

THE POWER OF COLLABORATION: ADVANCING THE FIELD OF PEDIATRIC DERMATOLOGY

A report of 2016 PeDRA activities

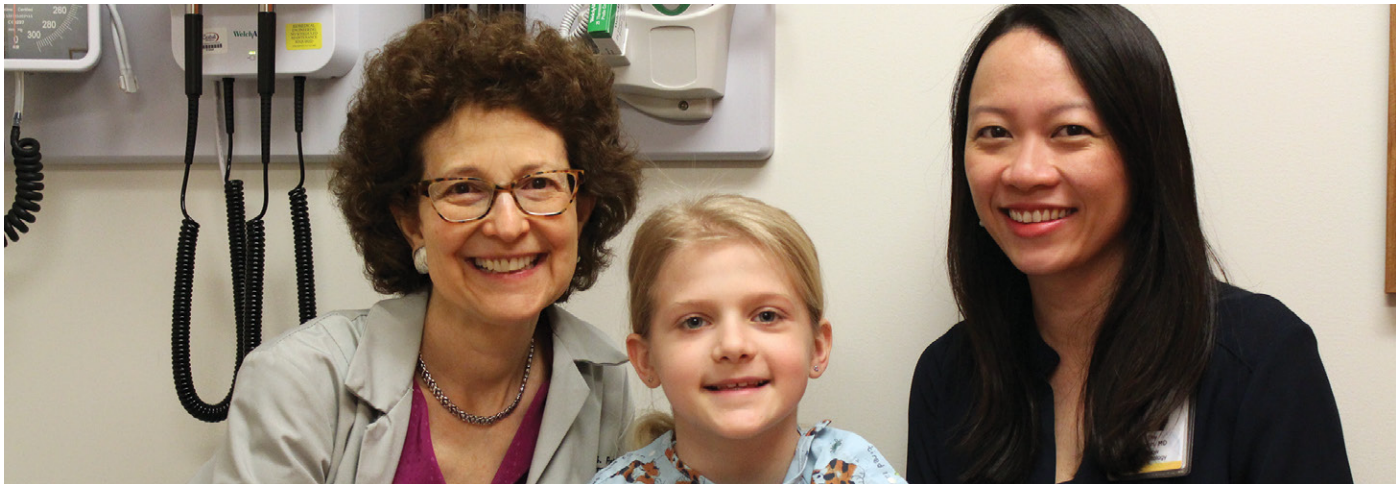


Pediatric
Dermatology
Research
Alliance

Helping Children with Skin Diseases through Collaborative Research

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Why PeDRA

Breakthroughs in the understanding and management of pediatric skin disorders are best attained through well-designed clinical studies executed by a cohesive network of expert pediatric investigators. Pediatric dermatology is a relatively young specialty that has lacked robust data about the natural history and long-term outcomes of skin disorders that begin in infancy and childhood. In addition, important guidelines of care, standardized treatment protocols, and FDA-approved therapies for the majority of pediatric dermatologic disorders are sparse. The Pediatric Dermatology Research Alliance (PeDRA) was founded in 2012 to address these critical yet unmet needs.

These gaps in the science and knowledge of pediatric skin diseases need to be urgently addressed, yet recruiting children for studies is challenging and projects are often underpowered, especially those aimed at rare diseases. With this reality, pediatric dermatology leaders recognized that investigations could not make meaningful progress without collaborative efforts.

Thus, PeDRA was founded as a research network in 2012. With investigators in the U.S., Canada, and Mexico partnering to conduct studies spanning several disease areas, PeDRA is beginning to positively impact the lives of children who live with difficult and life-altering dermatologic diseases and conditions.

Who we are

PeDRA is a network that multiplies the power of individual researchers by linking them together in collaborative studies. PeDRA, an organizing entity for multisite, collaborative research, is accelerating the pace of pediatric dermatology science, allowing clinicians to better understand, prevent and treat skin disease in children.

