

# PEDRAPOST



Pediatric  
Dermatology  
Research  
Alliance

AN E-NEWSLETTER TO UPDATE AND INFORM PEDRA'S COLLABORATIVE RESEARCH NETWORK | SUMMER 2016



## THIS ISSUE

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## Welcome

Welcome to the third issue of the *PeDRA Post*, an e-newsletter that shares research updates and efforts of the various groups that comprise our organization. As PeDRA begins its fourth year of expanding collaborative research, we are learning that when dedicated researchers band together, truly amazing things can happen. This partnership has allowed our Alliance to study rare diseases and help draw conclusions that impact patients afflicted with these often severe conditions. PeDRA is comprised of pediatric dermatology investigators spanning the globe that are committed to furthering research in the field while engaging the support of patient advocacy groups to help us shape priorities for studies. As we continue to grow and advance the field of pediatric dermatology research, there is reason for our patients to have hope for a brighter future!

– Kalyani Marathe, on behalf of PeDRA Communications Committee



## UPDATES FROM PEDRA STUDY GROUPS

Collaborative groups of investigators have formed under the PeDRA umbrella to pursue pediatric dermatology studies.

### *Birthmarks (and Procedural)*

The Birthmarks group has been actively conducting research on both vascular birthmarks as well as pigmented lesions. One of our first multicenter studies regarding propranolol use in premature infants was well received at the ISSVA meeting in Buenos Aires in April. Preliminary results from the multicenter genotype-phenotype study of children with vascular overgrowth syndromes were also presented at ISSVA and this study has recently been awarded a PeDRA grant for continued enrollment and analysis. A study examining the need for routine ECG screening in infants treated with propranolol has been accepted for publication in *Pediatric Dermatology*. Studies that continue to develop include topical rapamycin and pulsed dye laser for capillary malformations, procedural and insurance coverage issues regarding treatment with pulsed dye laser, 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 projects including developing vascular tumor cell lines. The pigmented lesion subgroup continues to work on studies that involve developing consensus guidelines for management of congenital melanocytic nevi, the role of MRI to detect neurocutaneous melanosis, and mapping primary congenital melanocytic nevi and their satellite lesions.

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#### **For more information, please contact:**

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

Anita Haggstrom ([aahaggstr@iu.edu](mailto:aahaggstr@iu.edu)) or

Joan Tamburro DO ([tamburj@ccf.org](mailto:tamburj@ccf.org))

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### *Genetic Skin Disorders*

#### **Epidermolysis Bullosa Subgroup:**

The Epidermolysis Bullosa Clinical Research Consortium (EBCRC) sites enroll patients in the EB Clinical Characterization and Outcomes Database (CCOD). The goals of the CCOD are to:

- 1) Identify well-described patient cohorts that may participate in future studies and therapeutic trials, and
- 2) Gather longitudinal data on the course, complications, and clinical interventions of EB, in order to develop and refine guidelines for best clinical practice.

There are nearly 500 patients enrolled in the CCOD. Having a broad cohort of EB patients eager to participate in additional studies was instrumental in the success of an observational study about pruritus (itch) in EB, led by Dr. Al Lane from Stanford University. In addition, a cohort of 104 patients with RDEB patients from the CCOD is being studied in order to better characterize the etiology of anemia in EB. This data will form the foundation for future studies regarding treatments for anemia in EB.

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#### **For more information, please contact:**

Anna Bruckner ([anna.bruckner@ucdenver.edu](mailto:anna.bruckner@ucdenver.edu))

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#### **Basal Cell Carcinoma Nevus Syndrome Subgroup**

Most of the patients with basal cell carcinoma nevus syndrome (BCCNS) are diagnosed between 17 to 35 years of age. Our research goal is to establish a centralized clinical database to enroll pediatric patients during early childhood. The registry will collect information including genotyping, disease progression, surveillance and interventions. Our objective is to improve the understanding of genotype-phenotype correlation of the disease as well as its manifestation and responses to interventions. We hope this coordinated effort will advance our knowledge about BCCNS that will lead to early diagnosis, interventions and better outcomes. There has been exciting development of therapeutics for basal cell carcinoma and BCCNS in adults. The safety of such new therapy can only be evaluated in pediatric patients through collaborative research. (See also BCCNS under STARC)

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#### **For more information, please contact:**

Joyce Teng ([jteng3@stanford.edu](mailto:jteng3@stanford.edu))

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## RESEARCH OPPORTUNITIES!



