

GlobalSkin RareDERM Environmental Scan

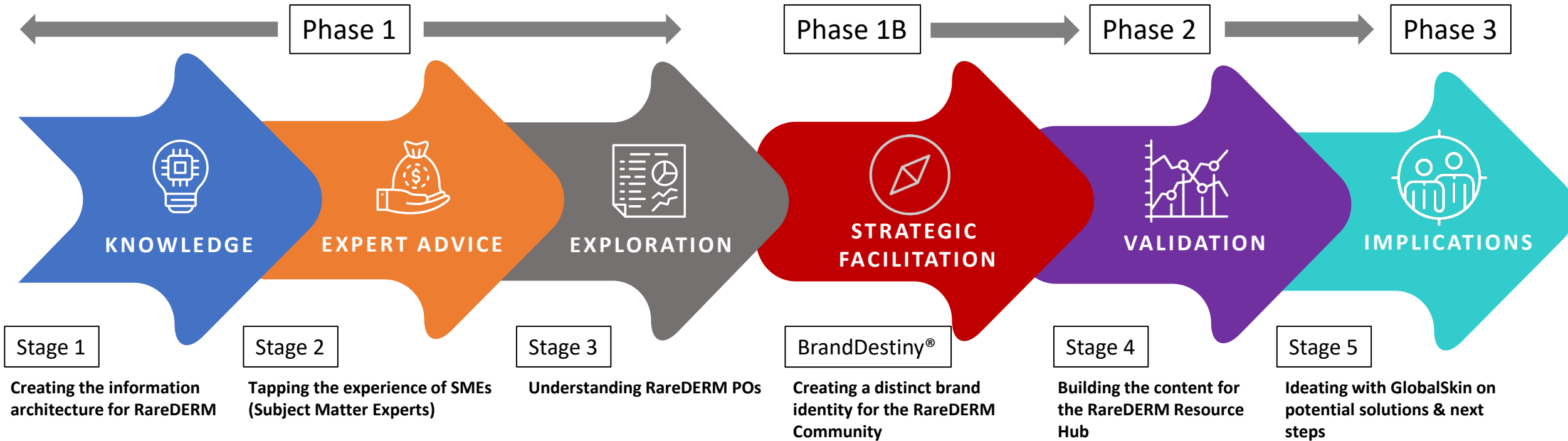
Presented by:



Scope of Work: Workplan



International Alliance of
Dermatology Patient
Organizations



What have we Learned so far?

1. Cleanslating® Rare Disease Websites
2. Expert Advice (SMEs) & Exploration with Rare Derm PO Leaders
3. Global Member Needs Assessment Study – Rare Derm Members
4. Learning & Implications for Phase 2: Validation of Environmental Scan

Environmental Scan Stages 1-3: Big Themes Uncovered

GlobalSkin

- Distinct in its ability to **build connections, nurture relationships, start dialogues**
 - Bodes well for what people want from RareDERM
- Credible data
 - Beyond Credible data is an **opportunity for Curated data**

Existing Rare Disease Orgs

- Don't demonstrate an understanding of the insights about POLs themselves
 - Too inward-focused, too complex, too technical, too features-oriented ... not enough PO-focus (i.e., needs, unmet wants, derived benefits)
- No differentiation between these rare disease organizations

RareDERM Resource Hub

- As a "Research hub" - thought to be untenable by many POLs
 - Who is going to do that work ("likely not me")? If GlobalSkin could, great, but skeptical

White Space Opportunity for RareDERM

- Psychological knowledge/mental health help
- Strategic help
- PO Registry (for best practices, experience, known disease incidence)
- True leadership in Advocacy and Partnerships

Cleanslating® Rare Disease Websites



U.S. Rare Disorders **Patient Advocacy Organization**
(1983; 300 PO members)



European **Alliance** of Rare Diseases Patient Organizations
(1997; 932 PO members)