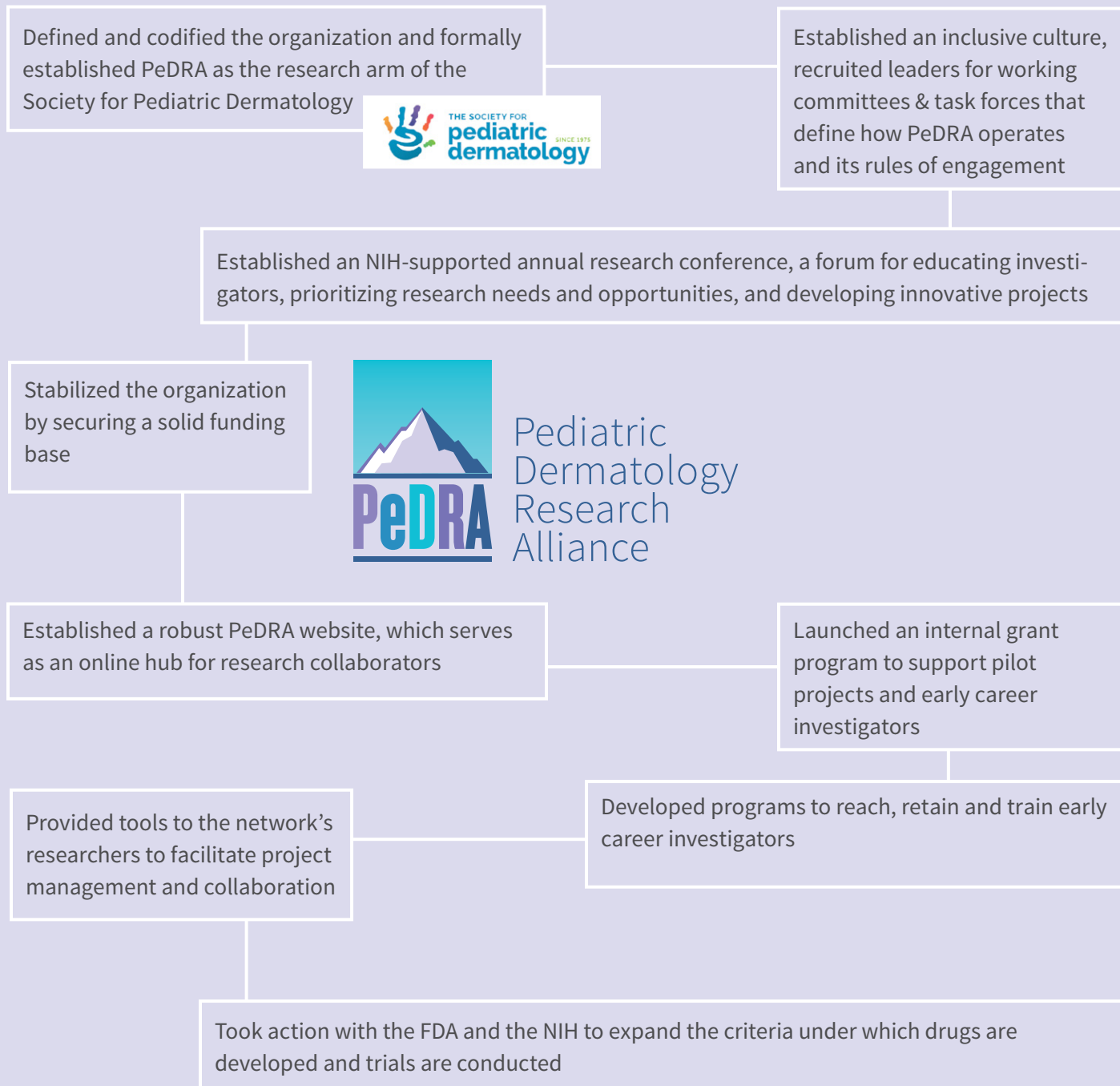
5 YEARS/ As PeDRA completes its fifth year, we are learning that when dedicated researchers band together, gaps in research can be addressed and solutions tackled for our young patients.

