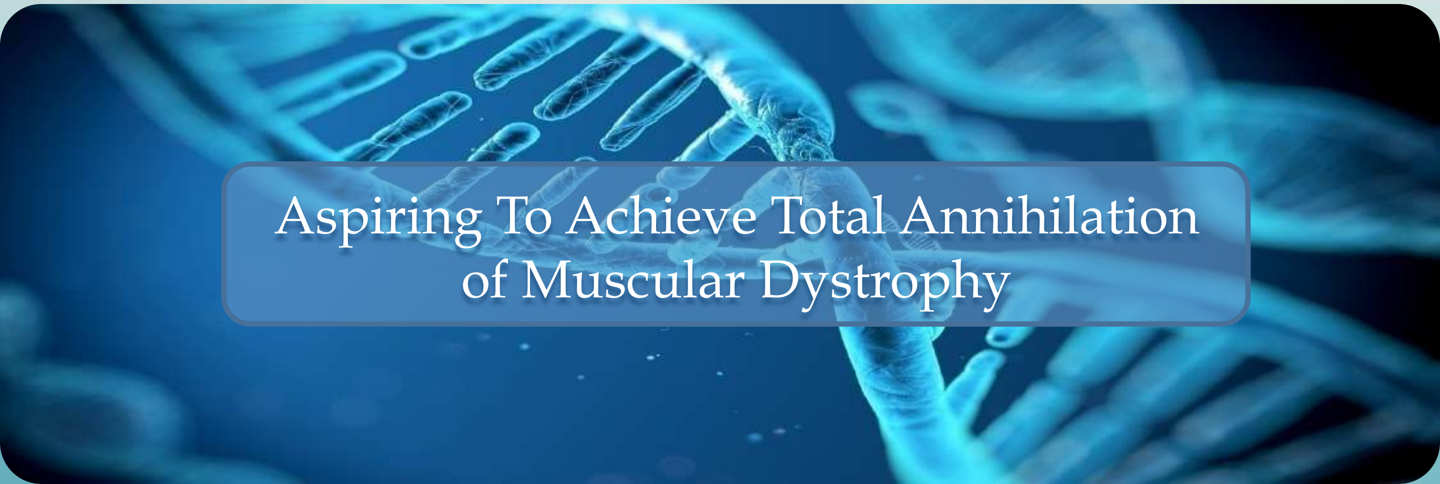
DARTians

The Quarterly Newsletter
from
Dystrophy Annihilation
Research Trust

2nd Edition

September 2020



Aspiring To Achieve Total Annihilation of Muscular Dystrophy

DARTians

The Quarterly Newsletter from Dystrophy Annihilation Research Trust

Issue 2, September 2020

Mission

- To bring together all Muscular Dystrophy parents and patients.
- To create awareness of Treatment, Drug Trials and Facilitate Research in finding solutions to eradicate Muscular Dystrophy.

Vision

To empower research for realizing the potential for full rehabilitation of those diagnosed with Muscular Dystrophy.

President's Message

To all the DARTians out there, Season's Greetings. It is really heart warming to see that despite all the mayhem being caused by the Corona virus Pandemic, the DART Team has been relentlessly pushing their cause.

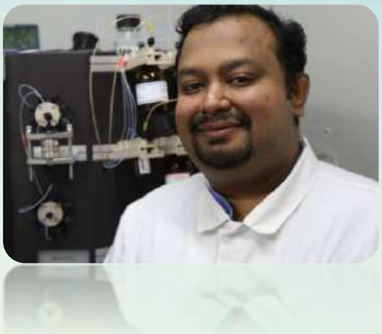
It gives me great happiness and joy to inform one and all that we have finally got our approval from the Indian FDA to conduct our Clinical Trials.

Over a 100 boys will directly benefit from this trial which will pave the way for larger groups in the near future thereby bringing hope and putting a smile on the faces of thousands of DMD families. God bless all the Doctors, Patients and Government agencies for all their support and help towards a brighter future for our kids.

\- **Ravdeep Singh Anand**, President DART

From the Research Desk

At DART, we have always been focused on translating research from bench side to bed side (Lab to Clinic). We have achieved tremendous success over the years purely on the trust, faith and support shown by the patients and their endearing families. I am also blessed to have a team of vibrant young researchers who have even got to the extent of shifting from one state to another to be part of DART. Thanks to the far sighted vision of our management we were able to establish world-class cell culture lab, Oligonucleotide Facility, Organic Chemistry lab and a GMP compliant manufacturing facility.



As of now to the best of my knowledge we are the first and so far only parent-funded research organization to be certified as a 'Scientific Industrial Research Organization' by the DSIR. Recently we got the go-ahead from the DCGI to start clinical trials and now in collaboration with pediatric doctors from various hospitals across India we are on the verge of starting the first ever human clinical trials for Exon Skipping in India.

I thank the parents, donors, state government and Central government funding and regulatory agencies for supporting our research. We are now making history.

- **Dr. Arun Shastry**, Chief Scientific Officer, DART

Internationally there are multiple initiatives to align efforts for the diagnosis and therapy development for rare disease patients. The international rare disease research consortium (IRDIRC) aims to have a genetic diagnosis for patients with inherited rare diseases within a year from the onset of symptoms. Furthermore, they want 1000 additional treatments for rare disease patients by 2027 – where the focus is not only on having these treatments but also on ensuring that patients have access to these treatments.

Within the neuromuscular field the TREAT-NMD alliance aims to make the field ready for not only clinical trials but also for treatment of patients with neuromuscular disorders. The World Duchenne Organization organizes the Duchenne World Awareness Day (WDAD) annually on September 7, an effort DART participates in as well. Furthermore, to enable Duchenne patient advocates to be equal stakeholders in the process and politics of rare disease therapy development, the Duchenne Patient Academy trains patient advocates from around the world, including representatives from DART.