*Note: All American NFPs in Rare Disease refer to themselves as “advocacy” orgs



International Rare Diseases **Research Consortium**
(2012; 60 organizations)



Latam **Network** of Rare Diseases Patient Organizations
(2013; 19 PO members)



Project to Create Asia-Pacific Action Plan for Rare Diseases
(2018; 50 Members, 31 Partners)

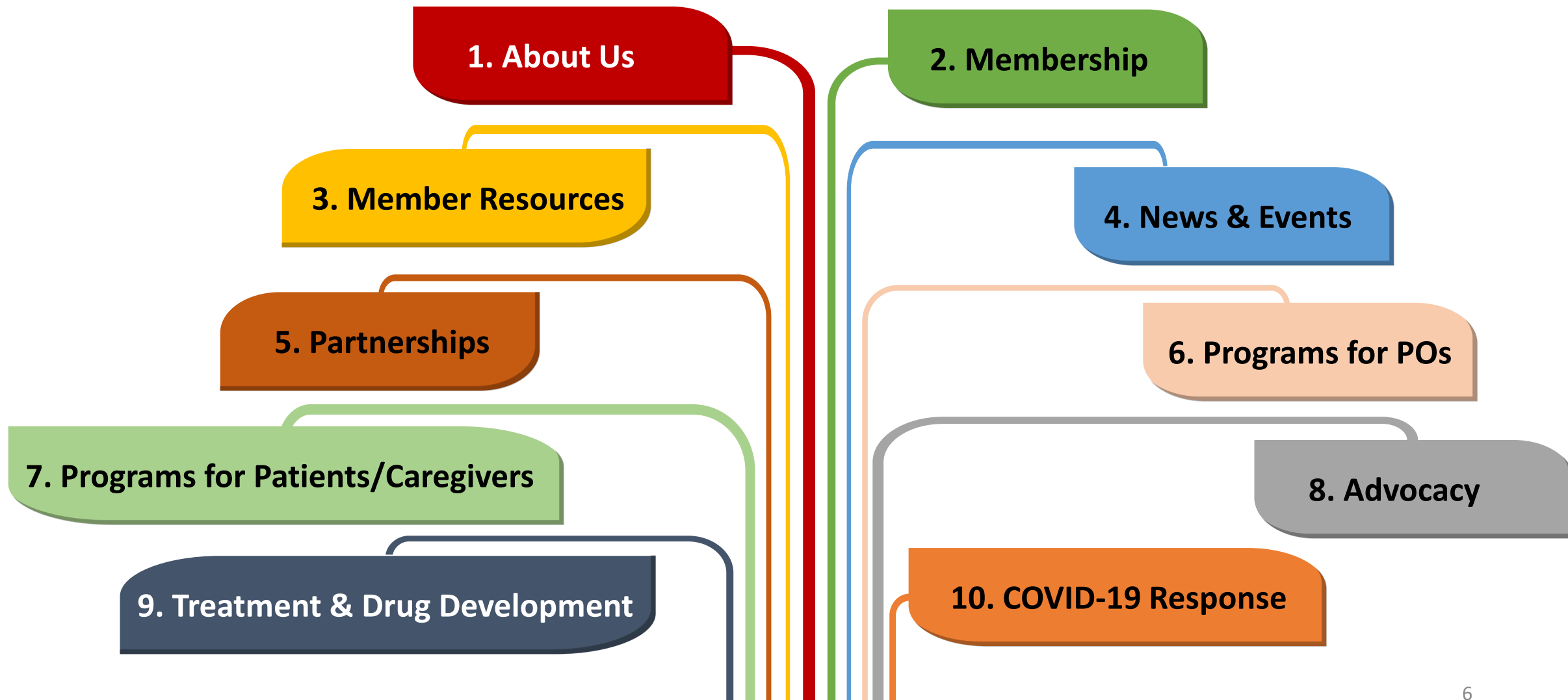


Global **Alliance** of Rare Diseases Patient Organizations
(2015; 70 PO members)



