WHAT DOES A PATIENT-FOCUSED CLINICAL TRIAL REALLY LOOK LIKE?

DISRUPTING THE CURRENT CLINICAL TRIAL PARADIGM

SCOTT SCHLIEBNER
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WHAT DO THESE TERMS REALLY MEAN?

PATIENT-FOCUSED
PATIENTRICITY
PATIENT-CENTRIC
PATIENTS AS PARTNERS
PATIENT-CENTERED
DIRECT-TO-PATIENTS
PATIENT CENTEREDNESS

“When you/your organization include and involve patients and their perspective in every decision you make”
IS TRULY “PATIENT-FOCUSED” REALISTIC?

• A CRAWL, WALK, RUN APPROACH
• AT WHICH STAGES IS PATIENT INVOLVEMENT MOST CRITICAL?
• WHAT DOES THE END USER/CONSUMER WANT?
• MINIMIZE THE BURDEN OF PARTICIPATION
THE WORLD’S FIRST CLINICAL TRIAL

- Recorded in the “Book of Daniel” in the Bible.
- Conducted by King Nebuchadnezzar of Babylon.
- Ordered his people to eat only meat and drink only wine, a diet he believed would keep them in sound physical condition.
- But several young men of royal blood, who preferred to eat vegetables, objected. The king allowed these rebels to follow a diet of legumes and water—for 10 days.

- When Nebuchadnezzar’s experiment ended, the vegetarians appeared better nourished than the meat-eaters, so the king permitted the legume lovers to continue their diet.

CURRENT PARADIGM: CLINICAL TRIAL LIFECYCLE

1. Approved protocol
2. Investigator selection
3. Approval process
4. Participant recruitment
5. Data filed & registration obtained
6. Presentation & publication of results
7. Data analysis
8. Data entered & reviewed

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WHAT IS WRONG WITH THIS PICTURE?

• PROTOCOL DESIGNED WITHOUT INPUT FROM SITE STAFF OR PATIENTS
• IS THE SCHEDULE OF EVALUATIONS REALISTIC?
• DOES THE STUDY APPEAL TO PATIENTS?
• HOW MUCH WILL PARTICIPATION COST ($)?
• HOW BURDENSOME WILL PARTICIPATION BE?
THIS IS OUR COLLECTIVE EXPERIENCE

BUT HOW DO PARTICIPANTS, PATIENTS, AND CAREGIVERS FEEL?

WE ASKED THEM.
WHAT WAS YOUR PRIMARY MOTIVATION FOR PARTICIPATING IN A CLINICAL TRIAL?

1. ADVANCEMENT OF RESEARCH INTO MY DISEASE

2. NO OTHER TREATMENT OPTIONS AVAILABLE

3. TO RECEIVE ACCESS TO HIGH QUALITY MEDICATION

4. CURRENT APPROVED TREATMENT IS NOT WORKING
WHAT WERE THE MOST CHALLENGING BARRIERS YOU FACED WHILE PARTICIPATING?

1. TRAVEL TO/FROM SITE VISITS
2. TIME COMMITMENT
3. FINANCIAL STRAIN
4. STRAIN ON FAMILY AND/OR CAREGIVERS
5. INVASIVE PROCEDURES; CULTURAL BARRIERS; ACCESSIBILITY; SIDE EFFECTS

PRA Health Sciences Rare Disease Patient Survey:
Data collected 01Aug18 – 30Sep18: N=47

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IF YOU HAVEN’T PARTICIPATED IN A CLINICAL TRIAL, WHY?

1. DID NOT MEET ELIGIBILITY CRITERIA
2. CLINICAL TRIAL WAS NO LONGER ENROLLING
3. THE TRIAL SITE WAS TOO FAR AWAY
4. IT WAS NEVER OFFERED TO ME
5. SOCIAL, EMOTIONAL, FINANCIAL; UNFAMILIAR WITH THE PROCESS; SCARED

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HOW DO WE OVERCOME THESE CHALLENGES AND BARRIERS?

HOW DO WE BRING CLINICAL TRIALS DIRECTLY TO PATIENTS AND FAMILIES?

WHAT DOES A SUSTAINABLE, FEASIBLE, AND PATIENT-FOCUSED PARADIGM LOOK LIKE?
CLINICAL TRIALS SHOULD FIT SEAMLESSLY INTO A PATIENT’S LIFE

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THE VIRTUALITY OF CLINICAL TRIALS

**WHAT DOES A PATIENT-FOCUSED CLINICAL TRIAL REALLY LOOK LIKE?**

- **SPIROMETERS**
- **GLUCOMETERS**
- **12-LEAD EKG**
- **BLOOD PRESSURE**
- **PULSE/OX**
- **SCALE**

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WHERE ARE WE HEADED?

THE BIGGEST HOTELIER IN THE WORLD…
…HAS NO HOTEL ROOMS

THE LARGEST TAXI SERVICE IN THE WORLD…
…OWNS NO VEHICLES

OUR FUTURE CARS…
…WILL HAVE NO DRIVERS

THE FUTURE OF CLINICAL TRIALS…
…WILL REVOLVE AROUND PATIENTS AND SEAMLESSLY FIT INTO PATIENT'S LIVES.
THANK YOU!

SCOTT SCHLIEBNER
Senior Vice President
Center for Rare Diseases
PRA Health Sciences
SchliebnerScott@prahs.com

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