We live in exciting times where treatments for rare diseases have now become a reality. This brings a new set of problems, such as ensuring as many patients as possible have access to the treatments. It is therefore crucial that we remain collaborating on a global level.

- **Prof. Steve Wilton**, Professor of Translational Genetics, Department of Human Genetics, Leiden University Medical Center, Netherlands

Disease Management

Medicine

DMD is one of the most burdensome genetic disorders. The affected boys live on an average for almost two decades or more, half of it on a wheel chair. I have watched the despair in the eyes of the boys and their parents for almost three decades hoping there will be effective treatment around next year. The therapies showing some promise are: exon skipping, read through of stop codons, gene therapy with mini and full gene, gene editing and utrophin augmentation.

Exon Skipping requires more effective Antisense Oligonucleotides with better penetration and less frequent dosing. In our study on Stop codon read-through without deletion/duplication were sequenced and about 50% had stop codons that would be helped by read-through technology. In January 2020 a team at the Munich Technical University corrected the gene defect using Crispr-cas9 technology. Using utrophin to replace dystrophin has led to success, but we would have to wait for more trials on this therapy. In conclusion I am of the opinion that we must develop affordable therapies in India, as DART has shown for exon skipping till then managing the disease with proper physiotherapy and steroids is our best bet. I am happy that DART has got the nod from the Drug Controller Government of India to manufacture their compounds in India and human trials will be mounted soon.

Dr. I C Verma – Senior Consultant, Sir Ganga Ram Hospital

Physiotherapy

Physiotherapy at DART involves screening, assessing and management and rehabilitation of children and adults affected by Muscular Dystrophy. We maximize the child's abilities in motor functions and work towards management of tightness, contractures and deformities in limbs and spine.

We strongly advise active and active assisted exercises rather than passive which involve active participation of the child. Therapy can also be ensuring in keeping all joints relaxed and supple. Exercises are taught to ensure in keeping joints in neutral position. Another exercise is to maintain forced expiration and forced inhalation lung volumes which optimizes and maintains good saturation of oxygen levels. We advise all our families to follow exercise regime as a daily routine leads to better outcome and helps attain our mutually desired goals.

Vishnu Vardhan Reddy, Physiotherapist, The Spastics Society of Karnataka

The UN and Rare Diseases

In September 2015, the General Assembly adopted the 2030 Agenda for Sustainable Development that includes 17 Sustainable Development Goals (SDGs). Building on the principle of "*leaving no one behind*", the new Agenda emphasizes a holistic approach to achieving sustainable development for all. Perhaps it is time to add #18 to the list – No Disease Orphan.

In India, within the frameworks of both the National Policy for Rare Diseases published in 2020 by the Government of India and the recently announced National Digital Health Mission on August 15th 2020, it is clear that the focus of the government is on technologies for better diagnoses and care delivery by leveraging digital platforms at population scale. The question of how all this focus on technologies can help build solutions for rare and orphaned diseases remains to be worked out. Let me suggest two examples of how progress can be made.

1. The University of Toronto through the spinoff company *Deep Genomics* uses deep learning platform *Spidex*, that provides "a comprehensive set of genetic variants and their predicted effects on human splicing across the entire genome." Its first success story appears to be a treatment for Wilson disease is a oligonucleotide therapy that caused approximately 70% skipping of exon 6. *It is important to note that the approach taken by DART and its sister concern Hanugen can now be scaled to have a much larger impact using these methods*.
2. Syndromic screening of orphan diseases can be facilitated by use of software like *Phenomizer (Kohler et al.,)* to carry out differential diagnosis and generate a gene list for a patient case. Or we could use *DeepGestalt (Gurovitz et al.)* or *Pedia (Krawiz et al.)* based on deep learning methods, which are exciting recent developments in the field of phenotyping that can possibly supplement the physical examination step.

To make real progress towards this goal #18 "No Disease Orphan" we need to promote an active dialogue among stakeholders in orphan diseases with a view to create a better world for persons with these disabilities.

Fortunately, there has been a growing awareness around orphan diseases and many stakeholders representing global, national and local interests in orphan diseases are active today with many information resources in web portals and foundations supporting diagnostic and therapeutic advances. Most important are the extraordinary heroic efforts by patients and their caregivers, such as the group at DART, that give us hope that the audacious goal of no diseases orphan is attainable.

Prof Vijay Chandru,
OPFORD Foundation, IISc,
(in collaboration with **Prof Chintan Vaishnav**, Tata Center and Sloan School, MIT)

Diet & Well Being

When we talked to doctors about our son Arun, we got to know that there is no treatment for DMD, so we went for natural remedies and started with ensuring he has a proper diet first. The following are the main points to consider:

✓Protein rich food such as egg and egg products, non vegetarian food.

✓Young and fresh shoots of wheat grass possess excellent immune building capacity and also keeps your blood count constant. Drinking a glass of wheat grass juice on an empty stomach is advisable to maintain normal blood count for a patient with DMD. Since wheat grass has no taste, a little sugar and salt can be added with a dash of lemon for better taste.

✓Food rich with protein, Vitamin E as well as Calcium plays an important role for these children through proper milk based diet or milk products like paneer and other items.

✓We have to always see that the food is producing oxygen enough amount because at later stages when the disease develops as the child gets older the oxygen supply and requirement should be proper.

To prolong survival in patients with DMD, there are several nutritional aspects that are important to consider. While dealing with the disease and after a lot of reading along with our own experience we came to know about the effects of over nutrition on mobility, respiratory and cardiac functions; the impact of hypo nutrition on muscle and ventilation; constipation and other gastrointestinal complications; chewing/swallowing difficulties with an increased risk of aspiration that predisposes to infectious diseases and respiratory complications; as well as osteoporosis with an associated increased risk of fractures.

