

**GOVERNMENT OF INDIA
MINISTRY OF HEALTH AND FAMILY WELFARE
DEPARTMENT OF HEALTH AND FAMILY WELFARE**

**RAJYA SABHA
UNSTARRED QUESTION No. 583
TO BE ANSWERED ON 25th July, 2023**

TREATMENT OF PATIENTS SUFFERING FROM RARE DISEASES

583. SHRI NARAIN DASS GUPTA:

Will the Minister of **HEALTH AND FAMILY WELFARE** be pleased to state:

- (a) the number of patients suffering from rare diseases registered in major centrally governed Hospitals all over the country and funds allocated to these hospitals for treatment of rare diseases;
- (b) the diseases defined as rare diseases and steps taken by Government for treatment of patients suffering from rare diseases; and
- (c) the number of patients who declined treatment or whose treatment is yet to be started, State-wise?

ANSWER

**THE MINISTER OF STATE IN THE MINISTRY OF HEALTH AND FAMILY
WELFARE
(DR. BHARATI PRAVIN PAWAR)**

(a) As per the data available with Indian Council of Medical Research (ICMR), 9,675 patients have been enrolled since November, 2019 on the portal of National Registry for Rare and Other Inherited Disorders (NRROID) out of which 4,408 patients are from centrally governed Hospitals. For financial year 2023-24, funds of Rs. 92.84 Crore (Rs. Ninety Two Crore Eighty Four Lakh Only) has been allocated for treatment of rare diseases' patients in the designated Centres of Excellence under the National Policy for Rare Diseases (NPRD), 2021.

(b) & (c) The term rare diseases, for the purpose of NPRD, 2021, shall construe the following groups of disorders identified and categorized by experts based on their clinical experience:

Group 1: Disorders amenable to one-time curative treatment:

- (i) Disorders amenable to treatment with Hematopoietic Stem Cell Transplantation (HSCT).
- (ii) Disorders amenable to organ transplantation.

Group 2: Diseases requiring long term / lifelong treatment having relatively lower cost of treatment and benefit has been documented in literature and annual or more frequent surveillance is required:

- (i) Disorders managed with special dietary formulae or Food for Special Medical Purposes (FSMP).
- (ii) Disorders that are amenable to other forms of therapy (hormone/ specific drugs).

Group 3: Diseases for which definitive treatment is available but challenges are to make optimal patient selection for benefit, very high cost and lifelong therapy.

- (i) Based on the literature sufficient evidence for good long-term outcomes exists.
- (ii) The cost of treatment is very high and either long term follow up literature is awaited or has been done on small number of patients.

In terms of NPRD, 2021, the steps have been taken by Government for treatment of the patients suffering from rare diseases include designation of eleven (11) Centres of Excellence (CoEs), which are premier Government tertiary hospitals with facilities for diagnosis, prevention and treatment; and financial support for treatment of rare diseases patients, which has been increased from up to Rs. 20 lakhs per patient to up to Rs. 50 lakhs per patient. Treatment of patients starts immediately after registration with the CoEs.