International **Alliance** of Dermatology Diseases Patient Organizations
(2015; 160 PO members)

Information architecture of Rare Disease - 10 major headings



In-depth content (219 topics) across 10 major headings

1. About Us

Who We Are

History

Vision/Mission/Goals

People

Funding/Donations

FAQs

Contact Info

2. Membership

Features:

Promotion of your Patient Organization

Access to other POs in RareDERM Community (including a PO Members List with contact info)

Sharing information with other POs in RareDERM Community

Sharing expertise with other POs in RareDERM Community

Sharing news with other POs in RareDERM Community

Rare Derm Disease Information/Education (what it is, care/treatment, research/medical, breaking news)

Advocacy & Policy-making (advocate for common positions, inform/shape global policies, connecting to major influencers, insights into policy process, analyses of regulatory and legislative activities)

Research (participate in HC and research projects for your specific disease globally; CAB (Community Advisory Board)

Benefits: (not addressed in detail on the 6 Rare Disease Org Websites)

Community/belonging

3. Member Resources

Capacity-building as a PO

Patient Registry

Patient Registry Platform:

- Cloud-based, mobile-friendly, safe/easy to use
- Platform team offers ongoing support and guidance
- Smart surveys target question participation and enable natural history data tracking
- Study Resources include standardized data dictionaries and access to rare disease IRB expertise
- Automated Survey Reminders encourage long-term user engagement
- Role-based permissions allow for flexible study design
- Analysis tools provide users with real-time data for comparison to other patients

In-depth content (219 topics) across 10 major headings (cont.)

4. News & Events

Org in the News/News

Press releases

Member News & Announcements

Newsletters

Conference Presentations

Social Media & Rare Disease Events :

- Rare Disease Day
- Member Meetings/Conferences
- Networking, Calendar of Events, etc.

5. Partnerships

International partnerships with umbrella orgs

Joint Actions on Rare Diseases (EURORDIS)

European Joint Programme on Rare Diseases
(EJP RD)

Commercial Alliances

6. Programs for POs

Conference Programs

Fellowship Programs - platform for networking
opportunities

Information/sharing experiences & Networking
Programs

Mentorship Programs

Capacity-Building Programs

Professional/Medical Education Programs &
Advisory Boards

Policy Programs

Research Programs (e.g., Rare Barometer by
EURORDIS)

7. Programs for Patients/Caregivers

Rare Disease Information Database

Rare Disease Resource Center

Rare Disease Educational Videos and Webinars

Patient Help Lines

Patient Empowerment Programs/Training

Patient Drug/Treatment Assistance Programs

Rare Caregiver Respite Program

In-depth content (219 topics) across 10 major headings (cont.)

8. Advocacy

What is a Rare Dermatological Disease

Estimating cumulative point prevalence of rare dermatological diseases for Advocacy purposes

Rare Disease as a Public Health Priority

Access to Diagnosis, Coverage, Therapies/Drugs, Telehealth

List of Resources (Medical, Psychological, Social, Financial, Caregiving)

Data to validate The Diagnostic Odyssey

Undiagnosed Initiatives

Information on Genetics/Genomics

Patient Stories

Impact of Rare Diseases

Advocacy Stage: UN, WHO

Advocacy Topics: Universal Health Coverage (UHC), Disability, Human Rights

State Report Card (interest in having a summary of “state” policies pertinent to the rare dermatological disease community)

9. Treatment & Drug Development

Medicines development and access

Accelerating Cures and Treatments: bringing together the experience of patients along with clinical data

Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP) is an integrated database and analytics hub that is designed to be used in building novel tools to accelerate drug development across rare diseases

Annual State of Play report: inform stakeholders at large of developments in the field of rare diseases research in order to support decisions of policy makers and research funders. Based on a systematic survey of published articles, in scientific journals and press releases

IRDiRC Recognized Resources label: a quality indicator, public label, accelerates the pace of translating discoveries into clinical applications