In conclusion I would say the aim of this article is to provide an analysis of the nutritional aspects and complications that can start in children with DMD and increase with aging. These aspects should be considered in the transition from pediatric clinics to adult services.

Pradeep and Jayshree Gangadharan, Parents

Mental Health

Focus on mental, social and emotional health needs to be consistent. Hence, some intervention is better than no intervention. Given the current scenario, Telehealth- therapy provided remotely (online) through telecommunication technology- is an effective and beneficial method for receiving intervention. Here's why I highly recommend this:

- There is a shift in the parent/ caregiver involvement in the therapy process- from third person to hands-on first person interaction with your child. This helps you to become fully aware of all the nitty-gritty of the therapy that your child is undergoing.
- This is an opportunity to spend quality time and strengthen the relationship you have with your child. You can tap into your creativity (with the therapist's right guidance) and make the sessions fun! -Be it by enacting social stories, using interactive online games etc. Through this new role you will discover interesting aspects of your child.
- Being the facilitator(s) enables you to then see the results of your efforts which are motivating, rewarding and empowering in itself.

Rather than dwelling on the uncertainty of things, your child can live how Jake Marrazzo, a DMD warrior, puts it- "*Don't live the diagnosis, live the life*".

Jesse Jeffrey, Developmental Therapist,

Education

'Inclusion' is a worldwide movement and in the field of education, it means appropriate education be made available for all students regardless of their abilities and disabilities and that the education system is an integral part of human rights and equity issue. As academicians, it is our responsibility to spread awareness of the different schemes available for children with special needs and utilize it to the maximum potential.

A trained and motivated teacher is at the heart of effective inclusion and therefore translating policy into practice will depend on the continued development of skills and confidence of teachers in meeting the diverse range of children's needs. In spite of all available schemes and programs, issues like economic, social, legal and technological issues may be a barrier in providing higher education for differently abled children. But I hope India has already begun to make earnest efforts towards the betterment of higher education for children with special needs.

Dr. Nagarathna, Principal, MS Ramaiah College of Arts and Sciences

Moms & Dads

We feel proud to introduce Amogh N Petkar to you all. He was born on 5th September 2005 (on the occasion of Teacher's day). He was the first grandchild for our (me & my wife) parents on both sides, so he was given all the attention & pampering. But we never thought the same kind of "special attention" would have to be given in a different way after sometime.

It was in the last week of June 2012, when we got to know about Amogh's condition. The doctor diagnosed it as Duchene Muscular Dystrophy. Trust me, we had never ever heard of this before in our life until then. Once we heard it from the doctor, we started to learn more about it from Google & it only made us more restless & scared. My wife was about to deliver the second child in 4-5 weeks time & the doctor told us that in case if the second baby happens to be a boy then he also might have the same problem.
I really take this opportunity to salute my wife for her courage which she showed during that testing time. We spent sleepless nights till her delivery & we're gifted with an angel on 4th August & that's when we heaved a big sigh of relief. I really feel that I am blessed to have a father in law (Mr. J B Kulkarni) who immediately decided to relocate from Hubli & stay with us to support us morally, physically, financially & especially unconditionally.

This article would be incomplete if I did't mention about Amogh's schooling support. He has been a student of "Chitrakoota School" since his pre-primary days & is now in 9th standard. We become speechless whenever we recollect the kind of support & help extended by his school. They constantly kept on asking us "how they can help us further".
Not only did they make his attendance flexible, kept his classroom always in the ground floor but also donated generously to DART. We honestly feel blessed to be associated with such a wonderful institution.

"Being challenged in life is inevitable, but being defeated is optional".

Our heartfelt thanks to entire team of DART

Nagaraj and Nisha Petkar, Parents

Heroes of the Quarter

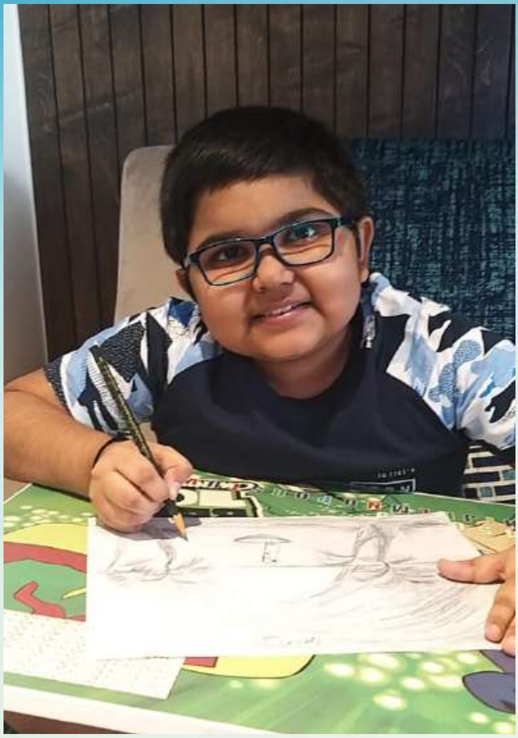
My name is **Karanveer** and I am 20 years old.
I am currently studying BSc Biotechnology at Ramaiah College of Arts Science and Commerce to accomplish my goal of finding a cure for rare diseases. I love sports, mainly cricket and football and my favourite sportsperson is AB De Villiers. My other passions are playing guitar and painting. I like watching movies and some of my favoured movie series are Fast and Furious, Mission Impossible and Avengers. Playing games on my play station is my treasured entertainment. I also cube and I have a huge collection of different types. Reading books is another hobby - Diary of a Wimpy Kid is my cherished series.
I am a foodie, my special dish being Chicken Biryani. I like to go on holidays and specially love road trips. I look forward to spend time with my pet dog; Shadow - she is truly adorable.

