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Xavier De Cuyper of the Federal Agency for Medicines & Health Products (FAMHP), Belgiumâ??s drug regulator, explains the FAMHPâ??s pandemic response and lessons learned, how the Agency is keeping on top of the rapidly evolving technologies being brought forward today, and the significance of a new early access programme for medicines in Belgium.

The COVID-19 pandemic had a huge impact on all healthcare stakeholders, especially on regulators who were pushed to approve treatments and vaccines within faster timelines than ever before. How did the pandemic affect the way in which FAMHP operates and what lessons from this period are you still drawing on now?

From the start of the pandemic, we increased our internal collaboration and transversal discussions for a faster exchange of information between concerned colleagues, with clear action lists as a result. As regulators, we furthermore had to think outside the box to find practical solutions to different issues, and this was only possible by setting up a strong collaboration with our national

stakeholders, but also with EU colleagues. We are still building on those networks in our current work. There is a precedent in working according to even faster timelines in crisis periods â?? which will certainly help in a future health crisis. Furthermore, Belgium stays actively involved and even coordinates the CT Cure project which aims primarily to cope with expedited timelines concerning the evaluation of clinical trials for COVID therapeutics.

It is certainly important to learn lessons from COVID for crisis preparedness. We are working on this at a national level but also at an EMA level, for example by our collaboration in the ETF (Emergency Task Force). However, one should be careful in extrapolating the experiences during a crisis period to the way of working under normal circumstances.

Innovation has never moved so quickly with the industryâ??s innovation cycles continuously accelerating and covering a greater scope of unmet medical needs. How challenging is it as a regulatory body to stay on top of the cornucopia of therapeutic approaches and technological platforms being brought forward today?

This is probably one of the most important challenges for regulatory agencies.

We try to handle this by training our internal assessors, creating collaboration agreements with academic experts, working in multinational assessment teams and recruiting specific expertise where necessary. We are also collaborating on a number of initiatives that have the objective to increase the capacity of the EU regulatory network.

Moreover, the FAMHPâ??s National Innovation Office (NIO) is an active member of the European Innovation Offices Network (EU-IN), a joint working group chaired by the EMA and HMA. In this context the FAMHPâ??s NIO acts as a critical interface between the EU-IN and the internal FAMHP divisions, including the strategic unit, to share critical info regarding new scientific and technological evolutions, horizon scanning activities, best practices from other NCAsâ?? innovation offices etc. This should contribute to the FAMHPâ??s ability to develop a more proactive and more agile strategic and operational way of working.

The FAMHP also takes an active part in different EU activities and platforms/initiatives to stay at the forefront of research and innovation and by doing so, developing new expertise and competencies. For example, the FAMHP is actively participating in the Accelerating Clinical Trials in the EU (ACT EU) initiative which aims to develop the European Union further as a competitive centre for innovative clinical research

Last time we spoke, you highlighted your ambition to position FAMHP as a â??regulatory trailblazerâ?• in vaccines and early-phase clinical development. What progress has been made towards this goal in the intervening three years?

Both domains remain of special importance for the FAMHP.

However, due to the constraints posed by the pandemic, we were not able to elaborate a long-term strategy for vaccines as a spearhead domain. During COVID all efforts were directed to having safe and effective vaccines authorized and accessible as fast as possible. It was a period of many unexpected situations and planning was needed more on a day-by-day basis. Now we are again developing a strategy for the longer term, considering the lessons learned during COVID.

Attention for early phase clinical development is now built into our standard processes, for example in the handling of applications for clinical trials, scientific advice and technical-regulatory advice and compassionate use programs.

Furthermore, several innovation support mechanisms and guidance tools have been developed on an ongoing basis by the FAMHPâ??s NIO to foster and accelerate innovation and clinical research in the domain of, for example, vaccines and in particular for early phase clinical development

A significant medicinal policy reform is currently under discussion at the Ministry of Health and slated for rollout this year, one of the most important items of which is the implementation of a more effective early access programme. How do you see this new policy reshaping the Belgian regulatory framework?

We have two different processes regarding early access with different owners and responsibilities: the early temporary authorization (ETA) under the responsibility of FAMHP and the early temporary reimbursement (ETR) under the responsibility of the National Institute for Health and Disability Insurance. Currently, both processes are split even if we are talking with each other about these programs.

In addition, it is the FAMHPâ??s firm belief that not only should focus be put on â??Access to healthcareâ?• (e.g., at the reimbursement stage), but also to â??Access to innovationâ?• in the preceding stages (eg. early regulatory acceptance) and more particularly a more â??Need-driven & patient-driven access to innovationâ?•.

