



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

 **215**
Members

 **62** Early Career Members (< 7 years in practice, or trainees)

 **130** Institutions represented by PeDRA members

 **5** Annual Conferences



130 Conference attendees annually

 **49** Studies

Research Proposal: 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
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BACKGROUND:

The understanding of Epidermolysis Bullosa has progressed in the past 60 years from a group of disorders characterized phenotypically to distinct diseases characterized by specific molecular and genetic abnormalities. While the methodologies clinically employed have advanced, the understanding of the pathogenic mechanisms of the latest international diagnostic schema optimally queries all these modalities for a comprehensive understanding of the individual patient. However, for practicing pediatric dermatologists, there are the critical problems of time, costs of available laboratory tests, and how to extend the utility of genetic testing has obvious implications for how we improve our understanding of the pathogenesis of these disorders.

diagnosis, how the utilization of tests have changed over time, and regional or institutional variations of test usage. When available, we will examine patient records to determine if the altered diagnosis resulted in changes in clinical management, including in prognostic information given to family members, treatment and genetic counseling.

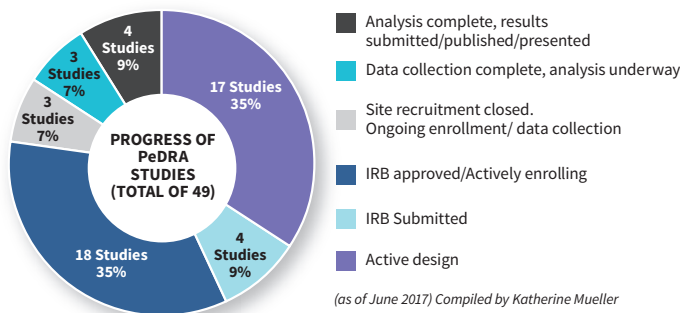
EXPECTED OUTCOMES:

- We anticipate that genetic testing has increasingly become relied upon as the sole diagnostic test for many patients in recent years.
- We further anticipate that when patients have had genetic testing in a timely manner, the IFM will add little clinical utility to the final diagnosis.

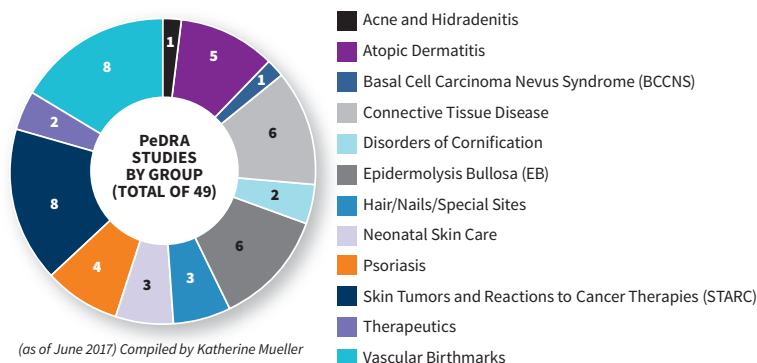
RESOURCES:

Unpublished study in epidermolysis bullosa cases which will require data extraction, analysis and statistical review. The database reflects contributions and medical histories provided by members of the Epidermolysis Bullosa Clinical Research Consortium (EB-CRC), a collaborative research group comprised of leading clinicians and researchers.

RESEARCH STUDIES



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.



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

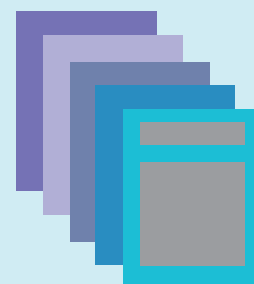
Totri et al. Prescribing practices for systemic agents in the treatment of severe pediatric atopic dermatitis in the US and Canada: The PeDRA TREAT survey

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 MENTEEES / 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.



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Helping Children with Skin Diseases through Collaborative Research

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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.