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ORPHANED BY THE POLICY AND BUDGETS: INDIA'S DIRE STATE OF RARE DISEASES AND ORPHAN DRUGS

Authored by - Eilin Maria Baiju
& Medha RL

INTRODUCTION

According to National Cancer Institute, an orphan drug is defined as a drug used to treat, prevent, or diagnose an orphan disease.[i] World Health Organization defines rare/orphan disease as a disease or condition with a prevalence of ≤1/1000 population.[ii] For this very reason, orphan drugs do not enjoy adequate support and resources from the market. But these diseases are not as rare as one would think. Orphan diseases are often life-threatening hence treatment is critical, especially when the majority of the patients are children. Even though only five percent (5%) of rare disease is known to have a cure with the right treatment many patients can have an improved quality of life.[iii]

India has the highest population who are affected by rare diseases. Four hundred and fifty (450) rare diseases are identified in India so far.[iv] This is due to the low market support that the orphan drugs enjoy which in turn makes the drugs highly expensive. Furthermore, the majority of these orphan diseases are chronic illnesses which makes it more difficult to afford treatment throughout a person's life. People who live in rural India, who account for Sixty-five per cent (65%) of India's population[v], cannot comprehend being able to afford such therapy. Even though India has many rare disease cases, the government's efforts are unproductive and sluggish.

In 2017, Government of India introduced the National Policy for Treatment of Rare Diseases (NPTRD). It faced several criticisms and faced several challenges in implementation, which led to the appointment of an Expert Committee in 2018. The committee was appointed to review NPTRD, to define rare diseases, to draft National Policy for Rare Diseases (NPRD), etc. NPRD essentially aims to lower the cost of treatment for rare/orphan diseases.[vi] This new policy also seems to have a lot of limitations. Thus this article seeks to do an overview of India's dire need for comprehensive legislation on rare diseases. Further, it seeks to suggest a better

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model for boosting access to treatment and medicines for rare diseases in India. Also in this article, a brief account of the international perspective regarding orphan drugs and where India stands in the race has also been included.

HOW TO BOOST ACCESS TO ORPHAN DRUGS AND REDUCE THE OCCURRENCE OF RARE DISEASES IN INDIA?

India is a signatory to the Universal Declaration of Human rights (UDHR). India has adopted many of its constitutional provisions keeping in mind human rights. Fundamental Rights and Directive Principles of State Policy in the Indian Constitution are greatly influenced by UDHR.[vii] Moreover, India is a country that has recognized the right to health and health care through various judgments. Supreme Court has reiterated the fact that the right to health comes under the right to life, which is a fundamental right under the Indian Constitution.[viii] In State of Punjab & Ors v Mohinder Singh Chawla[ix] the Supreme Court maintained that the right to health is inextricably linked to the right to life and that the government has a constitutional obligation to provide health care. In the case of Mohd. Ahmed v UOI[x], Delhi High court had observed that the state had a constitutional obligation to ensure access to life-saving drugs. Thus access to orphan drugs is also to be considered as a facet under Article 21 of the Constitution i.e., Right to Life.

First of all the citizens should be made aware of orphan diseases. Very few populations are aware of these life-threatening diseases. Conditions such as cystic fibrosis were thought to be very rare in India however, a genetic study has revealed that the condition is widespread, but it was previously undiagnosed.[xi] There is also a social stigma associated with these diseases. People do not reveal the fact that the disease runs in the family since it becomes a hurdle in their prospective marriage lives. Medical practitioners lack practical awareness of such diseases since some people shy away from receiving treatments.

In India, there is a lack of research and development in the area of orphan diseases. This is again attributed to the fact that these diseases are “rare”. Orphan diseases are very difficult to research since the number of patients is very few. There is very little known regarding the pathophysiology and natural history of these diseases. Thus, the physicians must collaborate with other regional and international researchers who work on the very same topic.[xii] This increases the scope of research and widens the sample of patients. However for the same to be

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possible the government should be willing to provide special grants for researchers who work on orphan diseases and drugs.

