Helping Children with Skin Diseases through Collaborative Research

PeDRA
Pediatric
Dermatology
Research
Alliance

A State of the Organization Report

2013 and 2014

The Mission

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

2013-2014

A State of the Organization Report
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Background

In 2012, a group of pediatric dermatologists created the framework for a collaborative research network, the Pediatric Dermatology Research Alliance (PeDRA). This report updates on the progress of this novel and innovative organization, which has already begun to have an impact on the volume and quality of research studies in the field of pediatric dermatology.

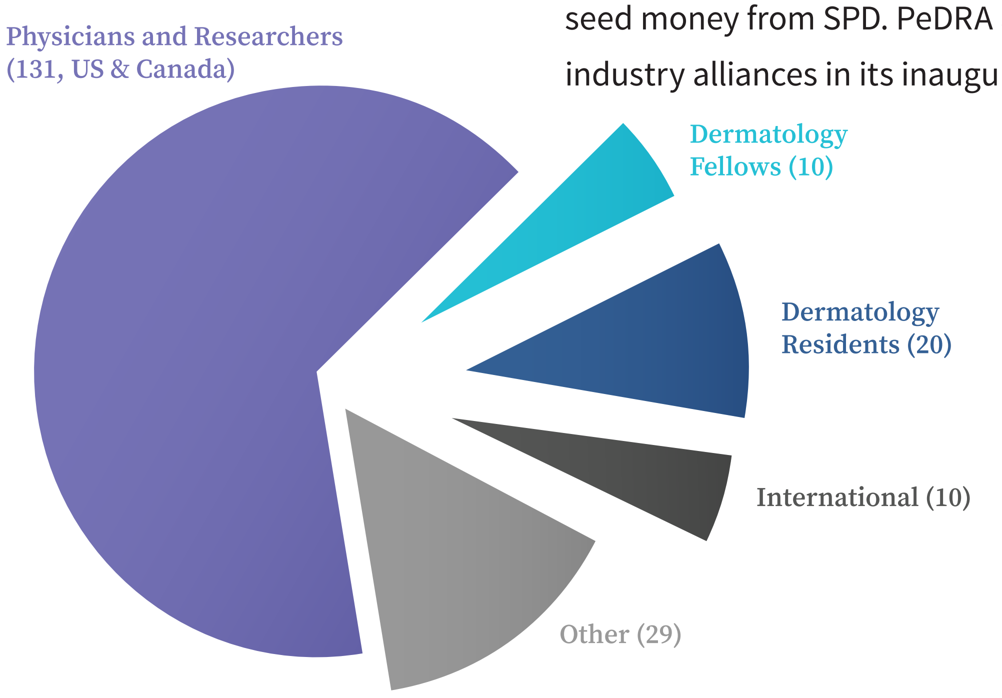
Defining Unmet Research Needs in Pediatric Dermatology

Establishing PeDRA has meant that dermatology leaders with a shared vision for increased "peds derm" research can begin to penetrate the many unmet needs in the field: Many pediatric skin diseases are so uncommon that meaningful study is difficult without collaborative effort. Conducting clinical trials in young children, even in common diseases, is also challenging in that recruitment of eligible subjects can be difficult and most pediatric dermatologists have busy clinical practices and little time, poor funding and lack of infrastructure to do high-quality research. These challenges mean that many – perhaps most – of our therapies for pediatric skin disease are based on anecdotal evidence, expert opinion and precedent. There is a lack of accepted clinical guidelines for many dermatology conditions, some of which are life-threatening. The types of standardized treatment protocols that exist in pediatric oncology and in pediatric rheumatology are sorely lacking in pediatric dermatology, and there is a paucity of NIH funded research dollars allotted to pediatric dermatology. Better evidenced-based management for children with skin disorders requires well-designed, multi-center collaborative clinical trials.

