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Ujwala & Siddharth Sen***

[***Abstract:*** *A major reoccurring problem, several nations tend to overlook is the problem of rare diseases. While most countries aim at eliminating or combating mainstream disease such as cancer, polio, AIDS etc., rare diseases such as Duchenne Muscular Dystrophy, Gaucher etc., have hardly been researched upon. Over the past century, some countries such as USA, Japan and Russia have diverted their laws and resources towards combating rare-diseases. India has only recently started paying heed to this problem. The lacking support from governmental bodies, including a complete lacuna of any kind of regulation on rare diseases was expressed for the first time in 2016 when the Delhi High Court ordered the Health Ministry to establish a "national rare disease policy".[1] This article primarily focuses on the National Policy for Treatment of Rare Diseases 2020 drafted by the Government of India. The Policy flashes several features in common with the laws in USA on Rare Diseases. With the withdrawal of the Policy for review purposes by the government, the issue of rare diseases treatment gains more importance. This article aims to analyze the implications of the said policy in a highly populated country like India.*]

I

Introduction

The medical industry plays a major role in the economy of any country, developing and developed nations alike. However, several nations have ignored or neglected this issue of growing genetic diseases and hence, extensive research is still required to counter them. In certain countries such as the United States of America, almost 17% of the GDP is being spent on healthcare.[2] These figures seem to be compliant with larger-than-life American economy, allowing countries with similarly sized economies to spend identical amounts on medical development. As a developing country, India had a budget of Rs.1900 Crores in 2019-20 for medical research, which seems grossly disproportionate to the healthcare needs of more

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1 *Mohd. Ahmed (Minor)* v. *Union of India*, 2014 SCC On Line Del 1508.

2 A. Gordon Smith, *The Cost of Drugs for Rare Diseases is Threatening the U.S. Health Care System*, HARV. BUS. REV. (2017), *available at*: https://hbr.org/2017/04/the-cost-of-drugs-for-rare-diseases-is-threatening-the-u-s-health-care-system (last visited 9 Sep. 2020).

than 135 crore population.[3] In 2017, the Indian government implemented a Policy directed towards addressing the dire need of research in genetic diseases and aid to patients suffering from such rare diseases.

Despite the steady increase in government support to medical research, this sector i.e. Rare Disease Research, has been neglected for an extended period of time. Subsidized medicines for treatment of patients suffering from rare diseases were unheard of. These subsidies are necessary for citizens suffering from rare disease, as majority of such patients are from economically backward strata. The concentration of wealth in the hands of a few in India, has resulted in several citizens falling under a bracket of economic deprivation. This pattern further extends itself into the rarity of these diseases and the sporadic nature of their impact. As with all genetic diseases, it is scientifically impossible to ground genetic anomalies to a particular lifestyle, thereby affecting a larger diaspora of the population.

Problems arise when the regulating policy is confined to strict definitions. The sheer magnitude of the action policy dilutes the very standard definition of rare disease. The major critique one stumbles upon in the policy is the lack of a comprehensive implementation model and the definition of the Policy itself.

This paper aims at highlighting the various issues faced by patients suffering from 'Rare Disease' or a Rare Genetic Disorder and further highlights the lacuna in the field, particularly in India by analyzing the existing law and policies on rare diseases and also comparing them to laws in USA to determine the viability of similar policies in India.

II

Meaning of Rare Disease

Rare Diseases are often serious, chronic and life-threatening. However, medical practitioners consider 'rare diseases' to be any abnormal genetic disorders, without giving a comprehensive and clear boundary to this category of diseases. This creates a legislative problem since, in the absence of a uniform comprehensive data, it becomes the duty of the State to fill this lacuna.

To define a rare disease is fallacious, as every country has a tailored definition for the same. In Europe, the Orphan Medicinal Products Regulation considers a condition as a rare disease if it arises in less than Five in 10,000 members of their

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[3] *See* Budget and Reports, Department of Health Research, Ministry of Health & Family Welfare, Government of India, *available at*: https://dhr.gov.in/documents/budget-reports (last visited 8 Sep. 2020).

society.[4] A disease or disorder is defined as rare in the USA when it affects fewer than 200,000 Americans at any given time.[5] The example of these two dominating nations in the medical field, highlights a major disparity in the definitions of a rare disease, as these States take into account the population, demographic occurrence and other such factors before defining a rare disease, whereas the nations still chasing the benchmark do not.

