Advocacy Groups and Physicians: The New Power Couple

Patient advocacy organizations partnering with dermatology physician-scientists offer an exciting new model for optimized patient care.

BY SHEILA RITTENBERG

While patient advocacy may not be controversial, the forms it takes and benefits yielded may well suggest some deviation from “normal” dermatology practice. Relationships between physician-scientists and patient advocacy groups often lead to unconventional associations and partnerships. This article describes some of these interactions and offers a model that has already yielded positive results for all parties.

CURRENT RELATIONSHIPS

Clinicians and researchers have typically served on medical boards or scientific advisory committees of patient advocacy organizations. In more robust patient organizations, they participate in fundraising events and may mobilize their staff to engage as well. Physician offices may disseminate materials that build disease awareness, attend and speak at patient conferences, attend support group meetings, and lead webcasts on the disease in question and its treatment.

Conversely, advocacy organizations impact research in their respective disease areas by awarding grants in support of specific investigators’ studies. Larger organizations have a significant effect on the direction and pace of research. Many of the organizations also advocate for increased funding of government entities such as the National Institutes of Health (NIH) in order to augment the nation’s investment in finding solutions and ultimately cures for the diseases these advocacy organizations represent. In some instances, patients and medical professionals visit policy makers and government agencies together to advocate for solutions to issues that pose the greatest barriers to patients receiving optimal care.

A RESEARCH ALLIANCE ENTERS THE PICTURE

In 2012, to respond to significant unmet research needs in pediatric dermatology, leaders of the Society of Pediatric Dermatology (SPD) envisioned and created the framework for a collaborative research network, the Pediatric Dermatology Research Alliance (PeDRA). Just a concept two and a half years ago, today PeDRA involves more than 130 clinicians and researchers in collaborative study groups and represents 68 institutions across the country. With more than 15 robust studies underway in five broad disease areas, the network proves that by linking investigators together in collaboration, we can multiply the power of each individual researcher many fold.

Early on in its formative phase, PeDRA successfully secured NIH funding for a freestanding research meeting and the PeDRA Annual Conference was born. Interestingly, NIH was particularly interested in seeing the patient perspective included in the meeting, as well as the endorsements from patient advocacy organizations that PeDRA had included in the grant application. From there, a conference planning committee—powered by pediatric dermatology clinicians and researchers—led the work and the first meetings were held in the fall of both 2013 and 2014.

These first two conferences became the platform from which attendees identified the most urgent gaps and unmet needs in pediatric dermatology research and formed collaborative working groups to launch studies focused on these priorities.

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NEW ENGAGEMENT STRATEGIES

A central strategy materialized in parallel with launching the annual conference: PeDRA decided to include patient advocacy organizations in the core purpose and activities of this annual research meeting. We recognized the value of the patient voice in informing and framing the critical studies that would take shape at the conference.

With some one-to-one outreach, PeDRA made personal contact with several patient organizations in disease areas covered under the broad categories of focus that PeDRA had established: Birthmarks, Genetic Diseases, Inflammatory Skin Diseases, Skin Tumors and Reactions to Cancer Therapies, and Neonatal Skin. We asked for their participation at the meeting and, in spite of lean budgets and competing priorities, the organizations also stepped up and provided some funding to support areas of the meeting not covered under the NIH grant.

ENGAGEMENT WITH PATIENT ADVOCACY GROUPS ADVANCES SCIENTIFIC RESEARCH

What followed was a serendipity of events that illustrates the power of the patient and physician-scientist interaction. The meeting agenda allowed time for the groups to make known their issues of urgency in patient care. These patient groups represented in many instances difficult and underserved diseases—not exactly diseases-of-the-month. Accustomed to fighting to be heard, these advocates found themselves working alongside some of the country’s leading pediatric dermatology investigators for whom the groups’ disease areas were actually the center of their world. Stakeholders from both sides were finding common ground. Patient representatives felt empowered, and could return to their respective communities reporting “a win.” This in turn fueled momentum and energy in both the staff and volunteer ranks and strengthened the advocacy organizations as a whole.

As a result of the involvement of patient organizations, PeDRA and its investigators also benefitted from a wave of serendipity. For example, connected to its participation in the PeDRA Annual Conference, the Foundation for Ichthyosis & Related Skin Types (FIRST) awarded a research grant to one PeDRA study in which collaborating investigators are studying newborn and early childhood complications and comorbidities of ichthyosis. As an active member of PeDRA, FIRST is deepening its involvement with and support of these investigators and their evolving role in advancing research.

Another patient organization, the Basal Cell Carcinoma Syndrome Life Support Network, was so moved by the potential synergies brought to light at the PeDRA conference, the Board voted to allocate a portion of a new grant (successfully secured for the Support Network) to PeDRA. The Network’s Philanthropy Director worked with the funder to help him understand the efficiencies of supporting the alliance of pediatric investigators and to envision how investment in PeDRA will enable research that individual investigators could not move forward on their own. “I am pleased to support the transparent, collaborative initiatives demonstrated by PeDRA, and hope others will also make unrestricted educational grants to support their endeavors,” said a spokesperson for the donor. “It is amazing...

Figure 1. Julie Block, CEO of the National Eczema Association, sits with Dr. Emma Guttman at the PeDRA 2014 Annual Conference.

Pediatric Dermatology Research Alliance

Website: www.pedraresearch.org

Founded: 2012

Mission: “To promote and facilitate high quality collaborative clinical, translational, educational, and basic science research in pediatric dermatology.”

Co-Chairs: Amy S. Paller, MD and Lawrence F. Eichenfield, MD

Annual Conference: November 6-7, 2015. Westin Hotel near DFW airport in Dallas, TX.
to envision what might be achievable under their coordinat-ed efforts, to benefit our vulnerable children with significant, debilitating skin conditions.”

Representatives of patient organizations are also integral members of various PeDRA collaborative study groups. And there we can find another important synergy in this patient/PeDRA interaction: Input from patient organizations helps investigators reframe pediatric dermatology studies. Advocacy organizations are the essential voice reminding investigators what matters most to patients. Whether it is defining how quality of life can be measured, or that itch matters more than body surface coverage, or establishing the reasonable safety risks parents are willing to take for their children who are enduring the harsh effects of difficult systemic skin diseases—these organizations play a vital role in shaping the research agenda. They are a powerful reminder of why we are all pursuing advances in dermatology in the first place.

ELEVATING THE FIELD FOR ALL PRACTITIONERS

Moving into another arena, PeDRA has aligned with patient organizations in advocating for access to new treatments for children with dermatology diseases. On March 9, 2015, the FDA held a hearing at which the panel heard arguments for allowing children into clinical trials in atopic dermatitis. It was a day of triumph for pediatric patients. Patients and physicians, including PeDRA leadership, made compelling, impactful testimonies and written statements that the panel recognized as game changing. The panel agreed that there is an urgent unmet medical need for systemic therapies for the treatment of children with moderate to severe atopic dermatitis.

The FDA discussion effectively signaled a green light for appropriate clinical trials of systemic therapies. Dr. Lawrence Eichenfield, PeDRA Co-Chair and spokesperson at the hear-