Early Successes

Organizing a Structure for the Alliance

An inaugural meeting to lay out plans for a collaborative clinical and translational pediatric dermatology research network was held in July 2012. Members of the Society for Pediatric Dermatology (SPD) dedicated to clinical research, SPD leadership, NIH staff, and other stakeholders attended this meeting and helped to create a roadmap for a new organization to help achieve these goals. Thus, the Pediatric Dermatology Research Alliance (PeDRA) was born, supported by multi-year seed money from SPD. PeDRA also caught the attention of industry alliances in its inaugural year and was able to secure some valuable support for additional capacity building. In 2013, PeDRA developed a leadership structure to drive this work, headed by an executive committee comprised of leading researchers and clinicians from around North America. Communications, Fundraising, Membership, and Science working committees have also been established. The Alliance established a seminal website, http://pedraresearch.org, to capture pediatric dermatology research priorities, progress on collaborative studies, proceedings of PeDRA annual conferences, and other resources relevant to clinicians, researchers and other stakeholders. The Alliance also solidified its administrative requirements and retained an Acting Executive Director to integrate work and ensure that organizational needs were met.



Engaged in PeDRA

The response from the field has been nothing short of outstanding. Just two and a half short years since its inception, PeDRA is able to claim some impressive statistics.

Establishing an Annual Conference

PeDRA leaders recognized the need for a free-standing meeting to create a bridge between basic scientists and clinicians and strengthen opportunities for translational research. PeDRA leaders secured funding for an annual conference from the National Institutes of Health resulting in two stand-alone conferences to identify gaps and challenges in current practice, establish research

> **“In a research world where we are too often told no, investigators are energized by all the promise and possibility that PeDRA’s new or ongoing collaborative studies suggest.”**
>
> —Beth Drolet, M.D., President,
> Society of Pediatric Dermatology

priorities, and develop collaborative clinical, translational, and basic science research. Patient advocacy organizations and industry also provided valuable support for the meetings. These 2013 and 2014 conferences involved new investigators, helping them become involved in studies and work alongside seasoned, senior investigators, supporting trainees and junior clinical pediatric dermatologists to enable them to develop the skills and infrastructure to support their research pursuits. Having research scientists at these conferences helps advance translational pediatric dermatology research initiatives.

The first free-standing PeDRA research meeting was held in Chicago in October 2013 with more than 80 participants,

and was met with great enthusiasm. It focused on teaching key strategies for recruiting and retaining study participants, navigating institutional review boards, writing grant proposals, and developing an infrastructure for multicenter collaborative research, with lessons delivered from our pediatric oncology colleagues. PeDRA’s second annual conference was held in Chicago in November 2014. More than 110 people attended, including pediatric dermatologists and researchers, pediatric dermatology fellows and dermatology residents, invited speakers, representatives of patient advocacy organizations and of the National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS), a branch of the National Institutes of Health (NIH). During the three day meeting, attendees focused on advancing collaborative networks for research to address important problems in pediatric dermatology, guided by a faculty of nationally and internationally recognized speakers. Speakers presented their ongoing research and addressed common issues in collaborative research projects, while break-out sessions provided attendees the opportunity to apply what they learned to new and ongoing projects. In both years, pilot and early project ideas were discussed with input from experts as well as from representatives of patient advocacy groups related to the diseases being studied.

PeDRA has focused its initial efforts to foster research and collaboration in several specific disease areas by forming these study groups:

- Birthmarks (Vascular & Pigment)
- Inflammatory Skin Disease (including Psoriasis, Connective Tissue & Auto-Inflammatory, Atopic Dermatitis, Acne & Hidradenitis, and Hair/Nail/Special Sites Disorders)
- Genetic Skin Disorders (including sub-groups in Epidermolysis Bullosa,
- Diseases of Cornification and Basal Cell Carcinoma Nevus Syndrome)
- Skin Tumors And Reactions to Cancer Therapies (STARC)
- Neonatal Skin Care

Launching Pediatric Dermatology Research Collaboration

Some 15 research projects are already launched or proposed under this collaborative umbrella. These include a multicenter study of vascular overgrowth syndromes, a morphea clinical registry and biobank, participation in a Yale-sponsored disorders of keratinization project to study a variety of ichthyoses and related disorders, and the Epidermolysis Bullosa Clinical Characterization and Outcomes Database. Two of these research areas have received partial funding from non-traditional sources, spurred on by the inspiring story that PeDRA tells of people joining together to shape and create the research necessary to make a difference for our patients.

