Shivangee Malik D Report 2

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MANAGEMENT OF RARE DISEASES: GAPS, CHALLENGES AND OPPORTUNITIES IN INDIA

BACKGROUND

Rare diseases are a group of conditions that affect a very small number of people, hence the term 'rare'. There is no standard definition across the world that defines the maximum number of people a disease should affect, for it to be considered a rare disease. For example, a disease is considered rare in US if it affects less than 200,000 people. In Europe a disease should not affect more than five people per 10,000 population to be called a rare disease. India still doesn't have a standard number that would classify a disease as rare.

There are approximately seven thousand rare diseases that are known to affect humans across the globe. Though there is no quantitative data available in India to estimate the prevalence of rare diseases, around four hundred and fifty diseases are known to exist in India, which affect more than seventy million people.

Out of the 7,000 known rare diseases, approximately 80% are genetic in origin. Being genetic in nature, most of such diseases are congenital i.e. present since birth. Approximately 75% of rare diseases affect children and approximately 30% of the children affected by any rare disease die before they reach the age of five years.

Drugs used to treat rare diseases are known as 'Orphan Drugs'. Most of the western countries have a separate legislation and regulatory standards when it comes to the development and distribution of orphan drugs. For example, the US Orphan Drug Act which came to existence in 1983. The presence of such regulations, which also include incentives and other provisions, help

keep the pharmaceutical industry interested in orphan drugs development. India, like many other developing countries, lack any such orphan drug legislation.

AIM AND OBJECTIVES

Aim

Aim of the study was to carry out an assessment of gaps, challenges and opportunities in rare diseases management in India

Objectives of Study

The objectives of this study are to analyze the following from the perspective of a health administrator:

1. To investigate the inherent gaps and challenges in management of rare diseases in India
2. To explore orphan drug development scenario in India as compared to other countries that have been actively engaged in orphan drug development
3. To list recommendations which can be helpful in overcoming the challenges identified

Expected Outcome of the Study

- To gain a valuable knowledge about the factors that impede the rare diseases management as a whole in India
- Explore opportunities that can help health providers to make India a better country to live in for people suffering from rare diseases

METHODOLOGY

Study Area The study was carried-out at ZS Associates, New Delhi and data was considered for carrying out an assessment of Rare Diseases: Gaps, Challenges and Opportunities in India.

Study Design Qualitative, descriptive study done using secondary data sources.

Study Duration: 04 Feb to 30 Apr 2019 in three phases. In first phase work protocols, framework, work culture was understood, and study approval taken. In second phase study tools were decided. In third phase data analysis was done. Data collection/extraction was done simultaneously during a period of three months.

Inclusion Criteria Relevant articles published after 2005 which were available for public use.

Data Sources Articles that met the inclusion criteria sourced from PubMed, research gate, google scholar, websites of relevant organizations and various news articles' websites.

Article Extraction By typing the keywords on the search bar of the websites listed above and applying time filters for extracting data that was made available for public use after the year 2005.

Data Analysis Content analysis of the selected articles will be done to summarize the relevant data

Presentation Statistical result, if any, will be presented in the form of tables, pie charts or graphs. Non-statistical results will be presented as summarized

Tool/Checklist for Data Extraction An excel workbook with the following items as a checklist.

(a) Bucket heading (Gap or challenge or opportunity)

(b) Date of article published

(c) Source from where article obtained

(d) Name of the journal

(e) Name of the author

(f) Location where the article was published

(g) Title of the article

(h) Key Findings & Inference from the article

Ser No	Publishing Date	Source of Article	Name of Journal/ Publication	Name of Author	Location Where Article was Published	Title of Article	Key Findings (Gaps/ Challenges/ Opportunities)