Though rare diseases have not been conclusively defined, they have the common denominator infrequency and rarity of their occurrence in human population. European Commission considers any disease affecting fewer than 5 people in 10,000 in European Union to be rare.[6] Some other countries have their own definition to suit their requirements.[7] This has resulted in heterogeneity in defining 'rare disease'. Additionally, ultra-rare or ultra-orphan diseases has been introduced as sub-category of rare diseases.[8] Recent statistics estimate that there are roughly 3.5% to 5.9% of the global population, affected by prevalent rare diseases.[9]

III

Regulation of Rare Disease in U.S.A.

The US Orphan Drug Act[10] was one of the initial laws catering to needs of citizens suffering from rare diseases. The Act promoted development of drugs for rare diseases through incentivizing research into orphan drugs with market exclusivity

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4 The European Parliament and of the Council Regulation 141/2000 of 16 December 1999, OJ (L 18) 1.

5 Orphan Drug Act 21 U.S.C. (1983).

6 *See* Communication from Commission to European Parliament, The Council, The European Economic and Social Committee and The Committee of the Regions on Rare Diseases: Europe's Challenges, COMMISSION OF THE EUROPEAN COMMUNITIES 2008, *available at*: https://ec.europa.eu/health/ph_threats/non_com/docs/rare_com_en.pdf (last visited 9 Sep. 2020).

7 Neil Khosla & Rodolfo Valdez, *A Compilation of National Plans, Policies and Government Actions for Rare Diseases in 23 Countries*, 7(4) INTRACTABLE RARE DIS. RES. 213 (2018), *available at*: https://www.ncbi.nlm.nih.gov/pmc/articles/PMC6290840/#bib22 (last visited 9 Sep. 2020).

8 *See generally* NICE Citizens Council Report on Ultra Orphan Drugs, NATIONAL INSTITUTE FOR CLINICAL EXCELLENCE (2004), *available at*: https://www.ncbi.nlm.nih.gov/books/NBK401721/pdf/Bookshelf_NBK401721.pdf (last visited 9 Sep. 2020).

9 S. Nguengang Wakap, *et al., Estimating Cumulative Point Prevalence of Rare Diseases: Analysis of the Orphanet Database*, 28 EUR. J. HUM. GENET. 165 (2020), *available at*: https://www.nature.com/articles/s41431-019-0508-0.pdf (last visited 9 Sep. 2020).

10 21 U.S.C (1983).

to pharmaceutical industry.[11] This Act was America's first major attempt to combat the problem of rare diseases. Drawing inspiration from this Act, several other countries have adopted laws relating to rare diseases, such as the Medicines Act 1991 in Singapore, Pharmaceutical Affairs Law 1993 in Japan, Rare Disease and Orphan Drug Act, 2000 in Taiwan, Rare Disease Act, 2016 in Philippines.[12] Some other countries have framed policies, guidelines, regulatory frameworks for treatment of rare diseases, like Orphan Drug Program 1998 in Australia, National Plans in several European countries, and Orphan Drug Framework in Canada.[13]

The definition of 'rare disease' in US laws includes a large number of diseases, while the EU only categorizes a genetic disease to be rare if it is found in less than 10,000 people. In USA, where the health care industry is at its peak of development and implementation, a wide definition of 'rare disease' allows for the coverage of a vast array of diseases under the same umbrella. Orphan Drug Act was enacted by the USA to support its rare disease framework. The legislation focusing on research is perhaps the most important reason for the success of the US Policy relating to rare diseases.

While drawing parallels with the US laws on rare diseases, one must bear two factors in mind, which are responsible for the successful implementation of the law. First, USA has a dedicated day for Rare Diseases on their annual calendar to increase awareness of these diseases. Although simple, it raises a pertinent question of whether the government of vastly populated nations like India, should address all sections of society, by focusing on mainstream diseases or tend to a minority section which suffers from rare diseases.[14] Second, the US medical industry has focused on exceptional research equipment. Early legislation such as the Orphan Drug Act ensured that substantial funds and donations were raised towards research in rare diseases.

