Together for Rare

Blueprint for Rare Diseases in India Initiative

Webinar Report

August, 2020

PPHF
PEOPLE TO PEOPLE HEALTH FOUNDATION

GRID Council

Takeda

Resilience Approach towards Rare Disease Policy in India

4th August 2020 | 11:00 AM-1:00 PM

Partners: GRID Council & Takeda Pharmaceuticals

Total Participation: 129 Attendees

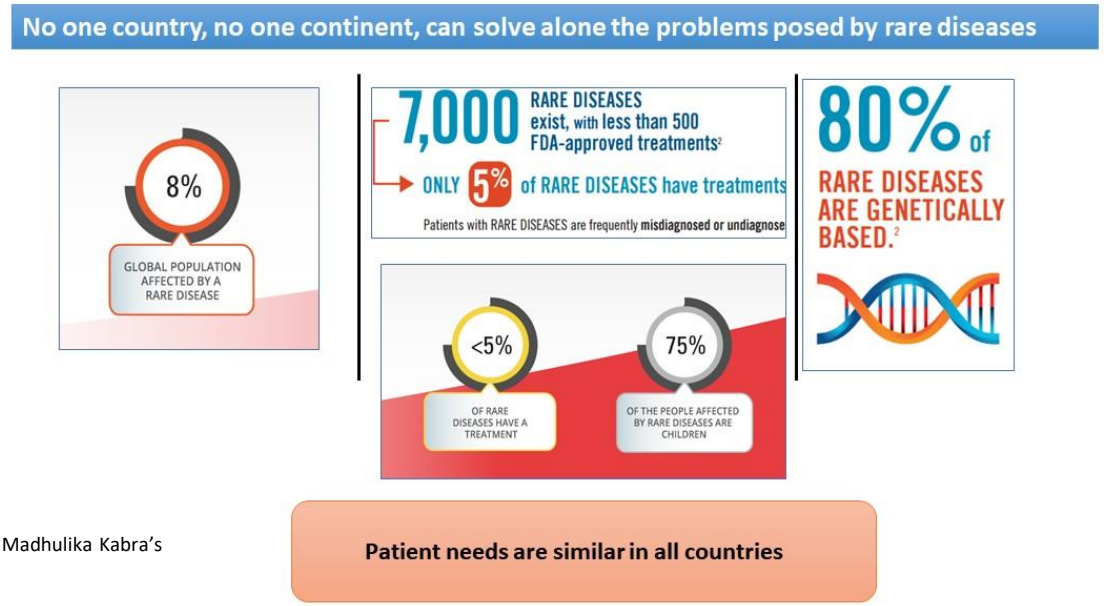
Executive Summary: On 4th August 2020, People to People Health Foundation (PPHF) in partnership with GRID Council and support from Takeda Pharmaceuticals organized a webinar on "Resilience Approach towards Rare Disease Policy in India" under the initiative- Together for Rare- Blueprint for Rare Diseases in India which is a pioneering initiative designed as multi-stakeholder initiative and collaboration focused on rare diseases (RD) in India. The initiative is foresighted and seeks to prioritize the needs of people suffering with RD by encouraging partnerships innovation and strengthening the nation's commitment towards delivery of greater impetus and better outcomes. The agenda for the webinar was to resurface and prioritize Rare Diseases as health priority in India.

The webinar was represented by a panel of esteemed speakers from National Organization of Rare Diseases (NORD-USA), AIIMS, State representative, OPPI and patient advocate. The webinar observed participation from various sectors including Government bodies, International bodies, patient advocacy groups, research and academia organizations, pharma industry, PSUs with a total participation of 129.

During the webinar, major stress has been given to multi-stakeholder collaboration models, capacity building for awareness generation and improving education for medical professionals and public, use of technology and other alternatives to combat issues related to access to treatment, clinical research especially for drug development and pyramidal approach to health service delivery planned out based on the locally collated data findings.