The newly combined PeDRA/SPD grant research program is calling for grant proposals. The application deadline for this current cycle is December 7, 2016. Grant recipients will be notified in February 2017. To apply, please visit <http://pedra-research.org/resources-for-researchers> and download application packets as appropriate.



Calling for volunteers! PeDRA is launching a task force to promote the submission of pediatric dermatology research grants for federal/NIH funding. Outstanding progress has been made in initiating our research studies. This task force will explore ideas for funding, and for mentoring early investigators, in order to increase NIH and other grant applications in support of PeDRA research studies. If you have grant writing experience and are interested in this task force, please contact Dawn Siegel, MD ([dsiegel@mcw.edu](mailto:dsiegel@mcw.edu)), 414-955-2819.



Do you have a survey you would like to disseminate to PeDRA members? PeDRA has to review and approve surveys before distribution. The application deadlines are August 15 and November 15! Please visit <http://pedraresearch.org/pedra-survey-policy> for application details.



2017-18 National Psoriasis Foundation research grants have expanded! Researchers interested in applying can now apply online at [www.psoriasis.org/grants](http://www.psoriasis.org/grants). Application deadline: Wednesday, October 12th, 2016. The NPF offers these grant opportunities: Discovery, Translational, Early Career Research Grants, and 2017 Public Health Challenge Grant.

### *Genetic Skin Disorders* Disorders of Cornification Subgroup:

The Disorders of Cornification Working group has obtained institutional review board approval and has begun to enroll patients in its Congenital Ichthyosis Prospective Study (CIPS) at selected centers. Protocols are in various stages of submission at several additional sites. This prospective study of newborns with ichthyosis is being conducted in order to help describe the early natural history of various genotypes and phenotypic groups. We hope that the results may ultimately serve as a foundation for the development of standards of care for the management of infants and children with these disorders. This project has been made possible by generous support from the Foundation for Ichthyosis and Related Skin Types.



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#### For more information, please contact:

Brittany Craiglow MD ([brittany.craiglow@yale.edu](mailto:brittany.craiglow@yale.edu))  
Keith Choate MD, PhD ([keith.choate@yale.edu](mailto:keith.choate@yale.edu))

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### *Inflammatory Skin Disorders* Acne and Hidradenitis Subgroup:

A new collaboration to describe patients with hidradenitis suppurativa has been initiated and is recruiting centers to participate. AbbVie has provided a grant to support this study, so participating centers will receive some funding to hire a student/trainee to collect data.

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#### If you are interested in participating, please contact:

Irene Lara-Corrales ([irene.lara-corrales@sickkids.ca](mailto:irene.lara-corrales@sickkids.ca))

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The PeDRA Acne Investigator Group (AIG) is interested in identifying and supporting more collaborative research into pediatric acne.

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#### If you are interested in participating and have any ideas for collaborative projects, please contact:

 Albert Yan ([yana@email.chop.edu](mailto:yana@email.chop.edu))

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### Atopic Dermatitis Subgroup:

The TREAT-US & Canada survey study has been accepted for publication in the *Journal of the American Academy of Dermatology*—thank you to everyone who participated! International efforts are underway to establish a core set of data/parameters that should be recorded as individuals receive systemic therapy. Please contact the study leads if you are interested in joining other ongoing projects: prospective, observational study of traditional systemic agents (Wynnis Tom, [wtom@rchsd.org](mailto:wtom@rchsd.org)); occurrence of topical antibiotic and antimicrobial resistance (Christine Lauren, [cat35@columbia.edu](mailto:cat35@columbia.edu)); and assessment of AD lesional morphology by race/ethnicity (Minnelly Luu, [mluu@chla.usc.edu](mailto:mluu@chla.usc.edu)). We also welcome other project ideas and encourage submission for discussion at the 2016 PeDRA Conference.

