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## **Clinical trial to treat rare skin disorder with siRNA technology**

*Salt Lake City.* PC Project, a public charity, announced that a Phase 1b clinical trial began today for treatment of pachyonychia congenita (PC) with a short interfering RNA (siRNA)-based therapeutic. PC is a dominant genetic disorder resulting from mutations in genes that encode keratins, which give structural integrity to skin cells.

Dr. Sancy Leachman, a University of Utah Dermatologist, is leading the study using a newly developed siRNA called TD101. This new drug has received the orphan drug designation (drug for rare diseases) by the US FDA under the Orphan Drug Act and was developed by TransDerm (Santa Cruz, CA) in collaboration with the International PC Consortium (IPCC), a group of physicians and scientists who agreed in 2004 to work together to develop therapeutics for this rare skin disorder.

Dr. Leachman, who heads the IPCC says, "Our siRNA formulation is designed to inhibit production of the mutated keratin so the normal gene can function correctly." Treatments based on the powerful new technology are just now coming to clinic. Recent siRNA clinical trials include treatment of age-related macular degeneration (AMD) and respiratory syncytial virus (RSV). TD101 represents the first use of siRNA technology in skin as well as the first siRNA which targets a mutant gene.

"We are pleased to be part of an effort to develop treatments based on our discovery in 1995 of the defective genes underlying PC. Although many of the gene mutations causing skin disorders have been identified, until now there has been a lack of effective therapy based on the genetic discoveries that we and others have made," says Professor Irwin McLean (University of Dundee, Scotland).

Dr. Roger Kaspar, CEO of TransDerm, says, "We feel fortunate to be part of an unprecedented effort involving a group of dedicated physicians, scientists, consultants, advocates and patients in pursuit of a common goal. We hope this drug can help the few PC patients that have the targeted mutation and that we can extend what we have learned to larger numbers of patients suffering from other untreatable dominant genetic disorders."

PC Project is a 501(c)(3) public charity dedicated to helping patients suffering from pachyonychia congenita. Says Ms. Schwartz, "This project is a remarkable collaboration between a lay public charity, industry, and academia working hand in hand." Contributors include the University of Utah (Sancy Leachman), University of Dundee (W.H. Irwin McLean and Frances Smith), Stanford University (Christopher Contag), Yale University (Leonard Milstone), Baylor College of Medicine (Dennis Roop now at University of Colorado), University of Saskatchewan (Peter Hull), BIOPOLIS/Singapore (E. Birgitte Lane) and University of Iowa (Rolland Poust). Industry partners include TransDerm (Roger Kaspar and Robyn Hickerson), Thermo Fisher Scientific/Dharmacon (Devin Leake), Elixin Pharma (Susan Srivatsa), Preclinsight (Doug Kornbrust), Visionary Therapeutics Corporation (Steve Hutcherson), Arena

Pharmaceuticals (Ajit Simh) , Agilent Technologies, Nucleic Acid Solutions Division (James Powell), Accugent (Ming Fai Chan) and GeneDx (Sherri Bale).

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