**WHAT IS PTX-022?**

PTX-022 is a topical formulation of rapamycin, under development by Palvella Therapeutics to treat Pachyonychia Congenita (PC). Rapamycin has been shown to inhibit the production of a kinase called mTOR, which is involved in the production of certain keratin proteins. In disorders such as PC that are associated with overproduction of aberrant keratin proteins, the inhibition of mTOR may decrease the translation of mutant keratin-expressing genes and potentially block signaling pathways linked to skin fragility, keratoderma, pain and ambulatory impairment.

**FOR MORE INFORMATION**

For more information on VALO, PTX-022 or to inquire about participating in VALO:

- Contact PC Project (www.pachyonychia.org)
- Visit the VALO study website at (www.valostudy.org)
- Visit clinicaltrials.gov (NCT03920228)
- Visit www.palvellatx.com

**PC PROJECT AND PALVELLA THERAPEUTICS PARTNERSHIP**

The VALO study is Sponsored by Palvella Therapeutics in collaboration with the Pachyonychia Congenita Project. Since its founding in 2003, PC Project has connected PC patients, researchers and physicians throughout the world in a united effort to help those with PC. PC Project maintains the only PC patient IRB-approved registry, through which patients can access the most up to date advancements in research and receive free genetic testing.

**VALO** is a multi-center, Phase 2/3 study, evaluating the safety and effectiveness of PTX-022 in approximately 60 adults with moderate-to-severe Pachyonychia Congenita (PC) with either KRT6A, KRT6B or KRT16 mutations.
VALO STUDY
Overview of Study Visits*

STUDY PERIOD 1: SCREENING
4 WEEKS
1 clinic visit
- Informed consent
- Eligibility check
- Lab samples
- Start daily diary and wearing activity monitor
- Continue normal routine (foot care and activity level)
- Optional video interview

STUDY PERIOD 2: OPEN LABEL
12 WEEKS
3 clinic visits
- Eligibility check
- Daily dosing with PTX-022
- Continue daily diary and activity monitor
- Continue normal routine
- Lab samples
- Optional video interview

STUDY PERIOD 3: RANDOMIZED, DOUBLE-BLIND
12 WEEKS
3 clinic visits
- Eligibility check
- Twice a day dosing with either PTX-022 and/or placebo
- Continue daily diary and activity monitor
- Continue normal routine
- Lab samples
- Optional video interview

STUDY PERIOD 4: FOLLOW-UP
8 WEEKS
2 telephone calls
- No longer taking PTX-022 or placebo
- Phone call from study doctor to see how you are doing
- Continue daily diary and activity monitor, return at end of study
- Continue normal routine
- Optional video interview

* This is not a complete list of all study activities.

SUB-STUDY
If you are not eligible to enter the Open Label study period, you may be invited to participate in a 28-day sub-study that will look at the amount of PTX-022 in the blood.

28 DAYS
2 clinic and 4 home nursing visits

TRAVEL ASSISTANCE
Travel assistance will be provided for participants in VALO and up to one caretaker to accompany them. Transportation (airfare, trains, car services) and housing (hotels) will be booked for you through a travel service that specializes in helping participants in clinical trials.