PERSPECTIVE

National Policy for Rare Diseases, 2021 – A critical perspective

Manju Dubey[1], Mohan Kumar[2]

[1]Assistant Professor, Department of Community Medicine, Raipur Institute of Medical Sciences, Raipur, Chhattisgarh, India; [2]Senior Resident, Department of Community Medicine, KMCH Institute of Health Sciences and Research, Coimbatore, Tamil Nadu, India

Corresponding Author

Dr. Mohan Kumar, Senior Resident, Department of Community Medicine, KMCH Institute of Health Sciences and Research, Coimbatore, Tamil Nadu, India
E Mail ID: kumar.mohan324@gmail.com

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Abstract

More than 8% of the global population is affected by a rare disease and >50% affected are children (30% of whom will die before 5 years). There are 6,000-8,000 classified rare diseases – 80% are genetically based – with less than 500 FDA approved treatments (or <5% have therapies) available to treat them. Lack of standard definition and community-based epidemiological data in India limits the estimation of prevalence and/or disease burden; though extrapolations at 6% to 8% show 72 to 96 million affected individuals. The Government of India approved the "National Policy for Rare Diseases, 2021" on 30th March 2021. It is a step in the right direction; at the time it necessitates optimal utilization of allocated resources and timely appraisal of the policy itself.

Keywords

Rare Diseases; Health Policy; India; Appraisal

Introduction

Rare diseases are defined as often debilitating lifelong disease or disorder with a prevalence of 10 or less, per 10000 population. However, different countries have their own definitions – ranging from 1 in 10,000 of the population to 6 per 10,000 – to suit their specific requirements in the context of their population, healthcare system and resources. (1) More than 8% of the global population is affected by a rare disease and >50% affected are children (30% of whom will die before 5 years). There are 6,000-8,000 classified rare diseases – 80% are genetically based – with less than 500 Food and Drug Administration (FDA) approved treatments (or <5% have therapies) available to treat them. Rare diseases affect >300 million people worldwide. (2) Lack of standard definition and community-based epidemiological data in India limits the estimation of prevalence and/or disease burden; though extrapolations at 6% to 8% show 72 to 96 million affected individuals.

The Government of India approved the "National Policy for Rare Diseases, 2021" on 30th March 2021 with an aim at lowering the incidence and prevalence of rare diseases based on an integrated and comprehensive preventive strategy encompassing awareness generation, premarital, post-marital, pre-conception and post-conception screening and counselling programmes to prevent births of children with rare diseases – within the constraints on resources and competing health care priorities. (3) Till the time country arrives at a definition of rare disease based on prevalence data, the policy had identified and categorized rare diseases/disorders as follows; Group 1 – Disorders amenable to one-time curative treatment (Hematopoietic Stem Cell Transplantation (HSCT), organ transplantation); Group 2 – Diseases requiring long term/lifelong treatment having relatively lower cost of treatment with documented benefit and annual or more frequent surveillance is required (Disorders managed with special dietary formulae or Food for special medical purposes (FSMP) or other forms of therapy like hormones/specific drugs); Group 3 – Diseases for which definitive treatment is available but challenges are to make optimal patient selection for benefit, very high cost and lifelong therapy (Sufficient evidence for good long-term outcomes exists, cost of treatment is very high and either long term follow up literature is awaited or has been done on small number of patients). (4)

Policy recommends financial assistance up to ₹20 lakhs under the Umbrella Scheme of Rashtriya Arogaya Nidhi (RAN) by the Central Government for treatment, of those rare diseases that require a one-time treatment (Group 1) – for those eligible under Pradhan Mantri Jan Arogya Yojana (PMJAY) (40% of the population), not limited to BPL families, for treatment in Government tertiary hospitals only. For patients with diseases/disorders under group 2 State Governments to provide support in terms of special diets or hormonal supplements or other relatively low-cost interventions whereas for those under group 3, government envisages to create an alternate funding mechanism through setting up a digital platform for voluntary individual and corporate donors (Voluntary crowdfunding) to contribute to the treatment cost of patients of rare diseases. (4)

