

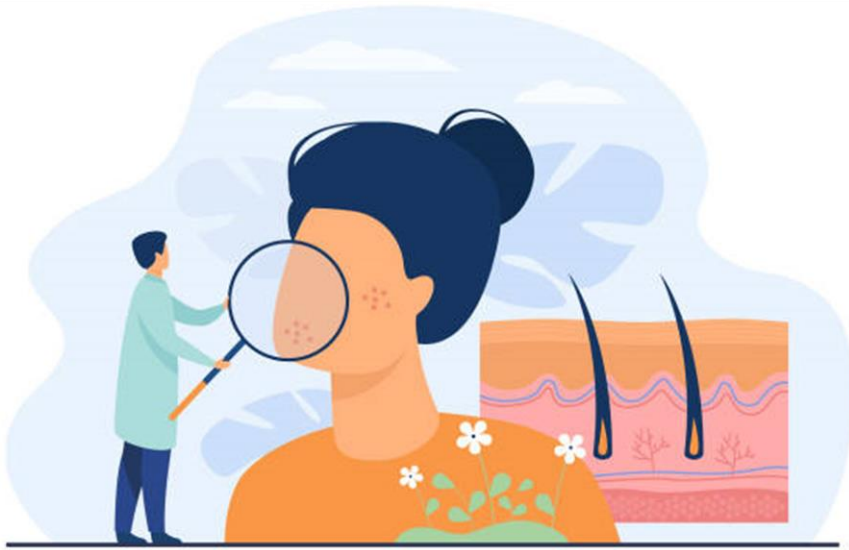


DakshamA Health & Education  
*Your Voices Your Choices*

# REPORT

## ROUND TABLE DISCUSSION

*"Rare Disease Policy- scope for inclusion and treatment of New Rare Diseases not included in National Rare Diseases Policy  
26<sup>th</sup> February, 2022*





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## INTRODUCTION

Rare diseases usually affect small populations of patients, sometimes fewer than 1 in 2,000 individuals. Most rare conditions are chronic, complex, degenerative, painful, and often fatal. Rare diseases affect around 4% of the world's population, or about 300 million individuals, a figure equivalent to other major non-communicable diseases. These rare conditions result in significant unmet needs for those impacted along with their caregivers, ranging from quality of life and health, equality, social inclusion, and access to education, as well as employment and other chances to contribute back to society.

Countries worldwide have their own definitions to meet their needs. Their definition of rare disease is constructed from the perspective of their own demographics, healthcare systems, and available resources. The use of different definitions and terminology can lead to confusion and inconsistencies, which has repercussions for treatment access as well as on research and development. India does not have a definition for rare diseases due to paucity of data and epidemiological evidence.

The landscape for Rare Diseases is constantly evolving due to its complexity and variety, as new rare disorders and diseases are discovered and described in the medical literature on a regular basis. Dermatological rare conditions are often overlooked as the specialty is not treated as priority. The identification and treatment of these rare diseases are difficult due to a number of challenges. Despite a number of global programs aimed at addressing concerns related to rare diseases, more work needs to be done to address the current challenges.

Ministry of Health and Family Welfare recently approved a national policy for rare diseases. The policy aims to reduce the incidence and prevalence of rare diseases through an integrated and comprehensive preventive strategy that includes raising awareness, early screening, counselling programs and enabling patients with rare diseases to access affordable health care within the constraints of resources and competing health care priorities. Financial support under the Umbrella Scheme of Rashtriya Arogya Nidhi has been provided for a defined set conditions. The biggest challenge remains the cost of treatment and funding for treatments of a wide range of diseases that are currently not covered under the policy. The disorders classified as Group 3 with definitive treatment accessible but at a very high cost and requiring lifetime care are currently not funded by any means. The inclusion of PSUs and corporate houses within CSR standards, as well as the recommendation of a voluntary crowdfunding system to generate resources even through digital media, does not guarantee that a patient would be able to raise enough funds for lifelong treatment in a timely manner.

DakshamA Health organized a round table discussion on the theme **"Rare Disease Policy- scope for inclusion and treatment of New Rare Diseases not included in National Rare Diseases Policy"** on the occasion of World Rare Disease Day, 2022. The focus of this round table discussion was to explore opportunities and scope for including rare dermatological disorders, and a uniform care continuum pathway for rare dermatological disorders into the existing Policy's ambit.



