Rare Disease Patient Journey Map

Participant Profile:

Patient Name:
Tina Stanley

Age: **32**
Sex: **Female**

Residential Location:
Bangor, ME

Occupation:
Accountant

Patient background:

- Insulin-dependent diabetic
- Bladder dysfunction
- Sleep disturbance

Other considerations:

- Sole proprietor of an accounting business
- Married
- Mom of 2 children

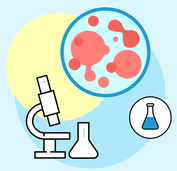
What characteristics do these patients have that may affect their ability to participate in a clinical trial/DCT?

- Travel
- Transportation
- Trust to site
- Technology Use
- Access to Tech and Internet

- Often experience vision loss:
- Many unable to drive
- Used to working with a KOL (doctor they know well)
- Patient works full-time so scheduling may be an issue
- Tech savvy but clinical trial naive

Clinical Trial Detail (Fictional Example)

Study Title:
A phase Ib/2a



Duration:
24 weeks

Number of Sites/Countries:
US only, 1 site in St. Louis, MO

Number of Participants:
15

Number of visits: **7**
In person: **4**
Virtual/Home Health Care: **3**

ePRO:

- Quality of life survey
- Medication diary
- Symptom dairy

Dose and format:

- Oral administration, stable at room temperature.
- Medication taken 2x/daily.

Inclusion:

- 18-64 years of age
- Lab-confirmed (via genetic testing) diagnosis of Wolfram-like syndrome

Exclusion:

- No clinically significant non-Wolfram related CNS involvement
- No unstable medical conditions other than Wolfram-like syndrome
- No unstable psychiatric disease

- Diary
- Home health nurse visit
- Smart watch
- Smart pill bottle
- Telehealth visit
- Study-related technology portal
- Additional diary on medication ingestion

Journey Stage

Pre-trial / Prescreening

How a patient learns about the study:

1 Direct to patient outreach via key opinion leader or patient registry

2 Patient referral from treating clinician, nonprofit, friend or family members

3 Patient reaches out and contacts a site or key opinion leader upon diagnosis OR reaches out to a nonprofit

Enrollment/Consent

Visit 1

Informed consent discussion takes place in person with study nurse though care partner or loved one may also be present

This is the first chance to build rapport.

Participant may want to share with referring clinician/local doctor.

Additional screening performed to confirm eligibility.

Medication dispensed via Smart pill bottle

Provide and train on study-related technology

Study Participation - Schedule of Activities

Visit 2-6

Information collected:

- Labs
- Vital signs
- Diaries/Questionnaires via eCOA/ePRO/Diary
- Diary entry after dosing
- Diary for surveillance during trial

What are the main challenges for the participant?

- Diagnosis
- Awareness of clinical research as a care option (CRAACO)
- Lack disease awareness
- Technology
- Engagement
- Risk of COVID w/ home visits?

Study Closeout - Schedule of Activities

Visit 7

Post-study closeout:

- Results shared through Snow
- Foundation as well as sponsor

Key considerations

Key Participants

Patient, treating clinician, caregivers

Patient, treating clinician, caregivers

Patient, treating clinician, caregivers

Patient, treating clinician, caregivers

Healthcare Team

Primary care doctorr

Primary care doctor; clinical trial team

Primary care doctor; clinical trial team

Primary care doctor; clinical trial team

Empathy Mapping:

Thinking/feeling/doing

Is it safe?

What is the likelihood the trial will benefit me?

What impact might my participation have on my loved ones?

What is the likelihood my participation will benefit others with my condition?

Can I trust this person/technology with my personal information?

Want to check with treating physician for endorsement?

Concerns about technologies. (eConsent, patient portals, etc) used

Consider how patients may feel if it is determined they do not qualify at this stage

Am I ready to give up face to face visits?

Do I have enough trust in capabilities?

How to build a relationship between patient and site?

Need to understand what comes next

Pain Points

Barriers retention and engagement blockers

Diagnosis

Awareness of clinical research as a care option (CRAACO)

Lack disease awareness

Technology

Engagement

Risk of COVID w/ home visits

General distrust of the medical or pharmaceutical establishment

Internet access

Lack of follow up from site staff

Study invitation email may got caught in spam

Lack of understanding

Concerns about risk

Trial commitment

Scheduling

Transportation

What is motivators to join the study?

Orphan disease with no approved therapy

Desire to contribute to science

Access to cutting edge therapies
Improved clinical care

Contribution to science

Retention Barriers:

- Lifestyle impact (elderly are retired)
- Length of trial
- Transportation/travel time
- Technology
  (Tech savvy? Access to smartphones / computers?)

Study results are rarely shared

DCT Components

Trial is hybrid - with onsite and telehealth options

Consenting:

- Share ICF for review prior to consenting/screening (read from home)
- Utilize an engaging eConsent
- Multiple modalities for information sharing
- Knowledge checks
- Opportunities to ask questions

Post consent:

- Dose is dispensed and tech is provided.
- Training and onboarding may occur in a number of different ways depending on the study (in-person, in home via home health, or via telemedicine)

On Site Visits

- Initial and final labs are collected on site
- Personal thank you notes with information on how to access study data
- Initial workup captured on site at first visit, basepoint for all trial interactions

Telehealth/Virtual Visits

- Can take place at home or work
- Medication is given to patient at first visit, contained in smart pill bottle

Information is collected digitally and should be shared with care teams

Personal thank you notes with information on how to access study data

What do sponsors, sites, and tech providers need to run a trial like this?

Buy-in from internal teams and support with change management strategies

Education on how DCTs add value and keep the role of the site as important as it is now

Access to fit-for-purpose technology - hardware and software

Provide and explain the options to participants, they know what is best for them.

Site's might need hardware (e.g., iPad) to support the use of software tools such as eConsent

Site needs ability to connect to patient via Telehealth.

Site and Patients need access to patient portal, telehealth tools, and smart pill solutions.

Patient needs long term access to trial information

Site needs to support patients long term access.