Recommendations

From the Roundtable Multi stakeholder Discussion on Draft National Policy for Rare Diseases

February 7, 2020, New Delhi

This is a consensus document reflecting key recommendations to strengthen the Draft National Policy for Rare Diseases prepared from detailed deliberations by a diverse group of respected experts and stakeholders in Rare Diseases, facilitated by PPHF and FICCI. Over 40 diverse stakeholders, representing government, non-governmental organizations, civil society, corporate leaders, industry networks, bilateral development agencies, patient organizations, medical institutions, academia, researchers, clinicians and Rare Diseases experts, met for the Roundtable Discussion on "Draft National Policy for Rare Diseases" on February 7, 2020 using a structured participatory meeting methodology called Open Space Technology. Open Space Technology is best used when there is an important issue to be addressed, there is a diversity of people involved and there is complexity.

The members, in a participatory manner, have worked as team to review and synthesize the list of urgent actions based on the need, experiences, evidence around Rare Diseases in India. Participants of the Roundtable came together in an effort to extend collaborative and practical recommendations towards strengthening the draft National Policy for Rare Diseases. Following are the recommendations from the deliberations in this exercise:

High Priority Recommendations:

1. We call for an **immediate fund allocation of Rs 500 crore towards treatment** of already diagnosed patients (approximately 400 patients majorly in Group III diseases) that are still awaiting treatment and suffering for the past over two years. Through this annually allocated fund, treatment to all patients for which drugs that are already approved by DCGI (and those that will be approved in future) can be availed. Such patients need to be urgently taken under the fold of treatment considering their Right to Life as outlined in Article 21of the Constitution of India.

2. We call for a **sustainable funding mechanism in place to ensure sustained treatment to the patients with rare diseases.** The potential sources of such sustainable funding could be:

   a) Centre and State contribution (in 60:40 ratio) through NHM, based on the existing PIP model could be adapted for rare diseases as well;
   b) Dedicate a portion of Health Cess;
   c) Create Prime Minister Rare Diseases Treatment Fund; pooling of CSR and PSU funds and provision for tax benefits to companies donating to it via amendment in Companies Act Schedule VII of CSR provisions;
   d) Develop a collaborative co-pay model with the Central Govt - State Govt- Industry partnership and define the percentage break up of cost sharing;
   e) Formulate guidelines for medical insurance to cover the costs for specific diseases requiring supportive therapies.

3. We call for **the committed financial assistance (of amount Rs 15 lakh as one-time payment) to be applicable for all falling under Group I diseases**, and not be limited only to those eligible as *Pradhan Mantri*

Jan Arogya Yojana (AB-PMJAY) beneficiaries. Inclusion of provision for disease specific financial packages or yearly packages in group 2 & 3 categories of diseases; and sufficient allocation to cover the cost of treatment for diseases requiring lifelong treatment and provision for inclusion for other disorders/ new diseases.

4. We call for **complete waiver of taxation (GST and customs duty) on drugs for treatment of Rare Diseases**. Rare disease drugs ought to be tax free in the same way as other life-saving drugs.

5. We call for **Earmark budget for prevention (education, communication, awareness) and research** on Rare Diseases in India. Establish a public–private partnership for a national Rare Diseases education and Awareness building program for the healthcare providers/physicians and community.

6. We call for creation of state **level diagnosis & treatment centers in all states** in addition to the Centre of Excellence at the national level. This can be initiate in an incremental manner starting with 5-6 states with existing capacity. Develop a selection criterion for identification of Centre of Excellence including private hospitals. The state level centers can also help ICMR for maintaining registry.

7. We call for a **mandatory provision for universal screening of newborns**. The Newborn screening can identify rare diseases in the first few days of a baby's life. The early intervention and treatment can produce lifesaving changes for these children, a dramatically improved quality of life for the entire family, and often significantly reduced costs to the public health care system.

8. We call for **a multi-sectoral convergent and linkages model to tackle rare diseases** in India. Define role for each Ministry and concerned departments and develop an implementation framework on measures to be taken by them on their sector wise response to tackling rare diseases. For example, use the infrastructure of rare blood disorders centers already available in the states.

***Additional Recommendations*:**

9. **Adoption of technological advantages offers new prospects for early diagnosis** (e.g. next generation sequencing) and **therapies** and offer better efficacy for rare and genetic disease.

