April 2019

Increasing our options for cancer treatment in Europe

Doing more with less

Various drugs already widely used for non-cancer diseases have shown promise for the treatment of cancer. Repurposing these drugs could allow new cancer treatments to be introduced relatively quickly and at low cost, thereby meeting the unmet needs of patients and healthcare payers alike. The Anticancer Fund urges EU policy-makers to commit to supporting drug repurposing to unlock its enormous potential.

Improvements in our understanding of cancer have led to important therapeutic advances in recent years. However, ensuring that all patients have sustainable access to high-quality care is a major challenge, as the costs of care are rising in the context of fiscal constraints on healthcare systems.

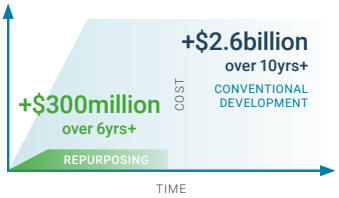
Cost of cancer medicines

7-Fold
PRICE INCREASE

Conventional development vs. drug repurposing

>€100.000
VS **€10-1.000**

Development costs over time



Cancer kills over 1.25 million people
each year in the EU - that is one in four deaths

Valuable high-quality, independent research is currently being conducted in Europe and has the potential to provide patients with sustainable access to effective treatment options. However, challenges to translating academic research into marketed products must be overcome. **Strategies are needed** for the delivery of scientifically validated treatment options to the patients who need them. **Drug repurposing** can be a valuable parallel pathway to the pharmaceutical industry development pipeline, and deserves to be given high priority.

The high-cost of new cancer treatments and discrepancies in access to cancer care across the EU is at the top of political agendas in most Member States. With the European elections looming in Spring 2019, candidates for office should understand what is at stake for cancer patients and health systems alike, and how solutions that aim to do more with less can help.

Repurposing of medicines

Repurposing (or repositioning) medicines offers a rational, evidence-based approach to help address these challenges and to provide patients, prescribers and payers with sustainable access to additional, cost-effective therapeutic options for cancer. Drug repurposing can take many forms. Some approaches, such as the reformulation of existing drugs resulting in new intellectual property and the commercialisation of previously shelved compounds, offer the potential for a return on investment that provides an incentive for industry. However 'financial orphans' emerge if these commercial incentives are lacking while patients are in need. The Anticancer Fund believes that dedicating funding to independent research will mean that patients will gain access to more scientifically validated treatment options.

Drugs that are good candidates for repurposing have the advantage that preclinical, pharmacokinetic and safety data are already available. This allows their development for cancer to be 'fast-tracked'. Moreover, repurposed drugs are often available as generics that are significantly less expensive than newer branded products that are still under a patent.

Today, over 280 candidates for repurposing have been identified in the oncology field alone. About 30% of these candidates are already being investigated in late-stage clinical trials for their anticancer activity. These trials will soon yield results that will need to be reflected in clinical practice as soon as possible to benefit cancer patients with urgent unmet medical needs.

But barriers exist

Researchers from academia or independent research institutes are active where incentives are lacking for industry to venture into drug repurposing – the so-called 'financial orphans'. However, their type of research is not compatible with the current drug development model. As well as poor overall awareness of the potential of repurposed drugs in both cancer and other diseases with high unmet needs, two key barriers to repurposing exist:

1

Lack of financial incentives and research funding

Repurposed drug candidates need to be tested in robust clinical trials, just like any other drugs. This requires significant investment and yet support is scarce when there is no industry sponsorship forthcoming. Currently less than 4% of late-stage repurposing trials have a commercial sponsor[1].

2

Lack of clear regulatory pathways

The current regulatory framework does not provide guidance on how to implement results from independent research when the pharmaceutical company that owns the marketing authorisation for the drug is not motivated to adapt the product label. If no regulatory solutions are found, these medicines will be used 'off-label' in the new indication (i.e. in ways that are not formally approved). This can entail a number of challenges. For example, off-label use can cause liability issues for prescribing physicians, uncertainties with regard to reimbursement, and patient safety concerns due to the absence of formal benefit-risk assessments[2].

[1] Pantziarka P, et al. ReDO_DB: the repurposing drugs in oncology database. Ecancermedicalscience. 2018 ;12 :886.
[2] Health Action International. Policy brief - Regulating the off-label use of medicines in Europe. 2018.

