

# PeDRA POST

Spring 2015

An e-newsletter to update and inform PeDRA's collaborative research network



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In 2012, leading SPD pediatric dermatologists created the framework for a collaborative research network, the Pediatric Dermatology Research Alliance (PeDRA). The organization was an idea just over two years ago and today, it is a novel virtual organization that has already begun to have an impact on the volume and quality of research studies in pediatric dermatology. Establishing PeDRA has meant that pediatric dermatology leaders with a shared vision can begin to penetrate the many unmet needs in the field. This E-Newsletter—our first—conveys some of the progress of the collaborative research work that is taking place under PeDRA and illustrates the generous support PeDRA has received. *We hope you enjoy this issue!*



Pediatric  
Dermatology  
Research  
Alliance

# Updates from the PeDRA Research Study Groups

Collaborative groups of investigators from across the country have formed under the PeDRA umbrella to pursue specific studies.

## Birthmarks (and Procedural) Group

The birthmarks group has experienced some exciting developments recently. Importantly, the group's efforts now encompass both vascular birthmarks as well as pigmented lesions. One of our first multicenter studies regarding propranolol use in premature infants has completed enrollment and is in the data analysis phase. A multicenter genotype-phenotype study of children with vascular overgrowth syndromes and the PHACE syndrome registry are actively enrolling patients. On the horizon are several new studies in various stages of development that address the following: topical rapamycin and pulsed dye laser for capillary malformations, procedural and insurance coverage issues regarding treatment with pulsed dye laser, the need for routine ECG screening in infants treated with propranolol, treatment thresholds for systemic therapy for infantile hemangioma, comparative incidence of PHACE syndrome in large facial IH, quality of life [measurement] for children with vascular malformations, and basic science work regarding kaposiform hemangioendothelioma and lymphangioendothelioma. The pigmented lesion subgroup has identified important areas of study and is actively working on studies that include developing consensus guidelines for management of congenital melanocytic

nevi, the role of MRI to detect neurocutaneous melanosis, and mapping of primary congenital melanocytic nevi and satellite lesions. Please contact:

Megha Tollefson, [Tollefson.Megha@mayo.edu](mailto:Tollefson.Megha@mayo.edu)

Anita Haggstrom, [ahaggstr@iupui.edu](mailto:ahaggstr@iupui.edu) or

Joan Tamburro, [TAMBURJ@ccf.org](mailto:TAMBURJ@ccf.org)

if you are interested in joining this group.

## Inflammatory Research Group

### Atopic Dermatitis (AD) subgroup:

The group is happy to announce that the TREAT-US & Canada survey study has been completed. Thank you to everyone who participated - we anticipate sharing results soon as to provider preferences in use of systemic agents for severe AD. Our prospective, observational study of systemic therapies is being modified to better facilitate the data collection process—a call for additional sites to participate will be forthcoming. On the advocacy front, PeDRA submitted a written statement to the FDA Dermatologic and Ophthalmic Drugs Advisory Committee for a March 2015 meeting, advocating inclusion of children in clinical studies of new systemic agents for AD. Other projects in development include the examination of MRSA

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## PeDRA RESEARCH GRANTS

PeDRA has issued its first Request for Proposals for grants to support collaborative PeDRA studies.

Funding is available up to \$5,000 USD per project.

The application submission deadline is May 15, 2015, 5:00 PM EST.

**APPLY NOW!:**

<http://pedraresearch.org/grants>



## PeDRA TAKES ON FOUNDATIONAL QUESTIONS

### What does it mean to be a PeDRA Study?

PeDRA has formulated a policy that will ensure collaborative studies under the PeDRA umbrella are in line with the mission and vision of the Alliance and that ethical considerations are upheld. Watch for the policy soon on <http://pedraresearch.org>.

### What are the ethical issues in conducting collaborative research?

How does PeDRA handle authorship issues, security of intellectual property, confidentiality agreements, and defining the 'culture' of PeDRA? These are just some of the fundamentals that PeDRA is examining in a code of ethics that will be a critical component of PeDRA's foundation. If you are interested in this committee's work, please contact Lionel Bercovitch, M.D., Ethics Committee Chair, [Lionel\\_Bercovitch@Brown.edu](mailto:Lionel_Bercovitch@Brown.edu).