At the European level, we would like to share our knowledge regarding the definition and assessment of unmet medical needs, which should not be predominantly driven by pharmaceutical companies. Defining the EUâ??s patient needs and stimulating the development of products in these areas is a challenging topic.

How important is the implementation of this early access program, given that Belgium sits in a lowly 23rd place in Europe in terms of average wait time between a drugâ??s EMA approval and national-level reimbursement?

The time needed to receive reimbursement after a positive opinion from CHMP is indeed high but regarding approvals with a high unmet medical need, this is being compensated by an efficient ETA process within 55 days.

In the reform, a distinction is made between early access, i.e., before CHMP approval, and fast access, i.e., after CHMP approval. Regarding early access, the FAMHP has already well-running procedures for handling applications for clinical trials, scientific advice, compassionate use programs and medical need programs. Fast access is more related to the activities of our colleagues at the National Institute for Health and Disability Insurance since it is related to the faster start of the reimbursement procedure for medicines addressing an unmet medical need.

What do you see as FAMHPâ??s role in helping Belgium maintain its position as one of Europeâ??s leading countries for clinical trials? Does the rollout of the EU Clinical Trials Regulation and a unified European trial database risk damaging this positioning?

The FAMHP intends to maintain Belgium as one of Europeâ??s leading countries for clinical trials. The CTR rollout certainly has an impact on clinical trials in Europe and Belgium. But we have shown in the previous years that we were preparing well due to our extensive pilot project for clinical trials, which paid off in the first year of CTR. Our processes are clearly established. We are one of the leading member states when it concerns clinical studies for which Belgium is requested to take the prominent role in its evaluation as RMS (reference member state).

We have competitive timelines, certainly for mono-national phase 1 clinical studies.

We also aim to collaborate and inform our stakeholders, including sponsors as well as possible and look for solutions together in case of start-up issues.

We keep a strong and close collaboration with our scientific advice department and with the College which coordinates with the ethics committees allowing us to follow up closely and proactively discuss potential issues.

The FAMHPâ??s NIO is also strongly interfacing with all stakeholders involved in the priority action PA7 under the ACT-EU Initiative (eg. CTCG, SAWP, EMA, HMA) to pilot and implement phase 2 of the European project on Simultaneous National Scientific Advice (SNSA). The SNSA pilot has a specific focus on, but is not limited to, multinational scientific and technical/regulatory advice (also called â??pre-CTA adviceâ?•) to facilitate and accelerate multinational clinical trials within Europe to the maximum extent possible. It facilitates sponsors and developers to obtain simultaneously clinical trial-related national scientific advice from multiple NCAs in the Member States where they intend to perform clinical trials under CTR, and as a consequence, the SNSA concept can increase significantly the clinical trial assessment and approval. Also in this domain, the FAMHP intends to keep its leadership position in Europe and to translate the experiences gained during the SNSA pilots to the benefit of both innovators as well as patients (eg. by gradually engaging both ethic committee experts and patient representatives in such SNSA procedures).

The FAMHPâ??s NIO also continues to develop and offer new types of early dialogue tools, support mechanisms and incentives to the specific needs of innovators in order to facilitate clinical research and innovation, e.g. portfolio meetings, project info meetings (PIM), pre-STA submission guidance, STA fee reductions/exemptions, etc.

Belgium recently moved to create a Health Data Agency of the kind that already exists in some other European countries, which industry sponsors are hoping will lead to greater levels of RWE utilisation, allowing promising medicines to prove their value and fostering higher levels of certainty throughout the entire process. What is your take on the potential impact of this move?

Big data, data analytics and RWE are probably some of the fastest-evolving fields at the moment. This includes efficient collection of relevant data during the development and use of the medicines, making these data accessible in an appropriate way and using often complex methodological tools for the analysis of the data. The FAMHP is currently developing a plan to coordinate this domain in a transversal way and establishing collaborations with external experts.

What is your final message for PharmaBoardroomâ??s international audience?

The FAMHP must guarantee its mission in a rapidly evolving technological, scientific and regulatory context. Timely support from all the different stakeholders in the Belgian healthcare ecosystem is

necessary to secure the FAMPâ??s required resource and expertise needs. This will allow the futureproofing of the FAMHP and maintain a key role for the agency within the European Regulatory Network for the benefit of the patient and healthcare providers.

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