Like any other drug manufacturing and development, orphan drugs development is also very expensive. Indian Pharmaceutical companies shy away from investing time and resources in developing orphan drugs. This is mainly due to the fact that the return profit is less compared to any other drugs since orphan diseases are rare comparatively. So the government should be ready to incentivize the manufacturing of orphan drugs. There should be a separate budget allocation for the same. Subsidies should be given to major Indian Pharmaceutical companies like how United States Food and Drug Administration (USFDA)[xiii], Japan, etc. provides to their indigenous pharmaceutical companies. Only by providing tax incentives and giving exclusive marketing rights and patent protection, the Indian government can encourage indigenous production of orphan drugs. However, the Government of India has not shown keenness in providing any incentives to any of the pharmaceutical companies for manufacturing orphan drugs. No Indian Pharmaceutical company manufactures orphan drugs. Thus people in India who are affected by these diseases rely on other developed countries (such as the United States of America, United Kingdom, Japan, et cetera) for orphan drugs. This makes the drugs very expensive and inaccessible.

One method to attract Indian Pharmaceutical companies to start manufacturing orphan drugs would be to give marketing exclusivity or patent to the companies over orphan drugs. Marketing exclusivity and patent also can run concurrently. Patent is a facet of Intellectual Property Rights (IPR) which is given early while developing the drug. However, marketing exclusivity is granted only when the drug is approved by the Food and Drug Administration (FDA) and all statutory legislations are met.[xiv] Patent and marketing exclusivity is generally granted to lessen drug competition. Under the Orphan Drug Act of United States of America, seven-year market exclusivity is granted to orphan drugs.[xv] In European Union, orphan drugs enjoy ten years of marketing exclusivity.[xvi] A research conducted in different countries on a particular drug which was given patent rights in those countries shows that giving patents and marketing exclusivity increases investments made by pharmaceutical companies on orphan drugs.[xvii] It also shows that patient treated by orphan drugs increased when patent and marketing exclusive rights were given.[xviii] This is due to the fact that the company was able to earn more profits when patents and marketing exclusivity was given. Patents help manufactures to secure market position, protect their research and development.[xix] Thus patents and marketing exclusivity will attract manufacturers.

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The government should be willing to reimburse the victims of orphan disease for the treatment. There should be a proper government-authorized insurance policy that covers all groups of rare diseases.[xx] Without the existence of a proper mechanism of insurance policies, a common man would not be able to afford such expensive drugs. United States of America, Italy, France, et cetera. have policies to reimburse for the orphan drugs approved by the government through their legislation.[xxi] In some countries, considerations such as equity access and the rule of rescue are followed. Rule of rescue essentially means value of rescuing a life regardless of cost.[xxii]

Another option in front of the government is to execute government-based crowdfunding and this should be tax-free, transparent, and timely. Many children die every day just because crowdfunding is not being done in time.[xxiii] Crowdfunding should also have government control as observed by Kerala High Court.[xxiv] But a mechanism solely based on crowdfunding would not work. The Government of India has recently launched a portal for crowdfunding for rare diseases. However, this is not tax-exempt.[xxv] This defeats the whole purpose of crowdfunding. Group 3 patients of orphan disease are completely left at the mercy of crowdfunding under the National Policy for Rare Diseases, 2021.[xxvi] This is another blunder on the part of the Indian government.

Any initiative taken on orphan drugs could only be executed with proper legislation backing it. India needs legislation that clearly lays out how to incentivize the manufacturing of orphan drugs indigenously, how to reimburse the patients for orphan drugs. A proper direction has to be provided in the legislation as to how to increase research and development on the same subject. We have seen how legislation has brought immense development in the area of orphan drugs in the United States of America, European Union, France, etc. Similarly, India also needs comprehensive legislation without which it would be impossible for a common man living in India who is affected with an orphan disease to get proper treatment.

INTERNATIONAL PERSPECTIVE ON ORPHAN DRUGS

As previously stated, rare diseases can include infectious diseases, genetic disorders and various types of cancers. Although there are more than 500 orphan drugs available and approved by various regulatory bodies, most of them remain ineffective for treatment purposes. With hundreds of new treatments entering the market over the last few years, it has further

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propelled the need for capital intensive research and development of the drugs, further encouraging the companies to invest further in such developments.