## INVESTIGATORS & PATIENT ADVOCATES

### The new research power couple

### A report from the Foundation for Ichthyosis & Related Skin Types

Engagement with patient advocacy organizations is an impactful initiative to advance scientific research. The Foundation for Ichthyosis & Related Skin Types® (FIRST), the global leader in ichthyosis patient advocacy, has witnessed tremendous growth within the patient advocate/medical investigator relationship.

In fact, since 2006, FIRST's Research Grant Program has independently funded over \$1.6 million in ichthyosis related research. FIRST's support has been instrumental in finding new genes that cause ichthyosis, unlocking the genetic basis for these diseases, supporting siRNA technology to directly target specific mutations, and helping the development of new topical products currently in clinical trial.

In 2014, FIRST awarded PeDRA member, Dr. Brittany Craiglow of Yale University, a \$50,000 research grant for her collaborative study of newborn and early childhood complications and comorbidities that accompany the



Jean Pickford, FIRST, stands with Keith Choate, MD, PhD, at the FIRST poster, 2014 PeDRA Annual Conference

ichthyoses. Dr. Craiglow notes, "At present, there is no standard of care for the management of babies and children with these disorders and therapeutic options are limited." This enhanced understanding will help these investigators establish accepted clinical standards of care that could be tailored to the genetic diagnosis. The study may also ultimately provide a foundation for the development of targeted therapies.

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## Investigators & Patient Advocates (Continued)

A thorough evaluation of infants and children with ichthyosis will help to fill a large void in the understanding of these disorders. FIRST anticipates that the results will be particularly helpful in counseling newly diagnosed families, which represents a significant part of FIRST's work as a patient support organization.

The physician-scientists funded by FIRST have enthusiastically engaged with the organization. Many have joined the Board of Directors, as well as FIRST's Medical and Scientific Advisory Board. Additionally, these physician-scientists are actively involved in patient communications, family conferences and patient support forums, and have welcomed the opportunity for advising and supporting the entire ichthyosis community.

As an active member of PeDRA, FIRST's deepening involvement with these investigators and their evolving role in the advancement of research, will serve as a model for other advocacy organizations. As is the cornerstone of PeDRA, FIRST embraces the strength of a collaborative network, realizing that the power of individual researchers multiplies by linking them together.

## Updates from the PeDRA Research Study Groups (Inflammatory, Continued from page 2)

decolonization practices and occurrence of mupirocin resistance, being led by investigators at Columbia University. Please contact:

Wynn Tom, [wtom@rchsd.org](mailto:wtom@rchsd.org) or

Larry Eichenfield, [leichenfield@ucsd.edu](mailto:leichenfield@ucsd.edu) if you are interested in joining this group.

### Psoriasis Investigator Group (PsIG):

The PsIG has several active projects and several in the planning phases. A few of note are as follows: The MAPP-US Survey (Management Approaches to Pediatric Psoriasis in the US) is in its final stages of development and will be deployed soon. The survey will assess 3 main areas of management of patients with moderate-severe psoriasis including severity measures, comorbidity assessment and systemic treatment approaches. Please watch your mail and participate in this important survey! A multi-center, international retrospective study to compare the efficacy and safety of systemic agents and phototherapy for pediatric psoriasis is in the data collection phase. This study will lay the foundation for desperately needed prospective studies. Studies in the planning phases include a series on patients with TNF-induced psoriasiform dermatitis in order to better understand the epidemiology, pathophysiology and treatment of this condition, and a project to evaluate and reconsider the current classification system of juvenile PRP. Please contact Co-Chairs: Kelly Cordoro, [cordorok@derm.ucsf.edu](mailto:cordorok@derm.ucsf.edu) and Amy Paller, [APaller@nmff.org](mailto:APaller@nmff.org) if you are interested in joining this group.

### Connective Tissue Disease subgroup:

The Connective Tissue Disease subgroup continues to conduct exciting research in a variety of disorders. Collaborations have been established with members of the Childhood Arthritis and Rheumatology Research Alliance (CARRA) in the fields of cutaneous lupus and amyopathic dermatomyositis. In fact, two PeDRA members will be attending the CARRA Annual Meeting in April to develop a multidisciplinary lupus project. Our morphea research also continues to expand, with receipt of a \$50,000 grant to fund

a multicenter trial to compare topical therapies for plaque morphea. Please contact: Yvonne Chiu, [ychiu@mcw.edu](mailto:ychiu@mcw.edu) if you are interested in joining this group.