PeDRA is a rapidly growing organization, run by leading dermatologists, mid-career professionals, and trainees who volunteer their time. These professionals also engage with the Society for Pediatric Dermatology (SPD), PeDRA’s “parent organization,” which launched PeDRA with generous seed funds and other important start-up resources.

With the streamlining of research and pooling of resources through collaboration and the resulting exponential expansion of the patient base, PeDRA is bringing us closer to new treatment opportunities and solutions for young patients.

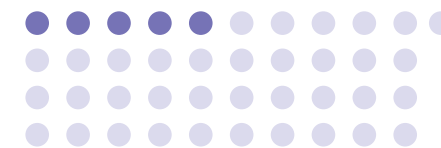
PeDRA's impact

The arc of PeDRA's work from inception in 2012 through the end of 2016 spans these highlights:



PeDRA research study groups: PeDRA has focused clinical, therapeutic and translational research and collaboration in several disease areas:

- » **Birthmarks:** vascular and pigmented (and Procedural)
- » **Inflammatory:** includes subgroups on atopic dermatitis; psoriasis; connective tissue disease; hair, nail & autoimmune; acne and hidradenitis
- » **Genetic Skin Disease:** includes subgroups on epidermolysis bullosa (EB); disorders of cornification; basal cell carcinoma nevus syndrome
- » **Skin Tumors and Reactions to Cancer Therapies (STARC)**
- » **Neonatal Skin**



There are now 41 PeDRA multi-center studies, five of which are completed, and PeDRA has provided internal seed grants to several pilot projects. Investigators are engaged at unprecedented levels in pediatric dermatology research.



Approximately 126 physician researchers are co-investigators in PeDRA studies,

representing some 68 teaching-based hospitals and institutions.



Approximately one-half of our annual conference attendees

are resident or fellow trainees and early career junior faculty.



These early successes have begun to bridge research gaps in our field, and demonstrate that PeDRA is changing the current and future landscape of pediatric dermatology research.

PeDRA annual conference

The long-term goal of PeDRA is to leverage research to improve the health outcomes of children with skin disorders. Established in 2013, our annual conferences have been integral to this success, providing a forum for identifying and prioritizing research needs and opportunities, and developing innovative projects. A combination of didactic lectures, expert-led interactive discussions, panel and poster sessions, and disease-focused small-group sessions are the foundation of this annual research meeting.

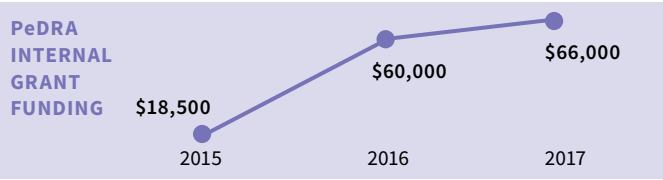
The Conference provides education and mentorship to new and established pediatric dermatology investigators in areas such as research methodology, grant writing, and project management. Conference attendees initiate new projects, advance existing ones and set new research priorities within PeDRA's five focus areas. The Conference also initiates research strategies that synchronize with organization-wide priorities.

These annual meetings generate productive interactions and partnerships that will enhance the productivity of clinical researchers in pediatric dermatology and lead to the conduct of impactful, high-quality studies. Because these activities are collaborative, they take the research farther faster and empower dedicated professionals who try to help young patients in an era when relatively few answers or solutions are to be found.



Other key operational areas

Grants: During 2016, PeDRA solidified its internal grant program. Having started in 2015 with a modest allocation of \$18,500 for collaborative projects, PeDRA stayed true to its core commitment in 2016 by increasing its allocation to \$60,000 for internal grants. In 2017, that number will be \$66,000. PeDRA and SPD also merged their grant programs into one unified effort, facilitating the application process, maximizing resources and harmonizing goals.



