

**22– 03 – 2024**

**News: Rare Disease Day**

- Recently, Rare Disease Day was observed on the last day of February. This international awareness day is dedicated to raising awareness of rare diseases and their significant impact on patients and their families.

## **Rare Disease Day**

- Rare Disease Day is a **globally coordinated movement dedicated to advocating for equity in social opportunity, healthcare, and access to diagnosis and therapies for individuals with rare diseases.**
- The theme for Rare Disease Day 2024 is “**Share Your Colours**”, which emphasizes collaboration and support.
- It was **established in 2008 and observed annually on 28<sup>th</sup> February (or 29<sup>th</sup> in leap years).** Rare Disease Day is coordinated by the **European Organisation for Rare Diseases (EURORDIS)** and over **65 national alliance patient organization partners.**

**It serves as a focal point for advocacy work on local, national, and international levels, engaging individuals, families, caregivers, healthcare professionals, researchers, policymakers, industry representatives, and the general public.**

## Rare disease

- A rare disease, also referred to as orphan disease, is any disease that affects a small percentage of the population.
- There are 6,000-8,000 classified as rare diseases, but less than 5% have therapies available to treat them.
- Most rare diseases are genetic, and are present throughout a person's entire life, even if symptoms do not immediately appear.
- About 95% of rare diseases have no approved treatment and less than 1 in 10 patients receive disease-specific treatment.
- 80% of these diseases have genetic origins.
- These diseases have differing definitions in various countries and range from those that are prevalent in 1 in 10,000 of the population to 6 per 10,000.
- However broadly, a 'rare disease' is defined as a health condition of low prevalence that affects a small number of people when compared with other prevalent diseases in the general population. Many cases of rare diseases may be serious, chronic and life-threatening.
- India has close to 50-100 million people affected by rare diseases or disorders, the policy report said almost 80% of these rare condition patients are children and a leading cause for most of them not reaching adulthood is due to the high morbidity and mortality rates of these life-threatening diseases.

- The most common rare diseases recorded in India are Haemophilia, Thalassemia, sickle-cell anemia and primary immuno deficiency in children, auto-immune diseases, Lysosomal storage disorders such as Pompe disease, Hirschsprung disease, Gaucher's disease, Cystic Fibrosis, Hemangiomas and certain forms of muscular dystrophies.
- Globally, 8% of the population suffers from rare diseases. 75% of the survivors are children who make parents run from pillar to post for their treatment, exhausting their resources and emotionally draining themselves in the process.