Friends constitute a major part in my life and I have been blessed with some very close friends since childhood. I have got many accolades throughout my school life. Some of my achievements are- getting 10 CGPA in class 10 and 95% in class 12, being appointed as the Head Boy of my school, DPS Bangalore North in class 12 and getting 100% scholarship into college.

Heroes of the Quarter

My name is **Amogh Petkar**
I stepped in to this world on 5th March 2005.
I study in Chitrakoota School, Bengaluru
My favorite subjects are Mathematics, Science and English.
My favorite food is Northern Indian Curry & chaat items.
My favorite online game is Free fire.
I am a die hard fan of action movies and I enjoy Harry Potter series. I also love drawing.
I have won a lot of medals in Olympiad Competitive exams .
I am now interested in making a short movie with my friends.

Heroes of the Quarter



My name is **Darsh Patel.**
My birthday is on May 30th .
I am 10 years old.

My hobbies are football, watching movies, playing videogames, drawing, painting, art and craft , swimming & cycling

My favorite food is Kichidi and my favorite movies are Captain America and Iron man.

Heroes of the Quarter

My name is **Tanzeem**.
My birthday is on 11th November.
I am 19 years old.

I am interested in drawing, watching TV, playing carrom, chowkabara and other indoor games with my siblings.


Name: Tanzeem Khan

This Quarter That Year (2015)

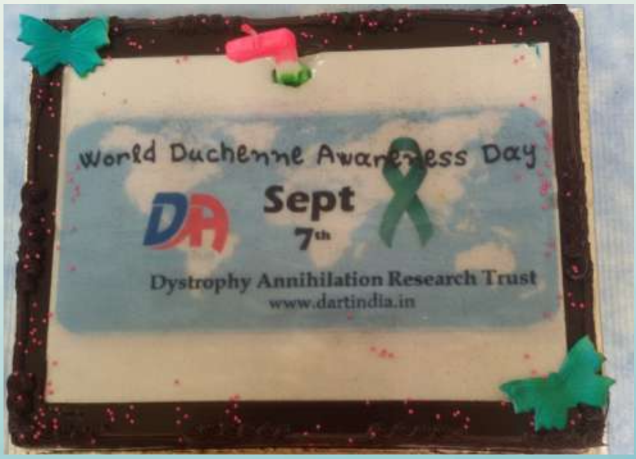
From 23 - 26 July 2015, Dr. Arun Shastry, Mr. RS Anand and Dr. Steve Wilton attended the prestigious INDO-USA Biotech Summit in Hyderabad.

On 27 July 2015 the State of the Art DART lab was inaugurated in Bangalore by Dr. Viswanathan and Dr. Steve Wilton

This Quarter That Year (2015)

From 15 August 2015 DMD children could avail of physiotherapy on a regular basis at DART which was conducted by Mr. Vishnu Vardhan who is a prominent physiotherapist in Karnataka

On World DMD Day [September 7] children, parents, staff and students all got together to celebrate life and had a lovely day of fun.



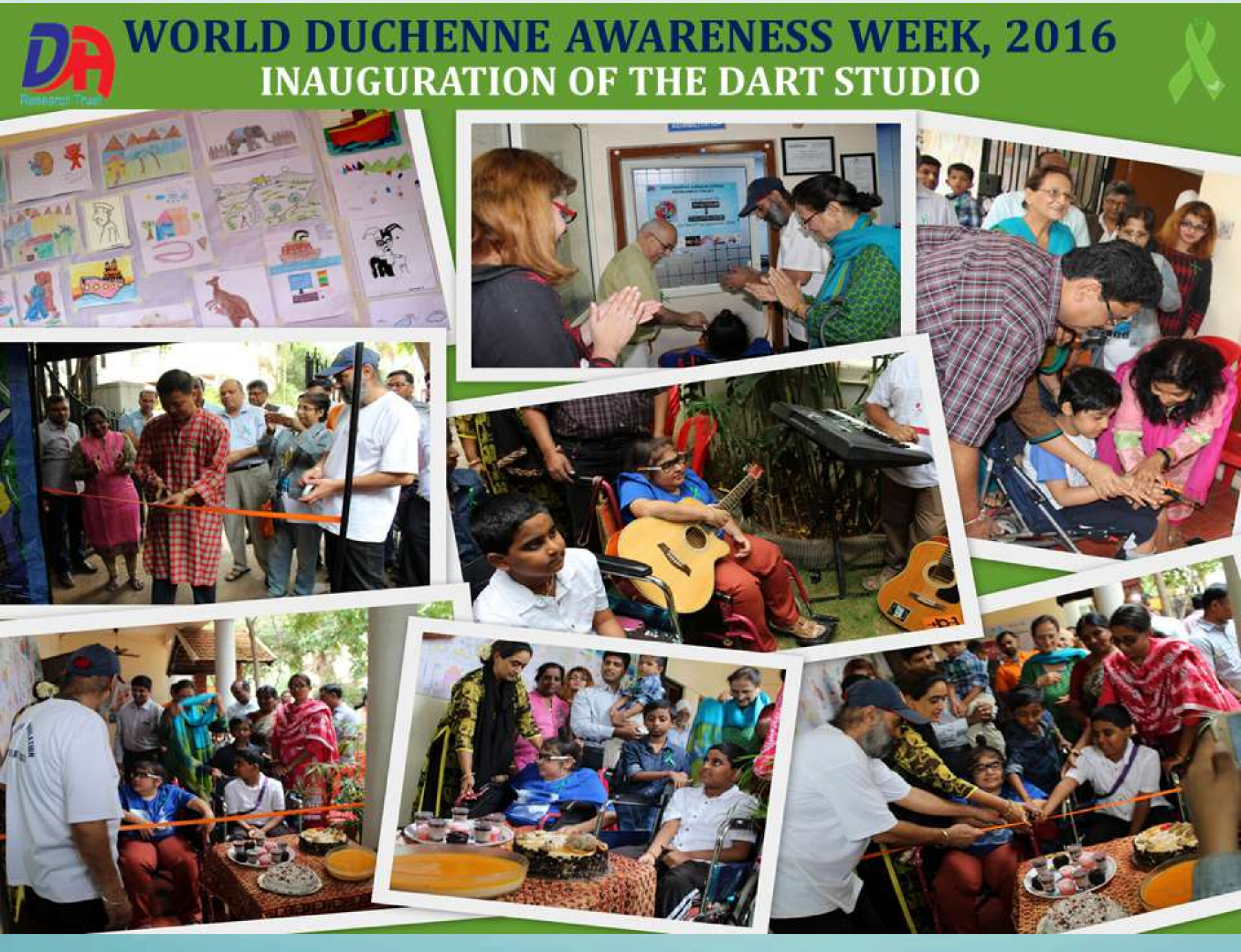
This Quarter That Year (2016)