IV

Regulation of Rare Disease in India

Indian Judgments on Rare Diseases

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11 *Id.*

12 *Supra* note 8.

13 Emily Harris, *Addressing the Needs of Canadians With Rare Diseases: An Evaluation of Orphan Drug Incentives*, 5(3) J.L. AND BIOSCIENCES 648 (2018).

14 S. Van Weely & H.G.M. Leufkens, *A Public Health Approach To Innovation*, WHO JOURNAL (2013), *available at*: http://www.who.int/medicines/areas/priority_medicines/BP6_19Rare.pdf (last visited 9 Sep. 2020).

The Indian courts have consistently taken a pro-active position on right to medical aid to be provided by the State. The Supreme Court has in several cases, emphasized to the government, to give priority to health of citizens under Article 12 and 47 of the Constitution of India. In *State of Punjab* v. *Ram Lubhaya Bagga*,[15] the court analyzed the Punjab's new policy on reimbursement of medical expenses of government employees and laid down that government's policies on healthcare should not be arbitrary, unreasonable or violative of any law or principle.

In another case, *Confederation of Ex-servicemen Associations and Ors.* v. *Union of India &Ors.*,[16] a public interest litigation was filed in the Supreme Court for recognition of right to free and full medical care to retired defense personnel and their families as a fundamental right, at par with serving defense personnel. The existing regulations of the armed forces related to medical care at that time excluded free treatment for serious diseases like tuberculosis, leprosy and mental disorders for ex-servicemen. While recognizing the right to medical aid as a fundamental right of all citizens and also acknowledging the services rendered by retired defense personnel, the court adopted a cautious approach, stating that the right to medical care is subject to the limitations of State financial stringencies on the health budget. The judgments of the court on right to medical care did not deal specifically with rare diseases till 2014 when a writ petition was filed in the Delhi High Court, in *Mohd. Ahmed (Minor)* v. *Union of India & Ors.*,[17] where the main issue was whether a child had 'right to health' and free treatment, which amounted to almost Rs.6,00,000 (Rupees Six Lakhs) every month. The petition highlighted the breach of the fundamental rights of the children of economically weaker section of India. As per the world standardized data by EUROID, children are a major section of patients afflicted with rare diseases.[18] This was the third case in a span of four years heard by the Delhi High Court pertaining to rare diseases.

Mohd. Ahmed's legal guardian and father filed a writ petition in the High Court of Delhi on the ground that the State was infringing the fundamental rights of his son under Article 21 and Article 14 by refusing to provide free medical treatment for his son's ailment called Gaucher disease, Lysosomal Storage disorder. The treatment for this rare and chronic disease is Enzyme Replacement Therapy, which was monthly, lifelong and at exorbitant cost of Rs. 6,00,000 every month. Interestingly, the State highlighted certain issues by citing two other cases, while justifying the difficulty in providing free treatment for the child. The challenge faced here was that despite having public health schemes to ensure the health and safety of the masses, it was difficult for the State to treat Rare diseases at par with

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15 AIR 1998 SC 1703.

16 AIR 2006 SC 2945.

17 2014 SCC On Line Del 1508.

18 *Rare Diseases: Understanding This Public Health Priority*, EUROIDS (2005), available at: https://www.eurordis.org/content/public-health-priority (last visited 9 Sep. 2020).

other mainstream diseases. This was due to the severe lack of funds in the hands of the government and also the limited funds allocated to rare diseases in the health and medicine budget. The High Court of Delhi mandated a policy to be formulated by the State for regulation of rare diseases in the form of funding for research and free treatment for patients afflicted with these diseases from economically weaker sections.

Indian Policy on Rare Diseases

Need for Policy on Rare Diseases in India

There are several reasons for India to incorporate a mandatory Policy on Rare Diseases. India has several communities which follow a tradition of incest and for generations, the tradition of incest has loomed over the Indian marriage culture.[19] Genetic disorders are a reoccurring theme amongst Indian masses. Increase in the population has led to increase in the average rarity of these diseases as well.

