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Navigating Intellectual Property Rights: Patentability and Market Exclusivity for Orphan Drugs in The Evolving Landscape

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Abstract

Intellectual property is an intangible property created with the endeavor of human intellect. This concept is formulated for the recognition and value for the inventions, creation of mind, literary or artistic work, designs, and traditional knowledge can be given recognition by providing Intellectual Property Rights. Intellectual property is necessary for the progress and well-being of the humanity so that new inventions and creations, further innovations are encouraged. Patent gives exclusive rights for product and process whereby without the permission of the patent owner invention cannot be commercialized. Patent Law is developed as per the standards prescribed in the TRIPS agreement globally. Patent rights are also granted for drugs as pharmaceutical research is expensive and unpredictable. Pharmaceutical sector protects their inventions by product or process patent rights. Nowadays rare diseases or orphan's diseases caused by genetic alterations have become a threat for human life. It has become a necessity to develop new drugs or orphan drugs to treat these rare disorders even though it is fewer in numbers. In US and EU once a drug receives the status of orphan is attained the protection is limited to 10 to 12 years of protection from commercialization. In such scenario the companies who have spent millions for their Research and Development will not be in a situation to take back the investment. High revenue or capital investment is hindering the development of orphan drugs. Orphan drug exclusivity with the patent protection will definitely help in encouraging more inventions for rare diseases thereby generating a world without disease. This paper surmises upon the importance of patent to orphan drugs and exclusivity to the manufactures which can be achieved only by appropriate policy decisions and regulatory framework.

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1. INTRODUCTION

Intellectual Property can be categorized as creation of intellect of humans. It includes Copyright, Patents, Trademarks, Geographical indications and Designs. The creation or invention of the human intellect is to be protected so that the person can enjoy the fruits of his creation. The inventor gets exclusive rights called Intellectual property rights for a definite period of time. Intellectual Property Rights is necessary for the growth and development of any knowledge. Knowledge can be achieved only by constant creativity and innovation. Public interest is one of the main aims to promote Intellectual property rights as it connects socio-cultural development and economic growth. As the benefit of all of the individuals is mandate it is very much necessary to promote arts and culture, traditional knowledge, biodiversity and progress in science and technology. Drugs are given patent protection in Intellectual property as it refers to the creation which can be used for the benefit of others. Pharmaceutical industries play a key role in developing new medicines and in building the strength of the nation. Due to very less population in need of orphan drugs biotechnology companies and Pharmaceutical industries fail to take initiatives to develop orphan or rare drugs.

2. CONCEPT OF RARE DISEASES AND ORPHAN DRUGS

World Health Organization define rare disease as lifetime disease or disorder with frequency of 1 or less, per 1000 population and it is infrequent in occurrence in the human population. There is also another category for rarest of rare disease called as Ultra rare disease where fewer than 2 patients

per 100,000 populations are affected. Every nation gives different definition for rare disease or orphan disease depending upon the population it gets affected. There are thousands of rare or orphan diseases around the world which does not have an appropriate and approved treatment. Without appropriate treatment the quality of life of patients is affected and their lives are shortened. Pharmaceutical industries face clinical issues in identifying an appropriate drug and fund for developing it[1].

3. REGULATORY FRAMEWORK DEALING WITH ORPHAN DRUGS – A GLOBAL SCENARIO

An orphan drug is used to treat rare diseases or rare medical condition. Countries like Unites States, Australia, Europe and Japan has laid down legal regulatory framework for combating rare diseases. Orphan Drugs Act 1983 which is formulated by the US Government has given incentives to the Research and Development thereby helping number of people. The incentive includes tax credit, funding for analysis and most importantly market exclusivity. By this Orphan Drugs Act more than 10 million patients in USA have been benefited. Other Countries also took initiative steps for orphan drugs by Japan enacting (Orphan Drug Regulation) and Australia enacted (Orphan Drug Policy). They also provide grants for clinical research for orphan drugs, protocol assistance, waiver of fees and market exclusivity to encourage manufacture of Orphan drugs. Recently in 2000 European Union has enacted orphan drug legislation, which seems to be far better than the U.S legislation. The definition of 'rare' disease varies from one country to another. The EU has defined rare disease as individuals no more than five per 10,000 of the population. In the European Union there are two Orphan Drug Regulations modeled on the US legislation. The first regulation (EC) No 141/2000 and second one is (EC) No 847/2000 both are offering incentives like market exclusivity and fee reductions for orphan drug development[2]. When European patent is granted for 20 years, the manufactures receive absolute monopoly to the drug and it can be further extended to 5 more years. This kind of extension helps the patent owner to increase the monopoly time period of the orphan drugs[3]. Thus, patent protection will provide additional protection for high revenue for orphan drugs. In Japan orphan drugs are regulated by Pharmaceutical and Medical Devices Agency under the supervision of Ministry of Health, Labour and Welfare took over the promotion of Orphan drugs. Japanese marketing authorization provides for incentives like development grant of 5 %, tax reduction, fast track review for approval and even patent issues are also granted based on reexamination period. In India the National Policy on Treatment of Rare Diseases 2017 (NPTRD) highlighted the need for defining the term Rare Disease but didn't provide one. The Hon'ble Delhi High Court in the case of Mohd. Kalim v. Employees State Insurance Corporation and others, the court kept the policy in abeyance contending that it was framed without the concurrence of most states in India. Subsequently the Health Ministry transferred the work of rare disease to National Health Mission. Orphan Drugs are defined in the New Drugs & Clinical Trials Rules 2019 as a drug "intended to treat a condition which affects not more than five lakh persons in India". Thus, comparing to US and EU, India lacks in appropriate regulatory framework in recognizing rare diseases as well as an initiative to encourage orphan drugs[4]. In India there are thousands of rare diseases which need a solution through invention of orphan drugs. A regulatory framework based on the global phenomenon is necessary at this point of time to cure the curse of orphan diseases in India.