On 7 August Dr. Arun Shastry and Mr. RS Anand visited the beautiful and timelessmonument - The Taj Mahal in Agra

On 24 July a team from DART took part in the Combat Carnival held in Bangalore and promoted awareness about Muscular Dystrophy

This Quarter That Year
(2016)

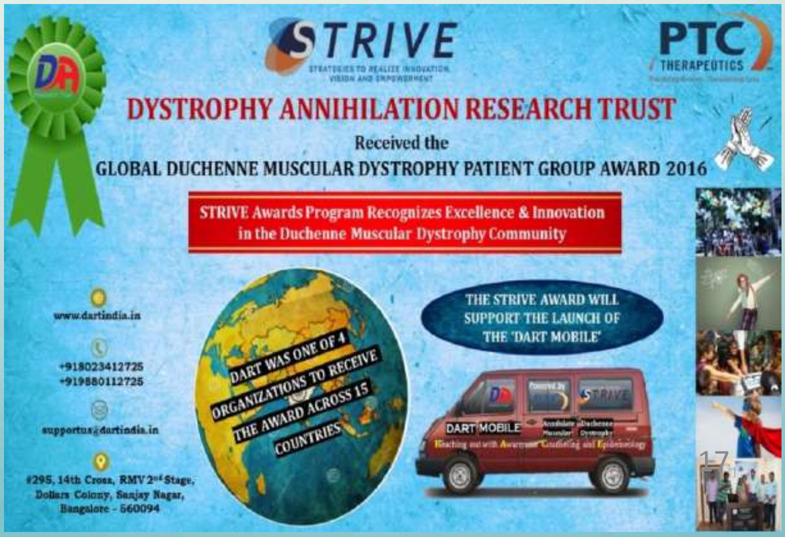
Throughout the entire first week of September in 2016, DART celebrated '*World Duchenne Awareness Week*' with multiple events across the country. This included awareness and donation drives in Bangalore, Chennai and Madurai which resulted in many families getting much needed relief, support and hope.



This Quarter That Year
(2016)

On 4 September the students and staff of Delhi Public School, Bangalore North joined DART in a walkathon at Cubbon Park, Bangalore to increase awareness of Muscular Dystrophy.

On 9 September PTC Therapeutics announced that DART had won the prestigious 'S.T.R.I.V.E for DMD' Global award


STRIVE
STRATEGIES TO REALIZE INNOVATION, VISION AND EMPOWERMENT

PTC THERAPEUTICS

DYSTROPHY ANNIHILATION RESEARCH TRUST

Received the

GLOBAL DUCHENNE MUSCULAR DYSTROPHY PATIENT GROUP AWARD 2016

STRIVE Awards Program Recognizes Excellence & Innovation in the Duchenne Muscular Dystrophy Community

DART WAS ONE OF 4 ORGANIZATIONS TO RECEIVE THE AWARD ACROSS 15 COUNTRIES

THE STRIVE AWARD WILL SUPPORT THE LAUNCH OF THE 'DART MOBILE'

www.dartindia.in

+918023412725
+919880112725

supportus@dartindia.in

#295, 14th Cross, RMV 2nd Stage,
Dollars Colony, Sanjay Nagar,
Bangalore - 560094

This Quarter That Year (2017)

On 19 July a team from acclaimed fashion icon Prasad Bidappa visited DART to get to know more about the work we do and meet and motivate the kids.

This Quarter That Year (2017)

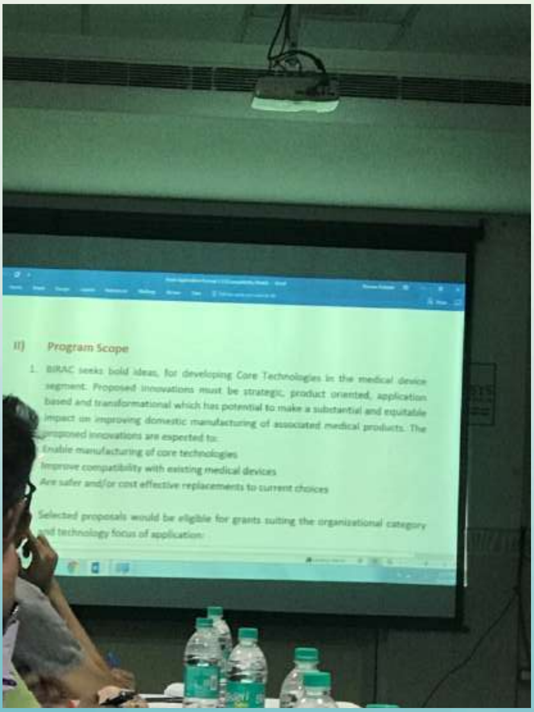
On the occasion of Independence Day, 15 August Dr. Arun, Mr. Anand and Mr. Ranjit represented DART at the cultural activities held at Shobha apartments and also manned the master muscle stall




This Quarter That Year (2017)

On 17 August a team from DART took part at an International conference in AVVM Sri Pushpam College, Tanjavur and gave inspiring talks on current trends in Biotechnology

On 27 August Dr. Arun Shastry gave a guest lecture at the prestigious Kalam Institute in Vishakapatanam.



