



Duchenne  
UK

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thestudio  
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# Welcome to our Parent Information Day

Saturday 2<sup>nd</sup> October 2021





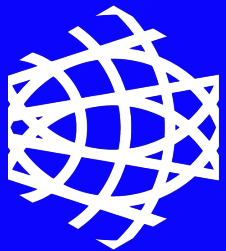
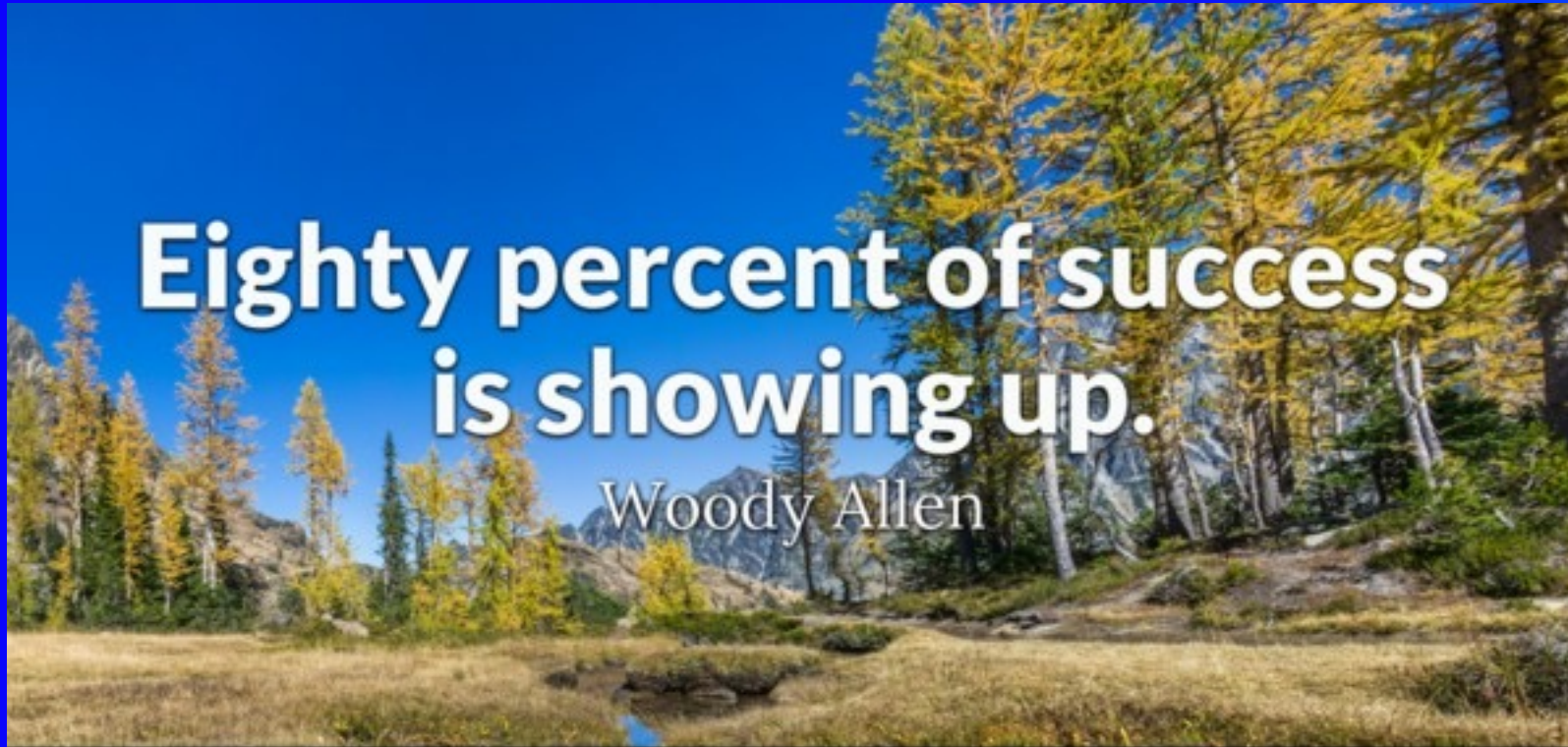
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# An introduction to newly diagnosed and first-time parents