### Hair, Nail and Autoimmune subgroup:

The Hair, Nail and Autoimmune Group is currently focusing on alopecia areata projects including comparative data on squaric acid vs. diphencyclopropenone. Parties interested in this project should contact Nanette Silverberg at [nsilverb@chpnet.org](mailto:nsilverb@chpnet.org).

### Acne subgroup:

The PeDRA Acne Investigator Group (AIG) is interested in identifying and supporting collaborative research into pediatric acne. Please contact: Albert Yan, [yana@email.chop.edu](mailto:yana@email.chop.edu) if you are interested in participating and have any ideas for collaborative projects.

## Genetic Skin Disease

### The Epidermolysis Bullosa Clinical Research Consortium:

Consortium (EBCRC) is transitioning leadership from Dr. Al Lane at Stanford University to Dr. Anna Bruckner at University of Colorado. The Clinical Characterization and Outcomes Database remains open but will be revamped after the move. The member sites of EBCRC are excited about the opportunities that change can bring and look forward to growing the EBCRC. Contact:

Anna Bruckner, [anna.bruckner@ucdenver.edu](mailto:anna.bruckner@ucdenver.edu) if you would like information about the Epidermolysis Bullosa Clinical Research Consortium.

### Disorders of Cornification subgroup:

The DOC working group is moving forward with a multicenter, prospective study of infants with congenital ichthyosis. Presently, there is a paucity of data to guide

the management of these patients and to predict which patient type will be at risk for serious complications, such as sepsis, electrolyte imbalance, and growth failure. The goals of the project are to describe the early natural history, evaluate incidence and severity of complications, and assess the efficacy of treatment methods among different clinical and genetic groups. We hypothesize that early complications and comorbidities are genotype-dependent, such that clinical standards of care can be tailored to the genetic diagnoses. Definition of clinical groups based upon phenotype may also provide useful predictors of outcome. Information gained from this study will provide the basis for the development of rational standards of care for the future management of children with these disorders. Please see Investigators & Patient Advocates article highlighting collaborative efforts between the FIRST organization, which funded the prospective ichthyosis study, and the DOC Study Group. If you are interested in this group, please contact:

Brittany Craiglow, [brittany.craiglow@yale.edu](mailto:brittany.craiglow@yale.edu)

Keith Choate, [keith.choate@yale.edu](mailto:keith.choate@yale.edu) or

Mary Williams, [elias.williams1@gmail.com](mailto:elias.williams1@gmail.com)

### Basal Cell Carcinoma Nevus Syndrome:

Currently there is still a lack of knowledge about the natural history of basal cell carcinoma nevus syndrome (BCCNS) in children. Thus, a long-term follow up study of children (and adults) with BCCNS would be valuable. Joyce Teng and Amy Paller are working with Kristi Schmitt Burr, Executive Director of the BCC Nevus Syndrome Life Support Network, to develop a centralized clinical database in the US. The registry is designed to contain complete clinical information regarding familial risk, clinical manifestations, disease onset, diagnostic tests results, screening and surveillance, and medical and surgical interventions received. With the

information, we will be able to: 1. Assess the prevalence of BCCNS in the United States; 2. Promote awareness, early and accurate diagnosis of BCCNS in pediatric population; 3. Characterize the disease manifestations based on genetic test results to make genotype phenotype correlations; 4. Improve management and early intervention of BCCNS in U.S. children.

There are emerging treatments for various manifestations of BCCNS in children. For example, Vismodegib was shown to be effective in reducing Keratocyst Odontogenic Tumors that presents in childhood for BCNS patients. However the toxicity in children is unknown. In addition, topical Itraconazole is currently being investigated to treat early BCC in adults. Centralized data collection will allow such collaborative research to evaluate the safety of new therapies in the pediatric population.

## Skin Tumors And Reactions to Cancer Therapies (STARC)

While skin tumors are rare in children, early detection and treatment can be critical to outcome. Currently, there is a lack of data on the diagnosis and management of both benign and malignant skin tumors due to the small numbers of cases seen at a single institution. Likewise, cutaneous complications of oncologic therapies, including drug reactions, GvHD, and secondary skin cancers, represent an understudied area of research investigation in pediatric dermatology. Collaborative multi-center efforts are essential in advancing our understanding of these conditions in children. Our research goals are to 1) Refine our understanding of the clinical presentation, histopathology, and management of primary skin tumors, 2) Generate longitudinal data on the natural history and outcomes of potential precursors of skin malignancy, and 3) Define and address unmet needs in cutaneous complications from oncologic conditions and therapies.



