

RareDERM Forum 2019 Outcomes Report



A Vision for a Better Future: Globally Empowering
the Rare Dermatological Diseases Community



GLOBALSKIN.ORG

International Alliance of
Dermatology Patient
Organizations

Milan, Italy
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"Individually, we are one drop. Together, we are an ocean."
Ryunosuke Sataro

Introduction

In June 2019, the International Alliance of Dermatology Patient Organizations (GlobalSkin) hosted the first-ever RareDERM Forum for leaders of skin patient organizations (POs) in Milan, Italy. Thirty-eight leaders representing over 20 rare skin diseases from 13 countries attended the two-day RareDERM Forum to work towards an overall objective—build a cohesive community of rare dermatology disease patient organizations and supporting stakeholders around a shared vision which will ensure the lives of these patients are positively impacted in a measurable way. Specifically, community leaders were brought together 1) to develop a global RareDERM network three-year strategy; 2) to build capacity among patient leaders; and 3) to create linkages between patient leaders and other key stakeholders around the world to leverage knowledge, experience and capacity.

The forum was structured around three components: informational and scientific presentations and group discussions focused around establishing a positive vision for the future for RareDerm patients; dialogue around RareDERM policy and advocacy issues locally and globally to identify challenges to the realization of the vision; and brainstorming activities to create a roadmap for the roles that PO's can play, individually, by working together and by working with other stakeholders.

The Process

The first day of the Forum was centered on research, education and experiences within the RareDERM world. Delegates identified common challenges and considered the importance of raising awareness of the vast impact of dermatological diseases. A series of presentations by patient organizations, pharma and industry stakeholders highlighting recent research developments and the need for supportive patient data within the RareDERM world provided further context. Stimulating more research to find solutions for people living with RareDERM diseases is a common challenge across all group.

The second day of the Forum shifted the focus to capacity building and group discussions centered on creating goals for the RareDERM community. Participants were introduced to communications resources and advocacy messaging so they may use these tools with their own organizations. Then, pulling together information from plenaries and working/advisory groups on challenges and obstacles faced by patients and patient organizations, delegates moved to brainstorming about changing the existing paradigm by developing a coordinated global strategy. Five working/advisory groups were formed, to address different goals as part of a three-year initial strategy.

Why it matters

The two-day Forum leveraged the diverse experiences of delegates from around the world and confirmed vibrant interest in and an approach for the creation of a formal RareDERM network, which has as its center the creation of a better future for people living with these rare skin diseases. This outcomes document highlights the vision, challenges and proposed roadmap that emerged during the two formative days of presentations, visioning, deliberations and collaboration. Ultimately, patient association leaders agreed that the RareDERM PO community must unite to determine and implement a collective three-year strategy that contributes to a wider RareDERM stakeholder community movement. The elements outlined in this document are expected to serve as the foundation for advancing the longer-term vision of the community.

The RareDERM Forum, with its rich, meaningful plenaries and discussions supported by industry leaders who believe in and support the cause of RareDERM patients and the organizations that support them. We wish

to thank and acknowledge these sponsors for their commitment to the RareDERM community: LEO Pharma (Platinum Partner), PellePharm (Patron), and Trend Community and Signify (In-kind Supporters).

The Vision

A world in which people living with rare dermatological conditions and diseases are able to lead healthy and productive lives.

The following comprise the key elements of a unifying future vision, as articulated by those representing what patients and their families really need and want –to be attained by 2030 by all stakeholders boldly working together to create a world where persons with rare dermatology conditions lead productive fulfilling lives.

Persons suffering with a RareDERM condition look to a future where they can thrive.

Patient leaders envision a world in which people with RareDERM conditions feel supported and empowered. In order to thrive, stigma must be addressed and so, the widely held perception, “but it’s just a skin disease” would no longer exist, thus eliminating the stigma associated with having a RareDERM condition. Patients and POs would feel they belong to a supportive community that provides and has easy access to resources, funding, and emotional support. Hope would flourish as cures for rare dermatological diseases would be commonplace, including genetic intervention to prevent conditions before birth.

Global healthcare systems are fully empowered to provide essential care and services to RareDERM patients.

In the future, RareDERM patients moving through the healthcare system would have a completely different experience than what they experience today. Early diagnosis would be followed by efficient access to care. Plenty of affordable and readily available, effective treatments are prescribed by empathetic health care providers who have training and expertise with rare dermatological conditions. This future, with appropriately aligned global healthcare systems, starts with universal guidelines for patient care and a research focus for RareDERM.

