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

LOK SABHA
UNSTARRED QUESTION NO. 1625
TO BE ANSWERED ON 16TH DECEMBER, 2022

GENETIC DISORDER

1625. SHRI PARVESH SAHIB SINGH VERMA:
SHRI VISHNU DATT SHARMA:

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

(a) whether the Government is conducting/ proposes to conduct any research on the treatment for rare Genetic Disorders in India and if so, the details thereof;

(b) the steps taken/proposed to be taken regarding cure for rare genetic disorders in India;

(c) whether the Government is making efforts to promote domestic manufacturing of medicines, for rare genetic disorders, considering their scarce availability and prohibitive cost;

(d) if so, the details of efforts made and outcomes achieved in this regard; and

(e) if not, the reasons therefor?

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

(a) & (b): The Indian Council of Medical Research (ICMR) task force in rare diseases includes twenty-four (24) studies addressing various issues in Primordial Dwarfism (Pd), Granulomatosis with Polyangiitis, schwannoma associated with Neurofibromatosis type 2, arterial calcifications in ADCC/CALJA (a rare disease marked by NT5E gene mutations), FibrodysplasiaOssificansProgressiva (Fop), Polymicrogyria Syndromes, Amyloidosis, Seronegative NeuromyelitisOptica, Bardet-Biedl Syndrome, Retinitis Pigmentosa Type 12, Arthrogryposis Multiplex Congenita (AMC), Primary Immunodeficiency disorders and Inborn Errors of Immunity.

Ministry of Health and family Welfare has framed National Policy for Rare Diseases (NPRD), 2021 for facilitating and providing financial assistance to the patients suffering from rare diseases. The policy has been amended and now financial assistance of upto Rs. 50 Lakh is permissible per patient.

Ten (10) Centres of Excellence (CoEs) have been identified, which are premier Government tertiary hospitals with facilities for diagnosis, prevention and treatment of rare diseases. In order to receive

financial assistance for treatment of rare disease and avail the benefits as per the policy, the patient of the nearby area can approach the nearest Centre of Excellence.

As informed, the Department of Biotechnology (DBT) has supported Research & Development (R&D) projects for developing treatment for rare genetic disorders in India. These projects include proof-of concept for treatment of hemoglobinopathies using genome editing tools, and clinical trial of lentiviral vector-based gene therapy for Hemophilia. Besides, research on modelling of rare genetic diseases such as inborn errors in metabolism using organoids and stem cells, used to test therapies and new delivery methods is also conducted by the Institute for Stem Cell Science and Regenerative Medicine, Bengaluru, an autonomous institution of the Department of Biotechnology. In addition, the public sector undertaking of the DBT, the Biotechnology Industry Research Assistance Council (BIRAC) has also supported projects for development of therapeutics for rare genetic diseases viz. Niemann-Pick Type C Disorder, and Duchenne Muscular Dystrophy.

(c) to (e): In order to make the country Atmanirbhar in pharmaceuticals, the Department of Pharmaceuticals implements the Production Linked Incentive (PLI) Scheme for Pharmaceuticals with total financial outlay of Rs. 15,000 crore and tenure from FY 2020- 2021 to FY 2028-29. The scheme covers pharmaceutical goods under three different categories. One of the product segment under the Category-1 is Orphan drugs, including those used for treatment of rare diseases.

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