

THE TOP 5 ISSUES IN MEDICINES POLICY FOR 2019 WHAT WILL DOMINATE THE AGENDA THIS YEAR

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1. THE FUTURE OF PHARMACEUTICAL POLICY IN THE NEW EUROPEAN COMMISSION

The fate of the Health and Food Safety Directorate General (DG Santé) and in particular that of pharmaceutical policy within the next European Commission are key uncertainties. We expect the new European Commission to be (even) more political than the current one. This means two things: a) We expect very few new pieces of legislation will be initiated by the Commission. If any, the European Commission will be inspired by and aligned with the aspirations of the most influential EU Member States in drafting any new legislation, b) if the predictions about the results of the European elections are confirmed and the European Parliament shifts further to the right, the new Commission with its limited scope and mandate might be more favourable towards business priorities and interests.

How will these developments affect decision-making in medicines' policies? In recent years, DG Santé's leadership kept sending mixed signals about its priorities. Instead of defending public health, Santé high-ranking officials would often sound more like representatives of DG GROW, the Commission's department responsible for EU policy on the single market, industry, entrepreneurship and small businesses, and they would repeat the usual pro-business arguments around the need to reinforce the pharmaceutical industry's competitiveness. For the time being, it seems that DG Santé will continue to exist in one form or another within the structure of the next Commission. Even if pharmaceutical policy remains under the remit of DG Santé, there are concerns its mandate will be eroded as different chunks will be split and shared across various DGs in the spirit of the so-called "synergies" approach, possibly under a newly-created Vice-President post. If this is the case, it will be important to ensure that the emphasis on pro-public health policies is treated equally to the rest of the areas under the new heading.

WHAT TO EXPECT IN 2019?

The issues of affordability and access to medicines will remain high on the political agenda and the



European Commission will not be able to dodge them, not least due to industry's aggressive pricing strategies which continuously grab headlines across the Union. Since the launch of Sovaldi with its paralyzing price tag and the political acknowledgement of the imbalances in the pharmaceutical systems via the [groundbreaking](#) June 2016 Dutch Council Conclusions, there has been a wide-ranging debate on access to medicines. Predictably, these disruptive developments have also mobilised industry forces. Nonetheless, industry lobbyists and their supporters have failed to regain control of the debate. Recent EU Presidencies, such as those of the Bulgarians and the Austrians, have followed in the footsteps of the Dutch keeping the pressure on the Commission to more boldly address the imbalances in the pharmaceutical systems.

This time, the most important legislative initiatives in the field of pharmaceuticals were launched during the Commission's final year in office, but they were at least five years in the making. Therefore, 2019 is of utmost importance in shaping what the Commission will work on in the next five years. The incoming Commission will have at its disposal a series of data-rich studies such as the intellectual property (IP) [incentives review](#), the Report on [Competition enforcement](#) in the pharmaceutical sector (2009-2017), and the orphan drugs study (coming soon). Member States who are concerned by the threat high prices of medicines pose to the sustainability of health care systems will need to guarantee the European Commission's priorities and initiatives are not skewed by the pharmaceutical companies towards harmful deregulation in the disguise of innovation promotion.

The public health and affordability arguments should not get lost in the onslaught of corporate lobbying.

2. THE FUTURE IS NOW: BENELUXAI AND OTHER REGIONAL INITIATIVES

In the coming months, we will see more products successfully negotiated via Beneluxai. The Valletta Group with all its shortcomings will slowly catch up and learn from the mistakes and successes of its northern neighbours. Truth be told, governments do not have much else at their disposal to tackle the issue of affordable access to meaningful innovation. Differently put, Beneluxai et al. are above all pragmatic, result-oriented, political choices for national policy-makers who are trying to address and mitigate the effects of a series of imbalances that undermine their negotiating power towards the pharmaceutical companies. In joining forces, governments are essentially learning from the pharmaceutical way of doing business, imitating the way pharmaceutical companies operate across different countries with their boards.

WHAT TO EXPECT IN 2019?

The successful negotiations on Orkambi (May 2017) and Spinraza (August 2018) are promising but more transparency is imperative, as public information on the details of the deals (or non-deals) is limited. In spite of their obvious significance, it is crucial that these initiatives are not reduced to simple



Turkish-bazaar type discount mechanisms, or become scapegoats for Health Ministers who wish to justify their inactivity or negative reimbursement decisions.

Instead, participating governments have a unique opportunity to decisively redefine the rules of the game and to reset the equilibrium via these regional initiatives. The launch of the International Horizon Scanning Initiative (IHSI) as a Beneluxai spin-off is indicative of the versatility and the evolving nature of these experiments. Indeed, everyone involved in these collaborative initiatives is [learning by doing](#). And, we should not forget that Beneluxai and the Valletta Group were only born in 2015 and 2017 respectively. It will take at least five years for these initiatives to demonstrate their full potential and benefit from the groundwork currently being undertaken.

