EUPATI: PATIENT INVOLVEMENT IN INDUSTRY R&D

23 July 2014, held at Bayer Healthcare, Berlin, Germany

WORKSHOP SUMMARY REPORT

SETTING OUT THE NEED FOR PATIENT INVOLVEMENT

This meeting was about taking concrete actions that help support patient involvement in research and development (R&D), said Nicola Bedlington, Executive Director, European Patients' Forum at the opening address of the workshop. Nicola outlined the explicit and implicit objectives of EUPATI as the starting point for our discussions. Through empowering patients to be involved at all stages of R&D and building capacity among the advocate community there is an opportunity to make patient centricity in R&D a reality.

To achieve this goal it is important that all stakeholders come together to implement true partnerships aimed to address common goals. This workshop focused on the need to involve patients and advocates actively and systematically at all stages of the R&D process. The EUPATI partners are ideally placed to make this a reality and have a fundamental role to play in creating a positive environment for all stakeholders.

During the day, every attendee would attend three breakout sessions designed to explore the benefits of patient and advocate involvement, the current barriers that exist and the relevant compliance codes and frameworks that need to be updated to enable smooth and transparent partnerships with patients and advocates during the research and development process. Best practice examples formed the basis of these discussions, allowing the participants to explore the lessons from these real-life examples and identify the approaches that made them successful. A summary of these three sessions is contained on the following pages. Full detailed notes were made during each breakout session. These will form the basis for discussions within the small groups being formed to drive the key activities forward.

PRIORITISED ACTIONS AND NEXT STEPS

The participants came together in the final session to discuss the outcomes of each of the three workshops and identify the priority activities that should be implemented. After a discussion of the issues and solutions identified during the day, all the potential activities were presented for a group vote. From this vote, the key activities identified were.

WHY

- **Clearly make the case for patient involvement in medicines development:** Working group to scope out key actions to document and communicate the impact and benefits including health, societal and business benefits of patient involvement in medicines development to all key stakeholders. This will involve creating a platform for sharing case studies of good practice and developing training for industry and regulators on the value of patient engagement

HOW

- **Develop a framework for patient involvement:** With the goal being to develop one set of guidelines for all parties (including regulators). A small group will be created to assist in reviewing the codes and making recommendations.

- **Outline the steps needed to involve patients and advocates:** Create a simple 'how to' check list for patient involvement in each phase of R&D

DO

- **Create key performance indicators for patient involvement:** Develop measures that cover quality, quantity and speed
- Create SOPs and guidance for good practice
- Develop EUPATI matchmaking as broker for patients and research

INVOLVE

- **Bring stakeholders such as regulators into the discussion:** Bring regulators into the working groups on 'How and 'Why' work streams so that they co-create the solutions we develop.

BREAKOUT GROUP 1: BENEFITS AND IMPACTS

During this workshop session, the participants discussed the benefits and impact of involving patients and advocates in the R&D process. Drawing upon concrete cases, participants identified examples of clear benefits of patient involvement across the entire value chain but noted that much of this was anecdotal at this stage and that there is a need to develop more evidence. It was agreed that both a top-down and bottom-up approach was needed to describe the benefits and show the strategic health outcomes benefits that this brings to the world. It was agreed that it was essential to identify and communicate the benefits and good practices in a relevant way to all key stakeholder groups.

- **High level benefits identified:**
  - Improving health outcomes for patients was utmost importance
  - Improving trust and stakeholder collaboration to bridge from bench to bedside

- **Specific benefits identified**
  - Better prioritizing of early research
  - Improved recruitment processes (increase enrolment rate, improve retention)
  - Improve trial planning and protocol designs including "ensuring that trials are designed around the lives of patients and not vice versa"
  - Improve trial conduct and operations, including improvement of factors such as patient safety, self-management and medication adherence support
  - Identify issues and address benefit risk early on
  - Improve communication and dissemination of research
  - Ensure patient-relevant trial endpoints are identified and have buy-in from all parties
  - Strengthen the broader patient community and public engagement
  - Improve wider health policy environment that governments care about
  - The economic benefit of conducting more focused clinical trials

- **Hurdles identified during this breakout session**
  - No structured systematic approach within companies and among stakeholders especially regulators and payers included
  - Need for industry to be accountable transparent and communicate collectively
  - Need for development of more evidence on economic benefits of patient involvement
  - Dedicated resources are required within the R&D organisation to ensure patient involvement
  - Important to maintain focus on broader horizontal involvement of patients and R&D
  - Educating physicians and other stakeholders on practicing patient involvement will be critical
  - So all stakeholders need to be involved as this process develops

Potential solutions identified during this breakout session

- **Create a working group to develop a platform of case studies** supporting clear key benefits and concrete examples that can be used across industry to show benefits across the value chain in the following key areas:
  - The setting of research priorities
  - Input into trial design and planning
  - Shaping research conduct and operations
  - Communication and dissemination of results

  Working group should ensure involvement of regulators in co-creating next steps
- **Create a simple 'how-to' check list for patient involvement in each phase of R&D**
- **Apply identified best practices and guiding principles for patient involvement in IMI2 projects wherever relevant**
- **Look at the benefits from each stakeholder group perspective** and ensure that there is an explanation of the benefits of patient involvement from each point of view
- **Build the economic evidence** to show that patient and advocate involvement really can reduce the cost and improve the focus of clinical research

BREAKOUT GROUP 2: COMMON CONCERNS, HURDLES AND SOLUTIONS

During this workshop session, the participants explored the barriers that currently exist that limit the involvement of patients and advocates in the R&D process. The groups was tasked with identifying the common concerns, the real hurdles that get in the way of patient involvement, the perceived barriers and misconceptions that exist and the potential solutions to overcoming these.