## UPDATES FROM PeDRA STUDY GROUPS

Collaborative groups of investigators have formed under the PeDRA umbrella to pursue pediatric dermatology studies.

### *Inflammatory Skin Disorders*

#### **Connective Tissue Disease Subgroup:**

The Connective Tissue Disease subgroup continues to grow with more members and more active studies. Collaborations with the Childhood Arthritis and Rheumatology Research Alliance (CARRA) have matured, and six PeDRA members attended the CARRA Annual Meeting in April 2016 to advance our interdisciplinary studies. PeDRA and CARRA are working together to develop consensus recommendations for evaluation, monitoring, and treatment of discoid lupus, plaque morphea, and juvenile dermatomyositis. Planning continues on a multicenter project to study the long-term outcomes of discoid lupus. The morphea registry continues to enroll patients, and genetic studies are ongoing. Data collection is underway for the morphea mapping study funded by a PeDRA collaborative grant. There are new projects looking at skin severity scoring of cutaneous connective tissue disease, and the JDM team is submitting a grant application to fund a comparative study of skin severity scores. The Connective Tissue Disease subgroup welcomes new members and is recruiting additional investigators for its studies; trainees and early investigators are encouraged to participate.

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#### **For more information, please contact:**

Yvonne Chiu ([ychiu@mcw.edu](mailto:ychiu@mcw.edu))

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#### **Hair, Nail and Autoimmune Subgroup:**

The Hair, Nail and Autoimmune Subgroup is currently focusing on hair AND nail projects. The alopecia areata projects include comparative data on squaric acid vs. diphenylcyclopropanone. There will also be a retrospective review of presentation, mycology, and response to therapeutics amongst children with onychomycosis.

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#### **Parties interested in this project should contact:**

Nanette Silverberg ([nsilverb@chpnet.org](mailto:nsilverb@chpnet.org))

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#### **Psoriasis Investigator Group (PsIG):**

The PsIG has been busy with several studies in various phases. The CAPP Survey (Clinical Approaches to Pediatric Psoriasis) has been deployed in the US and Canada and is now in the data analysis phase. The database is now closed for our retrospective review of systemic therapies and phototherapy for moderate to severe plaque psoriasis

in children. Data were presented at both the Society for Investigative Dermatology, the European Society for Pediatric Dermatology and the SPD. Ten PeDRA centers participated in the US and ten in Europe through their European Pediatric Psoriasis Working Group (EPPWG), and 445 patient records comprised the study population. A manuscript is currently in progress. PsIG was able to get funding from the International Psoriasis Council to support this study. A new natural history study for guttate psoriasis (GP) in 8-17 year olds (150 subjects) has been drafted with the goals of: i) determining the conversion rate to chronic plaque psoriasis (CPP) within 2 years; ii) identifying biomarkers in blood, cultures, and skin that may be associated with increases in conversion from GP to CPP; and iii) defining the gene expression and immune cell expression profile of GP. Severity, pruritus, and quality of life will be evaluated serially. PsIG is working with Janssen to fund this project with PeDRA, and we are looking for more investigator sites. Finally, a series on patients with TNF-induced psoriasiform dermatitis and a project to evaluate the current classification system of juvenile PRP are in the early phases.

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#### **If you are interested in joining this group, please contact:**

Co-Chairs Kelly Cordoro, ([cordorok@derm.ucsf.edu](mailto:cordorok@derm.ucsf.edu)) and Amy Paller ([apaller@northwestern.edu](mailto:apaller@northwestern.edu))

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### *Neonatal*

The Neonatal Skin working group project investigating pustules in afebrile neonates led by Christine Lauren has obtained IRB approval and data collection is in progress. The Neonatal Skin working group continues to grow and welcomes new research ideas and collaborations.

