Interaction of Patients, Regulators & Industry

Participants from regulatory authorities, patient organisations, academia, non-profit organisations and industry joined together in Berlin to advance and foster the interaction of us all on patient involvement and engagement in medicines R&D.

It is widely acknowledged today that patients' contributions to the discovery, development and evaluation of medicines enriches the quality of research and development, quality of evidence and opinion and transparency, trust and mutual respect. Patient involvement requires systematic involvement of all stakeholders: pharmaceutical industry, regulators and HTA bodies, patient and consumer organisations, health care professionals, non-profit organisations and academia.

Our Director, Jan Geissler, shared some preliminary data on the impact of EUPATI from a survey among EUPATI fellows who participated in the Patient Expert Training Course: they have increased their advisory roles significantly, comparing their engagement before and after the course:

- providing advice to the pharmaceutical industry increased from 8% to 52%,
- to regulatory agencies from 12% to 40% and
- to HTA bodies from 4% to 8%.

Despite this progress, challenges still remain, such as the lack of mutual learning, the lack of mutual trust, the lack of standardised metrics to measure benefits and impact, perceived and/or real barriers around conflict of interest as well as the lack of capacity in patient organisations. I highlighted that, based on the success of EUPATI in establishing the multilingual EUPATI Toolbox as well as the EUPATI Patient Expert Training course, decisions have been taken to continue

beyond 2016 as the only dedicated training structure and trusted brand on patient involvement in medicines R&D. The long-term sustainability of EUPATI is of major public interest, but securing public funding beyond the current IMI-funded project phase remains a challenge.

Isabelle Moulon, from EMA, highlighted that patients can engage in various ways in regulatory processes: they can be members of committees, act as representatives of an organisation or be individual experts, and she clarified how declarations of interests are evaluated for each of these roles. EMA has established a network of European patients' and consumers' organisations for collaboration on various topics. The EMA Patients' and Consumers' Working Party (PCWP) plays a key role in enabling these interactions. To support the best possible cooperation EMA recognises that patients involved with the Agency need to have adequate knowledge of the work of the Agency and therefore provides various training opportunities, also referencing EUPATI initiatives.

Matthias Gottwald, from Bayer, presented a practical "roadmap" for patient involvement in all steps of the R&D process. He also shared some preliminary findings from a DIA-Tufts University study:

- out of 20 pharmaceutical companies, only three consider themselves "very patient-centric" and
- four "not very patient-centric",
- most are "somewhat patient-centric".

The top planned initiatives were adaptive trial designs and adaptive licensing. The patient engagement function is placed differently in companies and mostly in clinical operations or clinical development. Corporate drivers to create a patient engagement function are company commitment and clear benefits for process improvement. Challenges for patient involvement in R&D are:

- concerns of patient independence,
- need for comprehensive guidance addressing all stakeholders,
- no infrastructure for “matchmaking”,
- no consolidated approach for patient involvement in industry and
- no metrics for impact assessment.

Two working groups – one on industry, the other on regulatory bodies

According to the Industry group, there is a need to gain more clarity on the conflict-of-interest question in order to be able to engage patients in an appropriate way. There is also a need to set a standard for compensation and fees across the industry. All industry partners are interested in a common framework for patient involvement across all stakeholders. It is time now to build upon what EUPATI has already done and develop clear, more detailed processes. Industry is willing to share best practices but need a platform to do so. Demonstrating value is also of utmost importance and perhaps this could be done by extracting metrics from existing case studies. Industry is eager to move forward and to have regulators be part of this journey of taking patient engagement to the next level.

The Regulatory group unveiled the very different approaches to patient involvement nationally and in EMA, as presented in case studies and discussions, and a need to continue that dialogue. What kind of patients/patient groups should be involved? And where in the work process is it useful? How to compensate participants? Sharing experience and developing best practices are needed, and EUPATI agreed to the start of a more structured exchange of experiences on patient involvement in regulatory affairs. Most national cooperation currently is informal and while there are some legal provisions, a wider consensus on processes and a legal framework may help. Commonly accepted outcome metrics on the value of patient

involvement is needed.

In the plenary, we considered the need of widening the EUPATI target audience: training could be expanded to hospitals, regulators, industry and other healthcare professionals, which will also help to further build collaboration and trust. Furthermore, there is a need for a matchmaking tool to connect patients to projects. EUPATI currently has a 'match-making' section online to connect stakeholders to projects and enable a two-way dialogue between stakeholders. Can this be built on?

Specifically for IMI and the future, the call topic text has been revised to indicate where patients can engage. The IMI Joint Undertaking also proactively indicates the role of patients when it launches its calls. Currently learnings are being gathered from the two projects where patients were involved. Patients who would like to get involved in IMI projects are requested to make sure that they are registered in the database.

The take home messages:

More clarity and common understanding is needed on the demands, expectations and restrictions on patient representatives in different roles as members of committees, acting as representatives of an organization, or being individual experts.

Should the EUPATI training be expanded to other target groups in addition to patients? And can IMI involve patients more proactively, e.g., through its new IMI Patient Engagement Strategy?

The very different approaches to patient involvement in national competent authorities and in EMA need to be aligned on the basis of more knowledge of current experiences and best practices, leading potentially to a more systematic involvement in the daily work of the national and European agencies. This will be taken forward in the European medicines

regulatory network.

The processes of patient involvement in industry have evolved, but the evolution of frameworks, mechanisms, metrics and processes happens ad hoc without much alignment and sharing. Establishing a neutral platform, e.g., DIA, to enable pre-competitive sharing and alignment is needed and will be explored further.

Both regulators and industry identified a need for clarity and alignment on compensation. This can be taken forward in multi-stakeholder discussions.

Both regulators and industry identified a need for development of commonly accepted metrics on outcome of patient involvement. Such an initiative could/should be taken forward by IMI.