In 2016, funded projects spanned topics such as:

- » Vascular overgrowth syndromes
- » Effective communication with atopic dermatitis patients
- » Cutaneous findings of a rare genetic disorder

At the time of this writing, the last set of 2016 grant applications is being reviewed and awards have not yet been determined. These applications include subjects such as:

- » Skincare in neonates
- » Ulcerated hemangiomas
- » Pediatric melanoma
- » Pediatric laser treatments
- » Eczema
- » Epidermolysis bullosa treatments
- » Propranolol treatment in infantile hemangiomas
- » Pediatric alopecia areata

PeDRA early career investigators: PeDRA places great emphasis on building a legacy for the future of pediatric dermatology research. That is why more than half of our conference attendees are trainees—clinical research fellows, dermatology residents and pediatric dermatology fellows. PeDRA's Early Investigators Committee is an energetic, dedicated group that focuses on the opportunities and possible barriers for early career people to advance in the pediatric dermatology field. The group sponsors training webinars, forums at PeDRA's annual conferences with tailored content, speakers and trainings, and an immensely successful one-on-one mentorship program.

Advocacy for our patients: From the outset, PeDRA has structured a powerful relationship with patient advocacy organizations working in the disease areas within PeDRA's focus. Representatives of these patient organizations attend and sponsor the PeDRA Annual Conference. At the 2016 conference, these leaders participated in an Advocacy Forum focused on ideas and approaches for physician investigators to partner with advocacy organizations. Patient advocacy representatives also join the disease-specific breakout sessions at the conference where they bring the patient perspective to research decision-making. Some go on to become an integral part of research projects. In these ways, along with extensive networking opportunities, patient advocacy organizations have a meaningful role in shaping the PeDRA research agenda.

"Our organization is the leading funder of research for epidermolysis bullosa (EB), a life-threatening pediatric skin disease that causes tearing, blistering and bleeding of the skin, stunted growth and disfigurement. As a rare genetic disorder, EB patients need every facet of science to band together for a cure. That is why PeDRA is the perfect partner. With investigators bringing together resources and working collaboratively on innovative EB studies, PeDRA as a research network, will go farther faster in EB research."

– Paul Joseph, Board of Directors, Epidermolysis Bullosa Medical Research Foundation (EBMRF)

Other key operational areas, cont'd.



Above: Xavion Chisley and Dr. Yasmine Kirkorian

Advocacy for our patients—at the FDA: When a child can't be a kid and his disease defines his life, it can shut him down—and take the family with him. Not so for Xavion Chisley, 8, who experienced the power of advocacy by speaking at a March 17, 2016 FDA meeting on Patient-Focused Drug Development for Psoriasis. Xavion is the patient of PeDRA investigator Dr. Yasmine Kirkorian, who worked with Xavion and his family to encourage them to attend the FDA meeting titled, Patient-Focused Drug Development for Psoriasis. Yasmine spoke on the effect of psoriasis symptoms for her young patients. Dr. Lara Wine Lee, attending for both the National Psoriasis Foundation and



PeDRA, also spoke. On closing, the FDA noted to Xavion and all in attendance, “you have been heard.” PeDRA went on to submit a formal statement to the FDA on pediatric psoriasis and its impacts. Along with the American Academy of Pediatrics and the National Eczema Association, PeDRA participated in a 2015 FDA hearing on the question of including children in appropriate atopic dermatitis trials. The FDA hearing subsequently took a favorable position in allowing children to join these trials. In 2016, PeDRA became a major sponsor of the Guidance Document for Industry (GDI) initiative, which was to generate a Guidance Document

to inform the conduct of pediatric atopic dermatitis. The GDI initiative was a collaborative process sanctioned by the FDA DDDP, and including experts from industry and multiple clinical specialties. The goal was to define optimal and uniform clinical trial design. PeDRA member, Dr. Elaine Siegfried, a longtime advocate for children’s rights in drug development, led this initiative. The two-year process began with regular conference calls among five committees, followed by a face-to-face meeting in conjunction with the PeDRA Annual Conference in November 2016. The outcome will be submission of a draft document to the FDA for review, (goal date May 2017) and ultimate dissemination.

