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

LOK SABHA
UNSTARRED QUESTION NO.3375
TO BE ANSWERED ON 12TH JULY, 2019
MORQUIO A SYNDROME

3375. SHRI PARVESH SAHIBSINGH:

Will the Minister of HEALTH ANDFAMILY WELFARE be pleased to state:

(a) whether the cure and treatment for Morquio A syndrome (MPS IV A) is available in India and if so, the details of types of treatment and places/clinics/hospitals where such treatment is available;

(b) if not, whether the Government plans to bring the cure by international agreements /MoUs/Inter-Governmental cooperation with other countries like USA and if so, the details thereof;

(c) whether the cure and treatment for Morquio A syndrome (MPS IV A) is too costly to be affordable by all people in India and if so, the details thereof indicating the average cost for the treatment of the abovementioned disorder in India; and

(d) whether the Government proposes any financial assistance for the patients of rare genetic disorder like Morquio Asyndrome (MPS IV A) and if so, the details thereof?

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

(a) to (d): Presently there is no cure available for Morquio A Syndrome (MPS IV A). The currently available treatment for MPS IV A is Enzyme Replacement Therapy (ERT). The treatment is in the form of weekly intra-venous infusions of the ERT that can be administered at any secondary or tertiary care hospital.

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.

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-I**) 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.

Annexure-I

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