Challenges and perceived hurdles identified

- **The timeframe and numbers of patients in the process**
  - Issue: Partnership working takes time, and sometimes this time to build sustainable partnerships can be perceived as a challenge

- **Some lack of understanding of what patient involvement is, how it can work**
  - Issue: There exists a lack of practical guidance that can help all partners understand how patients and advocates can be involved in the R&D process

- **There exists a lack of clarity around what happens as a result of patient and advocate involvement**
  - Issue: The expectations of what can be achieved are sometimes not aligned and there are no standard processes that all parties in the partnership can follow

- **Confusion or debates around the representativeness of the patient/advocate partner**
  - Issue: A common barrier is that some question whether a single patient partner or organisation can truly represent the views of a patient population

- **The need for a culture change from the very top of industry organisations and champions who will promote the inclusion of patients and advocates in the R&D process**
  - Issue: Across large organisations there needs to be a top-down approach to getting all departments to understand the value and impact of patient involvement

- **No clear metrics or evidence at the moment to support patient involvement**
  - Issue: There is no standard way at the moment for assessing the impact of patient involvement

- **There exists a lack of awareness of current patient involvement practices and approaches**
  - Issue: Although patient and advocate involvement is already happening to some extent, there is a lack of awareness widely across the industry on what is being done and how successful these partnerships are

- **Trust, anxiety of association and risk aversion**
  - Issue: It was acknowledged that there are inevitably issues around trust between partners, and patient associations and advocates not wanting to be too closely associated with an industry partner. On the industry side there is legal and compliance aversion to taking risks by partnering with patients and advocates.

Potential solutions identified during this breakout session

- **Overcoming the time taking to build partnerships:** EUPATI could act as a 'matchmaker' bringing together relevant EUPATI trainees with industry R&D opportunities to speed up this process
- **Addressing lack of understanding about the 'how and why' of patient involvement:**
  - Develop standard operating procedures (SOPs) and good practice guidance that informs on how patient and advocates can be involved in the R&D process
  - The group identified an aspirational goal to develop one single set of guidance or SOP across all stakeholder groups
  - Communicate patient involvement initiatives and practices both internally within industry organisations and externally to patients
  - Clearly define the processes for partnering with patients and advocates so that all parties know what to practically expect
  - Develop a set of key performance indicators (KPIs) for patient involvement that covers the quality of that involvement, the quantity of involvement and the speed
  - From the start of the partnership, be completely transparent on the processes, the expectations and the accountability from all sides
  - Deliver training for both industry and regulators on the value of patient involvement that helps overcome any lack of understanding that exists
- **Addressing the issue of patient representativeness:**
  - The group strongly felt that this is a 'myth' that needs to be robustly dispelled.
  - If the partners focus on the objectives of involvement and allow room for all perspectives to be captured then this perception can be overcome.
  - Review novel methods of engagement that can overcome this perception
- **To address the need for a culture change:**
  - Develop and provide the evidence of the impact of patient involvement to show that 'patient involvement is good for business'
  - Establish a practice of including evidence of patient involvement in regulatory dossiers
  - Develop a white paper in collaboration with EFPIA on the impact and value of patient and advocate involvement

BREAKOUT GROUP 3: CODES OF PRACTICE

During this workshop session, the participants explored the current codes that exist to govern patient and advocate involvement with the industry and to identify the exact and useful content within those codes that could be applied to patient involvement in the R&D process. Questions were shaped to help identify the relevant guidance and codes from over 40 identified guidance documents.

Code and guidance related issues identified

- **A full detailed methodological review as originally proposed was not considered necessary**
  - This kind of review was acknowledged by the group to not be required to address this issue

- **The current codes do not cover individual patient involvement in medicines R&D**
  - However, it was expressly stated by EFPIA that there is nothing in the code that prevents these partnerships

- **The term 'code' was found to be unhelpful in this group**
  - The participants felt that guidelines, umbrella frameworks and declarations may be better terminology to think about for empowering the partnerships of patients, advocates and industry

- **There needs to be a mind-set change on how codes are expressed and interpreted**
  - Overall there was recognition that language needs to be more directive towards patient involvement
  - There needs to be a clear default statement that everything is allowed unless expressly forbidden

- **Three key stakeholders must be involved in making the change: industry, patient groups and regulators**
  - In addition, journalist, NGOs, legislators, internal company legal representatives and doctors should be included in any new guidance development

- **Guiding principles have to be cascaded top down from the top of industry organisations**
  - The principles need to be pushed from the Chief Executive Officer (CEO) and the Chief Medical and Scientific Officers to ensure researchers across organisations involve patients and advocates

Potential solutions identified during this breakout session

- **A small group led by EUPATI need to take this guidance/framework issue forward in a stepwise fashion and scope out a number of possible options, which are not mutually exclusive to one another**
- **The aim of guidance should be clearly defined by this group working in partnership with other stakeholder groups**
- **EFPIA has offered to take the action from this workshop and drive discussion via their Compliance Committee**
- **Find the right forum to discuss and drive guiding principle forward;**
  - DIA EuroMeeting (Paris 2015)
  - DG Enterprise was another
  - Upcoming regulatory meetings
  - Upcoming HTA meetings

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