Stakeholders such as governments, industry, researchers, health care professionals, patient associations and advocacy groups seamlessly collaborate on international levels.

The vision for the RareDERM community is one of collaboration. With this collaboration comes dedicated funding for medicine and treatments accepted by all governments. Researchers and healthcare professionals work together to further the advancement of researcher as well as helping patients to gain access. Registries and data sets are shared and consolidated allowing all stakeholders to recognize and utilize the synergies that exist. Industry supports propositions for venture philanthropy. There is consolidation across disease groups for advocacy purposes and capacity building supports for patient organizations.

Challenges

With a ten-year vision of the future for the RareDERM community outlined, the challenges of achieving this future were identified. While delegates raised many issues, five key themes emerged from their discussions.

Patient organizations need to recognize and embrace the power of a collective movement.

There was an acknowledgement that a significant deterrent to a better future for RareDERM was the challenge of the organizations working together for a common purpose. Isolation amongst patient organizations easily grows for many reasons, but mostly due to a lack of information on RareDERM conditions in the medical community, the small number of people with each RareDERM condition, and a wide and complex system of access to support based on geographical and socio-economic factors. The silos these factors have created within the PO community has thus far inhibited building a global movement. However if POs embrace the commonalities across organizations and develop a genuine trust to work together for the common good, a true collaboration between POs leveraging the power of a very large and therefore more influential community of people leads to an increase in capacity to raise a collective voice with consistent messaging.

The information collected regarding RareDERM research data and patients is currently inconsistent, fragmented and not centralized.

Evidence-based information is scarce in the RareDERM world leaving patients with a difficult landscape to navigate. The research data and information about patients with a RareDERM condition that does exist is fragmented and inconsistent. Ultimately, this disjointed information makes it very difficult for the patients to find the right guidance and make decisions around their conditions. This lack of information leaves the medical community trying to provide the best options without necessarily having access to all of the best evidence. Global patient registries, regional centers for diagnosis and treatment and biobanks would alleviate these frustrations and provide patients, doctors and researchers with easier access to information, and centralize evidence and data to support best informed decisions regarding RareDERM diagnosis and treatment.

RareDERM patients need different channels to be heard by regulators and health system decision-makers in order to establish a greater connection between health care researchers, professionals and POs.

The needs of RareDERM patients present varied personal and systemic challenges. To address these needs, increased representation of patients within all levels of health care planning and the approvals processes is necessary. For instance, within the healthcare research realm, patients should be involved in shaping/informing clinical trials for rare dermatological therapies. And for health care systems, a seat for patient organizations in policy-making circles and national clinical guideline development should be provided by governments to ensure the patients' voices are heard and included. Providing these different types of channels for RareDERM patient voices leads to the establishment of essential relationships between researchers, dermatologists and POs.

RareDERM patients face challenges in accessing the best care.

RareDERM patients globally are need and deserve proper care. Patient leaders from countries all over the world agreed that the shortage of trained dermatological healthcare professionals, which compounds patients' challenges, is a key determinant. Amongst health care professionals, knowledge of rare skin diseases is inconsistent, and patients often face multiple appointments with several dermatologists and specialists before they receive a definitive diagnosis. However, even with a diagnosis of a rare skin condition comes the difficulty of finding an available treatment. And, currently, if an effective treatment has been developed, then cost and access to the treatment often become barriers. RareDERM patients encounter many hurdles to accessing the best possible care. Affordability of treatment should never have to be a consideration. This points to the importance of a universal health care movement for the RareDERM community.

Stakeholders need education about RareDERM.

Rare dermatological diseases are a unique, challenging environment fraught with misconceptions and lack of information. To bring hope and change for the RareDERM community stakeholders require more education about the needs and importance of rare dermatology. Raising awareness about the true impact of RareDERM conditions—what they are, onerous daily treatment regimes, barriers to access of diagnosis and treatment, psychosocial effects on patients and their caregivers— contributes to a unified vision of the needs of the community. With an understanding of RareDERM as a whole, governments, healthcare systems, industry, POs, healthcare professionals, researchers, and the biotechnology community can coordinate all their efforts to create a world where persons with rare dermatological conditions and diseases are able to lead healthy and productive lives.

