**Academy of Medical Sciences' response to the open consultation on the MHRA's Proposed Patient and Public Involvement Strategy**

**June 2021**

## Question 1: We are committed to improving the way we engage and involve patients. Do you consider that our overall approach in the strategy will deliver this, and if not please tell us why?

The Academy of Medical Sciences ("the Academy") promotes advances in medical science and supports efforts to see these advances translated into healthcare benefits for society. We are pleased to have collaborated with and supported the work of the MHRA on several occasions, most recently at a joint roundtable on *Advancing regulatory science for innovative medical products*.[1] We welcome this opportunity to further contribute to the work of the MHRA through this consultation on its draft Public and Patient Involvement (PPI) Strategy. Our response is based on our previous policy reports, as well as input from: members of our elected Fellowship, which includes some of the UK's foremost experts in academic and clinical medical research; grant awardees; and patient representatives - hereby referred to as 'our experts'.[2]

As emphasised in the report of our recent FORUM event with the AMRC, ABPI and NIHR, *Public involvement and engagement in research during the COVID-19 pandemic*, the pandemic offers an opportunity to make effective and sustainable changes to how PPI is conducted throughout the R&D pipeline.[3] Our previous policy work and FORUM workshops have identified numerous advantages of conducting PPI across the medical research landscape, namely to ensure that research meets patient needs; to enhance the quality, relevance and effectiveness of research; to build trust in science and medicines; to raise awareness of the players involved in generating medical evidence; to improve how research is prioritised, communicated and utilised; to identify new avenues for research; to accelerate the adoption of innovation; and to raise awareness of the regulatory process.[4,5,6]

The Academy applauds actions taken by the MHRA to adopt a more systematic approach to embedding the patient and public voice in its work. The PPI Strategy shows a strong commitment to improving how the MHRA approaches the use of PPI and to expanding the diversity and breadth of perspectives that input into MHRA decision-making. However, we believe that there are opportunities for improvement both in terms of the high-level objectives, messages and priorities that run through the Strategy, and the more specific actions required to deliver the Strategy.

We first list suggestions related to the Strategy as a whole and then make more specific comments under the five strategic objectives. Additional actions and challenges to be considered that do not relate to the existing content are included in the response to question 2.

### Recommendations related to the strategy as a whole

[1] Academy of Medical Sciences (2021). *Academy of Medical Sciences FORUM and MHRA joint roundtable on Regulatory Science.* https://acmedsci.ac.uk/more/events/academy-of-medical-sciences-forummhra-joint-roundtable-on-regulatory-science-advancing-regulatory-science-for-innovative-medical-products https://acmedsci.ac.uk/file-download/44970096
[2] Academy of Medical Sciences. *All policy projects.* https://acmedsci.ac.uk/policy/policy-projects
[3] Academy of Medical Sciences (2020). *Public involvement and engagement in research during the COVID-19 pandemic.* https://acmedsci.ac.uk/file-download/77957062
[4] *Ibid.*
[5] Academy of Medical Sciences (2017). *Enhancing the use of scientific evidence to judge the potential benefits and harms of medicines.* https://acmedsci.ac.uk/file-download/44970096
[6] Academy of Medical Sciences (2017). *Accelerating access to medical innovation: a research agenda for innovation science.* https://acmedsci.ac.uk/file-download/80863587

The strong commitment to improving how the MHRA uses PPI is evident throughout the Strategy. However, a greater emphasis on co-design and co-production would improve the sustainability of the implementation of the Strategy. The Strategy would benefit from identifying and articulating more specifically the ways in which PPI will involve genuine instances of co-production of regulatory approaches and the identification of best practices.

We have previously heard that patient needs have been poorly considered across the lifespan of clinical studies.[7] In line with the central recommendations of The Liminal Spaces project, we recommend that the MHRA adopts a whole system approach, which views the health research regulatory environment as an interconnected system.[8] The 'Partnerships' objective makes good progress in this regard, but there are opportunities to further consider how the Strategy can complement and support PPI across the whole R&D pipeline by engaging with researchers, pharmaceutical and medtech companies, other regulatory authorities and other stakeholders. This will be key to the sustainability and success of the Strategy. An example of an action that does use the whole system approach is the MHRA's pilot on patient involvement in new applications.[9] The Academy welcomes this project and hopes that the MHRA will continue to explore how this pilot could be extended to clinical trial applications and used to promote co-production of research, as well as how PPI in research can feed into regulatory decision-making.