What we call on you to commit to

As you step into your new role as a policymaker, there are a number of actions you can pledge to help make cost-effective, repurposed options available to cancer patients in Europe.

A parallel drug development pathway supported by public investment is vital to unlock the potential of repurposed medicines to provide new and sustainable cancer treatment options.

Rethink funding options for independent research

Europe has some of the brightest brains in cancer research, but they need your support. Given the public health impact of cancer and the potential value of repurposed drugs, a far greater level of EU and national funding to research in repurposing is warranted.

- Where commercial interest is missing and patient needs are high, commit to making public funds available for independent research and clinical trials for new cancer treatments.
- Creative funding models are required; these could be based on partnerships between public funders, health insurers, academic investigators and philanthropic supporters.
- The EU's next mission-oriented framework programme for research and innovation, Horizon Europe, should provide funding opportunities specifically aimed at supporting independent research into drug repurposing.

Unlock the regulatory pathway

The European Commission's Expert Group on Safe and Timely Access to Medicines for Patients (STAMP) has formed a working group on repurposing to explore barriers in the existing drug development route and devise a new regulatory pathway conducive to bringing repurposed medicines to the patient.

The STAMP group aims to define a simplified repurposing framework for independent clinical researchers that will lead to the registration of a new therapeutic use for a drug, and which is complementary to the current innovation pathway followed by commercial developers. This proposal will come under scrutiny by the Commission, and possibly the Parliament and Council. We urge you to stand behind this important work.

- Support the implementation of the new dedicated parallel regulatory framework, which aims to facilitate the prompt and efficient authorisation of new indications for existing drugs.

Promote awareness of the need for adequate infrastructure

Another barrier to innovation is the lack of infrastructure dedicated to drug repurposing. We ask you to call for much-needed investment in this area.

- Encourage understanding of the need for exploration of drug repurposing by promoting its value-potential
- Drive innovation in academic research by supporting the establishment of centres of excellence for horizon scanning and knowledge-sharing on non-cancer drugs that show promise in cancer
- Deepen cooperation across disciplines and especially including patients as experts in cancer research in Europe
- Support open science
- Collect post-launch phase and real-world evidence to get a clear understanding of a treatment's added value

Anticancer Fund

The Anticancer Fund (ACF) is a Belgian Public Utility Foundation dedicated to expanding the range of treatment options available to cancer patients, regardless of commercial value.

The ACF sparks solutions in valuable and affordable new treatment options that are outside the scope of industry. The ACF is convinced that clinicians, academia, government and foundations can play a bigger role in bringing promising, but commercially less interesting, cancer treatments into daily practice with added therapeutic value for patients. There is a need to provide a clinical, regulatory and economic framework in favour of independent clinical research to address the unmet need for cost-effective anticancer treatments that fill the gaps left open by the current R&D landscape. This is how the ACF sees sustainable innovation in cancer treatment.

Unlocking the repurposing potential

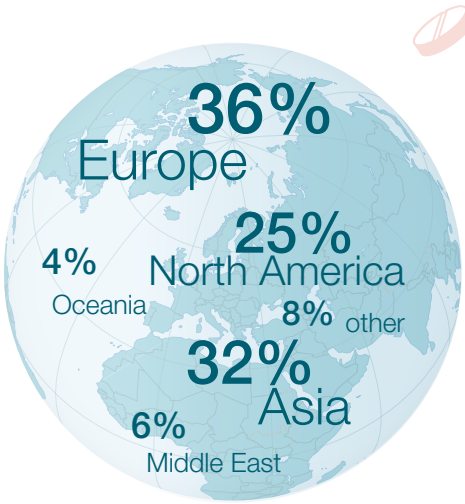
268 DRUGS

Generic
known safety ✓
cheap ✓

cardiovascular
insecticides
anti-parasite
alimentary tract
nervous system
hormones
sensory organs
anti-infectives
respiratory
genito-urinary system
dermatologicals
metabolism
musculo-skeletal system
blood

190 LATE STAGE ONCOLOGY TRIALS

brain & CNS
gastro-intestinal
hematologic
breast
lung
gynaecologic
paediatric



#VoteAgainstCancer
#MissionPossible
#EU2019

www.anticancerfund.org