PeDRA addresses unmet research needs

With significant foundational work under its belt through the initial years, PeDRA recognized in 2016 that we now had the opportunity to advance the field on a macro level. PeDRA leadership decided that one way to achieve this greater impact was to take on a larger, PeDRA-wide research project. Crossing all PeDRA study areas, testing the stigma of pediatric skin disease was decided as the focus for this work. With initial stigma work having been developed at Northwestern University by Dr. Amy Paller, past PeDRA Co-Chair, a PAN-PeDRA Stigma Study Group was formed and the concept received overwhelming endorsement by PeDRA researchers across all disease areas. The design of the PAN-PeDRA Stigma Study began in 2016 and the study is expected to launch in 2017. This study, which will assess anxiety, depression, and social functional issues in addition to stigma, has the potential to underscore the burden of disease—and highlight need for ancillary or integrated support services for kids with skin diseases, increased hospital support for interdisciplinary care, increased insurance coverage for diseases denied as “cosmetic,” and curricula for dermatology residents and pediatric dermatology fellows on what patients and their families experience and need.

THIS STUDY, WHICH WILL ASSESS ANXIETY, DEPRESSION, AND SOCIAL FUNCTIONAL ISSUES IN ADDITION TO STIGMA, HAS THE POTENTIAL TO UNDERSCORE THE BURDEN OF DISEASE ... FOR KIDS WITH SKIN DISEASES.

“During a time when my research efforts were isolated, PeDRA entered the picture and provided me with a rich network of colleagues for collaboration and networking. The broad PeDRA union includes trainees, early career investigators, as well as seasoned and luminary leaders. What a fantastic opportunity it was not only to plug into cutting edge research but also to find a professional community of like-minded people. PeDRA has made a significant impact on my career.”

– Lisa Arkin, MD, Director of Pediatric Dermatology, Assistant Professor of Dermatology and Pediatrics
University of Wisconsin School of Medicine



The road ahead

To frame PeDRA in a different context, the organization is like the adolescent primed to grow up but still childlike. However just as children's behavior today is nothing like that of 50 years ago, PeDRA is not your typical nascent non-profit organization. We stay nimble and laser-focused on the core mission, we test ourselves by continually expanding our vision, and we challenge ourselves by measuring our work in terms of impact—is what we are doing changing the field of pediatric dermatology and improving outcomes for children?



2017 With that view, PeDRA has set an ambitious but critical course for the next year. We have launched the PeDRA NIH Grants & Workforce Group whose mandate is to promote submission of pediatric dermatology research applications for federally-funded grants and to strengthen a research-trained pediatric dermatology workforce. PeDRA is also establishing a task force to focus on research vision and strategy—finding the ways of applying and leveraging research emerging from PeDRA to have a greater influence on the field. PeDRA is also looking at strategies for facilitating publications, consensus papers, and citations as these are some of the tools that can affect change in the specialty.

Longer term goals for PeDRA's research work include:

- » describing the burden of pediatric skin disease;
- » developing evidence-based consensus guidelines; and
- » conducting comparative effectiveness studies that will influence therapeutic recommendations.



How PeDRA operates

PeDRA's infrastructure is comprised of an Executive Committee and Board of Directors that drive the policies and priorities of the Alliance. There are also working committees and task forces that define how PeDRA operates and the rules of engagement for the network. Two examples among many include an Ethics Task Force, which defined principles for collaborating on research studies and a task force that defined what constitutes a PeDRA study. All this leadership is volunteer.

PeDRA has a part-time Executive Director and two PeDRA Fellows who lend some assistance with the administration of PeDRA. In 2016, we began to contract some hours from a project manager who acts as a liaison on membership, donations and external relations. A fiscal management firm handles PeDRA's accounting and budgeting processes.