We welcome investigators of all levels of experiences to join us. Please contact: Jennifer Huang, [Jennifer.Huang@childrens.harvard.edu](mailto:Jennifer.Huang@childrens.harvard.edu) Larry Eichenfield, [leichenfield@ucsd.edu](mailto:leichenfield@ucsd.edu) or Heather Brandling-Bennett, [Heather.Brandling-Bennett@seattlechildrens.org](mailto:Heather.Brandling-Bennett@seattlechildrens.org) if you are interested in joining this group.

## Neonatal Skin

The neonatal skin group is working to identify and prioritize research objectives that will advance our understanding of skin care and skin disease in neonates. We aim to expand our research network and develop collaborations with pediatricians, neonatologists, nurses, and scientists with expertise or interest in fetal/newborn skin. We are actively developing new projects, including a multi-institutional study characterizing pustules in the newborn.

Please contact:

Raegan Hunt, [rdhunt@texaschildrens.org](mailto:rdhunt@texaschildrens.org) or Kara Shah, [kara.shah@cchmc.org](mailto:kara.shah@cchmc.org)

if you are interested in working with the neonatal skin group.

## THANK YOU

PeDRA would like to openly thank as well as highlight the generosity of many clinicians, researchers, patient advocacy organizations and skin disease patients and their families who have provided financial support to the organization. Collaborative research has launched and so much progress has been made in two short years, including the exciting news that PeDRA is now offering its first grant cycle! There are too many contributors to list—watch for the PeDRA annual report on [pedraresearch.org](http://pedraresearch.org) to see all supporters—but we wanted to highlight a few exemplary donors here:



Gordon Dow, Founder,  
Dow Pharmaceutical Sciences &  
Philanthropist

**When asked why he provided a generous donation to PeDRA, Gordon Dow had this to say:**

“Having spent my career developing new therapeutics for managing skin disorders, I appreciate the importance of new ideas and research to advance the field of dermatology. It is tremendously exciting for me to give back by supporting PeDRA in its efforts to foster research in pediatric dermatology, a relatively neglected area of research in dermatology.”

**Adult patients, such as Elly Werner, also recognize the urgent needs of children with skin diseases:**

“I was diagnosed with morphea several years ago. It has been a slow and frightening process. I consulted with a number of well-known physicians and found very few answers. I discovered that it existed in children with frightening results. I began to realize that much more scientific research in morphea was needed. We need to increase the funding for research on this area, and to open opportunities for study that will end the suffering caused by this affliction.”



Elly Werner

Interested in adding your name to the growing list of PeDRA supporters? Just contact Sheila Rittenberg, Acting Executive Director, [sheila.rittenberg@gmail.com](mailto:sheila.rittenberg@gmail.com) or donate online: <http://pedraresearch.org>

# IMPORTANT DATES & REMINDERS

We have formally launched the PeDRA membership program! Please apply to be a member at <http://pedraresearch.org/membership-info> to:

- » Enjoy first access to the PeDRA Annual Conference and Conference Proceedings;
- » Communicate with PeDRA Study Groups;
- » Receive announcements about clinical trials and
- » Collaborate with other researchers on the PeDRA website.

PeDRA bylaws have been enacted! Please review when you sign up for membership <http://pedraresearch.org/membership-info>.

 Look for PeDRA on Facebook! And a special thank you to Cyndee DeKlotz for setting up the page. Please like our [PeDRA Facebook page](#).

## Complete PeDRA's new Research Capacity Survey !

A benefit for PeDRA members will be a password-protected online search function to find other researchers with similar interests and capabilities for collaborative research. Please remember to log on to your website account and complete the Research Capacity Survey so that our PeDRA network can maximize the benefit of this feature. Find the survey at: <http://pedraresearch.org/account>

PeDRA members are excited about the wonderful research projects being borne within this organization. We welcome new collaborators as we continue to develop ideas and a stronger research network. If you have a particular research interest, we invite you to join us and find others with similar interests with whom you can collaborate on projects.

## SAVE THE DATES:

May 6-9, 2015

**Peds Derm Research at the Society for Investigative Dermatology**

Atlanta, GA

July 9-12, 2015

**SPD Annual meeting**

Boston, MA

*Including PeDRA Update Meeting Friday, July 10, 2015*

November 6-7, 2015

**PeDRA Annual Conference**

Fort Worth, TX

This newsletter is brought to you by the PeDRA E-Newsletter Committee:

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