PeDRA Characteristics

> **“I think what is so unique about this meeting is the sense that we are forging new ground. We are change-makers, taking the reins and shaping the research necessary to make a difference for our patients. This is empowering for each and every one of us at the meeting.”**
>
> —Dawn Siegel, M.D.,
> PeDRA 2013 & 2014
> Annual Conference Chair

Morphea is one area that has been supported and an ichthyosis newborn study, designed to track babies born with the disease, record changes over time, and correlate the clinical outcome of ichthyosis, has been partially funded. This will generate historical data to help us better recognize and understand the natural course of the disease that associates with different specific types of ichthyosis.

PeDRA is also becoming active in registries. The psoriasis group that is part of PeDRA Inflammatory Skin Disease studies identified the need to gather data on various systemic psoriasis therapies. The group joined with a similar European one, and will be receiving a major grant for a retrospective review of use of systemic medications and phototherapy for pediatric psoriasis, followed shortly by the initiation of an international registry in order to collect data on a prospective basis.

Based on PeDRA’s successes in the first two years, we are now able to begin allocating some resources to funding of collaborative studies under the PeDRA umbrella. Further, the PeDRA study groups will be better positioned to pursue NIH funding as the collaborative structure of the work helps make a powerful case for support. In general, any project that is built across centers and taps into a network bodes well for the researchers involved and leverages the interests and resources of every collaborative partner.

Advocacy for Our Patients

From inception, PeDRA’s first priority has been to establish its leadership, organizational capacity, and a working framework for collaborative projects. Moving into its third year, PeDRA is beginning to undertake some advocacy to improve policies that have a bearing on how easily and effectively we in the field of pediatric dermatology can pursue research.

First, from the initial 2013 Annual Conference, PeDRA has been closely engaged with patient advocacy organizations. Several of these groups financially supported the 2013 and 2014 Conference, and many of them attended each meeting. This and other ongoing interaction helps PeDRA set direction and infuses the whole research process with an appreciation of needs and gaps from a patient perspective.

PeDRA’s members are concerned about the prospect of drugs in development not actually reaching our patients. PeDRA has engaged with the FDA Pediatric Division and is continuing dialogue to arrive at a more balanced understanding of patient needs and patient safety as related to drug development.

PeDRA also submitted comments on the National Institutes of Health draft policy for use of a central IRB in multi-center studies. NIH adopting this policy will eliminate the requirement for each participating center to acquire an IRB, and replace it with use of one central IRB. This will eliminate redundant work efforts, streamline research and encourage more collaborative work. Our expectation is that enacting this NIH policy will then have a precedent effect on other non-NIH funded studies.

Tackling new challenges

In 2015, an Ethics Committee, steered by some of the country's foremost pediatric dermatology leaders, will tackle guidelines for PeDRA's collaborative work and define inherent rights and responsibilities for participants in studies. The Grant Review committee, also run by dermatology thought leaders, will produce a modus operandi for allocating grants to PeDRA projects. We are also examining the best structure for PeDRA to make available study sites for industry sponsored trials. Another important area of focus for 2015 is determining the database infrastructure required to support a collaborative network. All of these are meaty, philosophical topics that reflect the growth, impact and sophistication of the organization.

> **"All the good work of PeDRA would be in jeopardy without ethical guidelines for conducting this extensive collaboration. Authorship issues, security of intellectual property, confidentiality agreements, and setting the 'culture' of PeDRA are only some of the fundamentals that PeDRA must tackle early on. The code of ethics we produce will be a critical component of a strong foundation for PeDRA."**
>
> —Lionel Bercovitch, M.D., PeDRA Ethics Committee Chair

Honor roll

PeDRA stakeholders believed in the early concept of the Alliance and invested in its capacity building and development of organizational structure. Funds raised in 2013, inclusive of SPD, NIH (Annual Conference), patient advocacy organization and industry support, totaled $164,000. In 2014, that number was $275,000.

PeDRA's Annual Conference was made possible, in large part, by R13 funding from the NIH, for which we are greatly indebted* Patient advocacy organizations also provided sponsorship to further support the conference.

