EU Action on Pancreatic Cancer

WORKSHOP 8

Pancreatic cancer can be placed at the interface between rare and common diseases; its incidence is increasing due to factors including demographic change.

The main topic covered in the session was the increased research and the changing regulatory environment around new drugs for pancreatic cancer, and how to bring this orphan disease to the top of the political agenda.

The panel was composed of international experts, researchers and policymakers: Nuria Malats, CNIO Madrid, Ricardo Baptista Leite, Member of Parliament, Portugal, Matthias Reumann, IBM Research, Lada Leyens, Swissmedic, and Angela Brand, EUPAncreas WG4 Coordinator, Maastricht University.
The session was chaired by Elke Anklam, Director, Joint Research Centre, European Commission.

What can be done – From research to policy

Regarding regulatory mechanisms, it has been suggested to have shorter trials and to grant exceptions for new orphan drugs once the additional added benefit is evident. One option here could be the concept of adaptive pathways. Adaptive pathways aim to improve timely access for patients to new medicines. It is a scientific concept for medicine development and data generation which allows for early and progressive patient access to a medicine. The approach makes use of the existing European Union (EU) regulatory framework for medicines.

Research still misses evidence on the causes of pancreatic cancer, and there are no validated bio-markers to support early diagnostics. Without bio-markers, early diagnosis is challenging as patient symptoms can have several different causes. This leads to late diagnosis, which furthermore leads to a small survival rate mid- to long-term, and poor prognosis (6,5 months from diagnosis to death).

Problems with research studies are: low incidence (need big data); high level of misdiagnosed cases; patients are too sick to participate in studies; only some patients undergo surgery, sampling difficulties and underfunded research work amongst others.

Once there are appropriate bio-markers it has been suggested to screen high-risk groups, although in discussions it was also argued that bio-markers are expensive and we should rather work on developing better primary prevention measures for pancreatic cancer.

Research shows that the mutation journey from a healthy cell to pancreatic cancer cell can take up to 17 years, so early diagnosis could prevent the disease, or lead to better treatment results. Countries should make the data they collect publicly available and collaborate in a way that researchers can use that data. Big Data can be useful if it is made actionable; data actions need to be transparent, standardised and holistic.
We need to characterise the problem first, and look at how pancreatic cancer is managed in different countries – from diagnosis to treatment, what are the differences and what are the problems.

An innovative way could be to use bio-markers and research evidence from other orphan diseases for pancreatic cancer (spill-over-effect). Looking at different kinds of cancers could be beneficial for pancreatic cancer diagnostics. Pharma companies should work together on "umbrella trials", to achieve more effective results.

It was also suggested to invest in Public-Private Partnerships (PPPs), using shared patent-schemes, to bring pharma, research and policymakers together to collaborate and foster data-sharing. This topic was controversial during the discussion with the audience, and ethical concerns were raised.
PPPs should have a common ethical background, and be transparent. This is a clear example of an unmet need, and we can learn from Norway's recent example of creating a consortium for unmet vaccination needs following the recent outbreaks of Zika and Ebola with the WHO, WEF and Bill and Melinda Gates Foundation: other diseases could also benefit from this approach.

As pancreatic cancer is an orphan disease which naturally has a low chance to achieve a critical mass for trials, it was suggested to enable cross-border clinical trials. Besides, reimbursement schemes should be included in the discussions on HTA, and different stakeholders need to be involved.

For the future, personalised medicine could be an answer. Personalised medicine is a medical procedure that separates patients into different groups - with medical decisions, practices, interventions and/or products being tailored to the individual patient based on their predicted response or risk of disease.

Partnerships should also centre on what patients and families are concerned about, rather than just looking at numbers i.e. survival rates. This is critical if we want to move towards a value based society. What do patients and their families expect and want?

Conclusion

Death rates for pancreatic cancer are increasing despite the general decrease in death rates for all cancers across Europe. As pancreatic cancer is a silent disease, it is of utmost importance to raise awareness among health professionals, patients and relatives, but also among the wider public and policymakers.

Researchers, health professionals, policymakers, donors and other stakeholders should all work together in a multidisciplinary way, "from cell to society", to achieve better health outcomes for the individual patient. This could be in the form of PPPs and policy-research networks such as the European Cooperation in Science and Technology (COST), with action EU Pancreas (BM1204) aiming to unite pancreatic cancer research groups across Europe and providing an innovative and unique platform for collaborating and sharing information, ideas and experience.

The "Pancreatic Cancer White Paper 2015" is a direct appeal to policymakers, legislators, regulators and stakeholders for collaboration, to raise awareness, and work on prevention and treatment of pancreatic cancer.
Delegates agreed that such approaches are essential to make a stand together to fight the increasing burden of pancreatic cancer.

Written by DIANA KOERNA & STEFANIE PRAXMARER

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NURIA MALATS, CNIO Madrid, Spain
RICARDO BAPTISTA LEITE, Member of Parliament, Portugal
MATTHIAS REUMANN, IBM Research
LADA LEYENS, Swissmedic, Switzerland
ANGELA BRAND, EUPAncreas WG4 Coordinator, Maastricht University

Chaired by ELKE ANKLAM, Director, DG Joint Research Centre, European Commission

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