OBSERVATIONS AND ANALYSIS

Orphan Drugs: Indian Perspective

- The speed of incidence and prevalence of rare diseases is much higher than the speed of development of drugs to treat rare diseases, especially in India
- The Indian pharmaceutical industry is not very interested in carrying out research for development of orphan drugs for the treatment of rare drugs because the number of people affected by them is small as compared to other diseases
- Looking at the financial gains, which is not very high with orphan drug development, makes pharma companies resilient towards it
- Orphan drug regulation in the western countries has people affected with rare disease a great deal in gaining access to orphan drugs, both physically and financially
- Pharmaceutical companies are provided with a lot of incentives when it comes to orphan drug development in countries like US and Europe
- There is no regulatory body in India to supervise the development of orphan drugs
- There is a lack of regulation of orphan drugs in India which has adversely affected the economic growth of Indian pharmaceutical industry
- For Indian payers, the affordability of orphan drugs has become a major issue

Developing Rare Diseases Resources in Developing Countries

- Will serve a lot stakeholders: rare diseases and disorders patient community, students and non-specialist readers, etc.
- Could evolve to incorporate rare diseases registry
- Will help researchers and key policy makers

Diseases Can be Rare, Not Remedies

(Healthcare Journal, Prasaana Shirol, 20 Feb 2018)

- Right to health is right for all people to have equal opportunities for access to quality healthcare
- Majority of common symptoms can be underlying causes of certain rare conditions, which might lead to a delay in diagnosis
- Most treatments are unaffordable due to exorbitant cost
- Inequality with respect to access to treatment for rare disease patients
- Rare disease drug development sluggish and poor
- Indispensable to give rare diseases precedence in public health domain
- States (as per recommendation of the national policy) must also recognise rare diseases as a health priority
- Have a comprehensive national approach for rare diseases to arrive at sustainable solutions
- Requirement to move beyond discussion stage

India's Rare Disease Policy Hangs In Balance

(Down to Earth, Banjot Kaur, 28 Dec 2018)

- Abrupt withdrawal of National Policy for Treatment of RD (NPTRD) by GOI w.e.f. 09 Dec 2018
- GOI reviewing policy in light of information & updates received for further improvement
- Kept in abeyance till a revised policy is issued

Rare Disease Day

(Vikaspedia, Editor, Online Portal, 05 Apr 2019)

- Celebrated every year on last day of the month of February
- Month known for having a 'rare' number of days
- First Rare Disease Day was celebrated on 29 Feb 2008
- Aim: Raise awareness amongst the general public and decision-makers about rare diseases and their impact on patients' lives

Summary of Findings of Data Extracted and Analyzed The details of summary of findings of data extracted and analysed of 35 articles/journals/online portals are attached as **Appendix**

RECOMMENDATIONS

Policy Priorities for Rare Diseases

a. Rare diseases should be made a priority in terms of public healthcare

b. Awareness should be raised about rare diseases among key stakeholders i.e. healthcare professionals, general public and health care practitioners

c. Efforts should be made to empower people and the community for a wider approach

d. Enabling a better management of rare diseases for patients so that they can be involved and be able to influence the decisions that affect them

e. Promoting continued research and development, building political commitment to drive research and innovation

f. Increasing collaborative research efforts to improve scientific understanding of all the rare diseases

g. Ensuring that people have sustainable access to diagnosis, treatment and care of rare diseases

h. Improving workforce capacity & developing infrastructural potential to treat rare diseases

i. Developing legislation for orphan drug development in India to entice pharmaceutical industry and improve access to orphan drugs

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ORIGINALITY REPORT

15%	7%	3%	7%
SIMILARITY INDEX	INTERNET SOURCES	PUBLICATIONS	STUDENT PAPERS

PRIMARY SOURCES

1	www.phama.org.my Internet Source	3%
2	Submitted to Griffith College Dublin Student Paper	3%
3	www.downtoearth.org.in Internet Source	2%
4	Romina G. Armando, Diego L. Mengual Gomez, Julián Maggio, María C. Sanmartin, Daniel E. Gomez. "Telomeropathies: Etiology, diagnosis, treatment and follow-up. Ethical and legal considerations", Clinical Genetics, 2019 Publication	1%
5	Submitted to Waterford Institute of Technology Student Paper	1%
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