What is an Orphan Drug? Orphan drug development process; 5 Myths About Orphan Drugs and the Orphan Drug Act; Orphan Drug Development Guidebook

Research Support: Research grants and disease-specific registries to support research

Research Policy and Actions: org contributes to rare disease research policy development, orphan medicine policies

Pharmacovigilance

Compassionate Use

Health Technology Assessment (HTA): goal is to support health care decisions and serve policy making through objective information; a multi-disciplinary field of policy analysis that examines long-term effects of any existing or new health care technology; evaluates medical, economic social, ethical, and legal implications of these interventions as well as playing an important role in determining reimbursement decisions

10. COVID-19 Response

Resource Centre

Leadership Series - providing education, training and resources to help with the additional challenges brought on by this pandemic

Rapid Response Videos

Seed Grants

Rare Disease Org Websites have complex information architectures

The tree image represents the established Rare Disease Organizations.



We found the information architecture of established Rare Disease orgs (6 evaluated) is too complex:

- Multiple topics
- Great depth within a topic
- Repetitive topics (same topic under different tabs)
- Lots of acronyms (not very easy to remember)
- Use of technical language (vs. simple, familiar).

Too difficult to see the forest for the trees in how information is arranged

While qualitative, this points to a **strategic miss** in the category of being a **differentiated player**.

It's **not obvious** what the **unique value proposition** is for these rare disease orgs.



Information architecture makes them navigationally challenging



Not an easy task to navigate these websites.

Easy to “lose your way”, because tree roots are overgrown, twisted and pointing in many directions. Difficult to even remember what you were searching for in the first place.

Maturity and number of POs represented correlates strongly with the website working and feeling like a tree with many underground roots (e.g., NORD, EURORDIS).

Compared to what’s available today, not many had:

- Search Function
- Scroll Tabs to move easily up or down the page
- Pop Up asking what you are looking for
- Chat Bot.

They focus inwards, instead of on their “customers”



Only one rare disease org (of 6 evaluated) **has tab “For Patient Organizations”** (NORD), that makes it easier for POs to find information that might be relevant to them.

Lots of information dedicated to **what a rare disease org does**, or wants to do, who it has collaborated with or advocated to ... not whole lot about **impact of org’s activities** on **patients or their stories** (exception = EURORDIS).

Focus is on **features versus benefits**. Only one benefit (community/belonging) is highlighted across 6 websites.

For example, **none provide impact examples** such as:

- Key **milestones** or achievements over **lifetime of org** (with dedicated tab)
- **Testimonials** from individuals who **matter to the patients** (e.g., doctors, researchers, PO Leaders).

Rare Disease Orgs don't look, feel, or act like "Brands"

While each rare disease org has "Vision & Mission" identified/prominent on website, as well as a slogan in most cases, there is **no evidence of a "Brand" at play**:

- The sum of associations, feelings, attitudes and perceptions related to the org;
- Relationship with member and promise to deliver a specific set of features, benefits and services.



NORD is as close as it comes to a "brand", but is limited to slogans, naming conventions (word marks), and sub-brands for activities of the org.

NORD posts its Brand Guidelines doc on its website.
https://rarediseases.org/wp-content/uploads/2019/09/NRD-1196-NORD-Brand-Guidelines_v3.pdf



NORD®
National Organization
for Rare Disorders

Alone we are rare. Together we are strong.®

NORD Owned Word Marks

- + NORD® logo
- + Alone we are rare. Together we are strong.®
- + Additional NORD Registered Word Marks

NORD Sub Brands

- + Rare Action Network®
- + NORD's Breakthrough Summit®
- + Living Rare Living Stronger®
- + Running for Rare®

Participation from Subject Matter Experts & RareDERM PO Leaders

16 in total recruited by GlobalSkin.