The COVID-19 pandemic has highlighted and exacerbated existing health inequalities, disproportionately affecting those from ethnic minority groups and socially disadvantaged communities. While we welcome the Strategy's ambition to reach out to societal groups who do not traditionally engage with research and governmental agencies, there is a need to consider how every proposed action will be performed in a manner that will lessen, rather than exacerbate, these disparities.

The Strategy would benefit from more context about the importance of embedding PPI in regulation, as well as clarity about what the Strategy is aiming to achieve. A clear statement of purpose explaining how embedding PPI will improve processes and outcomes would help clarify this for both the MHRA, outside parties and patients, as well make it easier to measure success. Such a statement should be co-designed with patient and public groups. A number of our experts felt that the Strategy puts emphasis on how the MHRA can have an influence on public and patients, rather than fully recognising the value that patients can offer to the MHRA. The Strategy should affirm PPI as a mutually beneficial activity, and care should be given in the wording and order of examples to highlight the value of patient and public groups. This is vital to gain trust and engage patients, especially those belonging to under-represented groups who are already sceptical about their voices being heard.

Another concern raised by our experts related to the intended timeframes mentioned in the document. Experts noted that embedding PPI within an organisation can take more time and logistical organisation than might be anticipated, particularly in order to achieve the necessary changes to internal culture. On the other hand, any changes to the guidelines on regulatory designs and outcomes need to be communicated in a timely manner to researchers and companies so that they can build the changes into their studies. Development of a detailed timeline with accompanying goals and points of evaluation, with consideration of the time pressures and dependencies between different actions, will be crucial to the success of this programme.

## Patient and public involvement

[7] Academy of Medical Sciences (2017). *Enhancing the use of scientific evidence to judge the potential benefits and harms of medicines.* https://acmedsci.ac.uk/file-download/44970096
[8] *Liminal Spaces: Driving a whole system approach to health research regulation.* https://www.law.ed.ac.uk/research/research-projects/liminal-spaces
[9] MHRA (2021). MHRA pilots patient involvement in new applications https://www.gov.uk/government/news/mhra-pilots-patient-involvement-in-new-applications

We are pleased to see commitments to build on progress that has already been made with respect to making patient safety information and public-facing material more accessible and usable. Access to reliable evidence presented in an accessible format is a key determinant of patients being able to make empowered and effective decisions about their healthcare.[10]

This objective offers ample opportunities for co-design and co-production. Specific actions that could benefit from co-design include the definition and measurement of patient-reported outcome measures (PROMs) and the design of public-facing information.

While PROMs are important for gathering evidence on impacts that matter to patients and are better predictors of long-term prognosis than other measures, it must be acknowledged that using PROMs does not automatically achieve the aim of including the patient voice in decision-making.[11,12] PROMs are mostly developed by clinicians or scientists, and often contain language that does not capture the true patient experience. There should be a commitment to the use of PROMs designed by the patient, and to developing these definitions and methods in conjunction with NICE, to streamline the regulatory and health technology assessment (HTA) processes. Transparency surrounding the weighting of different outcomes in decision-making is also vital for gaining patient trust.

It was noted that sharing draft guidance documents 'for comment before publication' and seeking 'patient and public views… at the appropriate time in the decision-making process' does not embed the patient voice. Co-design and co-production of guidance and decision-making processes from the start, with considerable and equal weight given to the patient/public opinion, is a more sustainable way of achieving meaningful involvement. The Academy recommends reconsidering parts of the Strategy, including but not limited to those mentioned above, that are suggestive of patient/public 'input' but not genuine 'involvement'.

The Strategy would also benefit from defining and distinguishing between the diverse ways in which patients and the public might be involved. Various verbs such as inform, communicate, involve, educate and engage are used throughout, but each require different strategies, management and forms of interaction.

## Responsiveness

The aim to develop a system that 'flags when more in-depth involvement of patient groups is needed' should be commended, but this section could be developed by being clear about when and how opportunities for co-production might arise, and considering how a whole system approach could be used to improve responsiveness (e.g. by identifying opportunities for other parties to be involved in reporting and ensuring patient safety).