Proposed Roadmap

In looking ahead to the future of the RareDERM movement, patient organization leaders agreed that the community must define a three-year strategic plan. The beginning groundwork for the development of a three-year strategy was laid on the last day of the Forum as the POs divided themselves into five working/advisory groups. Each working/advisory group focused upon one goal for the RareDERM community resulting in five focal points for the community at large. The proposed roadmap leading to the creation of the three-year strategy consists of the two following broad goals to be operationalized by GlobalSkin under the direction of the five working/advisory groups and the five focal points/goals each group created.

- I. Form and lead a RareDERM Coalition of Stakeholders that includes doctors, medical students, industry, government, researchers and biotech to create a long-term coordinated strategy to realize the vision set out by a network of patient organization leaders.

- II. In line with the coordinated strategy and utilizing existing resources, build programs, initiatives and resources to support all members of the RareDERM community to help achieve the shared vision of a world in which people with rare dermatological conditions and diseases are able to lead healthy and productive lives.

Working/Advisory Groups Five Focal Points:

- 1) Conduct an environmental scan of RareDerm
 - a) Assess the current RareDERM patient environment including consideration of industry, patient support and government support (orphan drug policies, etc).
 - b) Create a database that identifies rare dermatological diseases, by country and lists available data on:
 - Incidence
 - Access to treatment and care
 - Economic and social impact
 - Clinical trials
 - Publications
 - Registries
 - Patient organizations
 - Social media support groups
 - c) Build the database so it is usable by all members of the network to further the collective agenda – improving the lives of those living with RareDERM diseases

- 2) Build a RareDERM Coalition
 - a) Create, with GlobalSkin's support, a patient advocacy board and representative group
 - b) Collaborate on patient leader education and capacity building, using best practice examples from existing networks and organizations
 - c) Gather best in class examples of RareDERM tools and create a system for sharing amongst all Coalition members
 - d) Formalize the network under the GlobalSkin umbrella, using GlobalSkin staff/resources to provide infrastructural support to all committees and working/advisory groups
 - e) The Coalition to meet annually or bi-annually to build strategy, and capacity for all members to further the collective agenda
- 3) Leverage the Power of the RareDerm Community Network
 - a) Create a regular, multi-stakeholder meeting between pharma, payers and patient representatives.
 - (i) Establish points of commonality between stakeholders in order to turn challenges into opportunities
 - (ii) Demonstrate that this new network brings value to stakeholders and the patients they all serve
 - (iii) Create action plans for each stakeholder group to further the collective agenda
- 4) Create a Centralized Hub for RareDerm resources
 - a) Share RareDERM community information via the creation of a resource hub with consistent data standards
 - b) Initial key foci:
 - Infrastructure (security (DB)/integration)
 - Design
 - Define purpose (use) data to collect
 - Consent
 - Create guidelines
- 5) Influence Public Awareness and Deliver Global Advocacy
 - a) Influence public awareness about RareDERM through a global RareDERM/Skin Awareness Day.
 - (i) Provide materials and common speaking points for all stakeholder groups
 - (ii) Use the occasion to approach decision-makers locally and globally to address policy gaps
 - b) Use the Global Research on the Impact of Dermatological Diseases (GRIDD) data and other data sets on human and economic impact of disease; build a program that adds the validated voice of RareDERM community to the global discussion addressing pressing medical/research needs

Conclusion

RareDERM patients have unique and serious needs. They belong to a complex world that is constantly riddled with never-ending challenges revolving around a lack of awareness, research, and access to diagnosis, treatment and care. Looking at these problems from the perspective of different medical systems, geographical confines and a wide range of social and economic issues and it becomes apparent that the challenges for this community are significantly compounded. These challenges are certainly not new to patient leaders.

By building a roadmap to contribute to the realization of a collective vision, where challenges are minimal or no longer exist, patient organizations can bring hope to the patients they serve. The RareDERM Forum provided the time and space for patient leaders to collectively consider the challenges and turn them into opportunities for a better future with the creation of five goals for the RareDERM community. These efforts, combined with the support of stakeholders, sets out the roadmap that will lead the way to creating meaningful change for the RareDERM Community.

More than half of GlobalSkin's member organizations worldwide are categorized as serving people living with RareDERM. This positions GlobalSkin in the unique role of supporting rare skin conditions within the dermatology patient organization community globally and locally. GlobalSkin will continue to lead and work with these five new RareDERM Community Working/Advisory Groups and the broader RareDERM Coalition to significantly and measurably improve linkages for RareDERM patient organization leaders, build capacity and work towards a coordinated strategic approach to improve access to care, critical information and treatment options for people living with rare dermatological conditions. We, GlobalSkin and the RareDERM Movement, are committed to achieving the vision of a better life for RareDERM patients around the world.

