in balancing the undoubted advantages that data can deliver, particularly in the area of cancer, while still respecting the privacy and individuality of the person. A second consideration is the need to ensure that fair value principles are embedded within all aspects of the effective but responsible use of data. In this session, we considered (from the clinical, patient and industry perspective), how best to deploy data to accelerate innovation, how data intelligence can inform a more precise approach to patient care and what are the key enablers and barriers that we must consider to ensure that we deliver the optimal data-informed, patient-centred care for our society.

### Driving a data informed patient relevant agenda - lessons from DATA-CAN, the UK's Health Data Research Hub for Cancer

Mark Lawler (Queen's University Belfast, UK)

Mark Lawler, Professor of Digital Health, Queen's University Belfast and the Scientific Director of DATA-CAN, the UK's Health Data Research Hub for Cancer, described how during Covid-19 DATA-CAN pivoted to focus on the pandemic's effects on cancer.

- In 2017, it was acknowledged in a paper in [the New England Journal of Medicine](#), that to empower research and innovation, the cancer community needed to move from a closed



'selfish silo' mentality to a more open collaborative culture, and establish public trust in the use of big data.

- A citizen-focused data sharing cooperative approach was proposed, articulating a clear social contract where citizens (as data donors) were at the heart of decision-making. The idea was to democratise data analysis for maximum scientific, clinical and patient value, and support research by enabling Trusted Research Environments (also known as data safe havens or secure data environments). The resulting 'Roadmap for Restoring Trust in Big Data', published in [Lancet Oncology](#) in 2018, embedded the principles of the five 'safes': "Safe people, working on safe projects, utilising safe data, in safe places, with safe outputs".
- DATA-CAN, the UK's Health Data Research Hub for Cancer, was established to provide a cancer data ecosystem across the UK driving research and innovation, enabling more effective prevention and early diagnosis approaches, facilitating application of data-enhanced discovery to deliver innovative therapies, nurturing and empowering patient recovery and rehabilitation, and underpinning enhanced industry partnerships and innovation. Patients are involved at all levels of decision-making and have power of veto.
- With the Covid-19 pandemic, DATA-CAN pivoted to focus on addressing the pandemic's effect on cancer. DATA-CAN produced data, published in [BMJ Open](#) in 2020, showing that 7 out of 10 people with suspicion of cancer were either not going to their doctor for fear of contracting Covid or were not getting referred to cancer specialist services, while 4 out of 10 cancer patients were not getting access to chemotherapy at the appropriate time.
- DATA-CAN also undertook a modelling study, using a linked primary/secondary care dataset, predicting Covid-19 would be responsible for 7,165- 17,910 excess deaths in patients with cancer.
- The data drew attention of the government, academia, NHS and public to the disastrous effect Covid-19 was having on cancer services and people at risk of, and people with cancer.
- In light of the dramatic impact of Covid-19, the board of the European Cancer Organisation launched a Special Network on the Impact of Covid-19 on Cancer. The network, which convened a panel of experts from across Europe, developed a [7-Point Plan](#) to mitigate impact of Covid-19.
  - Additionally a pan European study was performed which showed that: European cancer specialists had seen 1.5 million fewer cancer patients in the first year of the pandemic
  - Over 100 million cancer screening tests had not been performed.
  - As a result, they estimated that as many as 1 million people with cancer could be undiagnosed due to presentational/ diagnostic backlogs.
  - The pandemic has also taken its toll on healthcare workers, with 4 in 10 feeling burnout and 3 in 10 showing signs of clinical depression.
- This intelligence informed a 'TIME TO ACT: Cancer won't wait-Neither Should We!' campaign, described in a 2022 article in the [BMJ](#), with messages including not delaying seeing doctors with warning signs of cancer, keeping screening and treatment appointments, and making getting cancer services back on track the top priority of the policy agenda. The campaign has been translated into 30 European languages.
- The experience of Covid-19 clearly illustrates the importance of data, suggesting in future it should be possible to use near real-time cancer data to help decision making.

## Data privacy by design is good for Patients and Drug development

**Giliosa Spurrier-Bernard (MelanomaFrance; Melanoma Patient Network Europe, FR)**

Giliosa Spurrier-Bernard, President of MelanomaFrance, considered the need to enforce data standards from the patient's perspective.