Alex Johnson & Emily Crossley  
Co-Founders of Duchenne UK, DMD mums

# Thank you for coming!



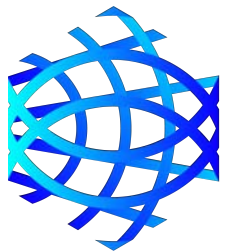
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# Alex Johnson & Emily Crossley



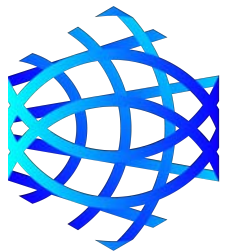
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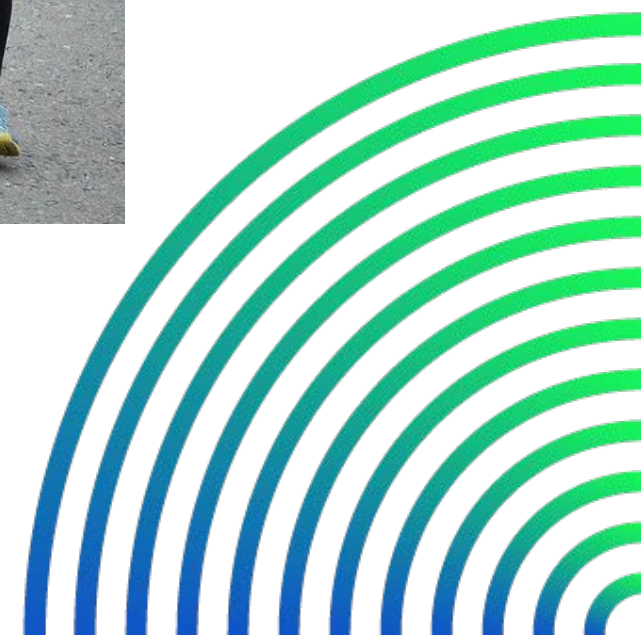
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# Alex Johnson, Co-Founder

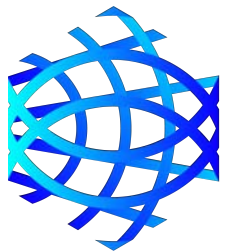


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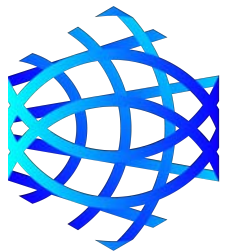
# Jack 13



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# Emily Crossley, Co-Founder & CEO

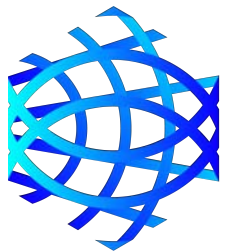


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# Eli 14



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# Format of the day

## Morning

- 1: Introduction and updates from Duchenne UK
- 2: Gene therapy update from companies & Q&A
- 3: Standards of Care

## Lunch & breakout sessions

Dad's and male carer's breakout session

or

Learning, behaviour and wellbeing in DMD: Working with school and college breakout session

## Afternoon

- 4: Other drugs in development - what's happening?
- 5: Adult life
- 6: Project HERCULES – what we've learnt
- 7: Parent panel Q&A & closing remarks

