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

LOK SABHA
UNSTARRED QUESTION NO. 3193
TO BE ANSWERED ON 12TH JULY, 2019

RARE DISEASES

3193. SHRI VINAYAK RAUT:
SHRI GIRISH BHALCHANDRA BAPAT:
DR. PRITAM GOPINATHRAO MUNDE:
DR. SHRIKANT EKNATH SHINDE:

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

(a) whether an estimated 72-96 million Indians are affected by rare diseases and if so, the details thereof;

(b) whether it is a fact that India has not arrived at an official consensus on the definition of a rare disease and if so, the reaction of the Government thereto;

(c) whether the Government is considering to formulate a national policy for treatment of rare diseases and if so, the details thereof;

(d) whether the Government has included rare diseases in its plan of action and if so, the details thereof; and

(e) the funds allocated by the Government for rare diseases to each State during the last three years?

ANSWER
THE MINISTER OF STATE IN THE MINISTRY OF HEALTH AND FAMILY WELFARE
(SHRI ASHWINI KUMAR CHOUBEY)

(a) to (e): Government of India does not maintain any such data at central level.

Government had formulated a National Policy for Treatment of Rare Diseases in the year 2017. However, the Government decided to review the Policy in the light of new information and updates available/received for its further improvement and effective implementation. Accordingly, the Policy has been kept in abeyance vide non-statutory Gazette Notification dated 18-12-2018, till the revised Policy is issued or until further orders, whichever is earlier.

Contd.....

Pending revision of the National Policy, under the Umbrella Scheme of Rashtriya Arogya Nidhi, a component for treatment of rare disease has been included w.e.f. 01.01.2019 for providing one-time financial assistance to patients belonging to families living below threshold poverty line for treatment of identified Rare Diseases (**Annexure**) in Government hospitals.

An amount of Rs.100 crore has been allocated for Rare Disease component of Umbrella Scheme of Rashtriya Arogya Nidhi during 2019-20. Umbrella Scheme of Rashtriya Arogya Nidhi is a Centrally Sponsored Scheme, under which no funds are released to State Governments. Financial assistance for eligible patients is released to the treating Government hospitals.

No funds were sanctioned under the National Policy for Treatment of Rare Diseases, 2017 during the currency of the erstwhile policy.

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Annexure

List of identified rare diseases covered for treatment under the Umbrella Scheme of Rashtriya Arogya Nidhi

1. **Disorders amenable to treatment with Hematopoietic Stem Cell Transplantation (HSCT)**

i. Lysosomal storage disorders in early stages for which Enzyme replacement Therapy (ERT) is presently not available (eg Metachromatic Leukodystrophy, Krabbe's disease and severe form of Mucopolysaccharoidosis (MPS) type I within first 2 years of age.
ii. Adrenoleukodystrophy (early stages), before the onset of hard neurological signs.
iii. Immune deficiency disorders eg Severe Combined Immunodeficiency (SCID), Chronic Granulomatous disease, Wiskot Aldrich Syndrome,etc
iv. Osteopetrosis
v. Fanconi Anemia
vi. Others to be decided on case to case basis

2**. Disorders amenable to organ transplantation**

i. Liver Transplantation -Metabolic Liver diseases and other Inborn Errors of Metabolism (small molecule diseases)

i. Tyrosinemia (failure to respond to nitisinone therapy or have documented evidence of malignant changes in hepatic tissue)
ii. Glycogen storage disorders (GSD) I, III and IV due to poor metabolic control, multiple liver adenomas, or high risk for Hepatocellualr carcinoma or evidence of substantial cirrhosis or liver dysfunction or progressive liver failure,
iii. MSUD (Maple Syrup Urine Disease),
iv. Urea cycle disorders,
v. Organic acidemias,
vi. Wilson's disease (Decompensated cirrhosis).
vii. Bile acid synthetic defects (Decompensated cirrhosis)
viii. CriglerNajjar Type 1,
ix. Alpha 1 antitrypsin deficiency (Decompensated cirrhosis)
x. Progressive familial Intrahepatic Cholestasis (PFIC)(Decompensated cirrhosis)
xi. Others to be decided - case to case basis

ii. Renal Transplantation-

i. Fabry's disease,
ii. Autosomal recessive Polycystic Kidney Disease (ARPKD),
iii. Autosomal dominant Polycystic Kidney Disease (ADPKD)
iv. Others on case to case basis

iii. Patients requiring combined liver and kidney transplants -Rarely Methyl Malonic aciduria may require combined liver & Kidney transplant) etc.