FORUM workshop participants previously applauded the Yellow Card System for its agility in enabling the reporting of side effects associated with COVID-19 medicines and experimental treatments, and we are therefore pleased to see mention of the System in this Strategy.[13] However, we believe there could be a stronger commitment to involve patients when designing innovations and changes to the System and in the reporting of adverse events generally.

## Internal Culture

The Academy strongly supports the approach of promoting internal culture change to empower staff to consider the patient/public perspective and recognises that this represents a significant positive change in the MHRA's approach to PPI.

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[10] Academy of Medical Sciences (2017). *Looking to the future: oncology endpoints.* https://acmedsci.ac.uk/file-download/41135280

[11] Academy of Medical Sciences (2015). *Stratified, personalised or P4 medicine: a new direction for placing the patient at the centre of healthcare and health education.* https://acmedsci.ac.uk/file-download/38266-56e6d483e1d21.pdf

[12] Academy of Medical Sciences. *FORUM.* https://acmedsci.ac.uk/policy/forum

[13] Academy of Medical Sciences (2020). *Public involvement and engagement in research during the COVID-19 pandemic.* https://acmedsci.ac.uk/file-download/77957062

Achieving internal culture change will be central to the success of other parts of the Strategy. It should be acknowledged that attempts to change internal culture rapidly by forcing ways of thinking risk certain actions becoming 'tick box exercises', leading to meaningless 'engagement' and threatening the sustainability and effectiveness of the Strategy. Meaningful delivery of this plan will require leadership from a dedicated public programmes team, who can provide assistance and training, as well as a committed team of staff with an open mindset who are already trained in the importance and value of PPI. Furthermore, there is an opportunity for co-production in the design and delivery of training, going beyond simply asking patient and public representatives to give presentations.

### Measuring outcomes

The acknowledgement that "Successful delivery of this approach will rely on our ability to align all activity and measures of patient engagement and involvement with our high level framework of outcomes" is welcomed. We suggest that this should be made more explicit and central to the Strategy as a whole.

Specific recommendations in relation to this section include the co-design of the patient engagement index with patients and the public and changing 'measure of trust' to 'trustworthiness', as it is trustworthiness that must be demonstrated and can be measured.

### Partnerships

As a partner of the MHRA through its membership to the Academy's FORUM, we are pleased to see that the Strategy considers how partnerships may play a role in developing PPI as a meaningful and valuable tool in regulation.[14] The Academy is keen to stay actively engaged throughout the development and implementation of this Strategy and to develop our relationship through other areas of shared interest and action.

Of relevance to our recommendation of utilising opportunities for co-production, we believe that it is essential that the overhaul of the Patient Group Consultative Forum is co-produced with patients and the public.

## Question 2: What additional actions should we consider to improve our strategy?

### Recommendations related to the strategy as a whole

In addition to the recommendations made in our answer to question 1, which relate to existing parts of the Strategy, our experts also identified a number of challenges and areas for action that are not considered in the current Strategy:

The Strategy uses 'patient' and 'the public' both collectively and interchangeably. The Strategy would benefit from defining these two groups and establishing how the different perspectives of these groups will be integrated to form a consensus view. We suggest that attention should be paid to how staff training will enable staff to effectively integrate these perspectives. Furthermore, a few important groups appear to be missing, namely charities and patient groups, especially those working with children and pregnant women, and under-represented groups. It will also require additional considerations of how to integrate the perspectives of groups with strong, polarised views. While we recognise that the scope of this Strategy does not cover healthcare professionals, there should be consideration of how this Strategy will complement the proposed healthcare professionals (HCPs) engagement plan, as laid out in the MHRA Business Plan, and how the views of HCPs and patients, which may differ, will be integrated.[15]

[14] Academy of Medical Sciences. *FORUM.* https://acmedsci.ac.uk/policy/forum
[15] MHRA (2020). *Business Plan 2020-21.* https://assets.publishing.service.gov.uk/government/uploads/system/uploads/attachment_data/file/889864/MHRA_Business_Plan_2020_to_2021.pdf

Secondly, specific consideration should be given to situations where the patient perspective is based on that of a single or small number of patient voices, for example when considering 'ultra-precision' medical technologies that provide bespoke treatment to a small number of patients, including for genetic and rare diseases. These technologies are likely to become increasingly common, and so the Strategy should commit to developing guidelines that detail how the patient voice can be embedded into decision-making in these cases.