Although, increasing percentage of funds are allocated to Research and Development in the country's budget, majority of the cash focuses on lucrative and profitable sciences. As per the annual budget of 2018-19, the Department of Biotechnology (DBT) received a whopping amount of Rs. 2,411.53 Crores, which will help the government in continuing its ambitious national biotech strategy.[20] This strategy aims to replicate the country's success in the IT sector. These figures sound impressive but create a huge problem for small/private laboratories. Grants for research are awarded on the basis of numbers alone such as the number of patients who would be benefitted by the treatment, the number of international institutions recognizing the treatment as feasible etc., The budget for Research and Development has no mention of 'rare disease' and it was not until recently that a considerate amount of the funds was allocated for the purpose of research. The Delhi High Court's direction to the government in 2014[21] pushed the State to take more proactive steps towards addressing rare diseases.

Complying with the order of the Delhi High Court, the Government of NCT, New Delhi constituted committees to investigate into Rare Disease regulation and draft a policy for the same. Three committees were constituted under Professor V.K. Paul, Head, Department of Pediatrics, AIIMS, New Delhi on Prioritisation of Therapy for Rare Genetic Disorders, a Sub-committee under Professor I.C. Verma,

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19 Nisha, *The Indian Family: Too Sacrosanct To Touch? Indian Women's Movement And Civil Society's Responses To Incest Abuse*, in AGENDA: EMPOWERING WOMEN FOR GENDER EQUITY: GENDER-BASED VIOLENCE TRILOGY 1 (2005).

20 *See generally*, Budget Gives Boost to New Technology Development, Ministry of Science and Technology, Government of India, *available at*: http://dst.gov.in/budget-gives-boost-new-technology-development (last visited 9 Sep. 2020).

21 *Mohd. Ahmed (Minor)* v. *Union of India*, 2014 SCC On Line Del 1508.

Director, Institute of Medical Genetics Genomics on Guidelines for Therapy and Management and finally, an interdisciplinary Committee under Dr. Deepak K Tempe, Dean, Maulana Azad Medical College, New Delhi.[22]

V.K. Paul Committee on Prioritisation of Therapy for Rare Genetic Disorders[23]

The V.K. Paul Committee focused on the available therapies and prioritization for genetic disorders based on resources, cost of therapy, outcome and quality of life. The Committee report categorized genetic disorders into three classes based on one-time treatment, long-term/lifelong treatment and no available treatment. Recommendations have been made for each category of genetic disorders along with providing genetic counseling and prenatal testing to families with history of genetic disorders.

I.C. Verma Sub-Committee on Guidelines for Therapy and Management[24]

The I.C. Verma Committee was tasked with reviewing the definitions of rare diseases, funding mechanisms, regulating access globally and creating a suitable definition and national plan on rare diseases for India. The Committee also considered the availability of treatment options and promotion of research in this sector. The Committee's report has developed an annual cost for the treatment of the most common rare diseases occurring in India and also evaluated availability and efficacy of treatment and cost of rare genetic disorders.

D.K. Tempe Interdisciplinary Committee[25]

Finally, the high-powered interdisciplinary D.K. Tempe Committee comprising of economists and stalwarts in the profession of bio-tech and medicine, was constituted. The Committee suggested a phase-wise treatment for those rare diseases for which clinical treatment is available and regularly review the policy with more information on treatment options, reduced cost of treatment, evidence-based outcome of clinical treatments etc. The Committee also listed certain rare Lysosomal Storage Diseases (LSDs) such as Gaucher, Pompe, Fabry, Neiman-Pick diseases etc., which are treated with Enzyme Replacement Therapy (ERTs). These treatments would challenge resource allocation and cannot be supported by the existing public health system due to the lifelong nature of their treatments and exorbitant costs involved. The report calculates the cost of treating such rare diseases with Enzyme Replacement Therapy at Rs. 1.8-17 lakhs per kg of body

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22 *National Policy for Treatment of Rare Diseases 2017*, Ministry of Health and Family Welfare, Government of India, *available at*: https://main.mohfw.gov.in/sites/default/files/Rare%20Diseases%20Policy%20FINAL.pdf (last visited 9 Sep. 2020).