Context: As per the trend in India, rare diseases have not been on the forefront due to the various challenges associated with the absence of policy directions, provision of services, lack of data and research in this area. Although the Government of India has been trying to establish a robust policy in place for the treatment and management of Rare Disease in INDIA, the whole process of policy formulation has been facing its own set of challenges. Government encouraged transparency and innovation as for the first time they released any draft national policy on 13th January 2020 in the public domain for review and inputs. Taking up this opportunity in February, PPHF invited the key stakeholders like State Government, research and academia, industry representatives, advocacy groups etc for a roundtable technical discussion along with FICCI and came up with the key recommendations after reviewing the policy. The recommendations were presented to the Joint Secretary at Ministry of Health and Family Welfare (MoHFW) for consideration and inclusion in the final policy which is still awaited.

The impact of Rare Disease - A Global Challenge



Slide from Dr. Madhulika Kabra's presentation

Apart from the ongoing challenges, another big challenge that has further laid back the responses in the rare disease space is COVID 19 which has affected health services across. There are financial implications on the drug manufacturers, transportation of drugs has become cumbersome, access to treatment due to restrictions in mobility etc are few of the many challenges to start with. Release of national policy has been delayed which has further added hurdles to the rare diseases environment since currently there are no standardized directions for execution which can be adapted or built upon to tackle the current situation.

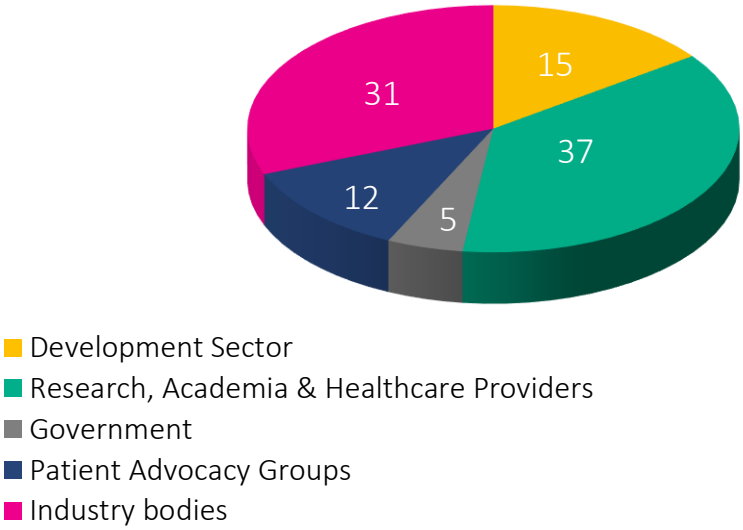
PPHF took the current scenario as an opportunity to highlight the special needs of individuals and families fighting rare diseases and to rekindle a dialogue between various stakeholders on possible solutions. With the objective of building a sense of urgency on tackling RD in India and increase policy attention, PPHF collaborated with GRID council in support from Takeda to start with the first in the series of rare disease technical events under the initiative of "Together for Rare: Blue Print for Rare Diseases in India" webinar on "Resilience Approach towards Rare Disease Policy in India" on 4th August 2020. The dialogue will continue Pre and post policy release with series of discussions, CME, seminars, webinars, presentations etc. to prioritize the rare disease agenda and which address the "how" part of policy implementation by generating concrete recommendations.

Proceedings: The webinar consisted of sessions on global policy priorities for rare diseases & how COVID 19 has amplified the need for access to tele-medicine, rare disease scenario in India, Karnataka State perspective and progress made in the rare disease management, Industry's contribution & insights for mobilizing partnerships and stories from the ground. Each presentation was followed by a poll for the participants.

Detailed agenda is attached as Annexure 1.

The event was attended by 129 participants (Fig: 1):

Sectorwise Partcipation (%) Total 129 Attendees



(Fig: 1)

The meeting commenced with a high- level overview of rare diseases in India by Dr Archisman Mohapatra, ED, GRID council followed by a brief introduction about the whole initiative by Dr. Laxmikant Palo, CEO, PPHF.

"If there is a bright side to this pandemic then it is the high uptake of tele-medicine. So many people in the rare disease community for many years have tried to get access to broadened tele-medicine and amid this pandemic we are seeing an uptake like we have never seen before."

"We are moving in the right direction and in the last decade India has really done well around rare diseases. Capacity building efforts for education, diagnostics, prevention, research, and community education have gone in but we need further strengthening on all fronts. A sustainable strategy is to be planned for specific treatment and supportive care needs strengthening at all health care levels. I am sure we will succeed if all stakeholders join hands together and work toward this cause".

