

# EPHA #A2MDialogues

Report on EPHA's new series of online discussions putting the spotlight on actionable solutions on European medicines policy in the European Union



## About the European Public Health Alliance

The European Public Health Alliance (EPHA) is a change agent – Europe's leading NGO alliance advocating for better health. We are a dynamic member-led organisation, made up of public health civil society, patient groups, health professionals, and disease groups working together to improve health and strengthen the voice of public health in Europe.



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# Introduction

In October 2020, EPHA launched the [#A2MDialogues](#), bringing together thought-leaders and policy-makers, academics, industry representatives and NGOs, for a frank discussion of European pharmaceutical policies and key access to medicines (A2M) priorities.

The following discussions took place during 2020:

19 October: [Unleashing meaningful innovation through regulatory reform](#)

27 October: [The EU's IP strategy: Enabler or barrier?](#)

19 November: [Pharma & COVID-19: Winners, losers, prospects](#)

25 November: [The Presidencies' perspective on the pharmaceutical strategy: Europe's to-do list on access to medicines](#)

8 December: [Getting it right: Recommendations for a European BARDA](#)

The European Public Health Alliance (EPHA) #A2MDialogues series of online events brought together key stakeholders and influential decision-makers to comment on the latest developments in pharmaceutical policies (access to medicines, A2M) in Europe and offered insights and recommendations into the shaping of future EU policy initiatives.

The poster for the EPHA #A2M DIALOGUES event features an orange background on the left with white text. It includes the event title, date, time, and topic. On the right, there is a background image of laboratory glassware with two circular portraits of speakers overlaid. Each portrait is accompanied by the speaker's name and title in an orange box.

**EPHA #A2M  
DIALOGUES**

**8 December  
16:00-17:00 CET**

**Getting it right:  
Recommendations for a  
European BARDA**

europaen public health alliance

Marie-Paule Kieny  
Director of Research  
INSERM

Michel Goldman  
Founder, I3h Institute  
Former IMI Executive Director

The main elements of the future Health Emergency Response Authority (HERA), to be proposed by the end of 2021, by the European Commission were presented during the December 2020 episode of EPHA's A2MDialogues: Getting it right: Recommendations for a European BARDA.

Such a structure would be an important new element to support a better EU level response to cross-border health threats. The COVID-19 pandemic has shown that Europe has paid the price of not being prepared in the biomedical research & innovation (R&I) front. It has demonstrated that the EU did not have adequate tools to support late phase clinical development and manufacturing capacity ramping up of innovative vaccines and other medical technologies.

EU research programmes are certainly welcome but experience shows that they are not flexible enough and cannot be adapted easily in times of crisis. The guest speakers agreed that HERA is a great opportunity to build on the excellent European science, to learn the lessons from the ongoing crisis and ensure that the public acts as a wise investor which steers meaningful, public health needs-driven innovation.

There was agreement that HERA should be a purely public organisation with a clear public health mission. Its mandate should not be conflated with areas of industrial policy. It should have a sizable budget which will provide for independent long-term planning. In acting as wise investors, governments should be ready to invest significant amounts of public money. Both speakers agreed that national governments should be mindful of the fact that not all R&D projects will come to fruition and some of them will not deliver the desired results. Put simply, the possibility of failure and the financial risk should be endorsed from the start, especially, as HERA will invest in risky competitive projects including the boosting of manufacturing capacity.

There was consensus that the new Agency will have to be independent, sustainable and protected from political pressure and evolving political priorities jeopardizing the continuity of its work. It should address the current lack of coherence between EU and national funding schemes and ensure that discoveries made with the support of EU funds will be translated into large scale industrial development across the EU.

HERA should have enforceable rules on Open Access (i.e. data sharing) following Horizon Europe. HERA should build on - but go beyond - existing access initiatives (e.g. the Medicines Patent Pool) and develop more transparent and efficient incentive mechanisms that de-risk private sector activity but at the same time guarantee universal access and public return on public investment.

Speakers agreed that its governance structure should be transparent and balanced, including both patient and public health organisations, as well as representatives of the research community. Whilst the industries will be important partners, they should not be part of any governance structure of this new public organisation. The definition of unmet needs will be done by the public health sector only and the goal will be to engage in development of new products to bring them to the market. This means that HERA will go well beyond the Innovative Medicines Initiative (IMI) which was limited to the pre-competitive phase only. It also means that affordability, availability, accessibility, socially responsible licensing and transparency conditions will be attached to the end products to reflect the substantial and multifaceted public support and investment. To this end, reasonable pricing clauses should be envisaged.