The International Alliance of Dermatology Patient Organizations (IADPO) - also known as GlobalSkin - is a unique global alliance of over 150 patient organizations in 45 countries representing over 60 disease areas.

Together, we are committed to improving the lives of patients worldwide. We nurture relationships with members, partners and all involved in healthcare - building dialogue with decision-makers around the globe to promote patient-centric healthcare. GlobalSkin's work is founded on three pillars: research, advocacy and support. For more information, please visit globalskin.org.

APPENDIX A: RareDERM Forum Patient Organization Participants

Working/Advisory Group Topic*	First	Last	Organization	Country
Growing RD Network	Kathryn	Andrews-Clay	Canadian Skin Patient Alliance	Canada
Create RD Hub & Registries	Neil	Bertelsen	Bertelsen Consulting	UK
Create RD Hub & Registries	Tina	Boileau	DEBRA Canada	Canada
-	Michelle	Capon	Canadian Association for Porphyria	Canada
Create RD Hub & Registries	Maryna	de Beer	Skin Cancer Foundation	South Africa
Growing RD Network	Catherine	de Vaal	Teddington Trust	United Kingdom
-	Brian	Fisher	Coalition of Skin Diseases	United States
Growing RD Network	Laurence	Gallu	Association Pemphigus and Pemphigoid France	France
-	Pamela	Gavin	NORD	United States
Leverage RD Network	Michelle	Hall	EB Research	United States
Deliver Global RD Advocacy	Trina	Harris	PEM Friends	United Kingdom
RD Landscape Analysis	Sharon	Hockey	PEM Friends	United Kingdom
RD Landscape Analysis	Lauren	Isbell	Nevus Outreach	United States
Deliver Global RD Advocacy	Daisuke	Ito	Japanese Albinism Network	Japan
-	Ira	Jain	DEBRA Singapore	Singapore
Create RD Hub & Registries	Ritu	Jain	DEBRA International	Singapore
-	Usman	Kahn	Eupati	Belgium
RD Landscape Analysis	Marion	Kloster	Verein Lichen Sclerosus	Switzerland
Leverage RD Network	Lucy	Lott	TedX talk	United Kingdom
Create RD Hub & Registries	Coumba	Makalou	The Salif Keita Global Foundation	United States
RD Landscape Analysis	Mary	McGowan	The Myositis Association	United States
Deliver Global RD Advocacy	Nicola	Miller	Teddington Trust	United Kingdom
Deliver Global RD Advocacy	Leigh-Anne	Nel	XP Society	South Africa
Growing RD Network	Marilyn	Perez-Mendoza	International Hyperhidrosis Society	United States
Growing RD Network	Jean	Pickford	Gorlin Syndrome Alliance	United States
RD Landscape Analysis	Angela	Roberts	South African Epidermolysis Bullosa Interest Group	South Africa
Leverage RD Network	Tricha	Shivas	The Myositis Association	United States
Deliver Global RD Advocacy	Annemarie	Sluijmers	Lupus Europe	Belgium
Growing RD Network	Lyne	St-Ours	DEBRA Canada	Canada
-	Alexey	Sucharev (Dr.)	Skin and Allergic Diseases	Russian Federation
Growing RD Network	Ellen	Swanborn	Stichting Lichen Sclerosus	Netherlands
Growing RD Network	Susan	Thornton	Cutaneous Lymphoma Foundation	United States
RD Landscape Analysis	Lex	van der Heijden	CMTC-OVM	Netherlands
Create RD Hub & Registries	Marjolein	van Kessel	Naevus Global	Netherlands
-	Karin	Veldman	Vereniging voor Ichthyosis Netwerken	Netherlands
Growing RD Network	Suzanne	von Seitzberg	Lichen Sclerosus Foreningen	Denmark
Deliver Global RD Advocacy	Sandra	Webb	XP Support Group	United Kingdom
Create RD Hub & Registries	Geske	Wehr	Selbsthilfe Ichthyose e. V.	Germany
Deliver Global RD Advocacy	Marc	Yale	International Pemphigus and Pemphigoid Foundation	United States

*Patient organization volunteers for working/advisory group topics