COVER STORY

INTERVIEW

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WORKING TOWARDS REALISTIC TREATMENT OPTION

BY DR VEENABHARATHI

When was DART established?

In 2012 with the idea of initiating research leading to treatment that would provide support to children affected by DMD (Duchenne muscular dystrophy) and their families.

What is the role of DART in DMD research? What scientific strides have happened in recent years, with respect to finding a cure or treatment?

DART is the first research laboratory in India focusing on DMD research. We are working towards a realistic treatment option to alleviate and reverse the dystrophy condition at the genetic level, thereby enhancing the quality of life of affected children. The research programmes have been partially funded by state and Union government agencies, including ICMR (Indian Council of Medical Research).

DART is a DSIR (depart-

BHANU PRAKASH CHANDRA

ment of scientific and industrial research, Union ministry of science and technology) recognised research lab. In February 2017, Hanugen Therapeutics was started as a spin-off of DART with the aim to make the skills and technology of antisense oligonucleotides (AOS: small pieces of DNA that can modify the production of protein by cells) available to those suffering from genetic disorders.

Currently, Hanugen Therapeutics has obtained the manufacturing licence from the Indian Drug Licensing Authority for the upcoming clinical trials of DART.

What is the strategy behind the process of exon-skipping?

In human genes, there are non-coding sequences (introns) and the protein coding sequences (exons). In patients with disease-causing mutations, skipping or masking the non-functional exons can work to establish a situation where cells can produce a shortened but functional form of the dystrophin protein (which is deficient or undergoes mutation in DMD).

FOUNDERS AND HEALER
DR ARUN SHASTRY WITH RAVDEEP SINGH ANAND, HIS WIFE MOVIN AND THEIR SON KARANVEER

In the future, the targeted next generation sequencing (NGS) may become a single platform to detect all types of mutations in the DMD gene.

An estimated 80 per cent of DMD patients have genetic mutations (alterations in gene) that are amenable to exon-skipping.

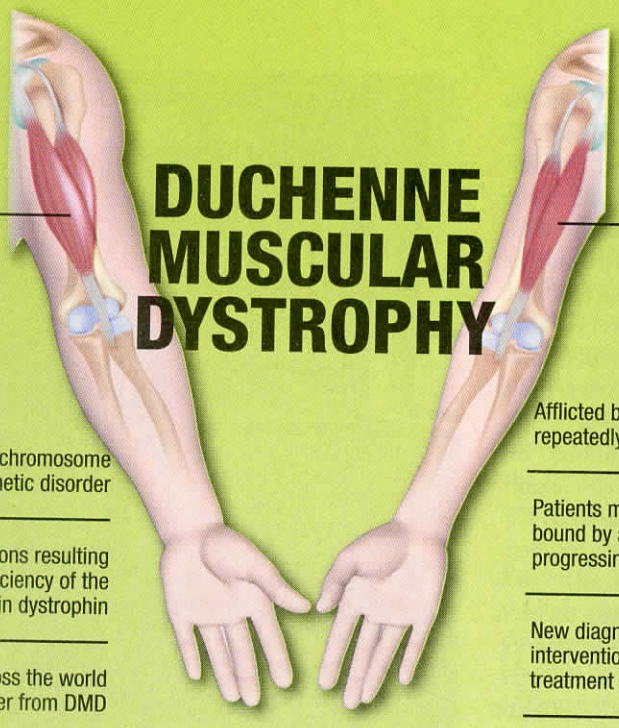
In DMD, an exon or exons are deleted. This interferes with the rest of the gene being pieced together. For the dystrophin protein to work, it must have both the ends of the protein. Hence, whenever there is a mutation, it results in a completely non-functional dystrophin protein and severe symptoms of DMD.

In exon-skipping, AOS are used to mask the exons that need to be skipped.

In the future, the targeted next generation sequencing (NGS) may become a single platform to detect all types of mutations in the DMD gene. NGS could provide precise genetic information for emerging gene therapies.

(DART was founded by Movin Anand and Ravdeep Singh Anand whose son Karanveer Singh is afflicted with DMD. Karanveer, 22, is wheelchair-bound. He is currently pursuing his BSc.)

DUCHENNE MUSCULAR DYSTROPHY



This is an X-linked (sex-chromosome linked) recessive, genetic disorder

Characterised by mutations resulting in the absence or deficiency of the muscle protein dystrophin

One in 3,500 boys across the world are known to suffer from DMD

The disease affects male children (mostly) since a male child carries only one X chromosome

The child manifests loss of muscle mass and progressive weakness of muscles

Afflicted boys tend to stumble and fall repeatedly by the age of two or three

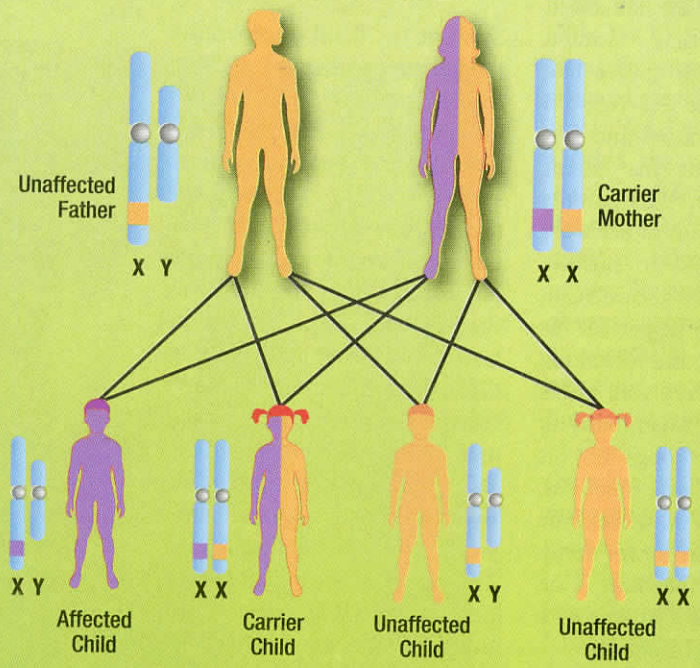
Patients may become wheelchair-bound by age 10-12 because of rapidly progressing muscular weakness

New diagnostic tests can enable early intervention—there is no cure, but treatment can improve quality of life

The Right of Persons with Disability Act, 2016, which aims to provide protection, equality and inclusion in society, covers muscular dystrophy

Care givers go through a lot of stress. Forming self help groups can help

HOW IT IS INHERITED



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