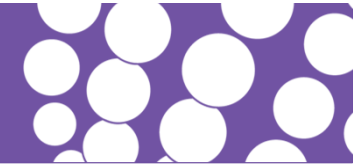


MOVING MEDICINES TO MOVE PEOPLE



MitoAction Seminar
February 12, 2021



Patients as Partners in Drug Development

Changing perceptions of roles in drug development

- We have come a long way since the streptomycin trial of 1948
- Previously, patients 'done to' as clinical research 'subjects'
- Now, drug developers, patients, regulators, payers and other stakeholders are talking but it's early days
- Future, full integrated timely involvement of all stakeholders including patients in the drug development process
- Why: Common goal of enhancing the quality and speed of drug development with better health outcomes for all

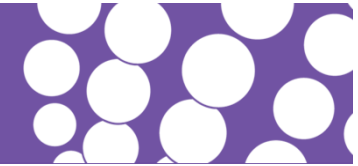


Oregon State TB Hospital open air pavilions
WikiMedia Commons

Drug development is difficult: how does partnership help?

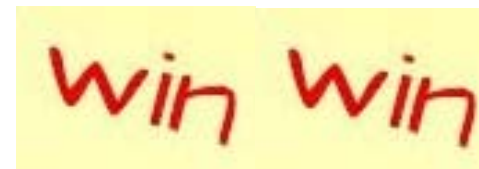
- Pharma is good at the science but patients and carer givers understand the burden of disease:
 - Unique perspectives based on the patient/carer understanding of the lived experience
 - This identifies important knowledge gaps and may inform research approaches
 - Patients can now access quality information on disease area and drug development (e.g. patient advocacy groups and internet)
 - Patients understand and expect more of their clinical study experience
 - Regulators ask about the patient experience (e.g. Patient Reported Outcomes [PROs])
- Paradigm shift from drug development directed solely by the science and the sponsor to one driven by the needs of real patients and their caregivers

What's in it for patients and pharma?

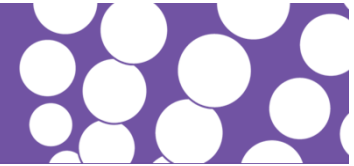


- These insights may lead to
 - Better **patient** experience which may lead to
 - Increased study participation with reduced study dropouts and a
 - Better **quality study**, faster than if patients are not partners in the process
 - i.e. better drugs for patients get to market faster with patients as partners than without

- *Patient perspective really matters*



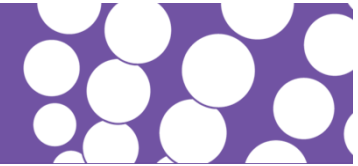
Closing the gap: Models of partnership



- Gap between aspiration and reality:
 - Explore models of engagement to see what works for patients, caregivers, sponsors, investigators and site staff
 - Identify where the partnership adds most value which will vary by programme
 - Staying within the guard rails

- What does pharma need to do to facilitate partnership?

Facilitation of partnership



- Pharma recognizes the value of patient partnership:
 - Pharma companies need to build frameworks to support the partnership process
 - People to lead experience and engagement activities
 - Consideration of Ethics/ Institutional Review Board approval for activities
 - Timing
 - Budget etc.
 - One size does not fit all
- In order to partner well, with patients in the design and execution of studies , pharma needs to invest time, thought, effort, people and resource into building effective collaborations

win win

Downsides

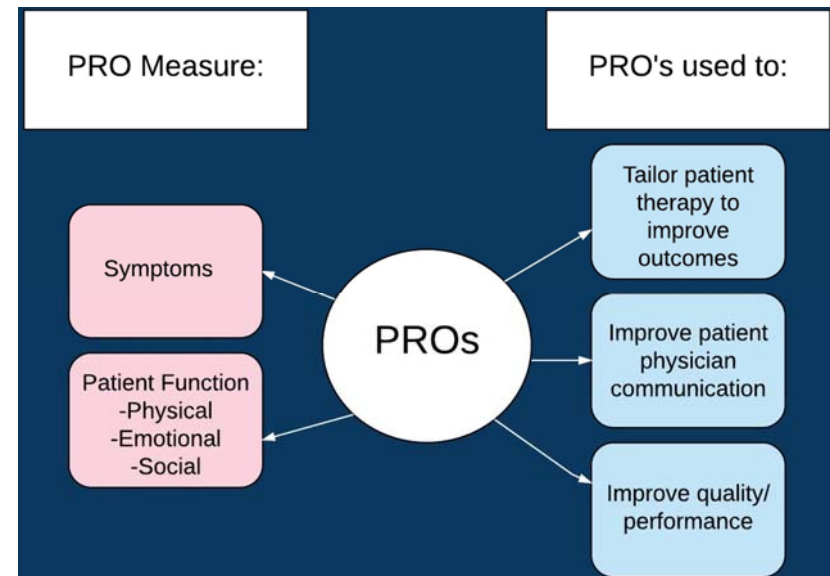
- Unreasonable expectations from either party- no magic wand!
- Lack of alignment
- 'Fashion' or jumping on a band wagon
- It takes time and consistency

