Event: IMI impact on patient involvement
Questions and answers

On 7 October 2021, the Innovative Medicines Initiative (IMI) held an online event on IMI's impact on patient involvement in research and innovation. This document sets out IMI's answers to the questions which, due to time constraints, were not addressed during the questions and answers session on the day.

Question: Are there special programmes to learn/educate (young) researchers how to communicate with patients or their representatives?

IMI's answer: Yes, EUPATI, which started life as an IMI-funded project, runs training courses on patient engagement for professionals from academia and industries. For more information, visit eupati.eu/training/patient-engagement/. In addition, EUPATI has issued guidance for different types of stakeholders on how best to involve patients in specific aspects of medicines development.

The Patient Engagement Toolbox developed by IMI project PARADIGM also includes resources on when and how to engage with patients at all stages of the medicines development process.

Question: Knowing what has been done through IMI, it would be interesting to clarify what is planned through the new entity to come?

IMI's answer: In February, the European Commission released a proposal to create the Innovative Health Initiative (IHI). IHI would be a new public-private partnership that would build on the successes of IMI. In September, the EU Member States reached an agreement on IHI and a number of other public-private partnerships. The Council is expected to officially adopt the legislation before the end of the year, and we will be able to say more about it then.

On the precise role of patients in IHI, the legislation is not yet approved. However, the legislative proposal does make it clear that patient involvement is critical and IHI will build on IMI's legacy and expand and develop the strategy further.

Question: Are there ways of engaging with the patients in drug development that should be recommended by regulators?

IMI's answer: As mentioned during the webinar, our PREFER project has developed a framework for industry, regulators and health technology assessment bodies for how to use patient preferences as input in medical product decision making. The project has asked the European Medicines Agency (EMA) and EUnetHTA to assess the framework and issue a public opinion on how useful PREFER's approach is from the regulatory and health technology assessment perspective. You can read more about this process here.

Following the webinar, PREFER announced that the EMA had published its draft opinion for public consultation.

Question: Are patients involved in the review and evaluation of IMI proposals? If yes, how? If no, why not?

IMI's answer: Yes! As we mentioned during the webinar, people from the IMI pool of patient experts have been invited to participate in a variety of IMI activities, including project reviews and proposal evaluations. To date, patients have participated in 17 expert panels that carried out project reviews and project proposal evaluations, working on equal terms alongside experts from other sectors.

Question: How does IMI make sure that truly independent patient organizations resp. patients become engaged in IMI projects?

Question: How do IMI projects address a potential conflict of interest of patient representatives?

IMI's answer: At IMI, we consider patients to be equal partners that can and should play an active role in the medicines R&D process. Including patients' perspectives in IMI activities and facilitating patient participation in projects is a top priority for IMI.

Over 30 patient organisations have chosen to become full project partners. Thanks to patient-led projects like EUPATI, growing numbers of patients have an in-depth understanding of medical research and development processes. This, coupled with their own experience and knowledge of their specific disease area(s), plus the funding provided by IMI, places them on an equal footing to other partners. Patient organisations' contributions to our projects are highly valuable and touch on many aspects of the project – examples of these were highlighted during the event.

Patient organisations rely on a number of sources of funding to finance their operations. However, the same is also true of many other project partners who receive funding from IMI. The issue of conflicts of interest is addressed in the IMI2 model grant agreement, which states that 'beneficiaries must take all measures to prevent any situation where the impartial and objective implementation of the action is compromised for reasons involving economic interest, political or national affinity, family or emotional ties or any other shared interest'.

From a pragmatic point of view, even though some patient organisations may receive funding from the pharmaceutical industry, this does not change the fact that they possess the highest level of knowledge, experience and expertise in patient advocacy and therefore are an important resource for our projects. Excluding these organisations from becoming full project partners would limit dramatically IMI's ability to have the valuable input they bring to many important aspects of a project like defining the outcomes that will genuinely benefit patients; determining the appropriate benefit-risk balance in new treatments; providing input into the best ways to involve patients in project governance. Moreover, their exclusion would also affect the ability of patients to be involved in our projects.

Furthermore, in practice, experience shows that patients are not afraid of speaking out and pushing their funders (including IMI at times!) to do more and do better on patient engagement.