II) Program Scope

1. BIRAC seeks bold ideas, for developing Core Technologies in the medical device segment. Proposed innovations must be strategic, product oriented, application based and transformational which has potential to make a substantial and equitable impact on improving domestic manufacturing of associated medical products. The proposed innovations are expected to:

Enable manufacturing of core technologies

Improve compatibility with existing medical devices

Are safer and/or cost effective replacements to current choices

Selected proposals would be eligible for grants suiting the organizational category and technology focus of application:

This Quarter That Year (2017)

On 2 August the kids and parents from DART took part in the making of the Master Muscle Marathon Anthem - 'Jaago re Bhaago re' Music Video

This Quarter That Year
(2017)

September 9, 2017 was an unforgettable day for DARTians as it was the day that Dr. Soumya Swaminathan, the then Director of ICMR, and present Chief Scientist of WHO visited DART and spent her valuable time with us. She shared her vision for our future and brought much joy and hope to the kids, parents, researchers and management at DART.

This Quarter That Year
(2017)

On 24 September DART hosted the very first Master Muscle Marathon on a grand scale at the GKVK Agricultural University Campus. Hosted and presided over by professional Ultra-Marathoner Coach Kay, hundreds of well-wishers and athletes took part and we were even serenaded by a brass band. It was a huge success and many people became aware of DART and the work we do.

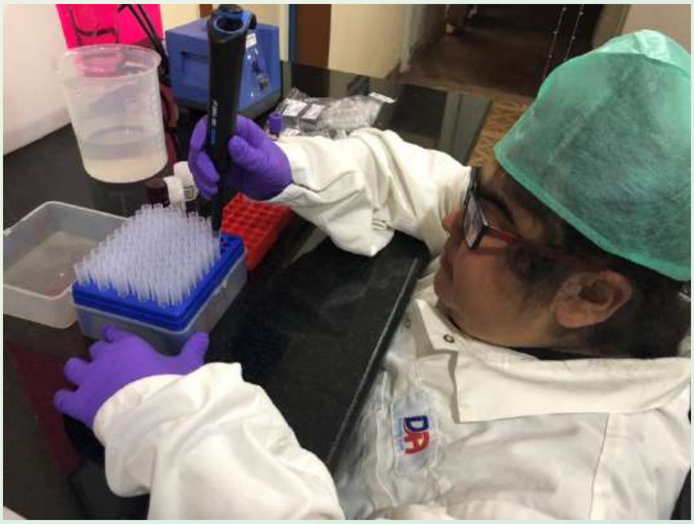
This Quarter That Year
(2018)

On 7 July the researchers and staff of DART went out for a Lab lunch to spend some time away from the lab and enjoy each others company outside the company.

On 19 July Dr. Arun Shastry gave a talk about Rare Disorders at the renowned Baptist Hospital in Hebbal, Bangalore

This Quarter That Year
(2018)

On 21 July Master Muscle Karanveer spent a day at DART as a young researcher, learning about the working of the lab. During the course of which he isolated and studied his own DNA and learnt about nucleic acids, Molecular genetics and the world of Molecular Biology. This experience shaped his desire to study Biotechnology in his higher studies.



This Quarter That Year
(2018)

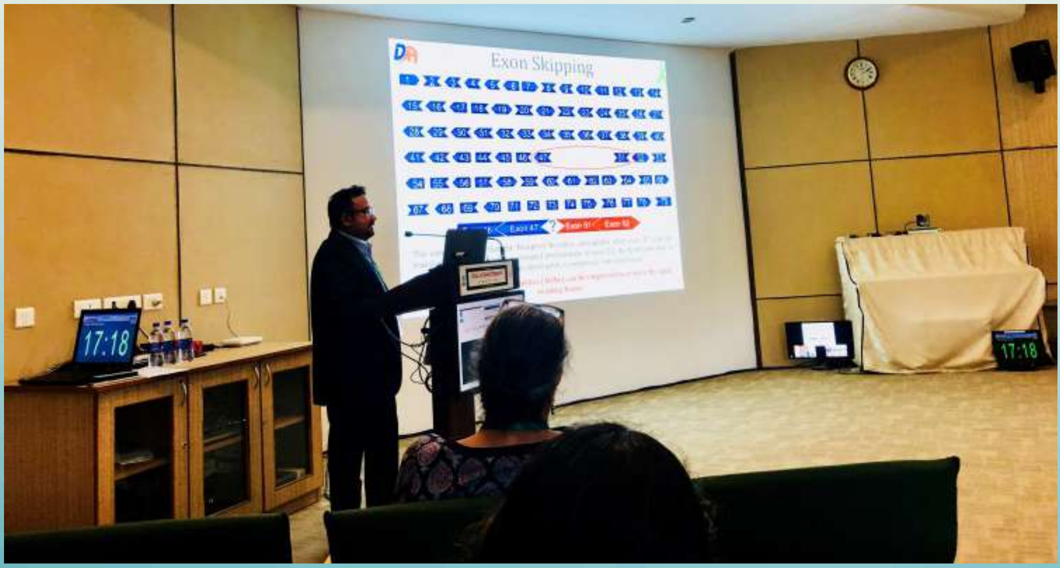
On 26 July Team DART along with a team from Center for Human Genetic (CHG) conducted an Awareness, Education, Evaluation and Physiotherapy camp at Tumkur. The camp took place under an initiative with the National Health Ministry. Many new families were identified who had children with Muscular problems and were given evaluation and physiotherapy with many finding relief and hope.