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#### **For more information, please contact:**

Raegan Hunt MD ([rdhunt@texaschildrens.org](mailto:rdhunt@texaschildrens.org))

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#### **HELP US HELP YOU:**

If you have moved, are no longer in a trainee program, or have changed any of your professional "stats," you can update your profile on the PeDRA listserv with a few easy keystrokes. This ensures collaborators and PeDRA-central can reach you when needed.

Please visit: <http://pedraresearch.org/account>, log-in, and then follow the prompts to User Profile and then Edit profile.

## *Skin Tumors and Reactions to Cancer Therapies (STARC)*

The Skin Tumors And Reactions to Cancer therapies (STARC) group has made great progress this year. We have 8 active projects. Four studies are in the data collection phase:

- 1. Melanoma and NMSC in children and young adults–**  
This retrospective study examines skin cancers in pediatric patients, with a focus on immunosuppressed children.
- 2. Retrospective evaluation of pediatric cutaneous mastocytosis–** This study looks at the clinical features and outcomes of children with cutaneous mastocytosis.
- 3. Pityriasis lichenoides chronica and mycosis fungoides–**  
This study aims to better characterize the presentations and outcomes of PLC and MF in children.

#### **4. Basal Cell Carcinoma Nevus Syndrome (BCCNS)**

**Clinical Registry–** This registry seeks to enroll patients to improve understanding of the clinical progression of BCCNS, evaluate treatments, and identify potential participants for future studies on BCCNS. (Please see also BCCNS under Genetic Skin Disorders).

The subjects of our other projects include chronic GvHD, pediatric melanoma, dermatofibrosarcoma protuberans, and patient education.

Our group welcomes new members and project proposals.

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#### **For more information, please contact:**

Jennifer Huang MD ([Jennifer.Huang@childrens.harvard.edu](mailto:Jennifer.Huang@childrens.harvard.edu)),  
Carrie Coughlin MD ([coughlincc@gmail.com](mailto:coughlincc@gmail.com)), or  
Larry Eichenfield MD ([leichenfield@rchsd.org](mailto:leichenfield@rchsd.org))

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## **INVESTIGATORS & PATIENTS**

This e-newsletter's research power couple: The PHACE Syndrome Community joins PeDRA's Annual Conference and helps shape research agenda.



**“It overwhelms us to see these experts paying attention to this underserved and under-appreciated disease syndrome.”**

– Jill Salas

Just three short years ago, the PHACE Syndrome Community was brand new and creating its volunteer leadership organizational structure. The group joined PeDRA's 2014 Annual Conference and quickly soared to become an advocate for research into PHACE syndrome, an uncommon association between large infantile hemangiomas, and birth defects of the brain, heart, eyes, skin and/or arteries. At the 2015 PeDRA conference, building on the discussions of the previous year, the Birthmarks Study Group breakout session turned its focus in part to PHACE and pressing research needs. Jill Salas, PHACE Syndrome Community founder, had an opportunity to highlight the critical role that early diagnosis plays in PHACE. Without early recognition and intervention, PHACE may manifest severe issues such as hearing problems, endocrine and growth problems, hemangiomas that

distort facial features, and disruption of other vital functions, such as breathing and vision. Other complications depend on structures involved but can include cardiac problems, developmental delay, seizures, headaches, and abnormal muscle tone, strokes and seizures – which are generally at their worst when the hemangioma is in its growth phase.

The Birthmarks group meeting at the 2015 PeDRA conference felt it could create a meaningful retrospective study looking at the diagnosis, misdiagnosis or lack of diagnosis of affected infants. In the study, Jill and the organization would reach out to the PHACE community for images of their children at early stages of the disease and would survey families about their experience with initial diagnosis. The physician scientists in the group agreed with Jill that early diagnosis is crucial from a patient perspective as well as a medical perspective.

Jill Salas says of the experience, “The more involved we get, the more we realize that PeDRA doing something great for research. It overwhelms us to see these experts paying attention to this underserved and under-appreciated disease syndrome. It's truly impressive to see how committed these doctors are—to their patients!”