The recent attempt by the European Federation of Pharmaceutical Industries and Associations (EFPIA) to [dictate](#) how governments should work together with the publication of the “Policy Principles on Cross-country Collaborations on Medicines’ Pricing and Access” speaks volumes about industry worries. Notwithstanding the dismissive tone of the article over the achievements of the cross-country collaborations so far, one can read between the lines, the industry’s agitation and discomfort as it sees its traditional “divide and conquer” policies being defied by EU governments. The pharmaceutical sector has long benefited from the power and information asymmetries which are now being addressed by these regional initiatives. Governments can no longer afford not to join forces if they want to rebalance market dynamics, ensure a level playing field and increase their leverage.

The industry should not be scared. There is a clear business case for them, particularly the smaller pharmaceutical companies who unlike big pharma do not have the means nor the capacity to enter markets in Europe. Companies with truly advantageous therapeutic products have nothing to worry about. The regional initiatives are therefore good for patients and will encourage healthier competition in the market.

The culture of collaboration is at the foundation of these regional initiatives and is also their greatest added value. The difficulty of establishing a common language of cooperation between civil servants and health systems in different countries should neither be underestimated nor taken for granted. This is invaluable especially in light of the limited resources and manpower Member States have in comparison to the infinite resources of the industry. In this respect, these “coalitions of the willing” create a sense of belonging and community for governments who feel weak on their own but stronger together. That said, it remains to be seen whether these experiments in policy-making will result in ad-hoc or systemic change.

Overall, they are game-changers and are here to stay.



3. THE POLITICAL CARTE BLANCHE OF THE EUROPEAN MEDICINES AGENCY (EMA)

It is not an exaggeration to say that the European Medicines Agency has been operating with a political carte blanche since its establishment in 1995. However, the increasing burden on health care budgets because of the high prices of some medicines has put the spotlight on the regulator's responsibilities in this debate. Unquestionably, the Agency remains one of the EU's crown jewels, but that should not prevent us from critically examining the course the Agency has steered over the past 24 years.

The EMA is a powerful global actor which, over the years, has developed an ever-closer partnership with the pharmaceutical companies it is supposed to regulate. Its growing authority and influence has, by and large, gone unnoticed by EU governments until the high prices of medicines started to bite. Pharmaceutical companies have been more strategic, prioritising and investing significant resources in fostering their partnership with the Agency since its inception. They regard the EMA as a very useful Secretariat which brings together the right people who can influence the moment they can start making money, through the granting of marketing authorisations. However, while companies grew closer to the top EU regulator, in most capitals, the Ministries of Health, Health Technology Assessment (HTA) Agencies, national medicines agencies and the payers grew farther apart. These silos left little space for mutual understanding and contributed to a lack of scrutiny, made even worse by competing priorities.

This is only now starting to change. The first ever meeting between the EMA and the payers' representatives took place only in September 2017. At last September's informal health council, the Austrian Presidency summoned Guido Rasi, the Executive Director of the EMA for a rare exchange of views with EU Health Ministers on the shortcomings of the EU medicines' approvals system and its implications for access and affordability. The problems due to the push for accelerated approvals (exemplified by initiatives such as the highly [controversial](#) adaptive pathways school of thought) and the use of the incentives by pharmaceutical companies for profit maximization in the field of orphan drugs for rare diseases were raised by several delegations, among other topics. The recently published, [must-read](#) WHO technical study on the pricing of cancer medicines and its impacts robustly reveals what governments are worried about: we have been paying too much for too little meaningful innovation. It is self-evident that regulators like the EMA and the U.S. Food and Drugs Administration (FDA) have contributed to this impasse; but they can help fix the situation.

WHAT TO EXPECT IN 2019?

During 2019, we will see the conclusion of the [European Ombudsman's own strategic inquiry](#) into the pre-submission activities or as Emily O' Reilly describes "the arrangements which the EMA has in place for engaging with individual medicine developers before the Agency receives applications for marketing authorisations from them". Currently, we know close to nothing about what is being discussed between



the regulator and the companies during this critical stage of drug development making it, so to speak, one of the black boxes of the Agency's processes. It is unfortunate that the European Ombudsman has had to intervene to shed some much-needed light into this critical phase of drug development, when the EMA itself ought to have proactively taken measures to promote transparency and public accountability of its activities.

The hope is that the Agency will eventually take on board and implement most of the Ombudsman's recommendations. This will undoubtedly shed light on the exchanges between the pharmaceutical companies seeking approval for their products and the regulator during the pre-marketing authorization phase and will be a step forward towards more transparency and public scrutiny in the provision of scientific advice from the regulator to the companies involved (see EPHA's own [recommendations](#) on how to achieve this).

