Patient Advocacy and Changing Paradigm in Drug Access

INTRODUCTION: WHAT IS PATIENT ADVOCACY

- Patient advocacy is a health care specialization involving patient advocates, who provide advocacy support for patients, survivors, and caregivers.
- 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 voice to patients, survivors, and their carers on healthcare-related (public) forums.

LEVELS OF PATIENT INVOLVEMENT

- 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 increased 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 is supported by legal and regulatory requirements.
- 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 patients' needs on several occasions.
- Misaligned priority between pharmaceutical innovation, research interest, and actual patient needs.
- Overlooking of questions vital to patient's needs.
- Substantial research and deliberation have been done to realize the value of patient and citizen involvement in drug development and approval process.

LEVELS OF PATIENT INVOLVEMENT

- Arnstein's eight rungs of participation ladder:
  - Information: Patients learn about drug development;
  - Consultation: Patients discuss drug development;
  - Collaboration: Patients work with professionals;
  - Delegated power: Patients have some decision-making power.

NICE (UK) APPROACH FOR PATIENT INVOLVEMENT

- Stakeholder Consultation: involves patient and care provider organizations in guideline development process.
- Direct Input: mandatory involvement of at least 2 patient/patient advocates in all NICE Guideline.
- Indirect Input: Patients written testimonials or video taped interviews.
- Guideline Dissemination: NICE guidelines are produced in patient friendly formats.

EMA APPROACH FOR PATIENT INVOLVEMENT

- 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

- Patient Representative Program:
  - Patients participate in FDA decision-making process.
  - Patient representatives recruited to advise on drugs, devices and biologics.
  - Drug Development Patient Consultant Program.
  - Patients participate in drug review and regulatory process.
  - Open Public Hearings.
  - Organized in every advisory committee meeting.

CASE STUDY 1: VERTEX PHARMACEUTICALS

- Orkambi, is a new combination drug combining two drugs and as tools to observe the effects of the drug.
- 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, symptom production, and difficulty breathing), as measured by respiratory domain of the Chronic Respiratory Questionnaire.
- Patients' and Consumers' Working Party (PCWP) since 2006.
- Patients' and Consumers' Working Party (PCWP) since 2008.
- Patients' and Consumers' Working Party (PCWP) since 2009.

CASE STUDY 2: SPROUT PHARMACEUTICALS

- Sprott Pharmaceuticals’ daily pill Fibranseer also referred to as the ‘little Pink Pill’ is intended to boost the female sex drive.
- Fibranseer 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 Fibranseer.

REFERENCES

- Penchina and Sons (2006).