In fact patients drove us to take our patient engagement work to the next level by creating the pool of patient experts. As we explained during the webinar, the pool counts 118 patients and 30 informal carers from 26 countries and with experience of living with a wide range of diseases. This pool has helped IMI to include patients in key activities such as the review of project proposals and ongoing projects. When carrying out these tasks, patients / carers act in their personal capacity and have to declare any conflict of interest they may have, as do all other experts.

IMI has the philosophy of managing perceived conflicts of interest through total openness and transparency with regard to how patients interact with projects.

Question: Were patients meaningfully involved in priority setting for IMI2? Are they being involved in the same process for IHI? I think that this stage is crucial and essential as it impacts the whole funding framework, which should not be co-decided only by the EC and the industry. Patients should be involved when the decisions on WHAT will be funded are made, not only in the implementation.

IMI's answer: EFPIA developed the IMI2 Strategic Research Agenda (SRA) following lengthy discussions with the EC and with input from more than 80 organisations, including patient organisations as well as regulators and academia. The final text of the SRA and IMI's Annual Work Plans (AWPs), which further develop the SRA, are endorsed by the IMI Governing Board and published online.

IMI's Scientific Committee (SC), which is made up of globally recognised experts and includes a patient representative, advises on the priorities to be included in the Annual Work Plans, which further develop the SRA. IMI2 Associated Partners also influence IMI topic design. For example, JDRF has generated three topic ideas on diabetes research and regards its input as a true co-creation process involving the type 1 diabetes (T1D) community.

The development of the Strategic Research and Innovation Agenda (SRIA) for the Innovative Health Initiative (IHI) is the responsibility of the European Commission and the IHI industry partners, not the IMI Programme Office.

The current version of the SRIA, elaborated jointly by the future IHI member industry associations and the Commission's services, takes into consideration the feedback received to an open consultation that took place in autumn 2019. The summary of the almost 100 responses, including patient organisations among them, is publicly available at www.euhealthppp.org/strategic-agenda.

Question: Is it still possible to participate in the IMI pool of patient expert?

IMI's answer: We will not open a new Call for expressions of interest to join the pool of patients under IMI. However, the proposed new partnership, IHI, is likely to include multiple opportunities for patient involvement.

Question: Could I ask about involving children or their caregivers in biomedical studies - I ask because many funders are now requiring PPI public patient involvement so most researchers are now doing their best to involve BUT isn't it tokenistic to ask children/parents about a very scientific biomedical procedure about which they can't influence? Is their involvement best suited to helping the researchers communicate more simply the science of what they are trying to do? Thank you.

IMI's answer: When done poorly, patient engagement can indeed be tokenistic. This is why IMI funds projects such as EUPATI, PREFER and PARADIGM, which provide patients and scientists with training and resources to make patient engagement meaningful. EUPATI's training courses and online resources ensure that patients are able to understand the terminology and processes involved in drug development. This makes it easier for them to follow and contribute to discussions within the project and allows them to have an influence in diverse project activities including communication, but also the design of clinical studies and project governance. PARADIGM and PREFER are delivering extensive guidance on the when and how of patient engagement – this should ensure that patients are not invited to comment on something they cannot influence!

Regarding the specific question of involving children and parents in research, the presentation of Ellen Vrieze-Hermans demonstrated that parents can contribute meaningfully to projects. More broadly, all the speakers demonstrated that properly involving patients in research results in better research.

Question: What is a recent project (IMI, EU funded or other) where you see a "novel" approach to patient involvement/engagement?

IMI's answer: Within IMI, we have a growing number of projects working on the question of how best to integrate the patient voice in health research and health care. Some were presented at the webinar, but there are others. For example, H2O is creating 'health outcomes observatories' that will amplify the patient voice both in their own healthcare and in healthcare systems more broadly. EU-PEARL is setting up adaptive clinical trial platforms, and patients are contributing to their design.

Questions addressed during the webinar

For information, the following questions were addressed during the webinar, either during the presentations themselves or during the Q&A session.

- How important is to have a wide patient representation (geographical, socioeconomic etc) and how to achieve this effectively?
- How can you 'provide' representativity of participating pats, for the respective 'study population'?
- How can researchers find patients to get involved in their projects?
- How do you document the value of PPI?
- Provide a concrete example of a case where having the patients onboard was a critical factor for reaching a milestone
- What was it like to participate in a research project as an expert by experience?
- How to convince patients to engage? What are the strongest arguments to get them in?