6 SMEs

- In-depth individual interviews for 1 hour
- Based in Netherlands, Singapore, U.S., Canada
- Interviews conducted via Zoom

10 RareDERM POLs

- 2 small group discussions for 2 hours each
- Based in Israel, South Africa, France, Congo, U.S., Canada,
- Interviews conducted via Zoom



Lisa Elder



Angela Muzzo



1. What do we need to understand about POLs as we create this new RareDERM Community?



2. What is weighing heavily on the minds of POLs?



3. How does the role of a RareDERM Community compare to *Non-Rare Derm* and *Rare Disease* initiatives?



4. What benefits do they hope for from a new RareDERM Community?



1. What do we need to understand about POLs as we create this new RareDERM Community?

What do we need to understand about POLs as we create this new RareDERM Community?

I consider this a personal mission.



Don't distract me away from the needs of our patients.

I'm committed to patients *and* their families.



Tend to the broad circle of those who need care and support, mentally if not physically.

I don't want to be just a 'taker'.



Help me share my lessons learned with others, to create best practices together, synergistically.

I couldn't be stretched more thinly.



Don't waste my time and energy or cost us money. Don't reinvent the wheel.

I need to feel rewarded for my efforts in the absence of being paid.



Help me have a voice. Help us have more wins.

I can't do this all by myself.



Help me amplify our impact.



2. What is weighing heavily on the minds of POLs?



What is weighing heavily on the minds of POs?



Finding better ways to help others

- Finding more patients with their disease in order to grow their knowledge of the disease.
- How to overcome the barriers to helping others.

Succeeding in running a PO

- Managing the burnout of volunteers
- Not being trained on how to effectively run a PO
- Managing technology, including moving to digital due to Covid-19
- Being HIPAA compliant

Making treatments more accessible

- Making known treatments more accessible, with the goal for everyone to have care that meets a universal standard.
- Not knowing the incidence of their disease, in their Country and the world.

Raising the visibility of their disease

- How to help their rare disease gain visibility in order to gain traction.
- Building confidence and reducing stigma.
- How to gain and share knowledge.



3. How does the role of a RareDERM Community compare to *Non-Rare Derm* and Rare *Disease* initiatives?

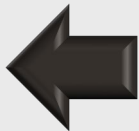




How does the role of a RareDERM Community compare to *Non-Rare Derm* and *Rare Disease* initiatives?

- Turns out that **Rare Derm has more in common with Rare Disease** than with Global Skin.
- Rare Derm needs to determine **what is unique about “skin”** to **leverage its position** in the **“rare disease” world**.
- It may not even be about disease per se, but about another differentiating factor.

Rare Disease



- May be ‘invisible’; told “it’s nothing” since lack of evidence that there’s a real problem
- May have strong awareness, even though are rare (Cystic Fibrosis, ALS)
- Already have created global associations
- Some robust genetically-oriented research

SHARED



- Missed by GPs, medical education needed
- Need better & quicker diagnoses
- Treatments exist but aren’t affordable or accessible
- Unknown incidence by country
- Need to gain knowledge of each condition
- Want to leverage/apply research from adjacent categories with similar bios
- Get learning & support from sharing stories
- Need higher visibility to gain money, research support
- Need to solicit funding even though not ‘commercially viable’

Rare Derm

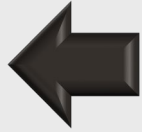


- May be less visible than other rare diseases
- May be less life-threatening
- Lack of global associations



How does the role of a RareDERM Community compare to *Non-Rare Derm* and *Rare Disease* initiatives?

