

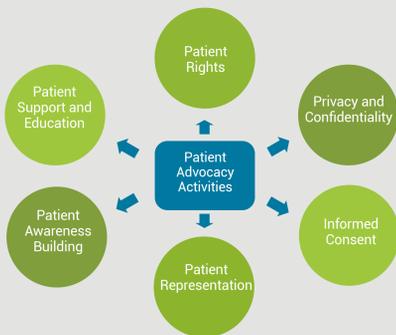
# Patient Advocacy and Changing Paradigm in Drug Access

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## INTRODUCTION : WHAT IS PATIENT ADVOCACY?

- Patient advocacy is a health care specialization involving patient advocates, who provide advocacy support for patients, survivors, and care-givers.
- A patient advocate may be an individual or an organization
- Often, patient advocates specify their involvement to a specific group of disorders
- Patient advocates give a voice to patients, survivors and their carers on healthcare-related (public) forums

## COMPONENTS OF PATIENT ADVOCACY ACTIVITIES



- **Role of Patient Advocacy in Drug Development :** to ensure appropriate patient representation in all aspects of drug development and approval

## PATIENT PARTICIPATION IN DRUG DEVELOPMENT AND APPROVAL

- In the past, patient involvement was limited to being recipients of drugs and as tools to observe the effects of the drug
- With increase in patient awareness, trend is changed in today's scenario
- In addition to disease-related measures, patient-reported outcomes (PROs) are increasingly being obtained during drug development, as a part of regulatory requirement
- Patient involvement was even enhanced in Patient-centric clinical trials (PCCT), where patients are involved from the stage of protocol preparation
- Currently patient involvement is considered imperative in the development, review and dissemination of evidence-based knowledge on health treatments, technologies and services
- Patient involvement is supported by legal and regulatory requirements
- Substantial research and deliberation have been done to realize the value of patient and citizen involvement in drug development and approval process

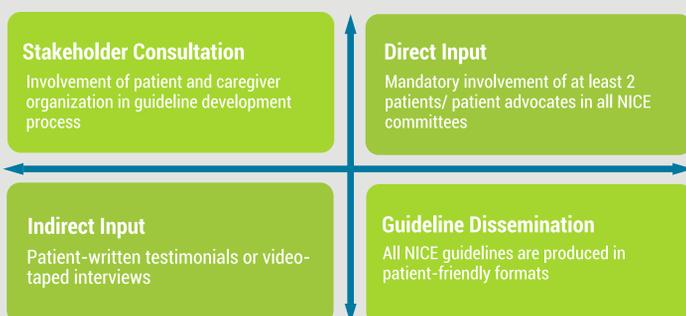
## PATIENT PARTICIPATION : MEETING THE UNMET NEED

- Pharmaceutical innovations failed to effectively meet patient's needs on several occasions:
- Mismatched priority between pharmaceutical innovation, research interest, and actual patient needs
  - **Overlooking of questions vital to patient's needs :** Researchers tend to emphasise more on chronic and severe health conditions, rather than acute and more common health problems
  - **Difference in end points of interest between researchers and patients :** For example, treatment of atrial fibrillation: physicians emphasis is avoidance of adverse consequences, whereas patient emphasis is avoidance of stroke as an outcome

## LEVELS OF PATIENT INVOLVEMENT



## NICE (UK) APPROACH FOR PATIENT INVOLVEMENT<sup>2</sup>



## EMA APPROACH FOR PATIENT INVOLVEMENT<sup>3</sup>

Patient advocates participate as full members with voting rights in the following EMA committees:

- Committee for Orphan Medicinal Products (COMP) since 2000.
- Patients' and Consumers' Working Party (PCWP) since 2006.
- Paediatric Committee (PDCO) since 2008.
- Committee for Advanced Therapies (CAT) since 2009.

## USFDA APPROACH FOR PATIENT INVOLVEMENT<sup>4</sup>

### Patient Representative Program

- Patients participate in FDA decision-making process
- Patient representatives recruited to advice on drugs, devices and biologics

### Drug Development Patient Consultant Program

- Patients participate in drug review and regulatory process

### Open Public Hearings

- Organised in every advisory committee meeting

## IMPACT OF PATIENT INVOLVEMENT ON RESEARCH PROCESS AND OUTCOMES<sup>5</sup>

- Increase in range of research topics
- Identification of new themes
- Highlighting issues of importance to patients
- Pushing science forward
- Adoption of a more ethically acceptable research design
- Improved trial consent procedures

## IMPACT OF PATIENT INVOLVEMENT ON PATIENTS AND PROFESSIONALS

### Impact on Patients

- Increased knowledge, confidence and support
- Feeling of overburdening, reliving distressing memories

### Impact on Professionals

- Deep understanding of patient issues
- Feeling of indifference

## CASE STUDY 1 : VERTEX PHARMACEUTICALS<sup>6</sup>

Orkambi, is a new combination drug combining Ivacaftor with a new drug, Lumacaftor and indicated for management of patients with cystic fibrosis. In clinical studies, Orkambi, demonstrated a modest 3 percentage-point improvement in lung function after six months. No clinically meaningful improvements were observed in self-reported respiratory symptoms (cough, wheeze, congestion, sputum production, and difficulty breathing), as measured by respiratory domain of the Cystic Fibrosis Questionnaire-Revised (CFQ-R). Surprisingly 12 of the 13 FDA panel members voted in favour of Orkambi. The main reason for the positive recommendation from FDA was the well-organized and compelling patient advocacy accompanying Vertex Pharmaceutical's presentation at the panel hearing. Seventeen patient advocates shared their experiences of taking Orkambi and claimed that the drug had a positive effect on their lives beyond what the clinical data indicated. In rare and orphan diseases where there is a desperate need for new therapies this example highlights the power of the patient voice in healthcare decision-making

## CASE STUDY 2 : SPROUT PHARMACEUTICALS<sup>7</sup>

Sprout Pharmaceutical's daily pill Flibanserin also referred to as the "Little Pink Pill" is intended to boost the female sex drive. Flibanserin was rejected twice by the FDA on the grounds of severe side-effects that far outweighed its benefits. However testimonies of women suffering from hypoactive sexual desire disorder, as well as the practitioners treating them swung the pendulum in favour of Flibanserin. The drug received recommendations by three quarters of the 24 FDA panel members

## PATIENT ADVOCACY IN INDIA<sup>8</sup>

- Many regulatory bodies, scientific review committee, ethics committee and Non-governmental organization (NGO) work towards patient rights and protection in India
- Prominent Health Advocacy Organizations in India include Alagille Syndrome Alliance, CARES foundation, CSS patient Group and Komen Advocacy Alliance
- Their main objective being to ensure patient's right and protection
- Unfortunately none of these organizations truly qualify as a patient advocacy group.
- India should aim to establish patient advocacy groups which provide special attention to patient care and protection of rights from the planning stage rather than at the troubleshooting stage

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