4. SHIELDING ORPHAN DRUGS

Orphan drug is necessary for curing rare diseases and help in the welfare of the country. Many people suffer from rare disease problems and face a life and death situation. It is a priority of all nations to give necessary mandate to orphan or rare diseases and to accelerate the orphan drug research. India has failed to encourage orphan drug research without providing government incentives or protocol assistance. Dr. APJ Abdul Kalam addressing the issues of rare disease said, "A coordinated effort at the national level is the need of the hour for more research and understanding rare diseases in the country. There is a need for a whole ecosystem consisting of doctors, a registry to record the prevalence of rare diseases, bio banks, support groups; more research on drug discovery, and of course, a regulatory framework. Each component is complex, and there is a lot of work ahead." Pharmaceutical industries are to be motivated as well as encouraged for better research and development. Series of issues are faced by the Pharmaceutical industries in the manufacture of drugs. One of the main issues faced by the industry or organization is funding, as government incentives are lacking for various drugs. Pharmaceutical industries spend years of research and development and funding for creating a new drug with limited resources. The recognition which they get from this manufacturing of drugs is revenue and novelty for their work. Two kinds of aspects are discussed in this scenario one is Patent Protection for Orphan drugs and the other one is market exclusivity. Patent is defined as the property right given to a creator which prohibits others from manufacture, selling, using for a time period. Generally, the term for patent protection is 20 years from the date on which the application for patent is filed. Product patents are given for drugs which also includes formulation and composition patents. Generally, market protection is received by the manufacturers from competitors for 20 years. In few instances the patent protection gets exhausted by the time the drug reaches the market. In addition to patent protection the manufacturers should also be provided with market exclusivity as an additional protection which is also a mechanism to generate revenue. Thus, even though the drug may have expired patent protection but it will receive market exclusivity at the time of approval there by acquiring the exclusivity[5]. Thus, it

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can be concluded by saying that an Intellectual Property policy combined with clinical development is the key to welfare and safe state.

5. RECENT LEGISLATIVE DEVELOPMENTS

The government of India has recently tackled the issue of rare diseases after seeing how urgent the situation was. The first effort got underway with the National Health Policy 2017[6], which addressed the management of rare diseases and sought to use a public-private partnership to address the shortcomings in public service[7]. After that, in July 2017, the National Policy for the Treatment of Rare Diseases (NPTRD) was drafted. Due to implementation challenges, among other reasons, it was then evaluated by an Expert Committee in 2018[8]. The Centre was instructed by the Delhi High Court to establish a "Rare Diseases Committee, a Rare Diseases Fund, and to finalize and notify the National Health Policy for Rare Diseases" by March 31, 2021, at the latest, in the matter of Master Arnesh Shaw v. UOI[9]. The Ministry of Health and Family Welfare authorized a thorough "National Policy for Rare Diseases 2021" in compliance with this order[10].Furthermore, "a hospital-based 'National Registry for uncommon Diseases' was developed by the Indian Council of Medical Research (ICMR) to include establishments across the nation that are dedicated to the identification and management of uncommon diseases[11]. Orphan drugs are classified as "drugs intended to treat a condition which affects not more than five lakh (500,000) persons in India" under "the New Drugs and Clinical Trial Rules 2019[12]". Orphan medications are subject to the same regulatory framework for clinical trials as other drugs, with the following exceptions[13]:

- The Central Medications Control Standards Organization (CDSCO), the primary drug regulatory body in India, has the authority to waive the need for local clinical studies when it comes to orphan medications.
- The CDSCO may be urged to expedite the approval process for an orphan medication by the sponsor of the clinical trial.
- There is no application fee required for an orphan drug clinical trial.