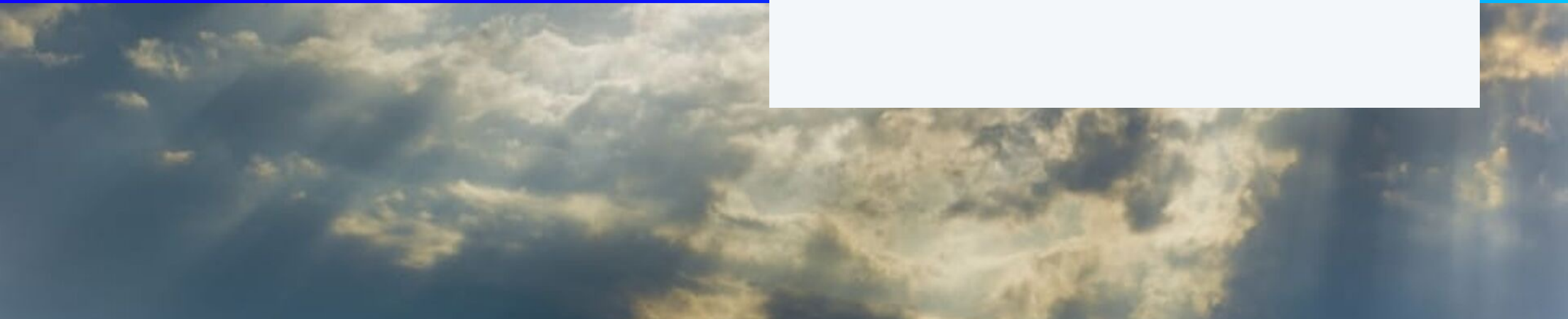
## Pie & Ale drinks reception



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**Balancing life after**

**DIAGNOSIS**





# How do you prioritise information?



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TAKE CARE OF TODAY

TAKE CARE OF YOURSELF

First steps

TAKE A BREAK

WHERE IS THE CARE?



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# You are your son's best advocate



**PRIMARY CARE**

**SCHOOL**

**COMMUNITY**

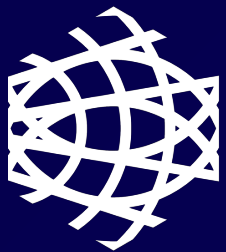
# YOU WILL FIND HERE

**SUPPORT**

**NEW FRIENDS**

**COMMUNITY**

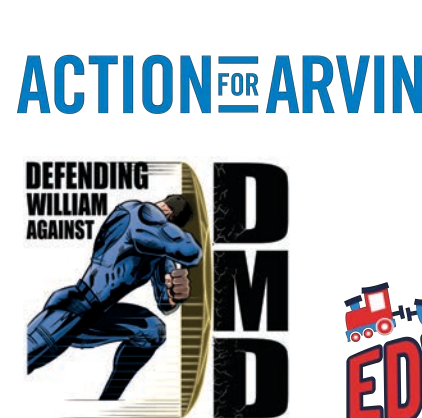
**KNOWLEDGE &  
UNDERSTANDING**




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# Family and Friends Funds - 42