In 2016, we undertook to provide some research tools to our collaborators. We invested in project management software, which

enables researchers on projects to share documents and communicate with their study team members. We also launched some extensive work to provide an online study tracking tool, which will enable us to systematically monitor the numbers of studies and the investigators running them, stages of development, resulting publications, etc. Dr. Wynn Tom, former Chair of the PeDRA 2014 & 2015 Annual Conference and current Chair of the PeDRA Science Committee, has led this effort and contributed many volunteer hours in both the design and launch of this program.

PeDRA's bedrock is made up of volunteer medical professionals who care for pediatric patients, run clinical trials, conduct bench research, lead translational research projects, consult on drug development, start registries, publish data critical to advance the field and—in their spare time—run PeDRA. The Alliance has a unique culture, shaped not by organizational preoccupations but by real needs in the field that these devoted professionals bring to PeDRA's work.

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THAT THESE DEVOTED
PROFESSIONALS BRING TO
PeDRA'S WORK.

Honor roll



THANK YOU to the **clinician/researchers** volunteering their time and expertise on PeDRA committees and task forces:

- Rich Antaya

Lisa Arkin

Lionel Bercovitch

Heather Brandling-Bennett

Anna Bruckner

Valerie Carlberg

Yvonne Chiu

Keith Choate

Kelly Cordoro

Colleen Cotton

Brittany Craiglow

Cyndee DeKlotz

Larry Eichenfield

Sheila Fallon Friedlander

Ilona Frieden

Maria Teresa Garcia

Maria Garzon

Sharon Glick

Anita Haggstrom

Elena Hawryluk

Adelaide Hebert

Marcia Hogeling

Kristen Hook

Jennifer Huang

Raegan Hunt

Irene Lara-Corrales
- Christine Lauren

Moise Levy

Marilyn Liang

Minnelly Luu

Julianne Mann

Kaliyani Marathe

Erin Mathes

Kim Morel

Judith O'Haver

Amy Paller

Thuy Phung

Elena Pope

Kate Puttgen

Jullian Rork

Dawn Siegel

Elaine Siegfried

Nanette Silverberg

Jeff Sugarman

Virginia (Ginna) Sybert

Joan Tamburro

Joyce Teng

Megha Tollefson

Wynnis Tom

Mary Williams

Albert Yan

THANK YOU to the **clinician/researchers** working tirelessly on PeDRA research studies:

- Smita Aggarwal

Regina-Celeste Ahmad

Hissah Al Shahwan

Mina Ally

Katelyn Anderson

Israel (Izzy) Andrews

Lisa Arkin

Smita Awasthi

Carl Baker

Karen Ball

Victoria Barrio

Cheryl Bayart

Susan (Susi) Bayliss

Emily Becker

Mark Beckwith

Leah Belazarian

Latanya Benjamin

Lionel Bercovitch

Julie Block

Kenneth Bloom

Markus Boos

Christina Boull

Heather Brandling-Bennett

John Browning

Anna Bruckner

Craig Burkhardt

Val Carlberg

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Larry Eichenfield

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Nika Finelt

Ilona Frieden

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Pedram Gerami

Sharon Glick

Maria Gnarra

Deepti Gupta

Monique Gupta Kumar

Emma Guttman-Yassky

Anita Haggstrom

Jennifer Hand

Elena Hawryluk

Adelaide Hebert

Marcia Hogeling

Kristen (Kristi) Holland

Kristen Hook

Kimberly Horii

Jennifer Huang

Steve Humphrey

Raegan Hunt

Marla Jahnke

Melinda Jen

Anna Juern

Megan Kinney

Andrew Krakowski

Lacey Kruse

Irene Lara-Corrales

ChristineLauren

Leslie Lawley

Moise Levy

Marilyn Liang



Honor roll, cont'd.