23 *Supra* note 16.

24 *Supra* note 17.

25 *Supra* note 19.

weight and submits that the said amount could treat 400 TB or HIV patients in a year. This report was compiled and sent to the Delhi High Court by the government.

National Policy for Treatment of Rare Diseases 2020[26]

The Government of India submitted the country's first ever National Policy for Treatment of Rare Diseases on the 17 May, 2017. The policy is based primarily on the recommendations of three committees, the I.C. Verma Committee, V.K. Paul committee and the D.K. Tempe committee report which investigated into the various issues related to regulation of rare genetic disorders. The Rare Disease Policy 2017 seems to mirror the international standards and maintains its objectives to be increasing awareness, subsidizing cures and promoting funding.

Analysis of the National Policy on Rare Diseases 2020

The National Policy on Rare Diseases was first drafted in 2017. It was withdrawn by the government due to implementation challenges and gaps. An expert Committee was constituted by the government to review the same.

The 2017 draft Policy contained a major lacuna in the area of the target audience for the Policy. Questions such as, whom does this policy concern, whether the specified concessions would be available to every citizen or only those who qualify below the poverty line have not been clearly addressed, can the Centre implement another policy focusing specifically on economically weaker sections, had been left unanswered. Given India's current financial predicaments, it would be safe to conclude that the benefit of subsidized treatment for rare genetic disorders may be limited only to patients from economically weaker sectors and not to all patients. The 2020 Policy has incorporated some clarity in this aspect by specifying the beneficiaries of the financial support under Rashtryia Arogya Nidhi to persons eligible under Pradhan Mantri Jan Arogya Yojana for those rare diseases that require one-time treatment. Some discretionary power has been delegated to State governments to determine financial support to patients suffering from other rare diseases that require regular treatment of special diets or hormonal supplements etc.

The 2020 Policy has also attempted to address the gap in the earlier draft of the Policy in terms of balancing competing public health priorities in a resource-constrained setting of India. However, a statement on measures on health problems of large number of persons by allocating relatively smaller amount being prioritized over treatment of rare diseases where greater resources will be required

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[26] *National Policy for Treatment of Rare Diseases 2020*, Ministry of Health and Family Welfare, Government of India, *available at*: https://main.mohfw.gov.in/newshighlights/national-policy-rare-diseases-2020 (last visited 9 Sep. 2020).

for smaller number of persons, will not be sufficient to address this public healthcare conflict.[27]

Another gap left by the draft of 2017 relating to lack of any provisions for creating awareness related to rare genetic disorders or treatment under public health system, has also been attempted to be filled by the Policy of 2020 in a collaborative manner between the Central government and State governments to raise awareness in all levels of healthcare system. More specificity in this regard, such as involvement of primary health centre workers, Anganwadi workers etc., would have strengthened the 2020 Policy.

Another major challenge in the policy is the lack of substantial empirical data as a Census of patients afflicted with rare diseases is yet to be conducted, though the Indian Rare Diseases Registry is a step in the right direction, albeit only supplementary in nature rather than an exhaustive measure. The National Initiative for Rare Diseases (NIRD) organized jointly by Indian Council of Medical Research (ICMR), All India Institute of Medical Sciences (AIIMS), Jawaharlal Nehru University (JNU) resulted in the launch of Indian Rare Disease Registry on 27 April 2017 for the citizens to report rare disease cases.[28] The registration process established by this policy is to ensure a statistical report of the number of rare disease cases in the country. However, the policy fails to take into account that majority of people in India are unfamiliar with the concept of online registration and the usage of technology for communication. Existing mechanisms like census, primary healthcare workers can be utilized for a wider and more effective collection of data relating to patients suffering from rare diseases.