**ACTION FOR ARVIN**




**ACTION FOR ZACH**




**Archie's Army**  
Helping Fight Muscular Dystrophy




**BACKING JACK**  
to help us beat Duchenne Muscular Dystrophy




**Ben vs Duchenne**




**CURE4GEORGE**  
Fighting Duchenne Muscular Dystrophy




**ACCESS TO LIFE**




Please help us in  
**Changing Charlie's Future**




**EDWARD STEAM TEAM**




**Elliot's Endeavour to End Duchenne**




**FOR FELIX**




**Duchenne UK Help Harry**




**CHASING CONNOR'S CURE**




**HENRY'S HURDLES**




**HELPING HAYDEN**




**HOPE FOR GABRIEL**  
FIGHTING DUCHENNE MUSCULAR DYSTROPHY




**JACK'S AIM**




**JACK'S MISSION**




**Jacobi's WISH**




**JOE'S JOURNEY TO END DUCHENNE**



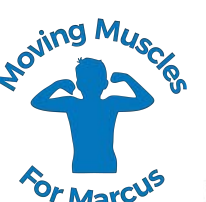
**LIFTING LOUIS**




**Love for Leon**  
Going the Distance to End Duchenne




**MISSION JENSEN**




**Moving Muscles For Marcus**




**MUSCLES FOR MITCHELL**  
strength through support  
FIGHTING DUCHENNE MUSCULAR DYSTROPHY




**PROJECT GO**




**RALPH'S FUND**




**Smile with Shiv**  
Fight Duchenne




**STRENGTH FOR STANLEY**




**Duchenne UK TEAM DEX**  
FUNDING RESEARCH INTO DMD



**team felix**



**TOGETHER for Rhys**



**WILLIAM'S FUND**

# Director of Research, Alessandra Gaeta

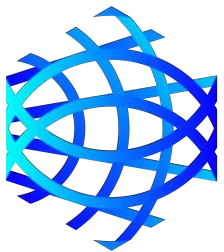


20-year R&D experience in academia, industry & the public sector in scientific, managerial and leadership roles

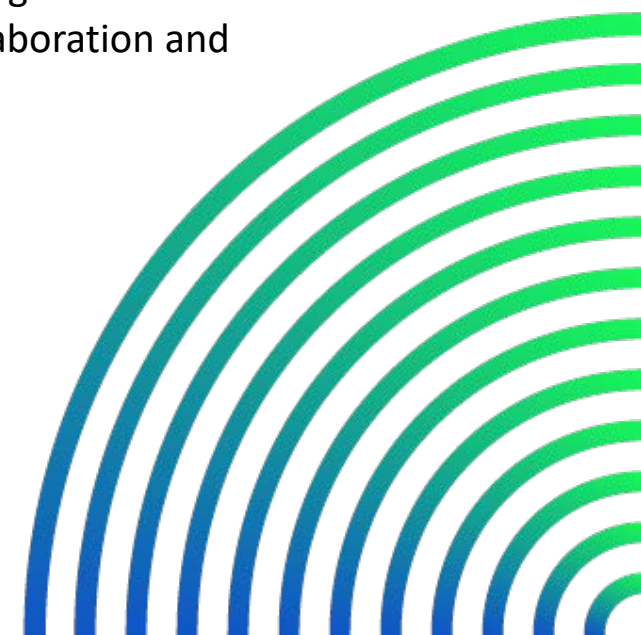
Pharmacy Degree and PhD in Medicinal Chemistry; post-doctoral Maplethorpe Fellowship Award for in pharmaceutical education and excellence in research at King's College London

Experience of preclinical, translational research, through to early phase clinical development

Led on the creation of high-profile, national R&D programmes to accelerate innovative treatments to the clinic, through collaboration and innovative funding models



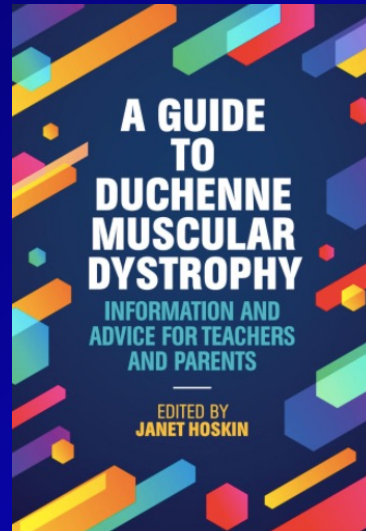
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UK**





# Ways Duchenne UK can help today

- Duchenne UK's newly diagnosed DMD family folder
- A Guide to Duchenne Muscular Dystrophy: Information and Advice for Teachers and Parents
- Steroid dependent alert wristbands



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Collect or order today for  
FREE



A LOT HAS

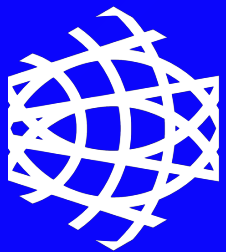
HAPPENED



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HOPE

IS HERE



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# THANK YOU!

