

Bringing Clinical Studies to the Home: A Case Study of a Pivotal Trial in Individuals with Pachyonychia Congenita

INTRODUCTION

In pachyonychia congenita (PC), mutations in keratin-expressing genes cause skin fragility and impaired skin barrier function, resulting in severe plantar pain, ultimately making walking difficult or impossible. Therefore, participation in a traditional clinical study, in which individuals are required to travel by air or car to a clinical site (possibly hundreds of miles away) several times during the course of treatment, can be challenging.

CASE STUDY

The design of the pivotal Phase 2/3 VALO Study of PTX-022 in individuals with PC was largely informed by first-hand patient accounts from the 2018 Externally-led Patient-Focused Drug Development Meeting with the FDA as well as recommendations from the Pachyonychia Congenita Project.

The study sponsor, Palvella Therapeutics, Inc. incorporated into the VALO Study protocol the use of:

1. Telemedicine
 - Clinical assessments including foot exams
 - Adverse event reporting
 - Compliance with medication
 - Technical support re patient diary application
2. Visiting healthcare practitioners
 - Collection of clinical samples, vital signs
 - Assistance with study drug accountability
 - Drug accountability

VALO VIRTUAL STUDY FACTS

- **203 home healthcare visits** have been conducted in the VALO Study (as of the date of this poster*)
- On average, study participants live **298.7 miles** from a study site
- The longest distance between a patient and a study site in the VALO study was **2,390 miles**

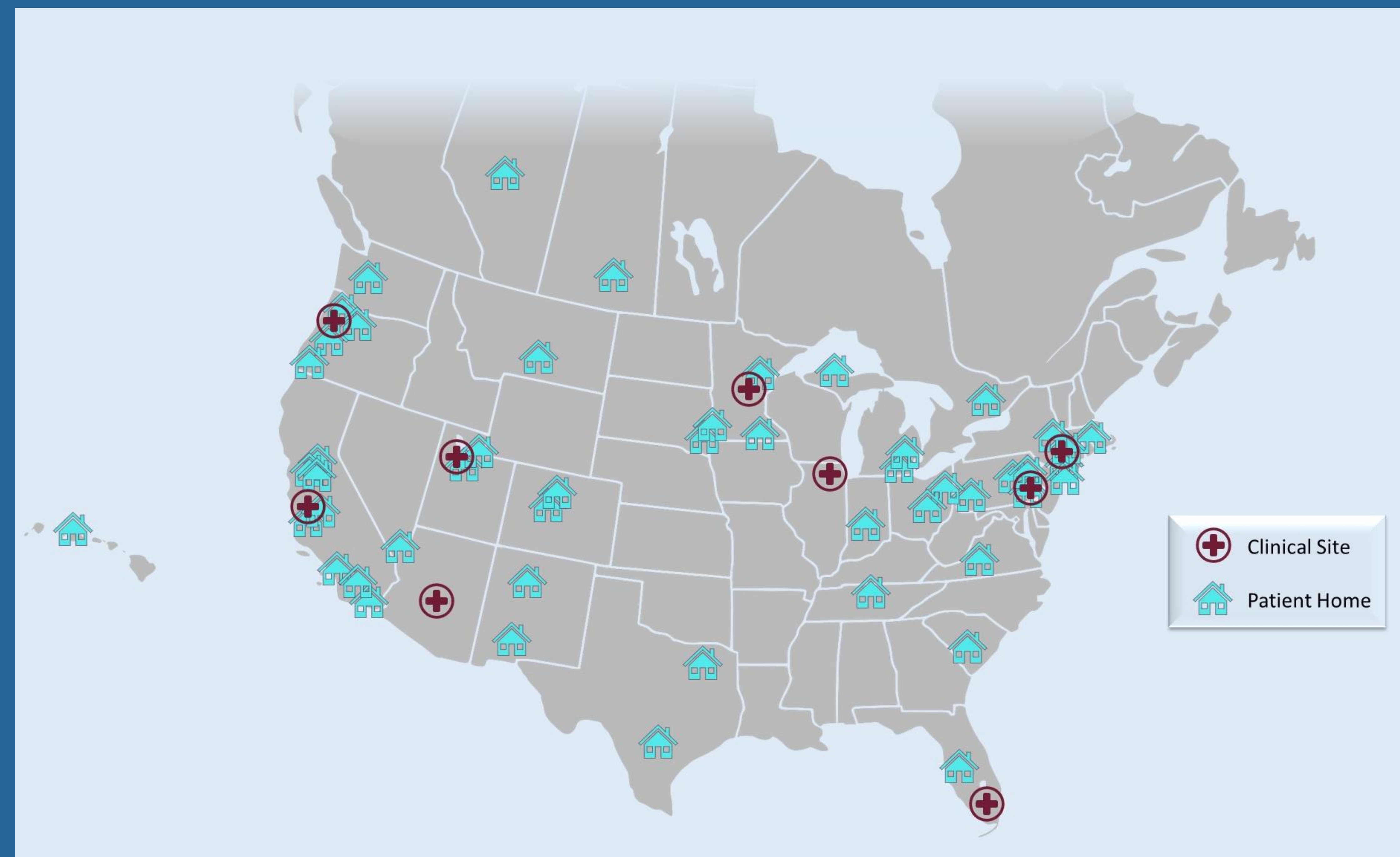
Patient-centric Approach Enables Individuals with Limited Mobility to Participate in Clinical Studies