Dr. Sanjeeva GN
Nodal Officer & Associate Professor, Center of Excellence for Rare Diseases, Indira Gandhi Institute of Children, Bangalore

"Rare disease being heterogeneous, one pill approach will not work in this situation. We need to have robust local data and depending on that local actions can be prioritized."

"We are going through a crisis of COVID-19 but let us take that crisis as an opportunity. I have seen a renewed focus on the health sector over the last 6 months. Building a comprehensive and sustainable policy for Rare Diseases is possible now, given the tremendous efforts of the Government in the health space. This can be achieved through multi-stake holder partnerships and collaboration."

KG Ananthakrishanan
Director General
OPPI

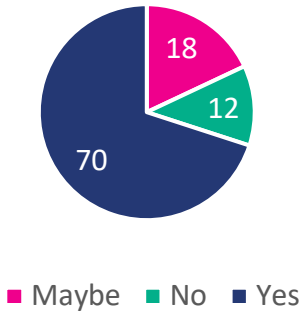
Saurabh Singh
Patient's Advocate

"Working on community awareness and medical insurance is important. Insurance companies can especially support supportive treatment."

Webinar Poll Results:

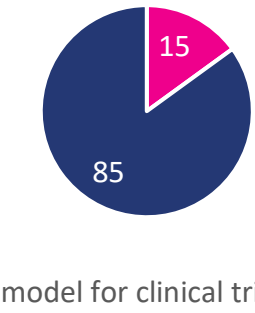
Poll Question 1:

Can telemedicine be a sustainable dynamic when it comes to rare diseases post COVID?



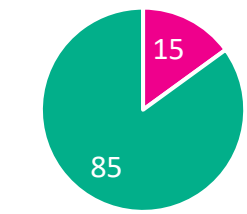
Poll Question 2:

To improve patient access and enrolment in clinical research during the pandemic, which strategy is best suited?



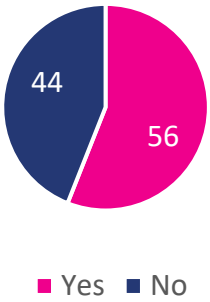
Poll Question 3:

How can patient's access to proper treatment be improved without compromising the quality of treatment in the most feasible manner?



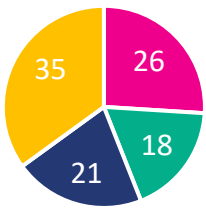
Poll Question 4:

Should an earmark budget with contributions from public-private sector for prevention and research on rare diseases part of the policy in India?



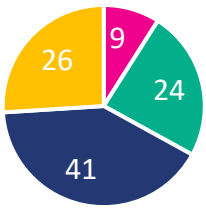
Poll Question 5:

What according to you is the main cause of the lack of investment and funding in rare disease space in India?



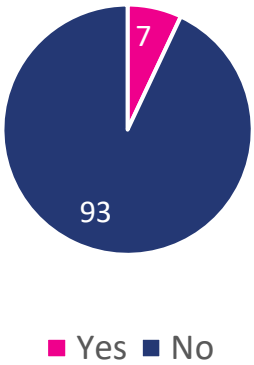
Poll Question 6:

Industry bodies should invest maximum funding in which of the following?



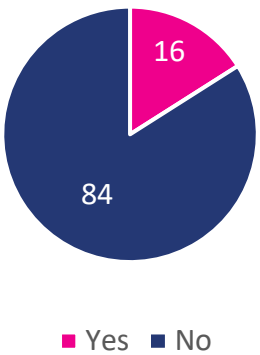
Poll Question 7:

Should new-born screening for rare diseases be made mandatory by the Government of India?



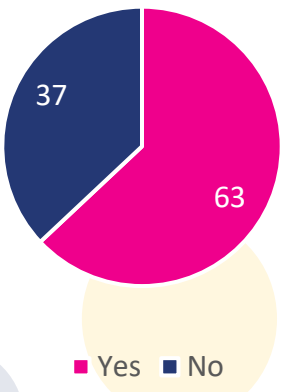
Poll Question 8:

Do you think it will be wise to direct Central Public Sector Enterprises (CPSEs) to place a certain % of their CSR funds into the said PM Rare Disease Treatment Fund?



