

Rare Disease Clinical Trials

Module 3: Team Approach to Managing a Clinical Trial Participant

Heather Lau, MD
Director, Lysosomal Storage Disease Program
NYU Langone Health

Clinical Research vs Medical Care

Misconceptions

- “Patients who are trial participants and who do not adequately appreciate the purpose and methods of research studies are ill-equipped to evaluate risks and benefits of study participations, and may fail ‘to recognize how personal care may be compromised by research procedures’.”

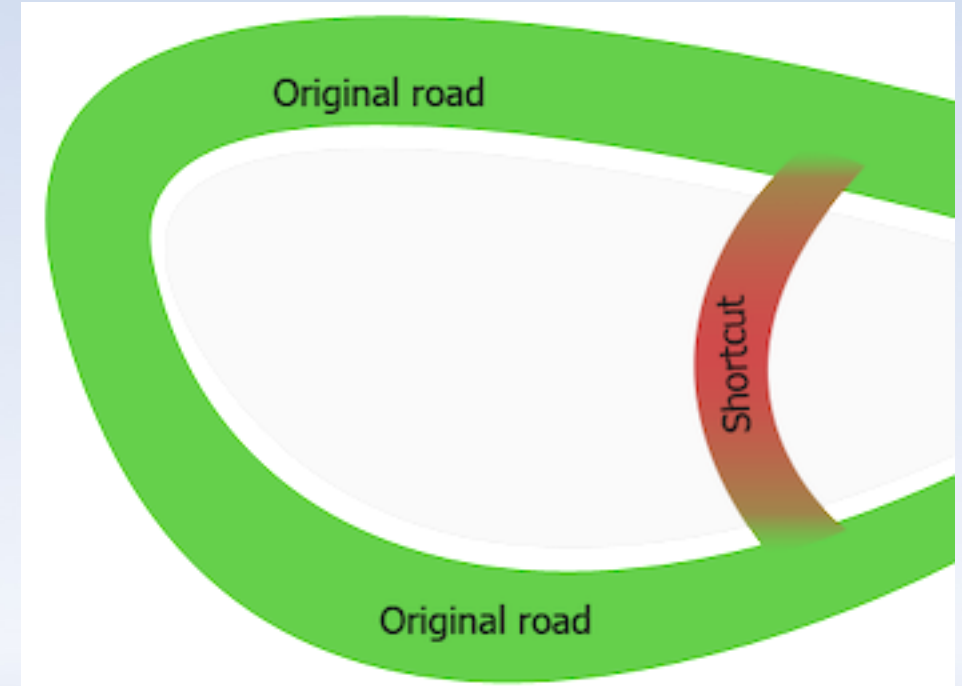
Patient's rationale for entering a trial?

	Major reason	One of the major reasons	Yes, but wasn't really why	Small factor	No, this was not a reason
Help future patients	34%	41%	13%	6%	6%
Improve their QoL	33%	36%	12%	7%	7%
To receive the best care	31%	32%	15%	7%	15%
To receive up-to-date therapy w/ot \$\$	25%	28%	14%	9%	24%
Extend their life	20%	21%	10%	11%	38%
Follow doctors orders	13%	19%	10%	9%	49%

Clinical Research vs Medical Care

Misconceptions

- Patients may view a clinical trial as a means to get better medical care
- While better medical care is desirable, the purpose of clinical trials is to generate useful information for future patients
- This misconception can lead to participants wanting to leave the study early



Supporting/Managing the Clinical Trial Participant

Support and educate the patient

- Make sure patients understand the commitment
- Make sure they understand that they can drop out at anytime
- Use language that is appropriate
- Review the study goals, outcomes measures
- Review timelines, procedures
- Review benefits/risks of the study



Roles and Responsibilities

Primary Care Team Role

- Contact Principal Investigator/Study Site to establish roles and responsibilities
 - Share with medical team
 - Share with patient/caregiver

Sharing Information/Duties

- What procedures/data will be performed by the primary care team vs clinical trial team?

Adhering to Protocol

Dos and Don'ts

- Being part of a clinical trial will require strict adherence to:
 - Scheduled appointments
 - Treatment protocol
- Participants may want to use medications/treatments that are not approved by the clinical trial

Communications

How to talk to a clinical trial team

- Establishing communication lines:
 - Best practices for working with research sites

Understand the key players

- Principal investigators and study coordinators
- Clinical Research Organizations (CROs) and their personnel

Communications

How to engage with the patient

- Best practices
- Support the patient and caregiver
- Extra time likely needed during appointments



Summary

- There are many misconceptions about clinical studies
- Goal of the care team is to manage the person so they can succeed if they enter a clinical study
- Goal of the care team is to support the patient's decision
- Communications is key to success