Any questions?



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# Welcome to our Parent Information Day

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# Introduction and updates from Duchenne UK

Alex Johnson & Emily Crossley  
Co-Founders of Duchenne UK



TODAY IS ABOUT

HOPE AND  
COMMUNITY

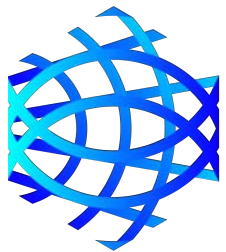


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# Alex Johnson & Emily Crossley



— IN PARTNERSHIP WITH —



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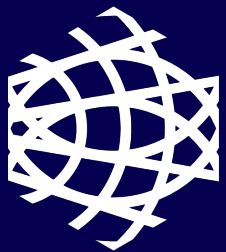


# FURTHER, FASTER, TOGETHER

**RESEARCH** - funding groundbreaking medical research

**SUPPORT** – DMD Care UK, Family folders and materials

**ADVANCE** – Project HERCULES, DMD Hub, Policy

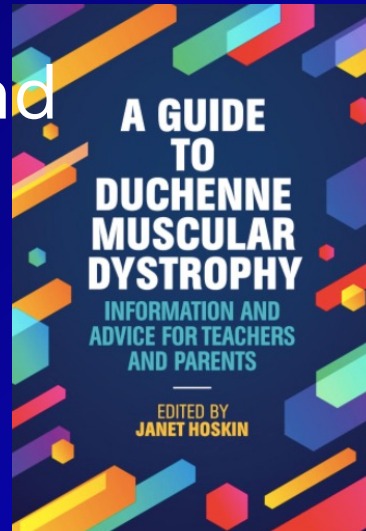


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- Steroid dependent alert wristbands
- Pre-order: Transitions folder for advice on adolescence and adulthood



Duchenne  
UK

Collect or order today for  
FREE

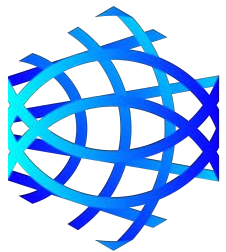


# Learning, behaviour and wellbeing in DMD: Working with school and college breakout session



Nick Catlin

Director/ SEN Advisor



**Duchenne  
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Janet Hoskin

Associate Professor UEL



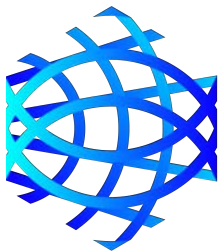
# Context

We are the largest funder of DMD medical research in the UK

To date, we have:

- ❖ Committed ~ **£ 13M**\* to preclinical and clinical research and research infrastructure
- ❖ Funded **69** research projects
- ❖ Worked in partnership with the **biggest industry players** in DMD therapeutic research

\* Since 2013



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# Our research goals

We are committed to drive research to find transformative treatments and technologies for all stages of DMD, through:

- Preclinical and clinical trials
- Devices & technologies

**Funding the  
best projects**



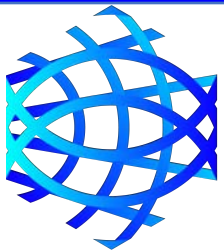
- Access expertise
- Leverage funding
- Influence priorities
- Drive change

**Collaboration  
&  
partnerships**



- Invest and grow DMD research infrastructure
- Knock down barriers and challenges in DMD therapeutic development
- Increase knowledge access and sharing

**Enabling the  
research**



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# Director of Research, Alessandra Gaeta