Global Skin



- More recognized by Doctors, more likely to be properly and quickly diagnosed
- Better support from Pharma: research, medicine
- Comfort in being among fellow sufferers

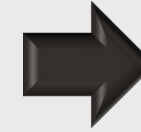
Coalition of Skin Conditions – we pay for a membership since it's our ticket to getting a ticket to the society of investigative dermatology where researchers assemble from all over the world

SHARED



- May be less life-threatening (and less noteworthy) than other diseases so less of a focus for Government
- Diseases to live with instead of cure
- “More than skin deep” affecting multiple systems
- Multi-disciplinary solutions may be required

Global Rare Derm



- Don't know but need to uncover incidence levels
- Have low instances and therefore low visibility
- Pervasive need for more accurate & quicker diagnosis
- Don't have treatments let alone cures
- Not commercially viable to Pharma
- Problems not being sufficiently treated
- Diseases can be even more easily ignored by Government
- More stigma due to rarity & ignorance



4. What benefits do they hope for from a new RareDERM Community?





What benefits do they hope for from a new RareDERM Community?

Psychological Health

- Treating the whole patient within their real-life context: school, family, new motherhood
- Helping with managing your disease psychologically, (re)gaining confidence and acceptance

Catalyst for Advocacy & Partnerships

- Adding credibility to action plans



A Meaningful Focus

- Life-changing for POs and their members
- Only possible "together"

Global Knowledge But Local Applicability

- Increased information & access
- Equitable care, regardless of geography, income level, language

PO Growth & Development

- Making more impact with the same effort
- Supporting dreams with plans

Increased Visibility

- Among HCPs, Government, Pharma
- Among global patients



What benefits do they hope for from a new RareDERM Community?

A Meaningful Focus

- Life-changing for POLs and their members
- Only possible *together*

- Have a **BHAG** with **achievable** objectives and **flexible** plans
- Inspire people with a goal to work together to achieve, that they could NOT do on their own and need global support for

No one would bite this off. So we must do it together. And it's a reason for coming together. And it gives people more hope that it'll actually go somewhere. (SME)

Global Knowledge But Local Applicability

- Need to span many "worlds"
- Increased information & access
- Equitable care, regardless of geography, income level, language
- Mapping specialists around the world (like Debra) to help everyone gain access to care
- Be a catalyst for better and quicker diagnoses
- Fully functional global operations, automated, SEO: privacy standards, accommodate for local language translation
- Host events that can serve complementary audiences productively
- House research (if doable?)

PO Growth & Development

- Making more impact with the same effort
- Supporting dreams with plans
- Create the network of POs
- Share best practices
- Train to lead effectively, plan your roadmap
- Bridge the different gaps in information that vary by PO
- Consider a development plan to address their stages and needs (and reward their progress)

70% of effort for best practices, 30% for being a repository of information (SME)

Increased Visibility

- Among HCPs, Government, Pharma
- Of POs and their diseases
- RareDERM could be invited to joint Dr/Patient events, visit Skin Boards in hospitals, could be the voice of the underrecognized
- Could forge new ways to familiarize Drs. on rare derm issues
- Learn how to obtain grants, funding
- Spread educational efforts to gain awareness and spread knowledge

Catalyst for Advocacy & Partnerships

- Adding credibility to action plans
- Need the power, influence, and knowledge of a strong, credible, organization to make these sizable connections
- Letters of support to get individuals the support they need... "we're not going to sacrifice skin to save money" (POL)
- Co-creation with policy makers i.e. with Ministry of Health & Welfare (SME)

Psychological Health

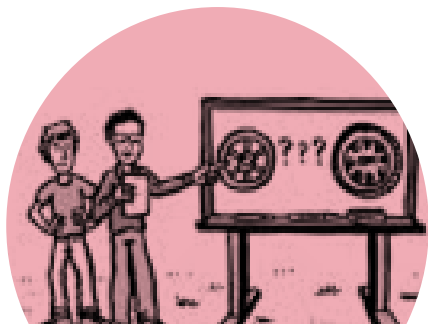
- Helping with managing your disease psychologically, (re)gaining confidence and acceptance
- Their psychological needs: stigma, fear of it worsening, the unknowns of aging, being a carrier
- Support of their care partners e.g. spouses
- Support of their families: siblings, Grandparents, children
- Measure your impact and your reach to help get support...not sure how to measure social impact... helping a patient find friends





What benefits do they hope for from a new RareDERM Community?