10. India need to have the **definition of Rare Diseases for the country**. Until India has its definition of rare disease, government can follow the definition of ICMR (1/25000 population) and clinical trial definition of orphan drug act (500000 patients in India).
11. As both government and private groups have shown increased interest in Rare Diseases, **public-private partnerships (PPPs) are essential to build capacity of providers and increase public awareness**.
12. **Industry can help in building capacity, providing equipment, reagents, personnel, support in meeting operational costs** through PPP model.
13. ICMR should **release the first report on patient registry** at the earliest. The Registry ought to be made agile in order to encompass future advancement in the rare disease arena.
14. The policy needs to be renamed more comprehensively as "**National Policy for Prevention and Treatment of Rare Disease**".
15. **Identify at least one Centre of Excellence in each State to generate state level data for the centralized Rare Disease Registry of ICMR.** All Centers of Excellence may remain connected and share data via digital platform owned and managed by ICMR.

16. Make provision for **dedicated corpus to improve the diagnostic capabilities of centers of Excellence & medical awareness**. Innovative access model is required to provide much needed access to advanced diagnostics facility and globally approved therapies.
17. **Include patient representative** within the relevant technical and scientific skills and ability to represent the patient voice in a nonpartisan and inclusive manner **in the Technical Committee**.
18. **We appeal that new diseases that fall under the definition of 'rare diseases' or a new treatment modality should be included in the existing groups.** Categorization of Rare Diseases should cover rare disease in children and adults, and diseases diagnosed in future. Current Categorization can be reworded as:

    Group:1: Rare disorders amenable to one-time treatment

    Group 2: Disorders requiring long term/ lifelong treatment

    Group 3: New diagnosed rare diseases where treatment documented in the literature have shown benefit.

It is a challenge that requires effective action across a number of sectors and actors (government, civil society, business, research, and development partners). Failure to intensify our actions and find solutions will cast a long shadow. To do so, we must act together, effectively and in alliances.

We have our personal commitment toward these recommendations and ourselves to work together in this effort with government.

Disclaimer: FICCI and PPHF facilitated the process and provided the administrative support for this document. The contents of this document do not necessarily reflect the views of FICCI and PPHF.

List of participants

Sr.No.	Name of Participant	Organization
1	Dr. Akash Sud	Fortis Healthcare Ltd.
2	Dr. A. Mahapatra	INCLEN India
3	Anil Bhushan	Pfizer
4	Amanat Thind	OPPI
5	Amitabh Baxi	Novartis
6	Avinash Tiwari	Takeda
7	Antonio Morell De Meer	European Union
8	Dr. Bhawna Dhingra	AIIMS Bhopal
9	Cheena Malhotra	PPHF
10	David Living Stone	World Vision
11	Dr. D S Ratna Devi	Indian Alliance of Patient Groups (IAPG)/ Dakshama Health
12	Dr. G.B Singh	MoHFW - NHM Punjab
13	Dr. Hitesh Muley	Pfizer
14	Harish Chand	World Vision

15	Jitendra Kumar	Takeda
16	Dr. K.C Aggarwal	VM Medical College Safdarjung
17	Dr. Laxmikant Palo	PPHF
18	Manisha Singh	Corporate Law Group
19	Manjit singh	LSDSS - Lysosomal Storage Disorders Support Society
20	Megha Gupta	PPHF
21	Dr. Nalini Kaushik	AEQUITAS Consulting
22	Nilovna Ghosh	Takeda
23	Prasanna Shirol	ORDI
24	Dr. P.C Joseph	World Vision
25	Prem Singh Rawat	OPPI
27	Parameshwaran Sitaram	Sanofi Genzyme
28	Dr. Partha Gokhale	BI - Boehringer Ingelheim
29	Dr. Rema Chandramohan	Institute of Child Health - Chennai
30	Dr. R K Mani	Batra Hospital
31	Dr. Ravi Gaur	Oncquest
32	Rakesh Chawala	IPSPI - Indian Patients Society for Primary Immuno Deficiency
33	Rubby Chawala	IPSPI - Indian Patients Society for Primary Immuno Deficiency
34	Dr. Sandeep Sewlikar	Roche Diagnostics
35	Sunil Tade Palli	Centogene
36	Dr. Shikha Shukla	Novartis
37	Swati Aggarwal	FICCI
38	Sanchit Singhania	FICCI
39	Sandeep Arora	Takeda
40	Sarita Chandra	FICCI
41	Tania Friederichs	European Union
42	Tansi Nayak	FICCI
43	Vineet Singhal	Takeda