6. THE NATIONAL RARE DISEASE POLICY 2021

The Ministry of Health and Family Welfare approved the “National Rare Disease Policy 2021” with an aim:

The aim of the policy is to reduce the expenses associated with treating rare diseases and to priorities local pharmaceutical manufacturing and research. Individuals with one-time treatment-requiring uncommon diseases (diseases listed in Group 1 of the rare disease policy) could receive up to Rs. 20 lakh in financial support through the Rashtriya Arogya Nidhi umbrella plan. "Roughly 40% of those who are qualified under the Pradhan Mantri Jan Arogya Yojana" will be covered by it. Crowdfunding will be used by the policy to cover the cost of treating rare diseases. Through an extensive IT infrastructure, businesses and people would be encouraged to make financial contributions. The creation of a nationwide hospital-based rare disease registry will guarantee that scientists and developers have access to sufficient data and comprehensive knowledge about these conditions. With the use of psychotherapy, District Early Intervention Centres, and Health and Wellness Centres, the approach aims to screen for and identify rare conditions early on, which will aid in their prevention[14]. On May 19, 2022, the Union Health Ministry released an office memo that increased financial assistance for patients with rare diseases under "the national policy of rare diseases, 2021 from Rs 20 lakhs to Rs 50 lakhs under the umbrella scheme of Rastriya Arogya Nidhi (RAN)." A limited number of conditions in Group 1 were covered by the previous twenty lakhs grant, but all rare diseases, including lung transplants, are now eligible for the new grant. An initiative of this kind will significantly increase treatment accessibility for thousands of patients in India who are suffering from rare diseases. it comes as a relief to patients suffering from rare diseases. a huge help to the stressed families comes from the hike in the grant[15]. In India, many medications for rare diseases are not available, and those that are have proven "exorbitantly expensive, putting enormous strain on resources." Few medications for uncommon illnesses are produced in India, therefore ultimately all of these medications are imported. Most orphan medications are prescribed on a regular basis and are meant to be used as palliative measures rather than as treatments. Certain individuals suffering from uncommon illnesses could need lifelong therapies, which are either unobtainable or excessively costly in India. Therefore, the policy's reform is advantageous for pharmaceutical companies looking to introduce uncommon drug therapies or treatments in India as well as for patients. Pharmaceutical companies can now introduce cutting-edge treatments for uncommon illnesses both in India and abroad.

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7. CONCLUSION

This is an era of knowledge and economy is driven by the benefits which derive from the knowledge. Recognizing the initiative made in the area of science and technology help in improving the health, welfare and development of people. Creativity and novelty can be encouraged by giving rights to the pharmaceutical industries private and public sectors, Research and Development centers, academia activities relating to the invention of drugs. Rare diseases are affecting few people which have left them with no option to live. It is necessary to cure and make their life worth living rather than choosing the option of mercy killing. By providing incentives for the industry for "orphan" research and appropriate framing of commercializing policies result in proper development of Orphan drugs. It is very keen to note that success of legislation is based on the global outcome which is based on similar purpose and content. With an aim to develop more orphan drugs, pharmaceutical industries should be given patent protection for a period of 20 years plus market exclusivity so that they can get back the revenue spend on the research and development. This step will encourage various manufacturers to come forward and improvise their research and development for creating orphan drugs for rare diseases. Patients with rare diseases can receive up to fifty lakhs in financial aid under the new policy reform, but only at the Centres of Excellence (CoEs) listed in the NPRD 2021. There are no hospitals in states like Kerala that have been designated as Centres of Excellence. Because of this, the majority of patients in these states who suffer from rare diseases are unable to benefit from the Scheme since it is nearly impossible for them to travel to neighbouring states to receive treatment in the CoE and so qualify for the benefits. Therefore, in order to guarantee the Scheme's successful implementation, Centres of Excellence must be established in each State. 96% of the funds provided by Rashtriya Arogya Nidhi to treat Group 1 Disorders have not been used in recent years. This makes it very evident that these people are not being treated effectively. Lack of institutional funding has already claimed the lives of several patients, and many more are in danger. It will be difficult to create an effective policy and successfully implement it without a clear description of rare diseases and their characteristics. Undefined terminology can cause confusion and inconsistency when used, which can affect research and development as well as patient access to therapy. To expedite the gathering of epidemiological data, we must thereby provide the National Registry for Rare Diseases with more staff and resources. This would help us in our own country to develop a suitable definition and other associated standards. In addition to saving lives, an early and precise diagnosis would lower long-term costs for the government and the patient. Therefore, it is essential to allocate more money and redirect it in other directions.

8. CONFLICT OF INTEREST

Conflict of interest declared none

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