Examples of specific areas in which partnership makes a meaningful difference – (rare diseases focus) (1)

- Understanding disease burden (epilepsy/FAOD examples)
e.g.:
 - limited published information focusing on the medical perspective
 - disease burden surveys across a wider population
 - natural history studies
- What is the unmet need?

Examples of specific areas in which partnership makes a meaningful difference – (rare diseases focus) (2)

- Identifying appropriate disease end points (PMM example)
 - There may be some established measures (which may be needed for regulatory reasons) but what endpoints really matter to patients?
 - Can these be identified early on and evaluated for utility in the early studies and then if useful carried forward into late phase studies?
 - Quantitative (a measurement- but what?) and qualitative (PROs)



BMJOpen <https://bmjopen.bmj.com/content/8/10/e021532>

Examples of specific areas in which partnership makes a meaningful difference – (rare diseases focus) (3)

- Education of pharma by the Expert patient- does pharma understand the disease area? (Neurology example)
- Advisory Boards
 - Study design -> protocol /review
 - Outcome: Are we studying the things that matter to patients as well as the regulators? (e.g. PROs)
 - Information for study subjects (Patient Information Sheet):
 - Does this make sense to a patient?
 - Is it too burdensome?
-

Examples of specific areas in which partnership makes a meaningful difference – (rare diseases focus) (4)

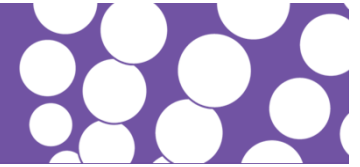
- Mutual discussion of benefits and risks
- What can the therapy do? E.g.
 - Adverse effects
 - No impact
 - Stabilisation of disease
 - Improvement
- My trade offs or those of a regulator in terms of benefit and risk may not align with those of the patients (e.g. HIV therapies; oncology; epilepsy; neurology etc)

Examples of specific areas in which partnership makes a meaningful difference (5)

Patient and patient advocacy partnerships with FDA/others:

- HIV treatments (1980's)
- Duchenne Muscular Dystrophy (2016)
- Mitochondrial disorders 2014: "Nutritional Interventions in Primary Mitochondrial Disorders: Developing an Evidence Base"
 - Pan-stakeholder workshop at NIH –nutrition/ interventions/ research opportunities AND forging collaborations among researchers, clinicians, patient advocacy groups, and federal partners

Personal learnings (1)



1) Listen

- Always listen carefully.
- Identify what is needed from the partnership and be brave!
- Do expectations align? Be honest.
- Many ways to collaborate fruitfully including for example,
 - Build relationships with patient organisations like this one
 - Focus groups
 - Interviews
 - Patient platforms and networks
 - Advisory panels and so forth

Personal learnings (2)

2) Use the tools at our disposal

Regulators increasingly ask about patient experience.

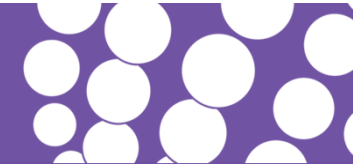
- The FDA's 21st Century Cures Act intended to promote innovation particularly in areas of unmet need, shows that patients are front of mind.
- Highly conscious of the tensions in rare diseases where patients have very limited treatment options/ research opportunities and show real altruism.



gg61048731 GoGraph.com



Personal learnings (3)



- 3) Build trust
- No misunderstandings
- Ensure clarity and alignment of goal and direction of travel of all stakeholders
- Where we cannot align, “own it” and agree to disagree.
- Once a goal is agreed, assign clear responsibilities, timelines and contacts
- Ideally, everyone over-delivers and under promises
- When you don't know the answer, say so

Why I believe in patient partnership:

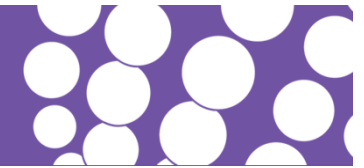


- Many positives have been discussed already.
- For me, true patient partnership is fundamental to
 - reducing patient burden,
 - answering the research questions that matter to the users of the medicine and
 - impactful drug development

i.e. getting the right medicines to the right patients at the right time



Patients as partners in drug development



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Thank you