As identified by participants at our joint roundtable on *Advancing regulatory science for innovative medical products*, one important role of regulatory science will be to explore how patients and the public can best contribute to regulation.[16] It should be ensured that the Strategy complements the MHRA's internal thinking about the development of the regulatory science field.

## Patient and public involvement

In addition to ensuring that this section identifies opportunities for genuine co-production, a number of additional actions are suggested:

The MHRA should attempt to make all public-facing documentation, both those related to PPI and more widely, accessible to people with a range of educational backgrounds. We are pleased to see mention of the website in the MHRA's strategic goals.[17] Specific attention should be given to making the language more accessible (e.g. avoiding the unnecessary use of abbreviations like SPCs, PILs and PARS), through co-design with patients and the public. We also recommend using PPI and co-design in the development of clinical trial information materials, which currently use dense language which is difficult to understand, and which may provoke anxiety.

In line with the whole system approach, the MHRA should explore how it could encourage, and eventually require, researchers to incorporate PPI within in their research. For example, it may be possible to build information about PPI into the framework by which the MHRA gives advice to researchers.[18] In addition, instead of simply recommending researchers to build PROMs into the design of clinical trial protocols (as noted in the Measuring Outcomes section), active engagement with researchers about PROMs will ensure this translates into an increase in the number of clinical trials protocols submitted that use PROMs. The MHRA's pilot on patient involvement in new applications is a welcome example of how this might be achieved.[19]

In line with our recommendation to consider the value and purpose of PPI in relation to the activities of the MHRA (question 1), we recommend that the Strategy identifies specific and relevant areas of regulation where PPI can add value. Such areas have been identified by participants at several FORUM workshops, for example:

1. Regulation of innovative medical devices and technologies. It should be acknowledged that the knowledge base and experience required for patients and the public (and MHRA staff) to engage with questions about medical devices (particularly those reflecting advances in artificial intelligence and related technologies) versus more commonplace medicines and interventions, is likely very different. The issues, concerns and relative balance of harms and benefits will also be difference. Consequently, engaging and involving patients in discussions about these technologies will require specific and additional consideration.
2. Use of real-world evidence. FORUM workshop participants previously highlighted the importance of public and patient 'buy-in' for the use of real world evidence in regulation

[16] Academy of Medical Sciences (2021). *Academy of Medical Sciences FORUM and MHRA joint roundtable on Regulatory Science.* https://acmedsci.ac.uk/more/events/academy-of-medical-sciences-forummhra-joint-roundtable-on-regulatory-science-advancing-regulatory-science-for-innovative-medical-products
[17] MHRA (2020). *Business Plan 2020-21.* https://assets.publishing.service.gov.uk/government/uploads/system/uploads/attachment_data/file/889864/MHRA_Business_Plan_2020_to_2021.pdf
[18] MHRA (2014). *Medicines: get scientific advice from MHRA*. https://www.gov.uk/guidance/medicines-get-scientific-advice-from-mhra
[19] MHRA (2021). *MHRA pilots patient involvement in new applications.* https://www.gov.uk/government/news/mhra-pilots-patient-involvement-in-new-applications

and HTA, and identified PPI as essential to addressing privacy and consent issues around data access.[20,21]

Other areas of regulation where PPI could add value include genome editing, adaptive trials, data-driven technologies, endpoints and personalised/stratified medicine.[22,23,24,25,26]

Stronger links between co-design and co-production and implementation and adoption could bring significant benefits. PPI is one way of ensuring that innovation lands in a receptive ecosystem and help overcome a major block in the current landscape in adopting change. Working with organisations that already practice PPI at scale, such as medical research charities, can help identify and support additional opportunities and benefits that PPI could bring.

## Internal Culture

As mentioned in the response to question 1, creation of a dedicated and experienced public programmes team which is embedded within all departments of the MHRA should be considered as central to the success of this Strategy.

As the MHRA is one part of the research and regulatory system, and works closely and collaboratively with other organisations, there is also a need to consider how the MHRA might contribute to culture change across the whole system and lifecycle of regulatory approvals.

## Measuring outcomes

The MHRA should commit to transparency surrounding the outcome of any evaluation of this Strategy, and to sharing best practices and lessons learned with the wider community.