The US made a path-breaking law on orphan drugs, under the name the Orphan Drugs Act, 1983 under the regime of President Reagan and managed to successfully tackle the profit motive of the drug industry to the needs of the patient. With this legislation US was successful in approving approximately 10 drugs per year and more than 250 drugs have been approved so far with an intention to treat more than 2,00,000 people in the United States of America.[xxvii] It is expected that the orphan drug market alone will cross USD 350 billion by 2028.[xxviii]

Whereas in Europe, orphan drugs are all medical products the pharmaceutical industry would be unwilling to develop under the normal market conditions and is a condition that affects not more than 5 people in 10000 citizens. It is the European Medical Agency (EMA) and Committee on Orphan Medical Products that play central role in facilitating the development and authorization of medicines for [xxix]rare diseases[xxx] In addition EU also established the Rare Disease Task Force in 2004 with European Commission Public Health Directorate to provide evidence to support the orphan drugs cause.[xxxi] Australia also has a similar appropriate threshold for orphan drugs under its 1997 legislation.[xxxii] Meanwhile Japan categorize Orphan Disease in the 1993 legislation as a condition that impacts less than 50,000 citizens (which is less than 3.9 per 10,000 individuals), coupled with the absence of adequate treatment and shows the development and needs for a new drug.[xxxiii] In recent years countries like South Korea and Taiwan have made considerable progress in legislation. In fact, China is also actively making new regulations for orphan drugs.[xxxiv] The Taiwan government has identified more than 160 rare diseases and developed 77 orphan drugs and 40 special nutrients for the treatment of rare diseases. The Taiwan Centre for Rare Disorders was established in 1999 with the motive to help patients Meanwhile South Korea legislated its orphan drugs Guidelines back in year 2003 and identified it rare disease that affects fewer than 20,000 people and stipulated exclusive marketing rights for 6 years to encourage medical research and development of orphan drugs.[xxxv] In addition it also established the Korean Rare Disease Information Database as well as Korean Organization for Rare Diseases to provide vast information on companies, researchers and patients.[xxxvi] Meanwhile China does not have any clearly defined legislation till now, but it has its own networks like Rare Disease in China Network and Chinese Rare Disease Academic Network. Similarly, Argentina, Egypt, Brazil and Algeria had a robust mechanism for reimbursement and allocation of funds from the healthcare budget for rare

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disease-related matters.

WHERE INDIA STANDS IN THE RACE

Despite severe child mortality associated with rare diseases, India's ambitious Ayushman Bharat does not have any provision to integrate the draft policy and effectively implement it. Meanwhile, the 2021 Budget as well as the 2022 budget shunned the orphan drugs blatantly and made no allocation for such plans despite one-fifth of its population suffering from it.[xxxvii] The existing National Policy for Rare Disease, embraces a very minimalist approach and maintains the position that it is a state matter. To add on, the policy also backed a parallel mechanism of the Indian Council for Medical Research formulation of the National Registry. Though the registry was implemented in 2017, it is yet to start nationwide data collection so that patients benefit from it. So in toto, the orphan drugs access were further orphaned by the draft policy and further caged by the National Registry. This coupled with our country's rustic health care creates unavailability of testing, prolonged diagnosis, limited access to specialists further worsens the misery. Though in general cases, early diagnosis can improve possibilities of survival and open doors to life-saving therapy, there is no ray of hope for Indian rare disease patients. The papers of draft policy are also silent on the inclusion of patients in International clinical trials. Under the draft policy the government has categorized patients into three groups. The first group comprises of people who would benefit from one-time curative treatment and the second group consists of people who need life-long treatment. Whereas the third group is concerned with people who would require long term life-saving treatment where the cost of the treatment is highly unaffordable and the treatment of patients in this category is relatively uncertain as per the draft.[xxxviii] For the first category patients, the central government will provide an assistance of Rs.20 lakhs through its schemes, Pradhan Mantri Aarogya Yojana and Rashtriya Aarogya Nidhi, provided that the patient belongs to the bottom 40% of the economy. The treatment involved is generally one-time curative treatment. For the second category patients, it is suggested that the states could consider patients falling under this category. There is no element of clarity in this categorization. The most problematic categorization happened with the third, which shook the rare diseases community, where the government has provided a straight jacket solution and resorted to digital crowdfunding platform because of "resource constraints". Practically analysing, the government diplomatically requested the third category to beg for donations for their treatment and receive donations. It is important to note here that crowd funding was always an option since the beginning and when the government doors shut, families resorted to it. The draft policy didn't make any improvement to the present situation of Group 3 patients and hence the new government portal has no effect or null in improving

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the existing circumstances.

