



Pediatric
Dermatology
Research
Alliance

OUTCOMES AT YEAR-END 2017

Founded in 2012, the Pediatric Dermatology Research Alliance (PeDRA) exists with one major goal in mind: **to increase the number of high quality research studies focused on children's skin diseases.**

PeDRA STATS





Research Proposal: Retrospective Analysis of the Structural, Molecular, and Genetic Methods Clinically Utilized in the Diagnosis of Epidermolysis Bullosa by Examination of the EB-Clinical Characterization and Outcomes Database

Laura Kaplan¹, Anne Lucky MD², Harper Price MD³, Anna Bruckner MD⁴, Sharon Glick MD¹

^{1,2,3,4}University of Colorado COM, ²University of Colorado, Dept. of Pediatrics, ³Phoenix Children's Hospital, ⁴University of Colorado SOM, Children's Hospital Colorado

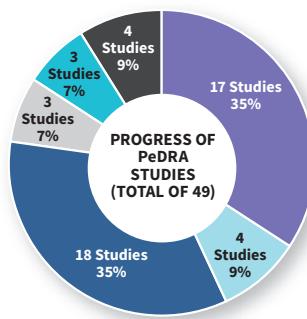


BACKGROUND:

The understanding of Epidermolysis Bullosa has progressed in the past 60 years. Today a group of disorders characterized phenotypically as distinct diseases characterized by specific structural, molecular and genetic abnormalities. Diagnostic methodologies clinically employed have evolved with the understanding of the pathogenic mechanism. Currently, a diagnostic approach schema ostensibly requires all these modalities for a comprehensive understanding of the individual patient. However, for a practicing pediatric dermatologist, the costs of diagnostic laboratory tests are prohibitive. Hence, tests are employed that are clinically useful and to the extent the utility of these tests is understood, testing has merit.



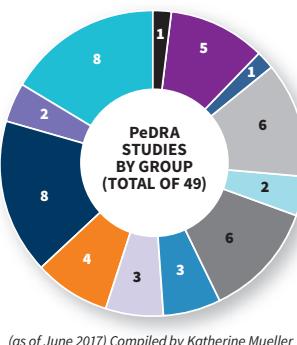
RESEARCH STUDIES



- Analysis complete, results submitted/published/presented
- Data collection complete, analysis underway
- Site recruitment closed. Ongoing enrollment/data collection
- IRB approved/Actively enrolling
- IRB Submitted
- Active design

(as of June 2017) Compiled by Katherine Mueller

There are **49 active multisite studies** within the PeDRA network run by **130 investigators**. PeDRA provided grants to **15 of these projects** from 2015 through December 2017.



- Acne and Hidradenitis
- Atopic Dermatitis
- Basal Cell Carcinoma Nevus Syndrome (BCCNS)
- Connective Tissue Disease
- Disorders of Cornification
- Epidermolysis Bullosa (EB)
- Hair/Nails/Special Sites
- Neonatal Skin Care
- Psoriasis
- Skin Tumors and Reactions to Cancer Therapies (STARC)
- Therapeutics
- Vascular Birthmarks

(as of June 2017) Compiled by Katherine Mueller

In addition, in 2017, PeDRA established our first cross-disease ("pan-PeDRA") study: **Stigma, Anxiety and Depression in Children and Adolescents with Skin Disorders: The "Big" Study**. This study uses a validated series of instruments to measure the effect of highly visible, chronic skin disorders on patients 8 years of age and above in causing stigma and psychiatric issues, particularly anxiety and depression.

BUILDING A LEGACY

An important goal for PeDRA is to develop a research-ready early career investigator community. PeDRA established the Early Investigator Committee to:

- **BUILD RESEARCH EXPERTISE**
- **FACILITATE MENTORSHIP**
- **ADVOCATE FOR THE NEEDS/ GOALS OF EARLY INVESTIGATORS**

5 PeDRA STUDIES PUBLISHED

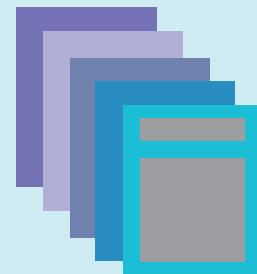
Totri et al. Prescribing practices for systemic agents in the treatment of severe pediatric atopic dermatitis in the US and Canada: The PeDRA TREAT survey

Osier et al. Pediatric Psoriasis Comorbidity Screening Guidelines

Hackethal et al. First Guideline on Pediatric Psoriasis Comorbidity Screening

Bronckers et al. Safety of Systemic Agents for the Treatment of Pediatric Psoriasis

Maguiness, et al. Pediatric skin cancer: Patient perspectives.