The policy relies heavily on the recommendations made by the three Committees whose reports are vague on the number of patients researched on which their reports were based which also lack clarity in empirical data relating to actual number of patients afflicted with rare genetic disorders. Further, India's ever-growing population does not help the economy in stabilizing and diverting funds towards various strata in society and the added aloofness of the government vis-a-vis unawareness towards the presence of this particular society does not help in curbing this problem. The judiciary was hence forced to bring this issue into the notice of the Ministry of Health and Family Welfare.

The problem with the Indian medical fraternity is lack of knowledge and resources on rare genetic disease. Research is a stigmatized profession in India, the general societal outlook towards research being a hurdle to capitalist success. Most medical practitioners are unable to detect these diseases due to lack of awareness or possibilities of such diseases. Further, in a scenario where the disease is traced,

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27 *Id.*

28 *See generally* Indian Rare Disease Registry, INDIAN COUNCIL OF MEDICAL RESEARCH, *available at*: http://bmi.icmr.org.in/irdr/index.php (last visited 9 Sep. 2020).

medication is far too expensive and becomes un-affordable for majority of the citizens who are unable to fathom such exorbitant medical prices.[29]

Another challenge is that the Centre has a very utilitarian outlook when it comes to diverting funds to the people who are in actual need of these funds. The D.K. Tempe Committee report blatantly declares certain treatments for rare diseases to be uneconomical in public health system by comparing the costs of the treatment with Tuberculosis and HIV treatments. In the earlier century, diseases like TB and HIV also required exorbitantly expensive treatments and research into their treatments led to the reduced cost of treatment. Applying a superficial and misplaced understanding of utilitarianism to a grave and multilayered issue like public health to unilaterally decide exclusion of rare diseases from public health system solely for reason of costly treatments, will only lead to a severely lacking and deficient policy on rare diseases. It would be defeating the essence behind the Delhi High Court's order to formulate a policy which remains a mere nod of acknowledgement to the existence of patients afflicted with rare genetic disorders without delving into the challenges of incorporating treatments of rare diseases within our public health system. It is defeatist and unbecoming of a Government to throw up their hands with reasons like optimal outcome, resource allocation, overburdened public health system without any attempts to formulate an inclusive policy.

Also, the biotech industry is growing exponentially in India. Research and manufacturer of various Orphaned Drugs is being done locally. This allows for a far more cost-effective solution and government wavers as in-house medicines are far more affordable. The Government's argument of challenging economic evaluation and resource allocation fall flat in the face of other third world countries like Nepal, which has managed to develop a certain policy on rare diseases.

One major shortcoming which has not been addressed by the 2020 Policy also is the lack of enforcement. Unlike the US Act, a mere policy on rare diseases in India would have no real enforcing power and even beneficiaries under this policy would not be able to enforce it in a court of law without the force of a statute.

Currently, the government has dealt with the issue by diverting funds from mainstream diseases towards rare diseases treatments. However, this solution is only a temporary relief. The issue of rare genetic disorder needs a long-term committed research and subsidized treatment programme.

The 2020 Policy also differs from its 2017 predecessor, by including an implementation strategy for the policy. The 9-point implementation plan rightly

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[29] A. Heston, *National Income*, in THE CAMBRIDGE ECONOMIC HISTORY OF INDIA 376-462 (Dharma Kumar & Meghnad Desai, eds.,1983).

includes pre-natal and neo-natal diagnosis of pregnant women and newborn babies respectively for rare diseases, as a preventive measure as well as raising awareness.[30]

The most vociferous step taken by the Indian government in funding Rare Disease research or subsidizing expensive medication was highlighted in the Centre's Campaign in the year 2014.[31] The efforts of the Ministry of Health and Family Welfare can be given due credit for the Policy drafted on the recommendations and reports of committees comprising of stalwarts in the field of genetic research. Further, the ICMR (Indian Council for Medical Research) has made certain attempts at providing certain State-run research laboratories with grants. However, these grants are limited by a cap of Rs. 10 lakhs on the amount provided by the ICMR to the medical laboratories for their research.[32]

Multi-national Corporations, private run research laboratories and other organizations refrain from investing in research, development and manufacturing of Orphaned Drugs for treating rare diseases. Capitalist and profit-driven institutions would consider the minuscule size of market for such drugs to be sufficient deterrent from investment.[33] Further, the credibility and prospects of investment in this field is very unpredictable due to faulty system of research in India where the general perception towards research is only as a last option of livelihood lacking prestige and success.[34] Then, this lacuna in R&D in rare disease treatment has to be filled by the government through research grants, promoting awareness and incentives to private laboratories and organizations for research in rare disease treatments etc.

