To
email

23 March 2023

RE: Letter on UMN definition in the Pharmaceutical Legislation & Request for a meeting

Dear

We as Cancer Patients Europe, Europa Uomo, Pancreatic Cancer Europe, and the European Association of Urology appreciate the effort of revising the European Pharmaceutical Legislation and the good intention that "The revision maintains the incentives as a key element for innovation, but they are adapted to better encourage and reward product development in areas of unmet medical needs and to better address timely patient access to medicinal products in all Member States." [1]
This revision is very timely, as currently, Europe is not a good place to live for many cancer patients who need to go to other continents to find clinical trials. This is the case for pancreatic cancer, for instance. Europe is losing its attractiveness for R&D investments. In fact, 48% of new treatments originate in the US, compared to 22% in Europe. So we applaud that the EU Pharmaceutical Strategy intends to allow Europe to become a world leader in medical innovation.

In the new EU pharma strategy, the Commission intends to establish a common understanding of unmet medical needs (UMN) through a definition and this definition will be the basis of a set of criteria that will be reflected in regulatory pathways. So, the concept of unmet medical
needs is one of the most relevant to us within the new EU pharmaceutical strategy, as it impacts the entire lifecycle of pharmaceutical products, from the prioritization of research to the discovery of medicines and pricing and reimbursement.

A proper definition of UMN plays a fundamental role in the entire strategy. What we read in Article 73 of the leaked version is a very narrow definition. The disease must be **life-threatening** or **seriously debilitating**, and any new products (if there is something already on the market) must show a **meaningful reduction** in mortality and/or morbidity over existing treatments.
We are afraid that this definition will lead to the opposite of what the EU Pharmaceutical Legislation intends to achieve, which is less R&D investment in the EU and fewer treatments for patients, as it will increase uncertainty.

A broad concept of UMN is fundamental to increasing rather than limiting the development of new drugs and ensuring that they reach patients.
The UMN is understood as a condition that is not adequately prevented, diagnosed, or treated by any of the interventions available. In this sense, UMN is not a one-time fixed concept as the "need" is constantly evolving in line with the availability of the latest science, technology, data,
infrastructure, collaboration, and the progression of the disease (chronic vs. acute), and as such it should not be crystallized in a fixed way within legislation.

What exactly constitutes an unmet need depends on the situation and context, and the views of the different stakeholders do not always converge. The burden of disease on the individual, quality of life, and new formulations of existing treatments less intrusive in daily life varies from patient to patient. The Unmet Need also depends on society in terms of incremental improvements in disease management, and on the healthcare system perspective in terms of resource allocation, and the cost-effectiveness of treatments. All this said, even the term unmet medical need is used improperly. We should consider using differently the terms unmet *medical*

[1] Leaked Version from Politico, March 2023

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needs, unmet *patient* needs, and unmet *societal* needs. Unmet medical needs as such can be determined by clinicians only and they need to be weighed against patients' and societal needs.

The stakeholders involved are very different and understand life-threatening or seriously debilitating or meaningful reduction significantly differently. Not to mention that what is life-threatening or seriously debilitating may change dramatically from the moment the R&D starts and the final product reaches the patients. For many cancers, progress is achieved not through paradigm-shifting breakthroughs but incrementally. Progress in cancer care is made with a stepwise approach, by gradually shifting treatments from 3rd or 2nd line to 1st line as they prove their value, and by combining therapies to achieve a greater response. Many of these innovations that have led to great progress in cancer survival and QoL over time could easily fall outside of the proposed UMN definition. Furthermore, this narrow definition will also have an impact at the national level in the context of Health Technology Assessment (HTA) and pricing and reimbursement decisions. Member States/payers could use this definition to prioritise what should or should not be reimbursed.
This will further increase disparities in cancer care and harm what we have been building in cancer care for the past 20 years.

The current UMN definition puts at risk the work done so far towards patient-centric innovation as it is not clear how patient preferences are taken into consideration.

A single definition of UMN should therefore be avoided and Unmet Patient Needs should always be considered along with Unmet Medical and Societal Needs.

For these reasons, we would like to call for an urgent meeting to discuss our views and make sure that these points are considered in the further revision of the text of the EU Pharmaceutical Strategy.

I am looking forward to hearing from you.

Thanks and kind regards,

Antonella Cardone

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European Association of Urology

EUROPA UOMO
The Voice of Men with Prostate Cancer in Europe

PANCREATIC CANCER EUROPE
The European Multi-Stakeholder Platform on Pancreatic Cancer

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