Research Trust

This Quarter That Year (2018)

On 27 July Dr. Arun Shastry proudly represented the Rare Disease consortium at the Biotechnology Ignition Grant (BIG) conclave conducted by the Government of India. During the course of which he spent valuable time with the secretary of the Department of Biotechnology (DBT) Dr. Renu Swarup

On August 6 Dr. Arun Shastry gave a lecture about Exon Skipping Strategies at a symposium held at CMC Vellore



This Quarter That Year
(2018)

On 7 August a team from DART spoke to the Asha Anganawadi workers who are government appointed Community Health workers who work solely in their village and focus on providing food supplements to young children. to ensure they are ready for a camp to be held at Bidadi.

This Quarter That Year
(2018)

On 17 August DARTian Abhay Ramkumar met his hero Sachin Tendulkar in Cochin and made him aware of Duchenne Muscular Dystrophy and its effects

On 18 August the Pooja for the beginning of the GMP-Manufacturing site was conducted ushering in a new era for DART

This Quarter That Year (2018)

For two days, 24-25 August, Dr. Arun Shastry, Dr. Aravind and Mr. Berty Ashley did a series of guest lectures for the students of Biotechnology at the Vignan's Foundation for Science, Technology, and Research (VFSTR) University, Guntur.
They also celebrated World Duchenne Awareness day by making the students aware of the disease and released the traditional red balloons.

This Quarter That Year
(2018)

On 7 September Mr. RS Anand spent a day with DMD children and their families at Dubai to extend the work of DART on a Global level.

On 18 September Mr. RS Anand was honoured by being asked to inaugurate the Pediatric Neuro clinic at Bangalore Baptist Hospital

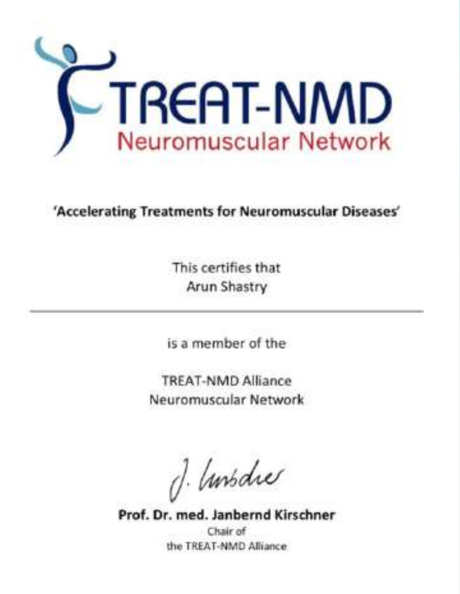
This Quarter That Year
(2018)

On 20 September the DARTMobile was revealed which was a mobile platform to RACE – Reach out with Awareness, Counseling and Epidemiology.

On 22 September Team DART and the DARTMobile conducted an awareness and evaluation Camp at Bidadi

This Quarter That Year
(2019)

From the 1 to 15 July, Dr. Arun Shastry attended the prestigious Leiden University Summer school conducted by the President of TREAT-NMD and well-wisher of DART, Prof. Annemieke.



TREAT-NMD
Neuromuscular Network

'Accelerating Treatments for Neuromuscular Diseases'

This certifies that
Arun Shastry

is a member of the

TREAT-NMD Alliance
Neuromuscular Network

Prof. Dr. med. Janbernd Kirschner
Chair of
the TREAT-NMD Alliance

On 7 September DART was honoured by being presented the 'Alert Being' award in Chennai which was picked up by DARTians Mahesh Karpur, Hitesh Chowhan, Arun Shastry and Berty Ashley

This Quarter That Year (2019)

On 9 September Dr. Arun Shastry spoke at the DBT rare disease mission in Delhi about the latest updates in Exon Skipping and its potential as therapy for genetic disorders.

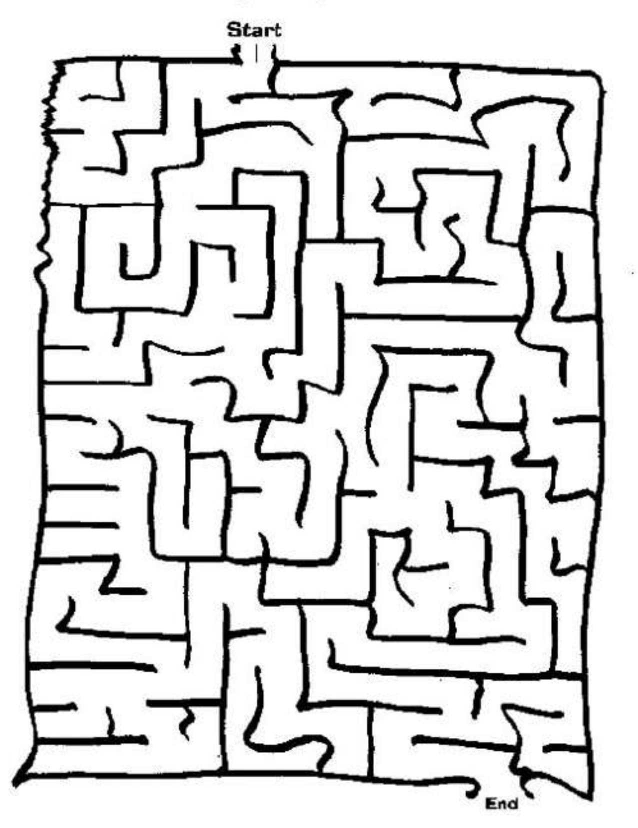
In September DART filed an application for conducting a multi-centric pan-India level clinical trials for Exon Skipping to Central Drugs Standard Control Organisation (CDSCO)