Critical appraisal

1. Need to emphasize quasi-vertical approach as health in India is a state subject – decentralization of care – with technical and operational guidance from Centre.
2. In terms of understanding the disease, policy mentions creation of a hospital based National Registry for rare diseases in India by ICMR. As hospital-based disease surveillance (though a feasible option in resource constrained setting) will not give the exact prevalence of rare diseases, policy had no plans at community-based screening and surveillance (data on few rare diseases available through newborn screening under Rashtriya Bal Swasthya Karyakram (RBSK)).
3. The policy identifies 8 Centres of Excellence and 5 Nidan Kendras under Department of Biotechnology (Unique Methods of Management and treatment of Inherited Disorders (UMMID) project). These along with Department of Medical Genetics (by State governments in at least one medical college in the State) are to be utilised for education and training of healthcare providers for screening for rare diseases. But the policy fails to focus on building a pool of medical practitioners trained in management of rare diseases (by providing diploma courses and fellowships).
4. The policy specifies increasing the government support for treating patients with a 'rare disease' (only Group 1) – from ₹15 lakh to ₹20 lakh – but rare disease organisations, patient foundations, advocacy groups and caregivers voice that this does not reflect actual costs of treatment.
5. The policy caps resources for different diseases – without considering the variation in treatment costs of different rare diseases, which are only bound to increase over time.
6. The new policy has absolutely no consideration (no assurance) for Group 3 patients, who require lifelong treatment support – lack of sustainable funding; and offers no support to patients awaiting treatment since the earlier National Policy for Treatment of Rare Diseases 2017 was kept in abeyance.
7. Lack of support/funding targeted at families and/or caregivers of patients with a rare disease to ensure their commitment to patient care (to overcome caregiver burden, treatment fatigue).
8. The policy fails to plan at making available tests (screening and/or diagnostic) at least at the regional level to start with.
9. Lack of timelines for setting up diagnostic and treatment algorithms.
10. Where drugs are available, they are exorbitantly costly – no domestic manufacturers in India except for those who make medical-grade food for those with metabolic disorders. Plans to promote in-country drug manufacturing capacity of bio-similar/bio-better/drugs for rare diseases – with contribution of Organisation of Pharmaceutical Producers of India (OPPI) – should be given attention. Possibility of Orphan Drugs Act (as in United States of America) in Indian context which incentivises industry by way of market exclusivity, grants to researchers and tax incentives on expenditure incurred during evaluation of drugs for their therapeutic potentials should be explored. (5)
11. The policy had considered collaboration with Ministry of Finance for reduction in custom duties on import of medicines related to rare diseases – but should also consider exemptions from Goods and Service Taxes (GST).
12. Considering the possibility of collaborative co-pay model with the Central and State government for industry partnership and guidelines for medical insurance to industry.
13. The policy passes the buck by identifying eight hospitals across India as 'Centres of Excellence,' leaving it to them to set up crowdfunding initiatives for patients requiring it, or for families to themselves look at crowdfunding options for treatment.
14. Due attention should be given to the role telemedicine in addressing inaccessibility to rare disease diagnosis, management and follow-up.
15. Considering use of ABC approach (of inventory management) to fund rare diseases.
16. Involving Ministry of Women and Child Development – Reproductive Maternal Neonatal Child Health + Adolescents (as majority are under 5 years) and National Health Mission (NHM) to mobilize resources in terms of manpower and money.
17. The policy should aim at strengthening international, regional and national collaboration for research, with physicians, patient groups and families – multi-stakeholder partnerships.

National Policy for Rare Diseases, 2021 is a step in the right direction – beginning of a new era for rare diseases. But effective implementation through multi-stakeholder

planning and equity consideration and strengthening sensitization/awareness efforts, screening and diagnostics, research, drug development and sustainable financing is the need of the hour.

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