

# **Treatment for Duchenne Muscular Dystrophy**

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### Why in News?

A collaborative effort between **doctors from Tamil Nadu, India, and scientists from Japan** has resulted in the development of a **disease-modifying treatment for** <u>Duchenne Muscular Dystrophy</u> (DMD).

## What is the Disease Modifying Treatment for DMD and Major Findings?

- This treatment utilizes a <u>food additive</u> **called beta-glucan**, derived from the N-163 strain of the yeast **Aureobasidium pullulans**.
- The six-month-long clinical study involved 27 children with DMD, of which 18 were in the treatment group and 9 in the control group.
  - In addition to regular treatment, all participants (age >3) received the beta-glucan as a food supplement.
- The study yielded the following notable findings:
  - Reduction in Muscle Weakness and Damage: Evidence indicated a reduction in muscle weakness and damage among the treatment group.
    - It also led to improved Muscle Strength.
  - **Safety and Lack of Adverse Reactions:** No adverse reactions were observed in the participants, and the treatment showed no side effects on the liver and kidneys.

**Note:** Beta-glucan is a polysaccharide (a complex sugar) that has **anti-inflammatory**, **immunomodulatory and antioxidant properties.** 

# What is Duchenne Muscular Dystrophy?

#### About:

- Duchenne Muscular Dystrophy (DMD) is a <u>rare genetic disease</u> characterized by the **inability of muscles to produce dystrophin**, an enzyme that aids in muscle wear and tear as well as its regeneration.
  - It affects only male children.
- The absence of dystrophin leads to muscle damage, resulting in muscle weakness and ultimately wheelchair-bound conditions in early teens and consequent premature deaths.

#### Common Symptoms:

- Progressive muscle weakness and <u>atrophy</u> (loss of muscle bulk) that begins in the legs and pelvis and later affects the arms, neck and other areas of the body.
- Difficulty walking, running, jumping, climbing stairs and getting up from a lying or sitting position.
- Frequent falls, waddling gait (abnormal walking pattern) and toe walking.

#### Prevalence:

- According to a 2020 study on the global epidemiology of DMD, the pooled global DMD prevalence was 7.1 cases per 100,000 males and 2.8 cases per 100,000 in the general population.
- Also, it has approximately 5,000 patients in Japan and 80,000 in India.

#### Current Treatments:

- Currently, there is **no known cure for DMD.** Treatment aims to control symptoms to improve quality of life.
- Available treatments for DMD include gene therapy, exon-skipping, and disease-modifying agents such as <u>anti-inflammatory medications</u> and steroids.

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