<http://www.phacesyndromecommunity.org/>  
Find us on Facebook: **PHACE Syndrome Community Friends**

## PeDRA & SPD TEAM UP ON GRANTS/SURVEYS

PeDRA is committed to boosting pediatric research in spite of hurdles and inherent difficulties related to funding research in both rare and common diseases in young children. To this end, PeDRA and the Society for Pediatric Dermatology (SPD) decided to maximize resources and harmonize their respective programs by merging grants into one single program. The PeDRA/SPD program is **calling for proposals to compete for research grants totaling \$85,000 in 2016** as part of a competitive award program.

In May, the PeDRA/SPD program awarded the first cycle of grants:

### SINGLE INSTITUTION PILOT GRANTS:

Grant Recipient: **Christina Boull**

“Cutaneous Findings in Fanconi Anemia and Patient Perceptions of Skin Changes”

Institution: University of Minnesota

**Amount: \$5,000**

Grant Recipient: **Christine Lauren**

“TADPOLES Study: Texting Atopic Dermatitis Patients to Optimize Learning and EASI Scores”

Institution: Columbia University, New York

**Amount: \$7,500**

### MULTICENTER TEAM GRANT:

Grant Recipient: **Beth Drolet**

“Multicenter Phenotype-Genotype Analysis of Vascular Overgrowth Syndromes”

Institution: Children’s Hospital of Wisconsin/Medical College of Wisconsin

**Amount: \$22,000**

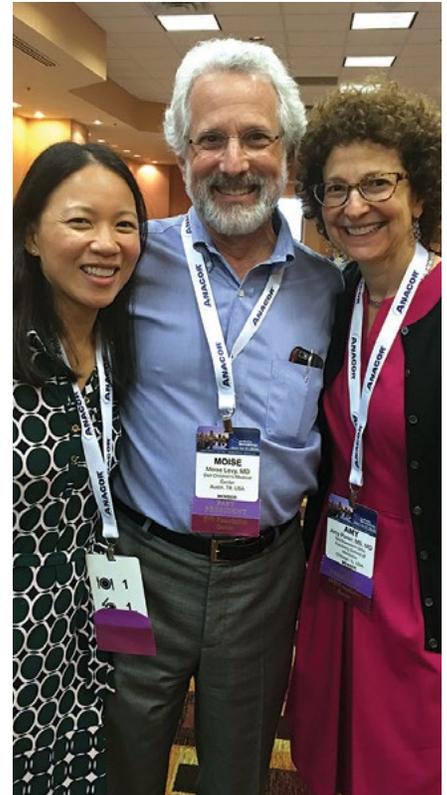


The application deadline for the second 2016 cycle is December 7, 2016. Grant recipients will be notified in February 2017. To apply, please visit <http://pedraresearch.org/resources-for-researchers> and download application packets as appropriate.

PeDRA and SPD have also aligned goals in vetting and staggering email requests for research surveys sent to our members. While in line with our mission to promote and facilitate high quality collaborative research in pediatric dermatology, we also are conscious of email and survey fatigue that may occur with too many requests. As always, participation in surveys is optional but very much appreciated, as your efforts will help our mission and vision!



PeDRA reviews and approves surveys before dissemination to our members. The application deadlines are August 15, 2016 and November 15, 2016. Please visit <http://pedraresearch.org/pedra-survey-policy> for application details.



### BASECAMP FOR PeDRA



PeDRA has invested in a multicenter document management program!

After reviewing a number of options, Basecamp was concluded to be the most user-friendly option with features that would facilitate centralized communications within PeDRA research subgroups. This will allow PeDRA members collaborating on research projects to share documents and improve editing flow, align project schedules, and more. Use of Basecamp is optional but we hope you find it to be a useful tool. The communications committee is teaming up with PeDRA research group leaders to help streamline the process (without sharing patient information or data, of course). We welcome any feedback you have on this process!

## EARLY INVESTIGATORS COMMITTEE MAKES PROGRESS

The mission of the Early Investigators Committee (EIC) is to provide mentorship and learning opportunities for junior faculty and trainees and advocate for their needs within PeDRA.

