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EDITORIAL

# Towards Meaningful Engagement for the Patient Voice

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Everyone is talking about the importance of 'the patient voice' in drug development [1–6]. New, more aggressive advocacy is shaking leaves and limbs at regulatory agencies and drug developers around the world. Legislation requires the US FDA to consider it as a regular part of its regulatory considerations [7], and pharmaceutical companies understand its value in their clinical development plans. But what is it, really, this 'new' patient voice? What is its current impact, and where is it going in the future?

## 1 A CIOMS Expert Working Group

The Council for International Organizations of Medical Sciences (CIOMS), an international, non-governmental, non-profit organization established jointly by the World Health Organization [WHO] and the United Nations Educational, Scientific and Cultural Organization [UNESCO] in 1949) [8] has created an international expert Working Group to help advance patient involvement in the development and safe use of medicines [9]. The purpose of the Working Group is to develop pragmatic approaches for the involvement of patients as key stakeholders in the development and safe use of medicines. Specific aspects of this involvement will include patient input into the development of medicines in areas of medical need; the collection and reporting of safety information; and the use of technologies involving patients for safety communication between stakeholders, including patients, healthcare professionals, pharmaceutical companies, regulators, and academia.

The Working Group goal is to formulate points to consider for the optimal consideration of patient perspectives and preferences to support the safe and effective use of medicines throughout their lifecycle—from discovery to clinical and market use—to improve the health of individual patients and the public. I am honored to be a member of the CIOMS Working Group. Having just returned from Geneva and a 3-day meeting of the Working Group, I wanted to share some thoughts and observations on this urgent public health initiative.

The traditional role of the patient voice in drug development has been to share the human component of disease. To date, this has largely meant the sharing of personal, emotional anecdotes. 'Save my child' (or 'my mother', or 'my spouse', or 'me'). These highly charged stories certainly help to make the drug development process more three-dimensional. But does 'playing the victim card' result in 'meaningful engagement'? Anecdotes have impact, but is it impact of the right kind, of the most powerful nature? No. The plural of 'anecdote' is not 'data'. Regulatory actions are, and must always be, data-based.

Patient passion is important but it must be combined with a more dispassionate, scientific understanding of regulatory paradigms. The 21st century patient voice can and must evolve into a tool used to impact regulatory decision making from both the heart and the head. That is the true pathway to meaningful engagement, the Tao of the patient voice.

## 2 Is the Patient Voice a Burden or a Benefit?

Senior voices from inside the FDA [10] and the European Medicines Agency (EMA) [11] speak out regularly about the importance of a more evolved patient voice in regulatory decision making. The same is true within the upper echelons of the pharmaceutical industry. I know these people. They mean what they say. But can the same be said for the rank-and-file drug reviewer and pharmaceutical executive?

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## 3 Enthusiasm is Common, but Commitment is Rare

Just as important as not allowing the emotions of an anecdote to overwhelm the realities of data (the dangers of vaccine denialism come to mind [12]) is not to assume that commitment 'from the top' equates to an enterprise-wide embrace of advancing the role of the patient voice in regulatory decision making. Based on my numerous conversations with line reviewers inside regulatory agencies on both sides of the Atlantic and around the world, there remains significant doubt as to the wisdom of more actively engaged patient advocacy. The same is also true within the legal and regulatory cloisters of Big Pharma.

How can we begin to address this invisible and insidious road block to progress? As Oscar Wilde quipped, "The truth is rarely pure and never simple". Shame on us all if we allow a recognized obstacle to go unaddressed, even if it is politically incorrect to do so. If you cannot measure it, you cannot manage it.

## 4 How do you Activate the Soul of the Reviewer and the Developer?

In the words of W. Edwards Deming, "Change is not required. Survival is not mandatory". What is the best way to win over drug developers and reviewers to the brave new world of advancing patient involvement in the development and safe use of medicines? Perhaps, as part of the CIOMS Working Group agenda, we can design and field a questionnaire to gauge the yin and yang of how more robust patient engagement is viewed at the working level of regulatory agencies and pharmaceutical companies.

The information gleaned from this type of research will help us to understand not just who, but also why. One likely truth is that those doing the work do not want the ground rules to change. That is just human nature. According to Rosabeth Moss Kanter, Professor of Business Administration at Harvard Business School, "The best tool for leaders of change is to understand the predictable, universal sources of resistance in each situation and then strategize around them" [13]. Change has an interesting way of affecting people that can often result in resistance. This resistance can range from fairly subtle, such as avoidance or passive aggressive behavior, all the way to outright defiance, hostility, and sabotage. The best way to avoid resistance to change is to seek to uncover potential resistance *prior* to implementing change [14]. Sometimes cognitive maps are the hardest to read. We need only undertake a cursory literature search to encounter the many passive/aggressive commentaries on topics such as patient-reported outcomes (PRO) [15] and quality-of-life (QoL) measurements [16] and their value (or lack thereof) in the drug review process.

## 5 How Can the Healthcare Ecosystem Become More 'Woke' to the Patient Voice?

Just as the FDA assiduously collects, reports, and learns from its various Prescription Drug User Fee Act (PDUFA) measurements [17], perhaps the agency can develop a metric to help identify where more effort is required to inculcate the karma of patient engagement in both the review and post-marketing surveillance of medicines. One idea is to develop a 'WOQUE' (Wide-Open Quality Engagement) score that can then be compared, review division to review division, in order to devise best practices and drive broader acceptance. Driving professional staff 'WOQUE-ness' can become a new PDUFA requirement. The time to have this conversation is now since the process for PDUFA reauthorization has just begun.

Similarly, inside pharmaceutical companies, how can scientists and their legal and regulatory review teams be similarly 'WOQUEd'? We must (perhaps with a polite shove from the CIOMS Working Group) design a 'learning health system' that enables research to influence practice and practice to influence research [18]. A timely example (and one to study carefully for the lessons it teaches us) is how a more educated and focused patient voice has changed and advanced the standards and practices of expanded access ('compassionate use') [19, 20].

## 6 Are Patients Really Equal Partners?

As with any ecosystem, the component parts of drug development and review are not necessarily equal to each other, but they are all requirements for success. What the patient voice must fight for is equal respect and a recognition of mutual value. It is not a question of 'equal' but of 'integral'.

In order to develop a more integrated role for the patient voice, to help advance patient involvement in the development and safe use of medicines, requires more than dynamic statements from the healthcare ecosystem's senior leadership. We must strive to develop the rules and measurements for success. One size will not fit all patients, regulatory systems, or pharmaceutical companies.

As the CIOMS Working Group effort moves forward, it will be useful for us to remember and internalize the wisdom of J.M. Eisenberg (former Director of the US Agency for Healthcare Research and Quality), "Globalize the evidence, localize the decision".

## Compliance with ethical standards

**Conflicts of interest** Peter J. Pitts reports no conflicts of interest.

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