Michel Goldman, the first Executive Director of the Innovative Medicines Initiative, said that the EU was supposed to be prepared, pointing to various programs across the bloc researching the first SARS virus thanks to EU research dollars. The issue is translating the great research from academics and small biotechs into the kind of large scale manufacturing needed during a pandemic.

***"All [the] science is certainly excellent, in my view as good as in the U.S. All regulatory agencies are excellent. ... But there is something lacking in terms of bringing the products rapidly to the citizens and to the patients."***



HERA will not only need to coordinate with the EMA and the European Centre for Disease Prevention and Control, but also will have to integrate and streamline efforts throughout the value chain from basic research to large-scale manufacturing and distribution, across public and private sectors. Building of vaccine manufacturing facilities that are on standby to be mobilised in response to emerging infectious threats should be considered, in light of the AstraZeneca vaccine debacle which has proven that rapid vaccine production at scale is a major challenge. At present, the landscape of EU research funding instruments is quite fragmented (IMI, Horizon Europe, EIB, EU structural and investment funds to name but a few) and insufficiently driven by the principle of public return on public investment. This is inefficient and counterproductive, particularly in times of crisis. HERA can be instrumental in aligning means with priorities with a clear legal framework, a substantial, sustainable budget and a strong leadership for a new independent and autonomous agency with a clear public health mission.





Marie-Paule Kieny, Director of Research, INSERM, reminded viewers that the U.S. BARDA (Biomedical Advanced Research and Development Authority (BARDA) which inspires the creation of the EU HERA is not a public-private partnership like IMI, Kieny noted. It's a public agency, with a whole bunch of money that coordinates well between a host of other U.S. agencies involved in health and research. This is what the EU should do as well.

The issue is whether EU countries want to put the money into it, and here Kieny was not optimistic, considering how they have rarely followed the Commission's guidance throughout the pandemic. Michel Goldman on the other hand, commented that private money is not necessarily a problem provided there's transparency - which he admits has been an issue with IMI. "There is nothing wrong in investing public money to provide the means, the incentives to share risks together with the private sector," he said.

**EPHA #A2M DIALOGUES**

**Unleashing meaningful innovation through regulatory reform**

**19 October**  
**17:00-18:00 CET**

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Beate Wieseler,  
Institute for Quality and Efficiency  
in Health Care (IQWiG)

Huseyin Naci  
London School of Economics  
and Political Science

Andrea Cipriani MD PhD  
University of Oxford

Lydie Meheus PhD  
Belgian Anticancer Fund

Speakers agreed that regulators should routinely inform patients and clinicians about what is and is not known about the benefits and harms of new drugs at the time of approval. In addition, regulators should proactively encourage companies to harmonise the designs of clinical trials within each therapeutic area. If trials share key design features, their utility for health technology assessment bodies and payers would be substantially improved. The European Medicines Agency should routinely require individual participant level data on clinical trials supporting its approval decisions, and allow re-analysis of this data by a pre-defined set of third-party organisations.

The issue of regulatory reform was highlighted during another of EPHA's access to medicines dialogues ("Unleashing meaningful innovation through regulatory reform") in light of the ongoing policy discussion on the extension of the mandate of the European Medicines Agency (EMA).

UNIVERSAL ACCESS AND AFFORDABLE MEDICINES

**RECOMMENDATIONS:**

**UNLEASHING INNOVATION THROUGH REGULATORY REFORM**

**EPHA #A2M DIALOGUES**

RECOMMENDATIONS PRODUCED FOLLOWING THE ONLINE DISCUSSION | 19 OCTOBER 2020

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As on the topic of HERA, the aim of the session was to articulate a set of recommendations, which can be downloaded [here](#)

Indeed, the quality and paucity of data have been a problem for clinical trials of new cancer drugs lately, said Lydie Meheus, Managing Director of the Belgian Anticancer Fund. She explained that the gold standard here would be studies taking overall survival and quality of life as their endpoint. But instead, many are looking at alternative metrics like progression-free survival, or even just patient response rate. And very innovative treatments sometimes are able to nab conditional approval, pending more data, in single-arm studies with no control comparator arm, she added.



Often more than one drug is developed in parallel in a given indication. Typically, each drug is investigated in isolation, missing a key opportunity to efficiently evaluate multiple drugs simultaneously. For indications with small patient populations, EMA, with input from European Health Technology Assessment bodies, should establish platform trials and require that suitable drug candidates are tested in this environment rather than in isolated insufficient trials. This approach could increase clinical trial efficiency and ensure early generation of comparative evidence to support decision making also for patients in rare diseases. On the issue of conditional marketing authorisation, when drugs are conditionally approved on the basis of limited data, post-approval, randomised trials should be routinely required to address those limitations. Currently, post-marketing studies are not designed to address the limitations of the evidence base at the time of marketing authorisation.