THANK YOU to the **individuals** who generously donated to PeDRA in 2016:

- \$1,000**

Thomas McIntee

Jeffrey Sugarman

Sharon Glick

Amy Paller
- Up to \$100**

Regina-Celeste Ahmad

Deniz Akkaya

Lisa Arkin

Maria Del Boente

Giovanna Ciocca

Francisco Colon-Fontanez

Colleen Cotton

Roselyn Epps

Esteban Fernandez Faith

Jill Nelson

Monica Novoa

Maureen Rogers

Dawn Siegel

James Treat

Mary Williams
- \$450-\$500**

Ilona Frieden

Harper Price

Albert Yan
- \$120-\$250**

Jeffrey Callen

Christine Cazeau

Yvonne Chiu

Lawrence Eichenfield

Christine Kilcline

Laura Levin

Thomas McIntee

Elena Pope

Nanette Silverberg

Megha Tollefson



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Medical College of Wisconsin
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Harvard Medical School
Boston Children’s Hospital

EXECUTIVE DIRECTOR

Sheila Rittenberg

PeDRA LIAISON

Jennifer Dawson

2016 PeDRA FELLOWS

Thy Huynh, MD

Andrea Waldman, MD



Honor roll, cont'd.

THANK YOU to the **patient advocacy organizations** that allocate funds to PeDRA when they are faced with many competing priorities. We sincerely thank these groups for their unwavering commitment to pediatric dermatology research:

NEVUS OUTREACH	\$9,000
NATIONAL PSORIASIS FOUNDATION (NPF)	\$5,000
FOUNDATION FOR ICHTHYOSIS AND RELATED SKIN TYPES (FIRST)	\$1,500
NATIONAL ECZEMA ASSOCIATION (NEA)	\$1,500
EPIDERMOLYSIS BULLOSA MEDICAL RESEARCH FOUNDATION (EBMRF)	\$1,000
NATIONAL ALOPECIA AREATA FOUNDATION (NAAF)	\$1,000
STURGE-WEBER FOUNDATION	\$1,000
BASAL CELL CARCINOMA NEVUS SYNDROME LIFE SUPPORT NETWORK	\$500
EB RESEARCH PARTNERSHIP	\$500
PACHYONYCHIA CONGENITA (PC) PROJECT	\$500
PARENTS FOR ECZEMA RESEARCH	\$500
PHACE SYNDROME COMMUNITY	\$500

PeDRA genuinely thanks the pharmaceutical and biomedical companies that have supported the Alliance’s growth and development in 2016. Their contributions provide essential funding that enable PeDRA’s capacity building and ensure a successful Annual Conference:

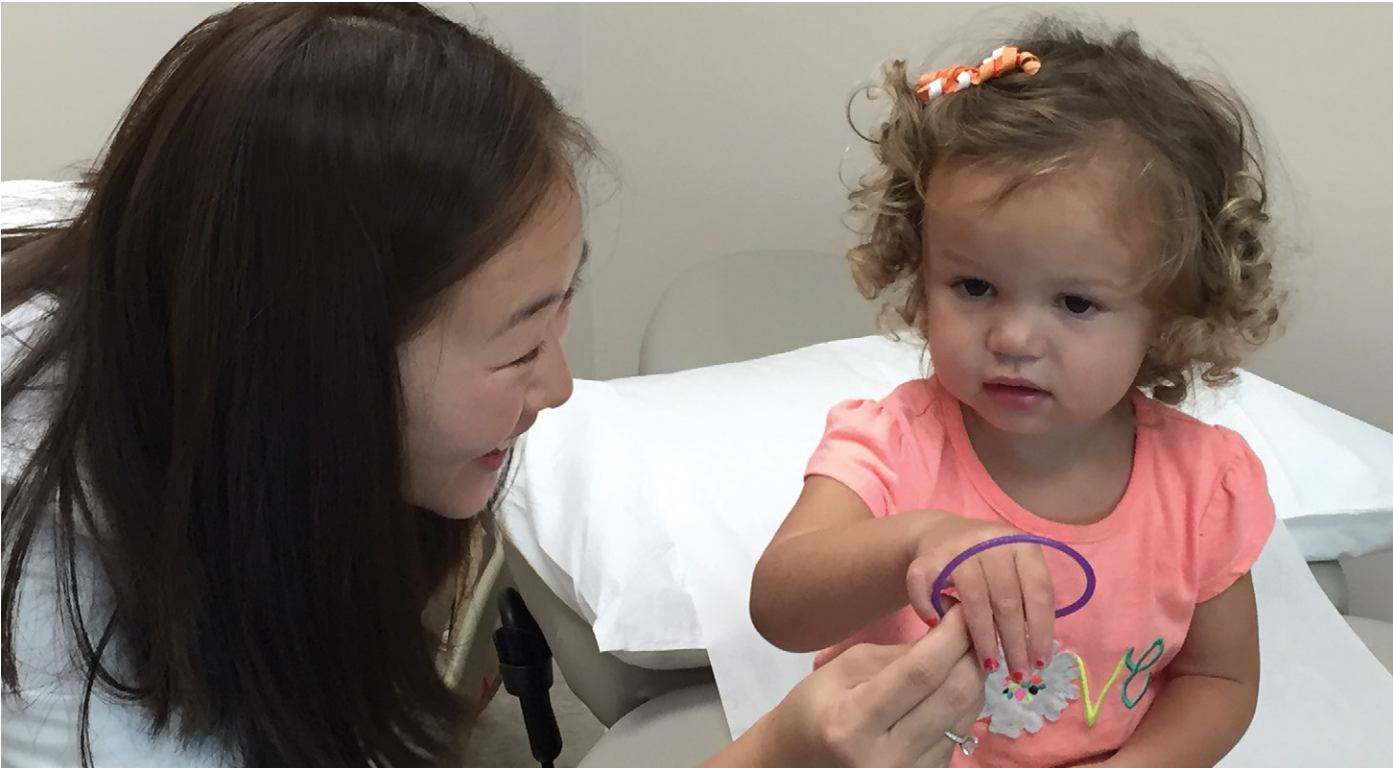


PeDRA also graciously thanks and acknowledges the Society for Pediatric Dermatology (SPD) for its critical support since PeDRA’s inception. The Society’s commitment to launching PeDRA is the reason we are now seeing important studies emerging from this research network.

PeDRA acknowledges the National Institutes of Health (NIH) for its funding of the PeDRA Annual Conference from 2013-2016. NIH recognition and early funding of the conference enabled PeDRA to establish this meeting as the central vehicle through which we achieve our mission. The Annual Conference could not have been developed without this vital NIH backing, particularly that of the National Institute for Arthritis and Musculoskeletal and Skin Diseases (NIAMS).

Conclusion

PeDRA has brought together many talented and dedicated individuals who collectively study rare and common pediatric disorders in a way never before pursued at a single site or even through a few collaborating centers. PeDRA is transforming the science of pediatric dermatology through this research collaboration. This work addresses the important gaps in our understanding of skin disorders of childhood and ultimately will translate to new solutions and strategies for helping children with difficult, high impact, and sometimes life threatening skin diseases.



“PeDRA provides the perfect forum for meaningful dialogue between patient groups and researchers, and this year’s meeting was thoughtfully designed to cultivate such exchanges. As an organization singularly focused on advancing patient/caregiver and research partnerships, PeDRA is our go-to meeting for connecting with the brightest minds in pediatric dermatology research with the goal of nurturing new ideas for research partnerships.”

–Korey Capozza, Global Parents for Eczema Research



PEDRA'S MISSION

PedRA's mission is to promote and facilitate high quality collaborative clinical, translational, educational and basic science research in pediatric dermatology. Along with this mission is the vision to create sustainable collaborative research networks to better understand, prevent, treat and cure dermatological diseases in children.



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Please visit www.pedraresearch.org for more information on PeDRA.

Questions? Please contact PeDRA Executive Director: **Sheila Rittenberg**
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Pediatric Dermatology Research Alliance—
PeDRA on Facebook.