Although 90% of its budget is financed by private sources (industry fees) the EMA is a public authority. As such, patients and consumers have a right to know what the regulated, i.e. pharmaceutical companies, are asking the Agency and what the Agency's answers are. As it is critical that citizens maintain their faith in the marketing authorisation system in Europe, the perception of the Agency's independence and integrity are as important as the reality itself. Therefore, it is the Agency's responsibility to proactively dispel any fears about regulatory capture.

The pharmaceutical sector is a highly secretive business but the industry should not be fearful of greater transparency at the EMA, unless they feel that the pre-submission activities yields them greater access and influence over the final decisions of the top EU regulator.

Taking the concerns expressed by Health Ministers at last September's informal council into account, the Agency's leadership team should change course and resist the pressure to move towards a regulatory environment which might lead to [a de facto lowering](#) of the evidentiary standards. To this end, the adaptive pathways school of thought, warmly [welcomed](#) by the industry, should not be revived. The approvals system in Europe is already flexible enough and further flexibility and new approval pathways will equal a euphemism for deregulation. Meanwhile, the payers will have their second meeting with the Agency in June. Should they wish to be listened to they will need to be more vocal and united in their requests towards the Agency.

It is up to governments to remind the EMA as well as their national medicines agencies that they are primarily regulators who are supposed to regulate and defend public health and patients' interests. They are not there to boost the competitiveness of those they are regulating. Thus, a mindset shift is necessary - one that treats pharmaceutical companies as a business sector in need of regulation and not as clients or partners, as they are currently viewed.



Nevertheless, after 24 years of pharmaceutical lobbying, silos and lack of political supervision, it will be a challenging mindshift, requiring political leadership and concerted action to revert this carefully plotted course.

4. HEALTH TECHNOLOGY ASSESSMENT (HTA): THE FIGHT IS NOT OVER YET. OR IS IT?

Much has been said on the regulation on HTA collaboration which the European Commission presented exactly a year ago. Our [predictions](#) over the thorny issues and the non-conclusion of the negotiations proved to be accurate. HTA is at the heart of national pricing and reimbursement decisions. Billions of euros are at stake, no matter how hard some attempt to frame the HTA process as a mere technical-scientific exercise.

One year later, there seem to be two dominant schools of thought in the Council: a) those countries who are confident about their national HTA systems and fear that the EC proposal will undermine them and b) those who are optimistic that the EC proposal on a new EU-wide HTA system will strengthen their capacity and HTA in general. The threat to the sustainability of their health systems posed by the high prices of some new medicines is the common denominator for both groups of countries. All are alarmed by this and are turning to HTA, hoping it will act as one of the gatekeepers in their systems. Most governments are also sceptical about handing over more power to the European Commission over this sensitive area of national competence. For the time being, it seems that the former school of thought is prevailing. It is also worth noting though that even in that first club of countries, HTA representatives are aware of their own shortcomings and limitations; they are under no illusion that they are not “winning the war” with the pharmaceutical companies, irrespective of how robust their national HTA systems are. To put it another way, they know they are losing but they are afraid that they will have much more to lose if they go down the path foreseen in the proposed EC regulation.

On the bright side, the HTA discussion has underlined the need to assess medical innovation and to address the problems caused by the lax standards of approvals endorsed by the EMA. HTA agencies (as well as pricing and reimbursement authorities) are regularly faced with the dilemma of having to assess and give negative opinions on medicines approved by the EMA on the basis of premature and/or incomplete data. This scarcity of evidence prevents them from doing their job properly as they do not have the information they need. The ongoing discussions under the Romanian Presidency in the first half of 2019 will focus on the less politically controversial aspects of the proposed regulation. It will be interesting to watch how these technical discussions contribute to giving HTA “more teeth” by raising the bar on the evidence required. This would mean that medicines’ developers aspiring to obtain a favourable appraisal by HTA would have to answer more questions and disclose more and better clinical trial data to prove that their products are true therapeutic advances.



WHAT TO EXPECT IN 2019?

Not much. The Romanian Presidency will continue the technical negotiations while the Finnish have expressed their intention to keep the issue on the agenda. The big question is what the priorities of the new Commission will be. The level of opposition to the proposal during the past year has been shockingly fierce, with the eventful June 2018 Health Ministers Council under the EU Bulgarian Presidency marking the red lines of numerous Member States quite early on in the negotiation process. The EU Austrian Presidency worked hard to reconcile the opposing views, trying to appease and lure sceptical Member States by putting forward a compromise proposal which gave a very limited role to the European Commission. That did not fly either. Immediately after the departure of the delegations from the concluding EPSCO Council in December 2018, the Ministers of the countries spearheading the opposition camp co-signed a letter emphatically reiterating that the core provisions of the EC-proposed Regulation are not acceptable.

