Priorities for Pediatric Dermatology Research – A Consensus Effort from the PeDRA Patient Advisory Committee

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Background
Every pediatric skin disease is associated with burdens unique to their own patient community and shared with the burdens of others. In 2019, PeDRA established a Patient Advisory Committee (the “PeDRA-PAC”) as a forum for engagement between parent and young adult patient representatives from these communities. With a direct connection to PeDRA, the PeDRA-PAC, interacts in the context of an active research network, with exposure to the latest research in the field and ability to influence emerging studies. As of October 2021, the PeDRA-PAC include 15 members representing 14 diseases and conditions (alopecia areata, atopic dermatitis, facial birthmarks, congenital melanocytic nevi, ectodermal dysplasia, epidermolysis bullosa, Gorlin syndrome, hidradenitis suppurativa, ichthyosis, pachyonychia congenita, PHACE Syndrome, pemphigus, psoriasis, and Sturge-Weber Syndrome).

Methods
THIS IS AN ONGOING STUDY. The PeDRA-PAC is conducting a consensus building exercise under the direction of an experienced facilitator. Following an introductory meeting, the PeDRA-PAC iterated via email to design a survey to identify shared disease burdens, curiosities, and priorities. An online version of the survey (created using Alchemer) was distributed by email to all 15 members of the PeDRA-PAC. Results from the survey were compiled and discussed during a virtual meeting of the PeDRA-PAC and are now being further refined into a second survey, which will be followed by a discussion during the patient breakout session at PeDRA 2021 and at least one more virtual meeting of the PeDRA-PAC to distill all information into actionable research questions.

HOW YOU CAN HELP
Please use the chat window or send an email to mike.siegel@pedraresearch.org.

Researchers:
1. Ask questions.
2. Share ideas and interpretations of the data so far.
3. Express interest in leading or participating in a study based on outcomes from this initiative.

Patients:
1. Ask questions.
2. Share ideas and interpretations of the data so far. Are we missing anything that matters to you?
3. Let us know if you’d like to get involved with the PeDRA-PAC or learn more.

Approach

Cross-cutting themes session: PeDRA 2020
Survey draft 1: PeDRA-PAC brainstorms session, draft survey circulated, feedback and input, survey revised, survey deployed
Data analyzed, reviewed with PeDRA-PAC
PeDRA-PAC presentation on methods and findings to date
PeDRA session to develop CER questions from most pressing research needs
Draft prioritized research agenda for review and revision by PeDRA-PAC
Final prioritized research agenda

Goal
To create a list of high-priority, patient-centric research questions relevant across multiple disease areas and actionable by PeDRA investigators. The hope is that the outcome of this work can influence near-term PeDRA studies, mid-term large scale studies (e.g., a “Big Study”) and future patient-centered outcomes research (PCOR) and comparative effectiveness research (CER).

Goal

Trending Priorities

More Specific
Connection to microbiome
Genetic mutations/ causes
Itch
Pain
Visibility
Treat existing cancer
Prevent occurrence of cancer
More Broad
Research to identify causes and disease triggers
Research to bring about new treatments that more effectively treat non-fatal, but significant symptomology
Research to bring about new treatments that prolong life/ limit risk of cancer
Research about navigation of the healthcare system, managing the disease, comorbidities, and treatments.
Research about psychosocial elements of disease (bullying, stigma, anxiety, depression, etc.)