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30 *Supra* note 26.

31 Anoo Bhuyan, *Government Submits Rare Disease Policy to the High Court*, THE WIRE, (12 Jun. 2019) *available at*: https://thewire.in/140229/rare-disease-policy/ (last visited 9 Sep. 2020).

32 *Guidelines for Extramural Research Programme*, INDIAN COUNCIL OF MEDICAL RESEARCH, available at: https://www.icmr.nic.in/sites/default/files/extramural/ Extramural Projects Guidelines.pdf, (last visited 9 Sep. 2020).

33 Song P, et. al., *Rare diseases, Orphan Drug, and their Regulation in Asia: Current Status and Future Perspectives*,1(1) INTRACTABLE RARE DIS RES. 3 (2012), available at: https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4204590/ (last visited 9 Sep. 2020).

34 John Forman, et al., *The Need for Worldwide Policy and Action Plans for Rare Diseases*, 101(8) ACTA PAEDIATRICA (2012).

V

Conclusion

It is important for a nation to improve the quality of the lives of its citizens.[35] Neglecting the rights of persons suffering from rare diseases, particularly children, violates the essence of our Constitution laid in the Preamble. It is only recently that the Indian economy has established itself in the international front. Due to this reason the country has never had the opportunity to cater to the needs of this small society. Rare Diseases and their treatment has been ignored and sidelined in India till 2017. The National Policy for Treatment of Rare Diseases 2017, despite its many inconsistencies, is the country's first step towards social justice in public health. Patients suffering from rare diseases, including a large percentage of children, live in suffering and pain due to lack of awareness of such disease or inability to afford the exorbitant cost of treatment. Medical scientists and researchers are also pulling out for reasons like unfeasibility of research, miniscule size of market for drugs, lack of government support etc. The slack created from lack of awareness and reluctance of private pharmaceutical companies to research has to be picked up by the State. The US Act was used as a mere tool in the American economy to care for the patients afflicted with rare diseases and also develop an industry by creating job opportunities.

Since rare diseases are not like most mainstream diseases, the number of such genetic anomalies is far larger than the number of citizens affected by it. There are nearly 3000-5000 rare diseases but the people affected are miniscule when compared to a mainstream disease. In this situation, it becomes extremely important to localize research, allowing for direct use of research in manufacturing medicine locally, reducing the cost of treatment. Another way to boost research would be by granting tax exemptions to research laboratories for research on rare disease treatments would promote more and bigger players in the rare disease research and development. This is extremely necessary as the mere existence of a policy and the importing of rare disease medicines becomes ludicrously expensive for the government, giving rise to justifiable but preventable arguments of resource allocation and overburdening the public health system.

The Indian government has made an attempt in treatment of rare diseases through the National Policy for Treatment of Rare Diseases 2020. Glaring lacunae in the initial draft policy of 2017, such as lack of awareness, lack of data relating to patients suffering from rare diseases, procedural implementation challenges in providing financial assistance have been noted in the current 2020 Policy.

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[35] Marius Pieterse, *The Potential of Socio-Economic Rights Litigation for the Achievement of Social Justice: Considering the Example of Access to Medical Care in South African Prisons*, 50(2) J. AFR. L. 118-131 (2006).

However, the 2020 Policy remains just that, a policy without any real detailed clarity or binding effect. Possible areas where the 2020 Policy may also fall short include Centre-State division of duties in the collaborative items of implementation strategy, conflict from prioritization in resource allocation between basic healthcare of large numbers and small number of patients suffering from rare diseases, raising awareness about rare diseases in all levels of healthcare system ranging from private swanky hospital, government hospitals to primary healthcare centres in rural India and last but not least an effective way to collect data relating to patients suffering from rare diseases in India.