Activity Page

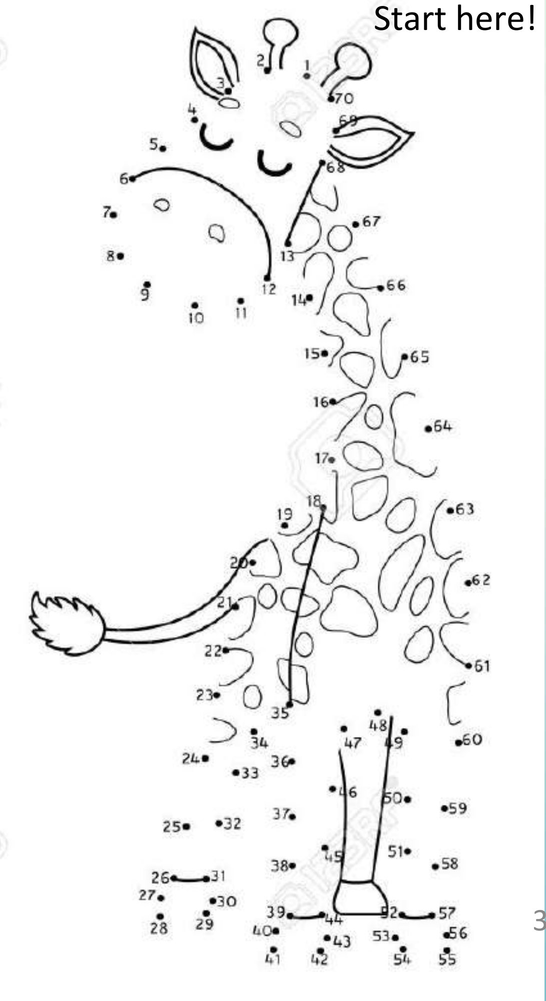
Fill out this fun maths puzzle!

	+		×		=	20
+	■	×	■	−		
	×	8	×		=	48
−	■	×	■	+		
	×		−		=	38
=		=		=		
0		80		10		

Get out of this maze!



Connect the dots and colour in!

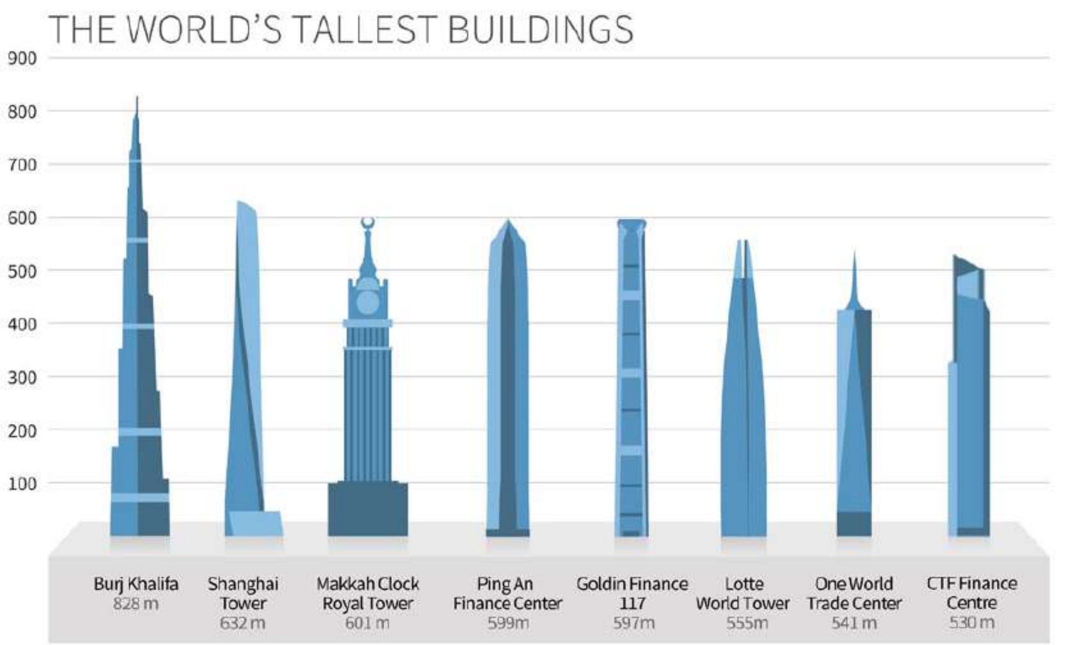


Find the Food Groups!

C	F	B	M	O	M	X	V	K
O	S	J	M	C	H	V	H	E
P	N	O	R	E	B	I	F	M
O	A	O	Q	S	A	U	Q	C
V	E	G	G	I	E	T	U	B
F	B	G	R	A	I	N	S	K
X	R	Y	X	N	U	A	F	Y
W	Q	U	T	T	D	I	R	C
Z	P	B	I	T	O	I	L	S
J	D	F	G	T	A	B	X	O
F	J	A	S	D	S	R	W	X

GRAINS
OILS
FRUITS
FIBER
DAIRY
MEATS
BEANS
VEGGIE

Trivia Time!



An Interview with a DARTian!

In this issue we speak to Sabareesan, Oligonucleotide Chemist

Us: Hi Sabareesh!! Tell us a few things about yourself.
SA: I am from Dharmapuri which is a beautiful green place. I like biking long distance and taking photo shoots!
Us: Yes! We love your instagram profile and wildlife pics. What is your favourite memory of DART?

SA: I enjoyed taking part in and working for the Master Muscle Marathon. It was a lot of fun for all of us. Another time was opening the DART Clinic to help the families. But my favourite moment was meeting Prof. Soumya Swaminathan. Though she was a very important personality she chatted and spoke with us like a friend.
Us: They are all amazing memories which wouldn't have happened if not for your help, thank you Sabareesh. What do you hope to be the future of DART?
SA: I think we will reach higher than what we are imagining right now!

Follow us on Social media!
Do show your support by donating to our cause!

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