Taking into account the June 2018 EPSCO, the December 2018 letter and the unwavering scepticism in the Council, one cannot exclude the prospect of the proposal being completely withdrawn by the next Commission. Should this be the case, the ball will be in the court of the sceptics to propose a credible alternative, one that guarantees that collaboration in the field of HTA in Europe continues beyond the expiry date of the current [Joint Action for EUnetHTA](#). So far, we have heard much about the problems with the Commission's vision but very little on what another way forward could look like.

As far as the pharmaceutical industry is concerned, they are happy with the status quo and the fragmented HTA landscape. They are used to working with even the "toughest" HTA agencies and they can navigate around them. This is why the industry has yet to mobilise its full lobbying resources around this file, preferring to let governments fight it out instead. Their best case scenario from the EC proposal would be an EU-wide HTA set-up, resembling the EMA or even better becoming one of its subdivisions - a prospect which at this stage enjoys very little support in the Council and beyond. In [our view](#), such a prospect would be disastrous.

Should the proposal survive, negotiations will most likely be concluded during the German Presidency of the EU in the second half of 2020. [EPHA's recommendations](#) on how to improve the proposed Regulation remain timely. One thing is certain; the end product will bear very little resemblance to the initial proposal launched by the Commission last January.

5. NO COMPROMISE: THE LOBBYING STRATEGY OF PHARMACEUTICAL COMPANIES IN EUROPE

The pharmaceutical companies' lobbyists were taken by surprise by the disruptive Dutch Presidency and the lasting effect of the [June 2016 Council Conclusions](#). For the first time, the industry lost control



of the debate and found itself engaging in damage control and having to provide real answers at a European level. Their top priority since then has been to ensure that “the Dutch fiasco” –in their eyes- is not repeated; and that the Dutch Conclusions and the processes they triggered will be weakened and/or quickly forgotten. In the meantime, the comprehensive and heated debate around pricing in Europe along with developments such as the intellectual property (IP) incentives review, the birth and growth of the regional intergovernmental initiatives has attracted the attention of Pharmaceutical Research and Manufacturers of America (PhRMA), EFPIA’s far more powerful sister organisation based in the U.S.

In terms of the alarms that went off on the other side of the Atlantic, it is worth remembering that it was February 2018 i.e. three months before the Commission’s actual proposal on the possible introduction of a Supplementary Protection Certificate (SPC) waiver was even out, when [PhRMA](#) and the Biotechnology Innovation Organisation ([BIO](#)) in an overtly threatening move, requested that the EU was added to the U.S. Trade Representative (USTR) watch list (Special 301 Report). They effectively called for the EU to be [blacklisted](#) due to the (still ongoing at the time) IP incentives review (requested by Health Ministers with the June 2016 Council Conclusions) and the introduction of an SPC manufacturing waiver (the European Commission finally [published](#) the proposal amending Regulation (EC) No 469/2009 concerning the supplementary protection certificate for medicinal products in May 2018).

WHAT TO EXPECT IN 2019?

As we [noted](#) last January, since mid-2017 we have been observing a steady escalation of the pharmaceutical industry lobby’s fight back in Brussels and the EU capitals, encouraged by PhRMA. The tone of recent and multiple EFPIA publications on, for example, the [SPC waiver](#) or the [regional intergovernmental initiatives](#) such as Beneluxai has been overtly belligerent, firing warning shots across the bow. It is now crystal clear that PhRMA/EFPIA member companies have chosen to show their teeth and are not being at all shy about it. “No compromise” also known as “take no prisoners” is the new lobbying strategy in Brussels and around Europe, irrespective of the official calls by EFPIA for “partnerships”.

They are baring their teeth at the next Commission, national decision-makers, at critics of the current pharmaceutical business model as well as at business competitors. The animosity between EFPIA and Medicines for Europe (the generics and biosimilars trade association) is expected to culminate in March when the final vote on the SPC waiver takes place in Strasbourg. Much can change in the coming weeks, but for now, it seems EFPIA might be (partially) defeated on the waiver battle. Looking back, perhaps the reason EFPIA chose to drive this surprisingly public debate to the extremes from the start and to set the stakes so high over this relatively insignificant - from the originators’ perspective- topic is to issue a warning to both the new Commission and the generics and biosimilar manufacturers



not to push for a legal review of the SPC regulation, a much more fundamental legislative file than the waiver. This will be decided by the next Commission. It remains to be seen if the generics lobby will be dissuaded from pursuing that path; but should the path of confrontation be chosen over a backroom deal, the fallout over the SPC waiver will prove to be nothing but a sneak preview of a full-scale war between the two sides of the industry.

At the end of the day, the protagonists within EFPIA should remember that no matter how tough they play, their profit in Europe comes from public sources. That should be enough to remind them to remain humble, to revisit their strategy and to come up with better arguments.



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