Combined 2013 & 2014 Patent Advocacy Organization Contributors:

Epidermolysis Bullosa Medical Research Foundation (EBMRF)	$8,000
National Psoriasis Foundation (NPF)	$7,500
Nevus Outreach	$5,000
Foundation for Ichthyosis and Related Skin Types (FIRST)	$2,000
National Eczema Association (NEA)	$2,000
Dystrophic Epidermolysis Bullosa Research Association of America (DEBRA)	$1,000
National Alopecia Areata Foundation (NAAF)	$500
National Organization of Vascular Anomalies (NOVA)	$500
Pachyonychia Congenita Project	$500
PHACE Syndrome Community	$500
Sturge-Weber Foundation	$500

*Funding for this conference was made possible (in part) by R13AR065364-02 from the National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS) and all co-funding support provided by National Institute of Child Health and Human Development (NICHD). The views expressed in written conference materials or publications and by speakers and moderators do not necessarily reflect the official policies of the Department of Health and Human Services; nor does mention by trade names, commercial practices, or organizations imply endorsement by the U.S. Government.

PeDRA also extends a sincere thank you to our industry alliances who have significantly helped to fund PeDRA organizational growth and development in 2013 and 2014, as well as support the Annual Conference.

Combined 2013 & 2014
Industry Alliance Contributions:

Glaxo Smith Kline	$65,000
Procter & Gamble	$35,000
Pierre Fabre	$20,000
Valeant	$15,000
TopMD Skin Care	$10,000
Anacor	$7,500

Family foundations

Family foundations have played a significant role in PeDRA's growth. These leaders have recognized the harsh discrepancy between the need for solutions for children with miserable and sometimes life-threatening skin diseases and the reality that there is a paucity of active research underway targeted to this pediatric population. We honor these supporters for their foresight and leadership.

Combined 2013 & 2014
Family Foundation Contributions:

Gordon Dow Foundation	$30,000
William D. Ginn & the Basal Cell Carcinoma Nevus Syndrome Life Support Network	$20,000
Hoefer Family Foundation	$5,000

Similarly, dermatology professionals in the U.S. and Canada recognizing the need and engaging with PeDRA in a variety of ways have made important contributions in 2013 and 2014:

Lionel Bercovitch
Anna Bruckner
Jeffrey Callen
Yvonne Chiu
Joseph Conlon
Lawrence Eichenfield
Esteban Fernadez-Faith
Ilona Frieden
Deborah Goddard
Adelaide Hebert
Leonard Kristal
Moise Levy
John O'Malley
Jill Nelson
Amy Paller
Julie Panepinto
Elena Pope
Dawn Siegel
Kirsten Turchan
Virginia Sybert
Mary Williams

Medical professional volunteers on PeDRA working committees

And finally, PeDRA would not exist or prosper without the tireless hard work of so many dermatology clinicians and researchers from early career to seasoned professionals. PeDRA's bedrock are these volunteers 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 advancing 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. Thank you to our capable and steadfast volunteers!

Rich Antaya
Emily Becker
Lionel Bercovitch
Heather Brandling-Bennett
Anna Bruckner
Yvonne Chiu
Keith Choate
Kelly Cordoro
Cynthia Deklotz
Lawrence Eichenfield
Ilona Frieden
Sheila Fallon Friedlander
Maria Garzon
Sharon Glick
Anita Haggstrom
Elena Hawryluk
Adelaide Hebert
Marcia Hogeling
Kristen Hook
Jennifer Huang
Raegan Hunt
Christine Lauren
Moise Levy
Marilyn Liang
Kalyani Marathe
Erin Mathes
Kim Morel
Judith O'Haver
Amy Paller
Thuy Phung
Elena Pope
Kara Shah
Dawn Siegel
Elaine Siegfried
Nanette Silverberg
Jeff Sugarman
Virginia Sybert
Joan Tamburro
Megha Tollefson
Wynnis Tom
Mary Williams
Albert Yan