BUILDING A LEGACY

Important initiatives have been built through the exceptional leadership of our Early Investigator Committee:

Successful Mentorship Program



20 MENTEES / 15 MENTORS



Education programs



4 WEBINARS (GRANT WRITING, JOB SEARCHES)
2 NEW 2018 WEBINARS ON: WORKING WITH INDUSTRY

At our Annual Conference, established the Early Investigator Forum.

Theme for 2017: Effective Communication of Science

PeDRA GRANTS

In 2015, PeDRA instituted an internal grants program to provide seed funding to collaborative projects under its umbrella.

3X GRANT INVESTMENT

From 2015 to 2016, more than tripled PeDRA's investment in grants from (\$18,900) to (\$60,000).

↑10%

In 2017, PeDRA committed to increasing investment in grants by 10% annually, bringing us to \$66,000 and \$73,000 for 2018.



Implemented a new online portal for grant submissions, streamlining the submission process.

\$75,000

Partnered on a special initiative with the **National Psoriasis Foundation** to create the **\$75,000 Pediatric Psoriasis Challenge Grant**.

LEVERAGING THE NETWORK

PeDRA recently held a retreat to chart the course for a **3-year strategic roadmap** that will help change the face of pediatric dermatology research. This strategic planning focused on: **Research Innovation & Infrastructure, Empowering Investigators and Advocacy**. Highlights:



- **ADVOCATE** for pediatric dermatology to be represented in policy formulation and political activities.
- **ENGAGE THE NIH** and other scientists in our work.

- **DIMINISH GAPS** that prevent success in "peds derm" research.
- **CREATE A TRAINING OPTION** for people interested in research-intensive careers.

- **USE INNOVATION** in research study development.
- **IMPROVE** "big data" gathering.
- **STRATEGIZE** consensus guidelines.

We leverage the strength and voice of PeDRA as an alliance of national prominence.



ADVOCACY



- **THROUGH TESTIFYING AND POSITION PAPER**, influenced FDA's decision to include children in atopic dermatitis clinical trials.
- **PROVIDED POSITION PAPERS TO THE FDA** on patient-focused drug development in alopecia areata and psoriasis.



- **"PUT A FACE" ON ALOPECIA AREATA AND PSORIASIS** by having PeDRA investigators and their patients participate in FDA open forums.
- **BECAME A MAJOR SPONSOR OF THE FDA GUIDANCE DOCUMENT FOR INDUSTRY (GDI)** that established guidelines for developing atopic dermatitis therapeutic agents for children and adolescents.
- **INTERACTED WITH NIH** on key policy questions, such as the use of a central IRB in clinical studies.

PARTNERSHIPS

PeDRA engages patient advocacy organizations to better understand pressing clinical and community needs and to help us shape our research agenda.

- **10-12 PATIENT ADVOCACY ORGANIZATIONS** both sponsor and participate in the PeDRA Annual Conference each year.
- **WE SET UP A PeDRA SCHOLARSHIP** to enable a patient advocacy organization to attend our Annual Conference, propelling the group into a network of scientific contacts and a deeper understanding of research.



- **SEVERAL PATIENT ADVOCACY ORGANIZATIONS** are sponsors of PeDRA's cross-disease study on stigma, anxiety and depression in children with skin diseases.

- **THE FOUNDATION FOR ICHTHYOSIS & RELATED SKIN TYPES (FIRST)**, provided a **\$50,000 grant** to a PeDRA multicenter project on ichthyosis.

PeDRA also partners with pharmaceutical and biomedical companies, who collaborate on special initiatives with PeDRA and provide generous financial support.



Pediatric
Dermatology
Research
Alliance

Helping Children with Skin Diseases through Collaborative Research

<http://pedraresearch.org> | Follow us: @PeDRAResearch @PeDRAResearch

PeDRA works in affiliation with the Society for Pediatric Dermatology, which generously provided seed funding in the initial years of PeDRA's work. PeDRA is also indebted to the National Institutes of Health (NIH) for partial funding of our Annual Conference each year.