



NORD[®]
National Organization
for Rare Disorders

RareLaunch: Research Ready

“Impact of Research When Your Organization Drives It”



INTERNATIONAL PEMPHIGUS & PEMPHIGOID FOUNDATION



Marc Yale,
Advocacy & Research Coordinator
marc@pemphigus.org



Impact of Research When Your Organization Drives It

Patient Organization Involvement in Research

Why

- Better health outcomes
- Improve doctor-patient relationships
- Research informs standard of care
- Leverage the patient voice
- Creates disease awareness

How

- Develop a research culture in your organization
- Identify research priorities
- Educate/communicate to your community
- Lead and design research projects
- Share results and data



Develop a Research Culture in Your Organization

What is a Research Culture

- Your organization's attitudes, values and expectations towards research
- Encompasses the behaviors of you, your team, and your patient community
- It influences researchers' career paths and determines the way that research is conducted and communicated
- Developing a research culture takes time and careful maintenance



Identify Research Priorities

- **Establish a group of medical advisors**
 - Find KOL's – researchers, clinicians, take multidisciplinary approach
 - Identify gaps in research
- **Create a patient advisory council**
 - Ask key questions about health research priorities/outcomes
 - Allow patient to lead - committee chairs or co-investigators
- **Engage research organizations like: NIH, academies and associations**
 - Meet with leadership
 - Hold meetings or symposia



Educate/Communicate to Your Community

- Community engagement is the link between basic and clinical research
- Educate patients on how to get involved and self-advocate
- Determine knowledge gaps
- Address relevant clinical questions
- Survey your community frequently
- Address patient burdens
- Provide access to research opportunities
- Connect patients and researchers



Lead and Design Research Projects

- Start a registry or natural history study
- Facilitate focus groups for researchers
- Fund a clinical scholarship – young investigators or a student
- Small seed grants – basic research or pilot studies
- Hold scientific meetings or symposia
- Publish - utilize medical students or researchers to include your name
- Establish a biobank to collect samples
- Work with drug developers on clinical trial design and enrollment



Share Results and Data

- Collect, collect, collect - the more data you have the better
- Establish agreements and guidelines on how the data will be shared
- Accelerates drug development in disease space
- Creates disease recognition and awareness
- Illustrates the power of the patient voice
- Leads to improved therapies patient outcomes



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Harness the Power of Research to Drive Your Organization!