

Taking Patients as Partners in Research

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INTRODUCTION

- 'Patient engagement' is an umbrella term covering all the interactions that patients have with their healthcare system.
- Patient engagement in research is an upcoming interest.
- Historically, "experts create, patients participate" in research.
- Nowadays, growing interest in involving patients from an earlier stage of research.
- There is growing interest in more fully engaging patients in the research process.¹

WHY INVOLVE PATIENTS IN RESEARCH DESIGN?

- Patients have a personal experience of disease that is not available to most researchers, which complements researchers' analytical skills and scientific perspective.
- Involving patients in research projects improves both the methodology and outcomes of the research.
- Patients often are able to provide invaluable additional insights about selecting appropriate research parameters and solving practical difficulties.

HISTORY OF PATIENT ENGAGEMENT IN RESEARCH

- The first major government-supported initiative to engage patients and the public in health-related research was the United Kingdom's National Health Service INVOLVE program, launched in 1996.²
- INVOLVE trains and encourages patients and the public to participate in all levels of research from design to review to dissemination.
- Activities included in INVOLVE project:
 - Working with research funders to prioritise research
 - Offering advice as members of a project steering group
 - Commenting on and developing research materials
 - Undertaking interviews with research participants.

METHODS OF PATIENT ENGAGEMENT IN RESEARCH

Patient Reported Outcomes (PROs), PCOR, and PCORI

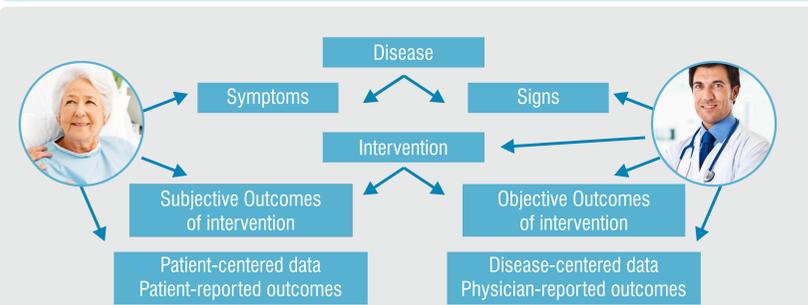
Patient Focused Drug Development

Patient-Centric Clinical Trials

Patient Advocacy

PATIENT-REPORTED OUTCOMES (PROS) AND PATIENT-CENTRED OUTCOMES RESEARCH (PCOR)³

- PRO are any report of the status of a patient's health condition that comes directly from the patient, without interpretation of the patient's response by a clinician or anyone else (USFDA)
- There has been an increasing call by the authorities that clinicians use PROs in healthcare decision making, and also in clinical trials
- Analysis of 'patient-centered' and 'patient-reported' data is the core aspect of PCOR
- Focus is now shifted from 'clinical effectiveness' of a treatment modality to 'quality of life improvement' it can bring



- In 2010, the PCORI (Patient-Centered Outcomes Research Institute) was created in the US.⁴
- PCORI is a U.S. non-profit non-governmental organization, which focuses on creating and promoting research that includes patients and caregivers.
- PCORI's aims include funding research that can help patients and those who care for them make better-informed decisions about the healthcare choices they face every day, guided by those who will use that information.

PATIENT-FOCUSSED DRUG DEVELOPMENT⁵

- Patient-Focused Drug Development (PFDD) is a new initiative from the Food and Drug Administration (FDA) intended to bring patient perspectives into an earlier stage of product development.
- The goal is that patients will be able to provide context for benefit-risk assessments and input to review divisions, and also aid in the development of new assessment tools, study endpoints, and risk communications.
- USFDA conducts regular PFDD meetings involving patients to determine the acceptability of new drug approval.

Public Meeting on Patient-Focused Drug Development for Autism

On May 4, 2017, FDA is conducting a public meeting on Patient-Focused Drug Development (PFDD) for Autism. FDA is interested in obtaining patient perspectives on the impact of autism on daily life and patient views on treatment approaches.

Date: May 4, 2017

Time: 1:00 p.m. - 5:00 p.m.

Location: FDA White Oak Campus
10900 New Hampshire Ave.
Building 31, Room 1503 B and C (Great Room)
Silver Spring, MD 20993
(Information about arrival to FDA's White Oak campus)

Registration: To register for this meeting, visit: [Public Meeting on Patient-Focused Drug Development for Autism](#)

PATIENT-CENTRIC CLINICAL TRIALS (PCCT)

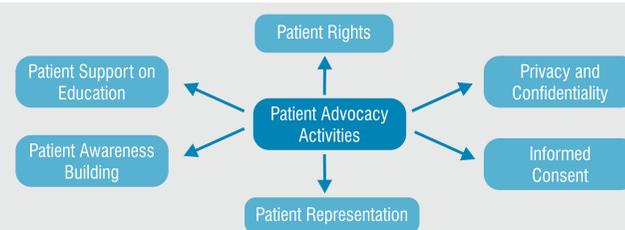
- PCCT is an emerging concept in the Western world.
- Patient participation in the RCTs is enhanced.
- Patients are involved from the stage of protocol preparation.
- RCT data is made easily available to the participating patients.

PATIENT ADVOCACY⁶

- A patient advocate is a healthcare specialist (individual or organization) who, as a liaison between patients and healthcare providers, argues with the best interest of the patient in view, in order to maintain or improve the quality of healthcare delivery to the patients that he/ she is representing.
- Role of Patient Advocacy Groups:
 - Promoting patient engagement in health policy decision making.
 - Promoting health literacy.
 - Ensuring the availability of adequate health information.
 - Building a strong patient's voice.

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BENEFITS OF INCLUDING PATIENT PERSPECTIVES IN RESEARCH⁷



IMPACT OF TAKING PATIENTS AS PARTNERS IN RESEARCH

- 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.
- Enhanced recruitment rates.
- Improved quality of data on using patients as co-researchers and interviewers.
- Enhanced power and credibility of the findings, leading to wider and more accessible dissemination.

PATIENT ENGAGEMENT IN RESEARCH: INDIAN PERSPECTIVE

PROs

- Clinical trials in India have started to use PROs as primary outcome measures.
- Indian researchers have started to realize the importance of PROs in improving the validity of their findings.
- At present there are no proper guidelines for conducting proper PCORs in India.

PCCT

- At present PCCT is lacking in India.
- Majority of Indian practitioners are yet to become familiarized to the concept of involving patient inputs in therapy and clinical trials.

Patient Advocacy

- 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.

CONCLUSION

- Effective patient engagement is a time consuming process and involves significant investment.
- Mutual trust and integrity are essential between all stakeholders of research.
- Patient engagement in research will allow the patient voice to impact clinical research.
- Studies involving engagement of patients as full-team members in their research programs would have a better reporting of patient-reported outcomes, which are more meaningful in the real world than pure clinical trial data.