## Partnerships

There is an opportunity to use this section to develop a plan for how the MHRA will take a whole system approach to PPI, including how the Strategy can complement and support PPI across the whole R&D pipeline by engaging with researchers, research funders, pharmaceutical companies, other regulatory authorities, and other stakeholders. Particular attention could be given to how the MHRA's pilot on patient involvement in new applications could contribute to a whole system approach, and how this pilot might be developed in the future.[27] Additionally, the new combined review process presents an opportunity to align PPI approaches across the MHRA and the Health Research Authority (HRA), and we recommend that this is explored further.[28]

We recommend that the MHRA continues to engage with and learn from partner organisations and regulatory authorities around the world, to learn from others' successes. We previously identified the US Drug Facts Box initiative, which has been shown to enable individuals to make improved

[20] Academy of Medical Sciences (2018). *Next steps for using real world evidence.* https://acmedsci.ac.uk/file-download/7021031
[21] Academy of Medical Sciences (2016). *Regulation and governance of health research: five years on.* https://acmedsci.ac.uk/file-download/14145196
[22] Academy of Medical Sciences (2019). *The promise of human genome editing for rare and genetic disease.* https://acmedsci.ac.uk/file-download/12657070
[23] Academy of Medical Sciences (2019). *Adaptive trials: acceptability, versatility and utility.* https://acmedsci.ac.uk/file-download/36842538
[24] Academy of Medical Sciences (2018). *Our data-driven future in healthcare.* https://acmedsci.ac.uk/file-download/74634438
[25] Academy of Medical Sciences (2017). *Looking to the future: oncology endpoints.* https://acmedsci.ac.uk/file-download/41135280
[26] Academy of Medical Sciences (2015). *Stratified, personalised or P4 medicine: a new direction for placing the patient at the centre of healthcare and health education.* https://acmedsci.ac.uk/file-download/38266-56e6d483e1d21.pdf
[27] MHRA (2021). *MHRA pilots patient involvement in new applications.* https://www.gov.uk/government/news/mhra-pilots-patient-involvement-in-new-applications

[28] HRA (2021). *Combined review.* https://www.hra.nhs.uk/planning-and-improving-research/policies-standards-legislation/clinical-trials-investigational-medicinal-products-ctimps/combined-ways-working-pilot/

judgements about medicines, as an excellent example of an innovative and valuable PPI initiative.[29]

More specifically, we suggest that the 'Insight Exchange' section could be strengthened by adding a commitment to identifying and sharing best practices and that 'Patient Engagement Mechanisms' section would benefit from a definition of what success of this programme would look like, and how this will be captured and assessed.

## Question 3: If we deliver our strategy, how do you think engaging with the MHRA would feel different from a patient perspective?

The experts that fed into this response had ranging opinions on the likely success of this Strategy as it stands. However, the consensus view was that if this Strategy is developed to provide more opportunities for genuine co-production with the whole system approach in mind, then it is likely that this Strategy will deliver on its objectives. Potential positive outcomes mentioned included greater trust in medicines, devices and clinical trials, and greater public understanding of the regulatory pathway.

Some mentioned that it is difficult to predict how patients might respond, and that different patient groups with different needs will engage in various ways. It is important to acknowledge differences in how different patient groups have been engaged historically. For example, cancer patients are a group that have been generally well engaged throughout the R&D pipeline. Engaging groups of patients that have often been ignored (e.g. mental health patients) will require more effort and careful consideration. There is of course the possibility that patients don't respond as desired in the short-term, and it should be considered how short-term challenges will be addressed so that they don't hinder long-term ambitions.

Finally, an important priority for the Strategy should be to ensure that patients feel genuinely involved as an equal partner and to avoid tokenism. As such, the two rounds of consultation used to develop this Strategy are warmly welcomed and will go a long way in ensuring that this priority is met.

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This response was prepared by Alice Fletcher-Etherington, Policy Intern, and informed by members of the Academy's Fellowship and previous policy work in this area. For further information, please contact Dr James Squires, FORUM Policy Manager (james.squires@acmedsci.ac.uk; +44(0)20 3141 3227).

[29] Academy of Medical Sciences (2017). *Enhancing the use of scientific evidence to judge the potential benefits and harms of medicines.* https://acmedsci.ac.uk/file-download/44970096