### This year, we launched our mentorship program!

- » We matched 20 mentor-mentee pairs who are meeting with each other quarterly. Mentees are providing questions to mentors in advance of their meetings to create a framework for discussion, and in turn, mentors are striving to share their pearls for success.
- » We will add participants to the mentorship program this fall, so stay tuned for an upcoming email posting!

### The PeDRA 2016 Annual Conference will feature an Early Investigator forum that includes:

- » A workshop on “What is a Good Research Question?” This session will focus on honing participants’ study ideas into informative and feasible study questions.
- » A panel discussion on “The first 5 years of my career.” This discussion will be led by mid-career and senior investigators who will discuss their challenges and accomplishments during their early careers.
- » A Mentor/Mentee “Meet & Greet” over dinner.
- » A Happy Hour for early investigators so we can get to know each other in a more casual setting!

### EIC position available:

- » We are recruiting a **Trainee Representative**. The representative will advocate for the needs of residents and fellows within PeDRA. He/she will have voting rights equal to other members on the committee. This is a great opportunity to get involved with PeDRA, and to ensure that our programs are inclusive of those aspiring to a career in pediatric dermatology! This position will be available to dermatology residents who have recently matched for pediatric dermatology fellowship.

### October grant writing webinar in the works!

- » Watch PeDRA emails for more details, coming soon.

You can reach the EIC with any questions or suggestions by emailing:

Jennifer Huang ([jennifer.huang@childrens.harvard.edu](mailto:jennifer.huang@childrens.harvard.edu)) or Lisa Arkin ([larkin@dermatology.wisc.edu](mailto:larkin@dermatology.wisc.edu)).

## DONOR SPOTLIGHT

We are indebted to the clinicians, investigators, patient advocacy organizations, industry partners, as well as skin disease patients and their families, who have been so generous to PeDRA and have made possible our rapid growth. In the year-end edition of the *PeDRA Post*, we will recognize many of these important supporters.



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



### GUIDANCE DOCUMENT FOR INDUSTRY (GDI) ON NEW THERAPEUTIC AGENTS FOR ATOPIC DERMATITIS (AD) IN CHILDREN AND ADOLESCENTS

PeDRA is a major sponsor of this initiative to optimize the substantial industry investment in anticipated clinical trials for pediatric atopic dermatitis (AD).

The lack of consistent approach to conducting pediatric clinical trials in AD, and the inconsistency in data reporting, affect the ability of physicians to evaluate comparative efficacy and safety of AD treatments and impacts on patient care. Consensus recommendations will help to standardize clinical trial design in this underserved population and may be an impetus (or even source) for an FDA guidance document on this topic.

Work groups have been meeting all year. Their work will culminate in a consensus meeting to be held in conjunction with the PeDRA Annual Conference in November.

For further information about this initiative, please contact

Elaine Siegfried ([esiegfri@slu.edu](mailto:esiegfri@slu.edu)).

## IMPORTANT DATES AND REMINDERS

### *Save the Date:*

**November 4-5, 2016**

The PeDRA 4<sup>th</sup> Annual Conference

Westin Hotel near Dallas-Fort Worth Airport (DFW)

Register here! <http://pedraresearch.org/upcoming-conference>

### *Reminders:*

- » **PeDRA grants:** Deadline for submissions for the next grant cycle is December 7, 2016. See article in this issue for details
- » **Membership in PeDRA is a two-step process!** Please apply for PeDRA membership if you haven't already: <http://pedraresearch.org/membership-info>. Once approved, you will receive a letter confirming your membership and inviting you to pay membership dues (\$150 for active members, free for trainees).
- » **Research Capacity Survey:** For those of you who have not yet completed the survey, please log in to the PeDRAresearch.org website and complete: <http://pedraresearch.org/account>

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

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<http://pedraresearch.org>

*Helping children with skin diseases  
through collaborative research*

Questions: Contact **Sheila Rittenberg**, PeDRA Executive Director, [sheila.rittenberg@pedraresearch.org](mailto:sheila.rittenberg@pedraresearch.org)



**Look for PeDRA on Facebook!** Special thank you to Cyndee DeKlotz, Facebook page master.