- Gillian Spurrier-Bernard described the situation in France (when her husband was diagnosed with metastatic melanoma) where patients can be responsible for their own data. They can keep their own records and take papers and imaging disks from appointment to appointment. Disappointingly, when clinical information was provided to an Innovative Medicines Initiative (IMI) pilot ('[GetReal](#)'), it was not made available to national melanoma registries.
- Spurrier-Bernard described her vision for an app storing clinical data (scans, blood, and radiology reports), explaining terminology and providing links to family history. Patient results would be seamlessly added to the portal with participants choosing what they wished to keep private and what they choose to share for improvement of health services. The app would direct communication with healthcare teams, with side-effects passed through a triage system triggering appropriate alerts. Patients would be informed how their data was used and by whom, with links to trials or research portals using the data. Any new knowledge relevant to the patient could be linked back to them.
- Spurrier-Bernard highlighted the development of [the European Cancer Patient Digital Data Centre](#), a virtual network of patient-controlled health data infrastructures where cancer patients and survivors deposit health information data in a standardised, ethical and interoperable manner. The centre would give patients easy access to their own data for decision making, enforce data standards, support good secondary data use, and give patients opportunities to initiate research.

## Industry perspective

**Bartek Madej (Novartis, CH)**

Bartek Madej, Head of Digital and Innovation at Novartis Oncology, Central, Southern and Eastern Europe, considered ways health data is benefitting European patients.

- Madej outlined three 'tangible' examples where health data have been turned into intelligence to benefit patients:
  - Novartis Oncology together with researchers from Uppsala University used machine learning to identify metastatic breast cancer (mBC) patients. Since treatment decisions for mBC are complex and patient subsets poorly characterised, identifying true prevalence of mBC has been difficult. The study, the first using machine learning to identify patient subsets in a national population health registry, estimated the number of mBC patients and described characteristics and survival outcomes.
  - Screening eligibility for clinical trials is labour-intensive. The 'Clinical Trial Matching Project' used IBM Watson technology to match breast cancer patients to clinical trials by evaluating patient data against inclusion and exclusion criteria. The project excluded 94% of patients automatically, reducing screening workload.
  - [MELLODDY \(Machine learning ledger orchestration for drug discovery\)](#) is a machine learning platform that makes it possible to learn from multiple sets of proprietary

data, while respecting their highly confidential nature. The technique, known as 'federated learning', allows datasets to remain behind firewalls, stored independently from each other. The algorithm goes back and forth between subsets of data from different sources, so that the data stays where it is but the analysis is "ported" to the data.

- [The European Health Data Space](#) (EHDS) has been created to enable the EU to support use of health data for better healthcare delivery, research, innovation, and policy. It should provide opportunities to unleash the power of data for benefit of patients, finding new cures and defining standards of care.
- EHDS faces three challenges: standardisation (introduction of unified electronic health records enabling sharing across EU MS); interoperability (need to legislate for connected data sharing systems); and fragmentation (overcoming GDPR limitations).
- EHDS should acknowledge different stakeholders including patients (currently underrepresented), academia (who know about standardisation of patient records), and industry (who could serve as mentors and supporters of projects).

## Panel Discussion

- Already cancer systems produce apps, allowing patients access to their own data. GDPR has helped create a level playing field, driving access to personal data. In secondary data use, there are a number of ongoing initiatives, such as the French guidelines around secondary use of data.
- Addressing cancer registry funding, Mark Lawler indicated he had a forthcoming 'Lancet Oncology. European Groundshot Commission being launched at the European Cancer Summit (<https://www.thelancet.com/commissions/European-Groundshot-cancer-research>) which among many activities explores the role of registries in national cancer control plans to deliver better health outcomes.
- With some innovative products unlicensed, there is a need to overcome confidentiality issues to allow industry to share data. In addition to MELLODDY, [DARWIN EU](#), established by EMA, is seeking to accelerate access to high quality real-world evidence for regulatory decision-making by standardising health care data to enable large scale access. The pilot phase involves a cancer registry.
- Incentivisation is needed to improve quality of data contributions. One approach is to enable hospitals to see their own data and also how they compare to other hospitals. The approach encourages participants to make data submitted more complete and more accurate.
- Minimisation of data collected would also benefit efficiency and security. Even though patients may be willing to share data, availability of data may create future issues for their children. There were also questions around national security, in small countries with homogenous populations health data could reveal new vulnerabilities. Public discourse is needed around security issues (both personal and national) of medical data. The way to avoid 'fishing expeditions', involving vast amounts of data, is to start with defined research questions. There is need to ensure data is well protected.
- To improve data sharing, standardisation is needed. Patient advocacy groups should come together to define minimal data sets needed for standardisation.
- Regarding the European Health Data Space proposal, an EC vision looking to make more effective use of health data, is currently out for comment.