VALO Participant Quotes:

"...it has been a great experience. Home visits were easy and ... even with international shipping and zoom calls, it was a very smooth process."

[The visiting nurse] "paid attention to my health and comfort ... as he worked through the step by step process guide. He was great to work with."

"The [healthcare worker] had to go above and beyond reason to venture my way. Did enjoy that I didn't have as many road trips."

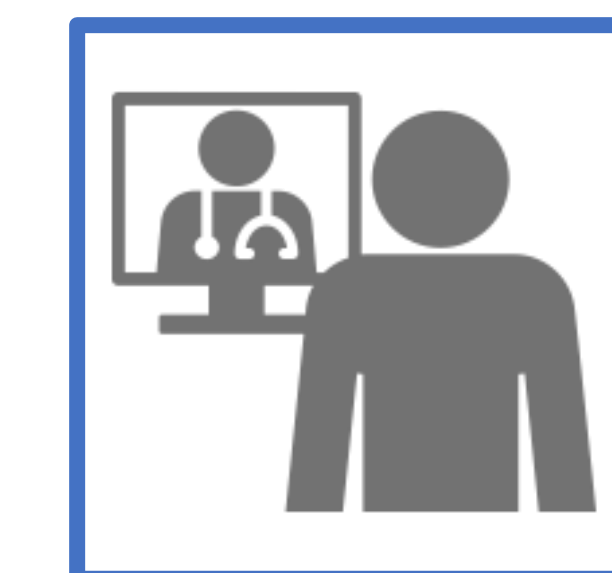


Given the challenges of enrolling sufficient numbers of rare disease patients in pivotal studies and the frequent geographic disparity among patients, virtual and home-based assessments offer significant benefits to sponsors:

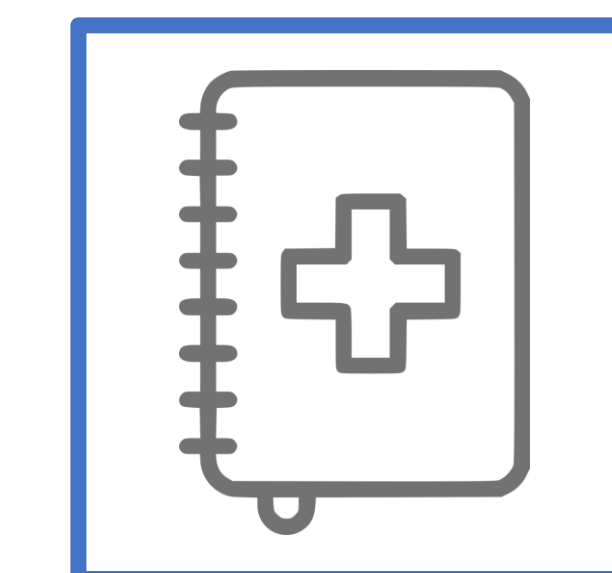
- Preserves one-on-one interactions
- Provides trial participants with access to expert physicians
- Encourages patient retention
- Allows for patient recruitment across a broad geography and access to underserved populations
- May reduce clinical trial costs and/or enrollment delays



Home health visits

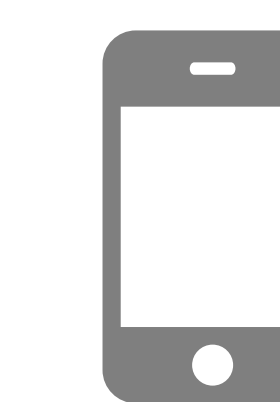


Telemedicine



Patient daily diaries

Take a picture to learn more



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Pachyonychia Congenita Project



www.pachyonychia.org

* NOTE: while recruitment has concluded in VALO, patient follow-up is still ongoing