

The Patient's Perspective: Patient Voices in Clinical Development

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About me

- South San Francisco resident
- MBA admissions consultant and business communications & etiquette trainer
- Rare disease advocate: **Epidermolysis Bullosa**



Journey to Epidermolysis Bullosa Diagnosis

- EB: A genetic skin condition that results in fragile skin
- Diagnosed with EB Simplex at birth
- Information on prognosis and treatments was relatively limited for most of my life
- Precise genetic mutation was confirmed much later (in my mid-20s)
 - Fun fact: Insurance denied the test 😊

My Introduction to Clinical Trials

- Until recently, no trials existed that were relevant to EB Simplex
- Visited the Stanford EB Clinic for the first time in January 2019
- Head physician connected me with the research coordinator for a clinical trial that was slated to begin later that year

My First Clinical Trial: Fall 2020 – Spring 2021

- **Oral anti-itch medication (Phase II/III)**
 - Hybrid: Two in-person visits (endpoints)
 - Weekly phone calls in between visits
 - Took 1 pill daily – received refills in the mail
 - Filled out a daily diary online (with an additional form weekly) for 3 months. Received email reminders
 - After conclusion of initial trial period, given the option to take open-label medication (3 months)

My Second Clinical Trial: Spring 2022 (6 weeks)

Topical gel to strengthen the skin (Phase I)

- One Zoom call prior to first in-person visit to review E-consent form
- Hybrid: 2 days of in-person visits at the beginning of the study, 1 day at the end of the study; Zoom calls weekly in between visits
- Underwent biopsies, blister formation procedure, and lab work during first visit and last visit
- Daily: Applied gel daily to 4 sites on body and filled out a diary on paper (in 2022!) for about 6 weeks
- Weekly: Filled out an additional form and took photos of each gel application site with a digital camera (in 2022!!)

What Worked Well

- Hybrid nature of trials
- Trial teams were compassionate, knowledgeable, and regularly expressed gratitude for my participation
- Goals of the trial were explained clearly in the consent form and during discussions
- Debit card was provided for ease of charging expenses
- Compensation was reasonable

What Was Challenging

Trial #1:

- Subjective scales in daily/weekly diaries
- Blood draw and EKG almost resulted in harm to skin
- False reading on EKG led to further testing
- Diaries were not mobile-optimized

Trial #2:

- Cumbersome nature of paper diaries and digital camera
- Same above issue with survey scales
- Initial confusion regarding duration of trial/suitable endpoint
 - Hyperpigmentation/redness referenced – common in non-Caucasian skin
- Technical malfunction of procedure machine
- Pain and healing time of biopsies

Patient-Centric Trials: My Recommendations (Part 1)

- **Patient journey** should be “mapped out” beforehand
- E-consent form review should be fully **digital**/online
- All diaries should be **digital** and **mobile-optimized**
- **Phones** should be used for photography
- Trial visits and procedures should be scheduled systematically, fully **considering the patient’s schedule** and upcoming activities in mind

Patient-Centric Trials: My Recommendations (Part 2)

- Patients' **physical limitations/needs** should be fully considered during all parts of the trial
- **Procedures**, after-procedure care should be fully **clarified** prior to participation
- **Telehealth** should be made available for urgent support
- **Survey scales** in diaries should be **calibrated** and discussed with patients in advance to **minimize subjectivity**
- Create open channel for **feedback**

Questions?