Beate Wieseler, Head of the Drug Assessment Department at Germany's Institute for Quality and Efficiency in Health Care (IQWiG), said that too many times that data on which the conditional approval was meant to depend arrives late or is inconclusive. After all, once drug companies get their drug on the market, the leverage to get them to conduct more studies is greatly diminished. Pricing here can help, Wieseler explained, with a new law

allowing for lower prices for approved medicines if the data isn't good enough.

In the post-marketing period, manufacturers should design their studies hierarchically: priority should be given to studies aimed at evaluating a product's net clinical benefit in randomised trials compared with current known effective therapy. At present, manufacturers have no incentive to invest in research to demonstrate the added therapeutic benefit of their products in the post-marketing period. Much of post-marketing research focuses on seeking new indications.





**Neha Dave**     
@NehaDave



"Companies need to standardise their approach to [#clinicaltrials](#), we have the tools, methods in place." Says [@Huseyinaci2](#) [#a2mdialogues](#) [@EPHA\\_EU](#)



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The role of payers was subsequently highlighted. In the guest speakers' view, payers should use their policy levers and negotiating power to incentivise the generation of better evidence on new and existing drugs, for example, by explicitly considering proven added benefit in pricing and payment decisions. Today, there is no clear association between drug prices and demonstrated therapeutic benefit. Health Technology Assessment bodies and payers across Europe should routinely disclose information on the comparative benefits and harms of new and existing drugs and whether there is proven added benefit. To this end, speakers called on governments to directly support and facilitate the production of comparative post-marketing data by investing in the development of collaborative research networks and data systems that reduce the

complexity and cost of rigorous randomised trials in the post-marketing period.

The gold standard remains randomized control trials, concluded Huseyin Naci, Assistant Professor of Health Policy at the London School of Economics and Political Science. He pointed to coronavirus treatments that originally had shown promise in observational studies as one example:

***"We're seeing now with COVID that if it wasn't for randomized control trials we'd have been misled."***





## EPHA #A2M DIALOGUES

**The Presidencies' perspective on the pharmaceutical strategy: Europe's to-do list on access to medicines**

**25 November**  
**16:00-17:00 CET**

european public health alliance



Rui Ivo Santos  
President INFARMED,  
Portugal



Momir Radulovic  
Executive Director  
Slovenian Medicines Agency



Marcel van Raaij  
Director Medicines & Medical Technologies  
Dutch Ministry of Health

EPHA's A2MDialogues also focused on topics of intellectual property reform and biomedical R&D as well as presenting the access to medicines priorities of Portugal and Slovenia as the countries taking over the Presidency of the EU during 2021. Member States officials commented on the EU Pharmaceutical Strategy at the EPHA online event, which took place on

the same day it was adopted. They welcomed the strategy as a useful inventory of action and as an important milestone in coordinating EU work in this area.

The secure supply of medicines will be a key priority for the Portuguese Council presidency said Rui Ivo Santos, the president of Portugal's drug agency Infarmed (*pictured top right*).

Santos said he fully supported the Pharma Strategy's goal of ensuring adequate availability of drugs, a goal also wholeheartedly supported by Momir Radulovic of the Slovenian Medicines Agency (*pictured top left*). Marcel van Raaij, (*pictured bottom left*) Director of Medicines and Medical Technologies at the Dutch Ministry of Health, said there are plenty of easy targets for the Pharmaceutical Strategy when it comes to improving legislation, pointing to the orphan drug regulation as one example where there was the opportunity to make the rules less burdensome on the industry.



**Marcel van Raaij**  
@MarcelvanRaaij

Invited by @EPHA\_EU, I was happy to discuss the pharma strategy of the EC together with Momir Radulovic and Rui Ivo Santos @INFARMED\_IP @AgencijaJAZMP. Thanks @YNatsis for leading the conversation.



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**Devaliere Alienor**  
@AlieDevaliere

Council conclusions are to be expected under the Portuguese EU Presidency building on the [#PharmaStrategy](#) [#A2MDialogues](#) [#MakeltHappen](#)

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All of the 2020 EPHA #A2M Dialogues can be viewed on EPHA's [youtube channel](#). The series will continue in 2021 - register for the latest event [here](#)

## Resources

[Recommendations from the event "Unleashing Innovation through regulatory reform"](#)



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