European Manifesto 2024

Best cancer treatments to address unmet patients needs

Making Europe a leader in the development of innovative, impactful and affordable cancer treatments

Anticancer Fund

Introduction

The cancer burden is expected to rapidly grow both at EU and global level.

At a global level, over 35 million **new cancer cases** are predicted by 2050, a **77% increase** from the estimated 20 million cases in 2022[1].

In the EU, **cancer deaths** are expected to **increase by more than 24% by 2035** and this will make cancer the leading cause of death. The **overall economic impact** of cancer is estimated to **exceed €100 billion annually**[2].

European decision-makers aim to bring the best possible treatments to every patient. However, if we desire a future where cancer is no longer a life-threatening disease, **all potentially impactful cancer research must be given equal attention**.

The current research in oncology predominantly focuses on developing new (bio) pharmaceuticals with substantial financial returns. **Europe urgently needs a complementary pathway** to bring **treatments, lacking commercial appeal**, to patients.

Objective 1: Incentivizing valuable research outside the scope of drug developers

Over the past decade, significant progress in oncology has been driven by substantial public and private investment, leading to better overall survival rates, especially in lung cancer, melanoma and blood cancers. However, the costs associated with innovative drugs are concerning.

While new therapies, such as immunotherapy, are available for various cancer indications, **rare cancers** - including paediatric cancers and **those difficult to treat**, like pancreatic cancer - **are underserved**. In these instances, drugs are often **used off-label***, sometimes without the necessary evidence base, putting patients **at risk**.

Commercial development should not be the sole driver of cancer research

Research using existing drugs should be high on the agenda of public institutions as **drug repurposing** could result in more affordable solutions. Further clinical research should be supported by subsidies and philanthropy. Final introduction in clinical practice will require **an adequate regulatory framework**, such as proposed in Article 48 of the draft Regulation.

Additionally, **innovation in surgery, radiotherapy, and their combinations with marketed drugs** could significantly improve overall survival and quality of life and could follow the same trajectory.

*use of a drug outside its approved indication(s)

Demands to policy makers

Pharmaceutical regulatory package: ensuring an effective role for not-for-profit organisations in drug repurposing

Regulatory changes are needed to translate positive outcomes from non-commercial clinical drug research into guidelines and clinical practice.
In the current review of the EU pharmaceutical legislation, **Article 48** of the proposed Regulation[3] **is crucial** as it enables not-for-profit organisations to submit clinical trial results to the European Medicines Agency (EMA) for assessment, potentially leading to new drug label indications.

What can you do as policymaker?

Ensure that Article 48 of the Commission proposal is adopted to achieve a concrete and effective role for not-for-profit entities in the repurposing of authorized medicines, including repurposing patented drugs for rare diseases.

Setting infrastructure for public clinical trial platforms across member states

The Covid pandemic highlighted the need for **public clinical trial platforms** across member states for **comparative effectiveness research**. The same concept should be applied for all promising cancer treatment modalities, including patented biopharmaceuticals, to ensure the best treatment for cancer patients.

Innovative funding models are essential, based on partnerships between public funders, private partners, health insurers, academic investigators and philanthropic supporters.

What can you do as policymaker?

Advocate, in all relevant European fora, for the establishment of public clinical trial platforms across member states, dedicated to cancer treatments.
Stimulate discussion on the creation of innovative funding models, involving the public and private sectors at both European and national levels.

Objective 2: Supporting pragmatic clinical research & ensuring an efficient assessment process

Pragmatic clinical trials are designed to evaluate the effectiveness of interventions in daily medical practice.

We advocate for **pragmatic trials** ensuring benefits that are clinically, not just statistically, meaningful. Interventions could include local, surgical, or radiotherapy treatments, repurposing licensed drugs and treatment de-escalation. Trials must be of high quality, as enforced by WHO's Resolution WHA75.84[4], to prevent the misuse of patients in futile trials.

Demands to policy makers

Empowering EMA

Publicly supported clinical trials, including pragmatic trials and clinical trial platforms, **should be EMA-approved rather than approved at national level**.

Joint Scientific Advice from EMA and Health Technology Assessment (HTA) should be mandatory to swiftly and efficiently demonstrate added patient value (Quality of Life and Overall Survival). The pharmaceutical industry agrees that **clinical trials in oncology should ideally meet the needs of both the EMA and HTA bodies** so that after EMA approval, no further studies are necessary before reimbursement can be approved.

EMA should establish a drug evidence watch process to monitor emerging efficacy data for drugs in new indications. This efficacy vigilance process can lead to label extensions and will help resolve issues relating to off-label use[5].

What can you do as policymaker?

Adapt the current regulatory framework to

- **Reinforce EMA's role in approval** of publicly funded clinical trials and clinical trial platforms in oncology.
- **Support mandatory Scientific Advice, related to registrational studies, from EMA and HTA jointly**.
- Support the creation of a **drug evidence watch process at EMA**.

Horizon scanning by HTA

The European Union should introduce an **automatic procedure for joint HTA reviews**, when drugs are approved for new indications through EMA drug evidence watch or under Article 48 of the proposed Regulation. Alternatively, not-for-profit organisations and patient advocacy groups should be able to apply for joint HTA assessment of a drug in an indication with high unmet medical need when the Marketing Authorization Holder is not willing to make the drug available on the European market.

What can you do as policymaker?

Advocate for an **automatic procedure for joint HTA reviews** applicable for drugs which received a positive scientific opinion for new indications under Article 48 of the proposed Regulation.

References

[1] https://www.who.int/news/item/01-02-2024-global-cancer-burden-growing--amidst-mounting-need-for-services

[2] https://health.ec.europa.eu/system/files/2022-02/eu_cancer-plan_en_0.pdf

[3] Proposal for a Regulation laying down Union procedures for the authorisation and supervision of medicinal products for human use and establishing rules governing the European Medicines Agency, amending Regulation (EC) No 1394/2007 and Regulation (EU) No 536/2014 and repealing Regulation (EC) No 726/2004, Regulation (EC) No 141/2000 and Regulation (EC) No 1901/2006 (COM(2023)193).

[4] https://apps.who.int/gb/ebwha/pdf_files/WHA75/A75_R8-en.pdf

[5] Pantziarka P. et al., The Lancet 2023; 402(10406): 945-947

About the Anticancer Fund

Who are we?

The Anticancer Fund is a Belgian not-for-profit organisation with an international scope. We are dedicated to expanding the range of treatment options available to cancer patients, regardless of commercial value. Our goal is to extend lives, increase quality of life and provide cures for cancer patients by complementing the commercial drivers of cancer care with exclusive patient-first thinking and a focus on evidence-based potential for new treatments.

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