## Session 5: Cross Border Access For Oncology Clinical Trials

### Introduction

Cross-border access to clinical trials is theoretically possible under the current 2011/24/EU Directive on patient rights in cross-border healthcare. However, a recently published study has shown that patients very rarely join trials outside their own country - despite a high need expressed by study respondents. In this session, the hurdles for patients to participate in research beyond the borders of their own country were outlined and potential approaches to increase cross-border access were discussed.

### Accessing clinical trials abroad. The experience of the Melanoma Patient Network Europe

**Bettina Ryll (Melanoma Patient Network Europe, SE)**

Bettina Ryll, founder of Melanoma Patient Network Europe, considered the challenges experienced by patients looking for cross border access to trials.

- Bettina described her experience when her late husband (who had stage 4 melanoma) was in a trial in London for a novel agent when they lived in Sweden. To access the trial, they paid for routine health care costs (beyond costs associated with the investigational agent) out of their own pocket.
- Clinical trials represent an important mechanism of accessing novel treatments for patients who have exhausted all available lines of treatment. However, the reality is that access to clinical trials across Europe is uneven, with disparities occurring in countries with similar socioeconomic situations, e.g., Denmark has many more trials than Sweden.
- In Europe, even large phase III trials often only recruit in a subset of member states, with the result that many patients can only access trials by travelling to other countries.
- Clinical trials can be financed through multiple sources, where health care systems pay for routine treatment scans and visits and trial sponsors cover the costs of investigational agents and other costs which are part of the additional treatment defined in the clinical trial protocol. When patients travel abroad, the sponsor still covers all the costs associated with the trial, but routine and follow-up care in their home country needs to be covered by the health system in their country of origin, but this may not be the case. With no universal mechanisms in place, access to clinical trials abroad is often only a possibility for patients providing some of their own financing.
- Denmark and Norway have established structures where if patients have exhausted all standard of care options and have no clinical trial available in their country, they can be considered by a committee for a trial abroad. If successful, the country's health care system supports trial participation, but also travel and accommodation (if these are not covered by the trial sponsor e.g. for academic trials). However, these systems do not appear to be working as efficiently as they could
- Other questions include who will cover additional costs resulting from toxicity experienced at home, and will there be protocols that health care staff at home can access on managing toxicity?
- Access to clinical trials abroad is inconsistent and currently depends too much on patient initiatives and finances.

## Cross-Border Access to Clinical Trials in the EU: results from an exploratory study

Teodora Lalova-Spinks (KU Leuven, BE)

Teodora Lalova Spink, a doctoral researcher at KU Leuven's Faculty of Pharmaceutical Sciences and at the Centre for IT & IP Law (CiTiP), provided an overview of her survey on cross-border access to clinical trials.

- Teodora Lalova Spink reported results of a mixed-methods exploratory study of cross-border access to clinical trials in the EU, published in 2020 in [Front Med \(Lausanne\)](#). The study objective was to achieve an overview of stakeholders' real-life experiences and identify needs, challenges, and potential for facilitation of cross-border access.
- EU Directive 2011/24/ EU, the 'Cross-border healthcare directive', does not include clinical trials within its scope.
- While there is no specific European legislation facilitating cross-border clinical trial participation, frameworks exist e.g., the Nordic Network for Early Cancer Trials (Nordic NECT).
- Altogether, 396 individuals responded to the survey (46% were investigators/physicians and 33% patients), and 38 individuals were interviewed (29% were investigator/physicians and 29% patients). Highest response rates were received from residents of Western European countries (38% for survey, 45% for interviewees), and lowest from Eastern Europe (9% of survey respondents, 5% for interviewees).
- According to study participants, cross border access to clinical trials occurs rarely. Some said it was 'close to zero', others 'less than 1%'.
- 92% of survey respondents answered 'yes' to whether we need cross-border access to clinical trials. However, it was felt cross-border access was only part of the solution, and that clinical trials should be brought closer to patients. In the survey, 68% of respondents felt there was need for reliable and easily accessible information, 67% for change in relevant EU legislation and 55% change in approach to institutional insurance for study participants.
- Challenges to participate in cross-border clinical trials included costs coverage, language barriers, procedural challenges, travel distances, vulnerability, cultural barriers and lack of information. Regarding lack of information, three challenges were identified: information about ongoing trials (eligibility criteria, sites, appropriate systems for patient's referral), information about value of clinical research in general, and information about best practices when joining a clinical trial abroad (legislation and regulation).
- Proposal for future actions from interviews included creating multi-stakeholder guidelines, optimising how information is disseminated, creating bilateral agreements between hospitals, amending the cross-border health care directive, increasing use of decentralised approaches to clinical trials (e.g. telemedicine), harmonising the EU clinical trials framework and creating a common ethics approval framework in the EU. The next step is to hold multi-stakeholder, multi-national discussions to create guidelines including easy access to information, collection of best practice and ways to navigate the system.

