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European Federation of Pharmaceutical Industries and Associations

EFPIA Patient Think Tank – Issue Summary July 2014

EFPIA's Patient Think Tank serves as a forum for both EFPIA members and European patients' organisations to come together in an active forum to discuss how industry can best meet patient needs. Following each meeting, an issue summary is posted on the EFPIA website. Below is the issue summary of July 2014.

Benefit/Risk

The current landscape is defined by the following elements:

- The current regulatory pathway is expensive and slow in getting new therapies to patients;
- General response rates from medicines is very poor;
- Science is going faster than the current regulatory system;
- Pathways require large trials to target small populations;
- Reimbursement needs to reflect the reality of new therapies.

To respond to these challenges, EFPIA is pursuing the medicines adaptive pathways to patients (MAPPS). In the future, multiple sources of data will be used to support and sustain medicines approval. (i.e. real world data). The Innovative Medicines Initiative provides a framework for testing these new approaches.

From a patient perspective, there is a clear need for patient engagement in the area of B/R evaluation – for example, take the case of Thalidomide and Duchenne, where patients had played an important role in shaping regulatory decisions and risk management plans.

We are not starting from a blank page. Patients have been engaged in reviewing EPARS and PILs, however further evolutions is needed. The focus is on training, capacity-building and awareness. Rather than being restricted by the idea of a "patient-expert", part of the contribution of patient representatives is to access and assimilate real patient experiences from their networks.

The EMA is evolving a patient-centric culture, including in the field of B/R assessment. The EMA will be piloting trials for adaptive licensing, which will enhance the capability to consider the perspectives of patients and families.

For many regulators, this is a difficult change. To give a powerful example, patient perspectives on the trade-off between *quality* and *quantity* of life may be quite different from those of a regulator. These fundamental differences have been shown and replicated by a number of studies that have examined patient choices. Regulators may be prepared to accept that patients will take more risks, but have more difficulty integrating that knowledge into their decision-making.

To date, the ethical dimension of decision-making has not been a significant factor in policy debate. Most discussions of benefit-risk are based on public health concerns, although researchers have been concerned to assess whether patients are in a position to make reasoned choices in serious or life-threatening situations.

Finally, although the role of patients is accepted in principle, we need more studies that address the benefits of patient involvement in decision-making. EMA's own analysis of patient input to protocol evaluation suggested that patients have made a significant contribution in more than 50% of cases, but the evidence base is too thin to be a platform for dissemination of good practice.

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From an industry perspective, technology is offering a new type of evidence package. Today, new methodologies for evaluations are available, while multiple cycles of learning can generate adaptive approaches. There is a spectrum of mechanisms for obtaining patient input currently in existence – a ranking of these mechanisms would be useful. While the patient voice is important, the drug approval process still lies ultimately with regulators.

Although trends are positive and experimentation with new decision-making structures and assessment methodologies are part of the process, there are balancing concerns. We need consensus on methodologies between patients, industry and regulators – without this, industry can't know how to prepare trials.

Some discussion is also needed on how to use data. The assessment of a medicine has to end in a decision about whether it will be made available to patients. This is characterised as a judgment about the balance of benefit and risk. In fact, this is a condensation of a complex set of trade-offs that (assuming a certain level of safety) need to be made available to patients. In evaluating next steps, it is important to recognize the diversity of situations that need to be addressed.

Also covered in the benefit-risk discussion:

- Ongoing work of the benefit-risk group at EFPIA
- The PCORI report & capturing patient-relevant outcomes (http://www.pcori.org/assets/2013/11/PCORI-Methodology-Report.pdf)
- Role of EUPATI in furthering the discussion

EFPIA's benefit-risk group and the PTT agreed to continue the dialogue through a sub-group to be established.

Access to Medical Congresses

In some cases, current regulations place restrictions on full participation by patient representatives in medical congresses, medical scientific sessions and exhibitions. It was noted that this is in some way contradictory to the expertise of patient representatives.

Other Topics Covered

- Framework and objectives of the Think Tank.
- Patient organisation involvement in the upcoming EFPIA-PhRMA October conference.
- EPF/ NPO Patient Access Partnership.
- The results of the recent EU elections.
- Increased transparency around the Think Tank, i.e. with increased website presence.
- IMI-2 as a tool to explore new B/R models.

Full copies of EFPIA Patient Think Tank meeting reports are available from EFPIA, upon request: communications@efpia.eu