Medical professional volunteers on PeDRA Study Groups

Smita Aggarwal
Regina-Celeste Ahmad
Mina Ally
Hissah Al Shahwan
Katelyn Anderson
Israel Andrews
Lisa Arkin
Smita Awasthi
Victoria Barrio
Cheryl Bayart
Susan Bayliss
Emily Becker
Leah Belazarian
Latanya Benjamin
Lionel Bercovitch
Kenneth Bloom
Markus Boos
Christina Boull
Heather Brandling-Bennett
John Browning
Anna Bruckner
Craig Burkhart
Val Carlberg
Leslie Castelo-Soccio
Sarah Chamlin
Carol Cheng
Karen Chernoff
Yvonne Chiu
Keith Choate
Heather Ciliberto
Heather Irina Cohn
Kelly Cordoro
Jonathan Cotliar
Colleen Cotton
Carrie Coughlin
Jerome Coulombe
Edward Cowen
Brittany Craiglow
Cynthia DeKlotz
Lucia Diaz
Beth Drolet
Lawrence Eichenfield
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Monique Gupta Kumar
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Anita Haggstrom
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Adelaide Hebert
Marcia Hogeling
Kristen Holland
Kristen Hook
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Steve Humphrey
Raegan Hunt
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Anna Juern
Megan Kinney
Andrew Krakowski
Lacey Kruse
Irene Lara-Corrales
Christine Lauren
Leslie Lawley
Moise Levy
Marilyn Liang
Anne Lucky
Sheilagh Maguiness
Anthony Mancini
Kalyani Marathe
Ash Marghoob
Erin Mathes
Catalina Matiz
Catherine McCuaig
Patrick McMahon
Leonard Milstone
Adnan Mir
Maria Elena Miyar
Kimberly Morel
Amy Paller
Holly Paugh
Thuy Phung
Dominique Pichard
Ingrid Polcari
Elena Pope
Harper Price
Lori Prok
Kate Puttgen
Michele Ramien
Jennifer Reeve
Adam Rubin
Julie Schaffer
Kala Schilter
Birgitta Schmidt
Wendy Schumacher Kim
Kara Shah
Cathryn Sibbald
Dawn Siegel
Elaine Siegfried
Nanette Silverberg
Sarah Skillman
Aimee Smidt
Jeff Sugarman
Virginia Sybert
Joan Tamburro
Joyce Teng
Brook Tlougan
Megha Tollefson
Wynnis Tom
James Treat
Lily Uihlein
Ruth Ann Vleugels
Mary Williams
Karen Wiss
Albert Yan
Kevin Yarbrough
Andrea Zaenglein

Special mention goes to:

PeDRA leadership volunteers

Executive Committee Co-Chairs

Lawrence Eichenfield, M.D., University of California, San Diego, Rady Children's Hospital, San Diego

Amy Paller, M.S., M.D., Northwestern University, Ann & Robert H. Lurie Children's Hospital of Chicago

Executive Committee Members

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Ilona Frieden, M.D., Univ. of California, San Francisco, Benioff Children's Hospital

Moise Levy, M.D.,Dell Children's Medical Center, UT-Austin, Baylor College of Medicine

Kimberly Morel, M.D., Columbia University, Morgan Stanley Children's Hospital of New York-Presbyterian

Dawn Siegel, M.D., Medical College of Wisconsin, Children's Hospital of Wisconsin

PeDRA Administrative Fellows

Colleen Cotton, M.D., Rady Children's Hospital, University of California, San Diego

Isabel Haugh, M.D.,

PeDRA Acting Executive Director

Sheila Rittenberg

> **"Despite the unparalleled scientific advancements in cutaneous biology and new therapeutics that are emerging, there has been a relative paucity of discovery related to pediatric skin disorders and certainly few translations into new interventions. Importantly, PeDRA has brought together many talented and dedicated individuals who will collectively study rare and common pediatric disorders in a way that we've never before been able to pursue at a single site or even through a few collaborating centers"**
>
> —Amy Paller, M.S., M.D. & Lawrence Eichenfield, M.D., PeDRA Co-Chairs

Conclusion

By forming a network of researchers, PeDRA has the potential to transform the science of pediatric dermatology by multiplying the power of individual investigators, linking them together through critical collaboration. In this way, PeDRA can target the important gaps in our understanding of skin disorders of childhood.

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www.pedraresearch.org