ENRF Briefing Note on STOA workshop on healthcare research

Thursday 10 January 2019 saw a workshop run by the European Parliament's Science and Technology Options Assessment (STOA) Panel on the topic of innovative solutions for research in healthcare. The high-level forum discussed ideas around developing a novel approach to deliver better precision medicine in Europe and had as speakers, among others, the Belgian Health Minister, Maggie De Block, and the head of EORTC, Denis Lacombe.

At the meeting, Paul Rübig MEP, who is **STOA** Vice Chair, set the ball rolling by saying that data plays an important role in healthcare, especially in cross-border healthcare situations. The use of data offers an opportunity to save lives and to know which kind of medicines work together. Rübig's view is that the establishment of a new framework between industry, patients, governments and other stakeholders could mitigate the somewhat problematic current situation.

The speech of Maggie De Block, Belgium Minister of Social Affairs and Health, but also of Migration, was, as always, very practical and upfront. She advocates that political decisions needs to be evidence-based and as such real-time evidence is needed, stressing the importance of safety and wellbeing, next to financial sustainability. Everybody knows nurses are key when discussing personalised medicine to optimise dose and duration. Frontline nurses can have the data which are key for HTA. Quality of survival and applied clinical research is key to identify practices that improve daily quality of life. Therefore, HTA agencies need other sets of data than regulators.

Questions were also asked about the effectiveness of the general data protection regulation (GDPR), with EMA chief Guido Rasi surprising many by saying that he is not sure that the digital revolution and the regulatory environment are compatible. "There is a need for clarity "immediately" on two points", he said, citing secondary data use for health research and asking who is responsible if someone manages to identify data anonymised in good faith.

On the topic of modern concepts in healthcare in the 21st century, participants heard that there are very effective innovative new drugs which have been approved on the basis of a very limited number of patients, but they are used in a large number of patients for long periods of time afterwards. More real-world data and evidence is needed.

The workshop also heard that, in terms of challenges of new technologies in the research and societal environment, for researchers these lie mainly in the area of bio informatics solutions, benchmarking technologies and data interpretation. The situation is complex for researchers as well as for those bringing medicines to market, with the latter being challenged by new treatment approvals and off-label use. These issues go on to create challenges for pricing, health technology assessment (HTA) and new therapeutic guidelines.

Regulatory trials which aim to document new drugs are certainly needed, but as downsides their primary end-points are frequently purely drug centred and are based on heavily selected populations. The control arm may not, in fact, represent real practice, leading to the possibility of poor external validity, which doesn't adequately serve day-to-day patients and health professionals. Today's clinical research looks at optimal patient populations, drug combinations and sequences, and duration of treatment, **but a way needs to be found to re-engineer how to work together.**

The workshop heard about an EMA study which showed that, out of 48 cancer medicines approved between 2009 and 2013, only just over one-third showed a prolongation of survival. Attendees were told that the critical gap to be addressed, at the European level, is to understand how to move from drug-centred research to **patient- and society-centred research**, while also ensuring the interests of all stakeholders, especially on how to create a collaborative research space to make a quality change. Also raised was the need to break down silos in developmental phases and between research and care, with the latter two needing to be brought closer together.

Important was the intervention of Ewan Birney from the European Molecular Biology Laboratory (EMBL) talking about genomics and saying that in clinical practice, stratification can help with better diagnosis and prognosis, better use of medicines, such as in respect of personalised medicine, and with specific care pathways optimised for individual cases. The workshop heard that for the best stratification, four pillars are required. These are at-scale genomic assays, a clear legal basis to access appropriate data and approach patients, a very large virtual cohort, ideally with population scale ascertainment, and harmonised representation of key aspects of electronic health records (EHRs).

Finally, Wim Goettsch intervention on HTA stressed the importance of also social, economic and ethical, not just the medical assessment.

Research in healthcare is an ongoing discussion and nurses have always been vocal in this important field. The broad topic, and its many elements, is the subject of continuous engagement by the ENRF. Europe needs to be more proactive when looking at how best to bring innovation into healthcare systems, not least when it comes to interoperability and real-time data collection.

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