In addition, there is no law in India or provision in the draft policy exempting the drugs that are used to treat rare genetic diseases from 'price control'. This aspect is very important considering the nature of the drug market is oligopolistic and often there is a tendency to jack up the price of orphan drugs. For example, there was a global outrage when the price of Daraprim, the life-saving drug that treats the liver that does not produce bile acids, were shot up from $13.50 per pill to $750 per pill.[xxxix] To worsen it further, this hike of 5000% was further approved by the US Food and Drug Administration.[xl]

PRECEDENTIAL FRAMEWORK OF INDIA ON RARE DISEASE

As per data available, almost 300 million people are suffering from rare diseases and with more than 7000 rare diseases out of which 450 have been found in Indians.[xli] With the help of the draft policy the Indian Government has made an allocation of Rs.20 lakh through its umbrella scheme of Rashtriya Arogya Nidhi.[xlii] Recently in the case of Master Arnesh Shaw v. Union of India and Anr.,[xliii] Justice Pratiba M Singh directed the Centre to finalize the National Policy for Rare Disease as the revised policy raised questions on matters relating to sharing of expenditure and cost-effectiveness between the centre and the state. The judgement also stated that the draft policy has not seen the light of the day despite being introduced for consultation in 2020[xliv] and further stated that the treatment of children with rare diseases is a fundamental right under the ambit of Article 21 as "Right to Health and Health Care". The bench also observed that the Centre cannot wipe their hands clean in matters involving rare diseases and contend that they cannot be made a party to the proceeding.

The Delhi High Court constituted the Renu Swarup Committee expert committee on 12th March 2021, which requested the Union of India to specify its budget of health in the preceding 5 years and also show details of the amount unused so that the same can be contributed to the treatment of rare disease and indigenous development of therapies for treatment. The committee also suggested the constitution of National Consortium for Research and Development on therapeutics for rare diseases further effectuated by the draft policy in papers. The committee also suggested the formation of the National Expert Committee on Rare Diseases to evaluate the progress of research and provide advice on matters pertaining to

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orphan drugs and rare diseases.

One interesting observation, in this case, was the submission of accounts details of the Ministry of Health and Family Welfare. The affidavit showed that Rs.200 crores were allocated for the purpose of the expenditure in respect of rare disease, out of which the total expenditure was merely Rs.7 crores over the last three years.[xlv] This showcased the failed mechanism of fund allocation for thousands of children and the parents out there seeking help for treatment.

Recently, in the case of Arif v. State of Kerala and Ors[xlvi], the bench headed by Justice P.B Suresh Kumar directed the Centre and State to disseminate guidelines to overview the crowdfunding scheme. The court commented that "every Tom and Harry out there might be collecting funds with ill-motive via crowdfunding and the amount might be getting deposited in private accounts." The court noted that the amount raised often is so huge amounting in crores, that is enough to stifle the economy if not properly regulated by the government.

CONCLUSION

There exists no denial in the fact that rare diseases and orphan drugs are a mammoth human issue faced by thousands of children and other people. As rightly pointed out by the rare disease community, the present draft is a mockery that lacks foresight and is utterly thoughtless to the needs of the community. India has a long journey to trek to console the woes of its rare disease community. As previously stated, the price of the drugs involved for treatment is extremely exorbitant making it important to incentivize the research trials and further delve for sustainable options. Advanced Prenatal scanning is one way through which rare disease occurrence can be curbed. But these tests are ritzy and requires high expertise. The government should take active steps in establishing the Centre for Excellence (CoE) in every state to enhance research and training. The government should also consider alternatives like indigenous research and try to produce low-cost substitutable drugs and therapies for its population.

The government should work towards the actual establishment of the National Consortium for Research and Development on therapeutics for Rare Diseases and the National Expert Committee on Rare Diseases. The government should make sure that the treatment and funding are streamlined in such a way, that patients should not be unnecessarily dragged into the realms of litigation to get some relief from the government. The assistance should reach the needy on time. It is also important that the government sees the allocated funds for rare diseases reach

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the correct hands-on time and the budget is effectively utilized. As stated by the Kerala High Court it is also very important that there is a government overview in crowdfunding for these treatments and to remove the corruption involved, if there is any. The groundbreaking Technology of CRISPR Cas9 (Clustered Regularly Interspaced Short Palindromic Repeats) used for genome editing is raising new hopes for the rare disease community. The government should try employing this technology within the specified ethical means to help rare disease patients.

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