## Can we improve cross-border access to clinical trials?

Ingrid Klingmann (Chairman at Board of the European Forum for Good Clinical Practice, BE)

Ingrid Klingmann, chair at the Board of the European Forum for Good Clinical Practice, describes her vision for EU Cross Border Trials (EU-X-CT) initiative that provides information for patients.

- Harmonising the EU framework by adding cross-border access to Directive 2011/24/EC is probably not an option and changing the clinical trials system would be too complex. The solution is to identify concrete options and tangible solutions.
- EU Cross-Border Trials (EU-X-CT) is a new multi-stakeholder group with plans to bring information on cross-border access to clinical trials in Europe together in a way that is relevant for patients all over Europe.
- EU-X-CT, driven by the [European Forum for Good Clinical Practice \(EFGCP\)](#) and [European Federation of Pharmaceutical Industries and Associations \(EFPIA\)](#), hopes to develop a consortium of 50 to 70 organisations from different countries and disciplines. The idea is to create a registry of relevant national information from all European countries and offer it free on a dedicated, independent website, and make the initiative known to patients, patient organisations and treating physicians.
- Essential participants include patient advocates and patient organisations, academic institutions involved in clinical trial infrastructures, not-for-profit organisations, pharmaceutical and medical device companies, CROs, and medical societies (physicians, pharmacists, HTA networks and ERNs).
- Proposed registry topics include: National healthcare systems' abroad costs' reimbursement conditions; national healthcare systems' conditions for follow-on treatment availability and its financial coverage; national trial liability coverage requirement for trial participants from abroad; national contact points for support (for patients and treating physicians); description of practical challenges of investigator sites for investigators enrolling patients from abroad; description of practical challenges for patients wanting to join trials abroad and examples of best practice.
- A kick-off meeting to define EU-X-CT's work plan is being held in Brussels. To create balance, the aim is to involve a wide range of stakeholders. The meeting will be hybrid and can be joined both in person and online. Anyone interested in participating should contact Ingrid by email. [Ingrid.klingmann@efgcp.eu](mailto:Ingrid.klingmann@efgcp.eu)

## Panel Discussion

- Interest in developing electronic systems to help in gathering data supporting decentralised trials. The EMA currently has the [Accelerating Clinical Trials in the EU \(ACT EU\) programme](#) to facilitate improvement of decentralised trials; a multi-stakeholder meeting which was held after this event on 4 October 2022.
- Regarding clinical trial information, the [clinicaltrials.gov](http://clinicaltrials.gov) website does not include complete information. A more accurate picture can often be obtained from patient organisations who have full protocols and personal contact with investigators. Also, oncologists from centres of excellence are more likely to be aware of trials abroad. Participants need to be confident trials are appropriate before personally investing time and money.

- Countries vary widely in willingness to accept trial participants from abroad – the Netherlands does not accept trial participants from abroad, Belgium does, and Germany sometimes does but has a requirement for participants to speak German to understand informed content.
- For physicians, time taken to enrol participants in trials (particularly when playing catch-up after Covid-19) creates disincentives. Hospitals are also likely to incur additional costs around investigations, which may not be covered by the trial sponsor. Workloads would be reduced by simplifying clinical trial protocols, including reducing number of laboratory tests required. Currently, number of tests per patient in a clinical trial can be as high as ~ 150.
- Even at national levels, it may be difficult to access trials in other parts of a country due to competition between institutions for trial activity. To overcome such conflicts, participants need independent information so that they can decide on the options that works best for them.
- Participants can be deterred from taking part in trials by practical considerations, such as travel costs. To facilitate trials, pharmaceutical companies already play a role in helping with organisational issues (e.g., booking flights). For non-commercial sponsors, funding should cover costs to prevent disparities with commercial trials. Anyone applying for a relevant Horizon Europe project could include funding for cross-border access to clinical trials in their application.
- Evidence is irrefutable that [cancer patients treated in clinical trials have better outcomes than those who are not](#), making cancer research and cancer clinical trials a necessity rather than a luxury. But messages are not getting across to all stakeholders, including physicians and a significant percentage of physicians thinking that they should not be asking patients to participate in clinical trials.
- Clinical trial investigators need greater support and should have dedicated time to perform trials in addition to their clinical duties. Communication gaps exist between people designing clinical trials and those doing the day-to-day work. The topic of clinical trials is not covered sufficiently in medical curricula (undergraduate or post graduate levels). One suggestion was for commercial sponsors to fund research fellowships where health care professionals receive clinical trials training.