It must avoid...



Reinventing the wheel

- Caution against this but also admit to not knowing enough about what already exists: ERN, Orphanet, GRIDD
- Collaborate with larger groups that are in alignment
- Springboard off of systems that work



Overpromising

- Pilot test success – do one thing or disease well and then expand/roll-out from there
- How can it be possible to assemble all the research? It would be great, but could it be executed to work?



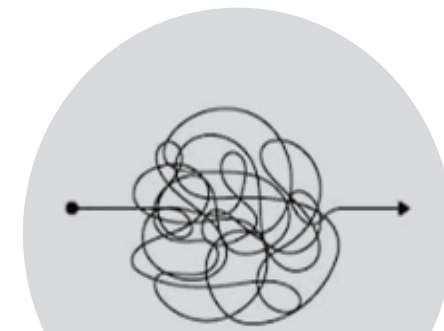
Being global at the expense of local

- Commitment should be to provide equal access regardless of government, health care system, stigma, geography, income, language, tech access, everyday product access.
- Show flexibility in execution



Getting sidetracked

- Can get distracted from the mission by following the “shiny lights” – like money
- Can seek power at the exclusion of working with others and building or collaborating
- As you get more successful, others will pull you into what they need you for



Being too complicated

- Already lack time to do what they need to do, be involved in organizations
- Want outcomes not process

I'm going to get lost in it. (POL)

Key Learning: Profile of Rare Derm Members

- Regional representation: 49% in Europe, 35% in North America, 13% in Africa, 1% in South East Asia
- Represent 19 rare skin diseases
- Mix of tenures: 19% less than 6 years, 32% 7-10 years, and 49% 11+ years
- Mix of tenures by GlobalSkin membership: 28% 1 year or less, 38% 2-3 years, and 34% 4+ years
- Four-in-ten Rare Derm POs have paid staff
- Majority have volunteers (87%), and among these, there is varying volunteer size: 19% have at most 1 volunteer, 44% have 2-5, and 25% have 6+ volunteers
- Seven-in-ten (72%) have English as one of their official languages, and all POs have English-speaking staff

Key Insights: Needs of Rare Derm Members

- Longer tenure as organization and as members of GlobalSkin (vs. non-Rare members)
- Yet, don't seem to have gotten very far in their quest to help their patients
 - Only apparent explanation is simply the rarity of the diseases they represent, and the minimal number of organizations which represent the same dermatological disease
- Resulted in strong need to connect with other organizations in the rare derm disease space (why the GlobalSkin Conference is the most valued benefit of membership)
- Also the underlying reason for their single biggest challenge - fundraising, which is so difficult without evidence of enough disease patients to get anyone's attention

Key Insights: Needs of Rare Derm Members

- Key concern is helping patients discover existing treatments for their disease/condition. Without funding, however, they cannot get off the ground
- Patient stories are powerful tools, with multiple roles. In absence of disease incidence, they “become the evidence”. They give the disease a “face to the skin”, demonstrate the physical and emotional impact of the disease, and serve as both an education and advocacy tool to stakeholders
- As result, the GlobalSkin member benefits, proposed services and educational topics explored, all have lower interest. Without viability (i.e. funding), offerings by GlobalSkin currently on the table are simply out-of-reach for their current stage of development
- Patient research (GRIDD) is slated to play a major role in bringing evidence to the table about these rare skin diseases and their patients

Key Insights: GlobalSkin needs a Segmented Member Strategy by Disease Type

- For **majority of measures** examined, there is a **difference between Rare Derm and Non-Rare Derm members**. Specifically, they differ on:
 - Tenure as an organization and with GlobalSkin
 - Number of dermatological diseases represented
 - English as official language
 - Top challenges of the PO
 - Benefits of membership
 - Advocacy engagement
 - Interest in future services and educational topics from GlobalSkin, and
 - Usage of GlobalSkin touchpoints