**National and International speakers shared their views on various topics during the discussion. The speakers were:**

**Chair:**

**Dr. Ramesh Bhat**, *Vice Dean, Father Muller Medical College, Kankanady, Mangalore, Director ILDS, Past president IADVL*

**Speakers:**

**Dr. Murlidhar**, *Rajagopalan, Senior Consultant and Head Dermatology, Apollo Hospitals, Chennai*

**Dr. L. Swasticharan**, *Chief Medical Officer, Directorate General of Health Services, Ministry of Health and Family Welfare, New Delhi, India*

**Dr. Madhulika Kabra**, *Professor, Division of Genetics, Department of Pediatrics, All India Institute of Medical Sciences, New Delhi*

**Mr. Paul Albert Mendoza**, *Vice President of Psoriasis Philippines and President of Psoriasis Asia Pacific*

**Dr. Ritu Jain**, *Board director at Global skin, International Alliance of Dermatology Patient Organizations, founder & President of Dystrophic Epidermolysis Bullosa Research Association (DEBRA)*

**Dr. Sunil Dogra**, *Prof. of Dermatology at PGIMER Chandigarh*

**Key takeaways from the discussion points:**

**1. Awareness:**

- Understanding of rare diseases is limited, especially those related to the dermatological conditions both amongst patients as well as HCPs.
- People in the community lack knowledge about the disease's etiology, signs and symptoms as well as the disease life course. This in turn leads to poor understanding of managing their condition.
- In India, there is no awareness material or resources available on dermatological conditions for patients and caregivers that is easily accessible
- There is currently no reliable source for patients to identify and learn about their treatment and diagnosis.
- Lack of awareness results in inadequate screening and delayed treatment.
- Dermatological conditions have a significant impact on people's quality of life, but they are rarely addressed in the community, leading to stigma and impact on mental health of patients

**2. Access to treatment:**

- Misdiagnosis, limited treatment options, and limited access to care, cause unnecessary suffering to patients with rare disorders like Generalized pustular psoriasis (rare dermatological disorder)
- Due to high out-of-pocket costs, treatment unavailability, or difficulty accessing medicines even when they are available, patients have additional challenges in obtaining such therapies.
- ILDS is requesting WHO to recognise biologics as essential drugs.



- HCPs are unaware of new treatments options and often do not follow treatments and screening protocols
- Not all dermatologists are trained to diagnose and manage all dermatological rare diseases and continuing medical education is very necessary.
- Request for inclusion of new rare disorders can be sent to the central technical committee by the treating clinician with the relevant information for inclusion into the National policy after which it will be reviewed and included.

### 3. National registry for dermatological rare conditions:

- ICMR has initiated a hospital-based 'National Registry for Rare Diseases,' which involves centres throughout the country that deal with the diagnosis and treatment of rare diseases.
- Patient registries for rare diseases that will assist in determining the burden of rare diseases, such as dermatological problems, as well as the burden of patients' quality of life are an urgent priority.
- The registries will help arrive at a proper definition that accounts for the specificities of our own healthcare system as well as define the national disease of burden in terms of rare diseases.
- Need to list all rare diseases for the purpose of insurance and other financial coverage

### 4. Research and development:

- There are limited studies to understand the cost benefit analysis of the interventions to treat the dermatological rare diseases.
- More treatment options for treating rare dermatological conditions need to be researched.
- There is a paucity of scientific evidence on the use of biologics to treat these conditions.
- There are no studies on quality of life and economic loss to patients due to dermatological conditions.
- Research into the various social, economic, and cultural aspects that influence treatment access, and the duration patients seek treatment is the need of the hour
- Networking between regional care centres, tertiary centres, state medical colleges, periphery and interactions between basic science institutes on drug development and promote research is needed.

### 5. Standard treatment and operational guidelines:

- There needs to be a constructive policy and system of regulations in place to identify conditions that can be considered rare.
- There is a need for standard treatment and diagnosis protocols for people who are living with rare dermatological disorders.
- Although the government covers a significant number of expensive drugs, there must be a system in place to track how these treatment options are used by patients and prescribed by physicians.
- The National policy has already set a precedent by including three new rare diseases and inclusion of dermatological rare diseases will not be an issue.

### 6. Insurance coverage:

- Involvement of insurance stakeholders to help them understand the importance and purpose of including dermatological disorders in the insurance package.



- A national portal for crowdfunding exists and the funds raised go directly to the Centers of Excellence. Patients are screened as per eligibility criteria.

### 7. Lack of trained manpower:

- Lack of access to medical specialists such as trained dermatologists is a key gap area. To ensure the best treatment and management of the condition, specialists must have adequate training.
- Continuous medical training is essential for maintaining uniformity and improving care quality for persons suffering from rare dermatological conditions.
- Nurses, pharmacists and other allied health professionals also need orientation and training to provide supportive services.
- Centers of excellence are mandated to raise awareness and train healthcare professionals to manage rare conditions

### CONCLUSION:

The present national policy for rare diseases includes a provision for incorporating rare dermatological diseases. This can be accomplished by bringing together all stakeholders and policymakers, as well as the voices of patient advocates, on a single platform. The policy does not debar any rare disease and is open to inclusion with a formalized procedure that includes a request from the treating clinician to the Central Technical Committee. It is critical that we handle dermatological diseases as a public health issue. Patient organisations have been at the forefront of disease awareness and advocacy for national supportive legislation. This can be further strengthened by prioritizing some of the discussion points and recommendations listed above.