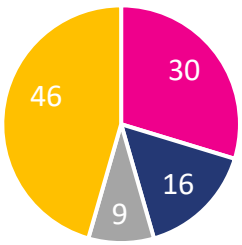
Poll Question 9:

Do you think there has been improvement in the patient care services in the last five years?



Poll Question 10:

What can be the best sustainable funding mechanism for sustained treatment of rare disease patients?



- Center & State Contributions
- Create PM Rare Disease Treatment Fund pooling CSR and PSU
- Dedicate a portion of Health Cess
- Develop a collaborative co-pay model with central-state-industry partnerships

The key suggestions and recommendations from the webinar are:

1. Prioritization of preventive strategies at all levels (e.g. genetic counselling, prenatal screening and diagnosis, Newborn screening) is important.
2. Strengthening capacity building efforts on various aspects of rare diseases including improving education and awareness among medical professionals, health care workers and public are essential.
3. Specific therapies and supportive care should be universally available for patients with rare diseases including insurance coverage.
4. Sustainable funding mechanism should be in place to ensure treatment of people living with rare diseases and state and central government partnerships for funding and along with the obligation of Corporate Social Responsibility funds for Rare Diseases.
5. Strengthening and funding research on rare diseases particularly in drug development must be focused upon.
6. Networking of patient care services from the primary health care level to tertiary care centers is an urgent need.
7. India can set up a national and/or regional (South/South East Asia) Reference Network along the lines of the European Reference Networks.
8. States must focus sharply on creating robust program for local prevalence data, a quasi-pyramidal model of service delivery, technical and operational research, and scale up in a phased manner.
9. Successful state-level interventions can be evaluated and scaled up at a national-level.
10. India can replicate/adapt successful initiatives from other countries
11. Multi-Stakeholder platform with representatives from Central Government & the State Government, academia, industry, and civil societies must be created to address challenges.
12. India must set-up Centre's of Excellence in each State to generate state level data for the centralized ICMR Registry
13. Coordinated efforts on simplifying and organizing systems for counselling, awareness, diagnosis, management, drug procurement, financial and peer support systems are critical.

Way Forward

Rare diseases are rare in nature but impact millions across the globe. Cost to the system and community is so high that it requires collective solutions. We need to take small but concrete steps consistently to ensure a robust rare diseases service delivery system at all levels.

India needs to start with a standard definition followed by estimation of prevalence and simultaneously work on releasing the national policy for necessary directions for all the states with the provisions of adaptability moving forward based on the new learnings from the states and other countries.

Building greater collaboration with the stakeholders working on Rare Disease is essential to achieve greater visibility for rare diseases in India backed by a better rare disease policy.

Annexure 1

Length	Time	Content	
11:00-11:05 AM	5 mins	Welcome remark	Dr Laxmikant Palo, CEO, PPHF
11:05-11:15 AM	10 mins	Introduction and High-level overview on RD scenario in India	Moderator Dr Archisman Mohapatra Executive Director, GRID Council, Delhi NCR, India
11:15-11:25 AM	10 mins	Policy Priorities for NORD & how COVID 19 has amplified the need for access to tele-medicine?	Ms Rachel Sher, Vice President Policy and Regulatory Affairs, National Organization for Rare Disorders (NORD), USA
		Poll	All Participants
11:30-11:40 AM	10 mins	Rare Diseases scenario in India	Dr Madhulika Kabra, Professor, Division of Genetics Department of Pediatrics, All India Institute of Medical Sciences (AIIMS), New Delhi, India
		Poll	
11:42-11:52 AM	10 mins	State perspective & progress made in RD management	Dr Sanjeeva GN Nodal Officer & Associate Professor, Center of Excellence for Rare Diseases, Indira Gandhi Institute of Children, Bangalore, Karnataka, India
		Poll	
11:54 AM-12:02 PM	10 mins	Industry's contribution & insights for mobilizing partnerships	Mr KG Ananthakrishnan, Director General, Organization of Pharmaceutical Producers of India (OPPI), India
	30-60 secs	Poll	All Participants
12:05-12:15 PM	10 mins	Voices of the patients- Opportunities & challenges	Mr Saurabh Singh, Patient's Advocate, India
		Poll	
12:15-12:45 PM	30 mins	Questions & Answers	Moderator
12:45-1:00 PM	15 mins	Closing remarks and Vote of thanks	Moderator