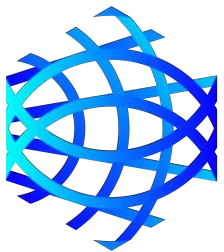


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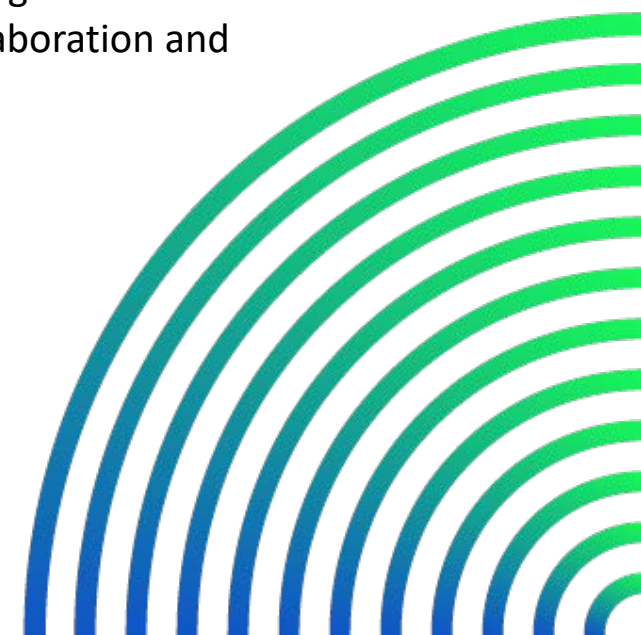
Pharmacy Degree and PhD in Medicinal Chemistry; post-doctoral Maplethorpe Fellowship Award for in pharmaceutical education and excellence in research at King's College London

Experience of preclinical, translational research, through to early phase clinical development

Led on the creation of high-profile, national R&D programmes to accelerate innovative treatments to the clinic, through collaboration and innovative funding models



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# Achievements to date: examples of research projects in key areas of focus



The first UK patient ever dosed with a **gene-therapy**

**TAM-DMD**

The largest **drug-repurposing** trial in DMD, which is creating invaluable insights for the community



Supported the early-stage clinical development of the first **steroid alternative** drug, now at late-stage clinical development and showing promise



Leveraged over £1M funding from People's Postcode Lottery to drive the development of a 'Dream Wheel-Chair', specifically designed with and for DMD patients



# Our projects to address some of the key challenges in **DMD gene therapy R&D**



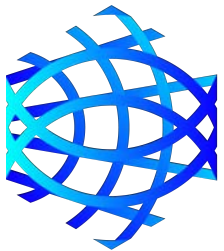
Exploring approved drugs to modulate the **body's natural immune responses** to viral vectors, to allow for repeated dosing of gene therapy



Investigating **alternative delivery mechanisms for gene therapy** which could carry a larger construct, enable repeat dosing and promote specificity to the muscle



Looking at the **body's innate immune responses** and stopping it from mounting a response to an AAV vector



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# Examples of projects in the pipeline

**Power  
suit**

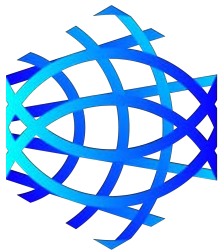
**Assistive wearable device** to enhance upper body function and strength



Pioneering research programme evaluating the use of **muscle stem cells** in the regeneration of muscle tissue

aparito

**Digital, video app** developed for and with people with DMD for home based physical assessment



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# Hydrotherapy clinical trial funded by Duchenne UK

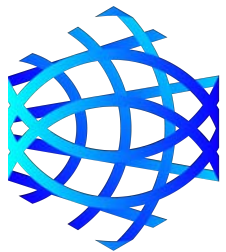
**What:** Clinical trial to prove impact of hydrotherapy

**Where:** Derian House Children's Hospice, and a second North West site TBC

**Who:** 44 boys and young men age 6+ and not receiving daytime ventilation

**Duration:** 24 months

**How to apply:** Sign up to the DMD Hub for updates [dmdhub.org](https://dmdhub.org)

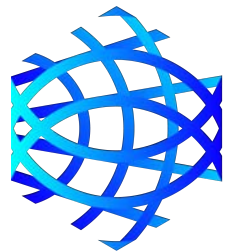
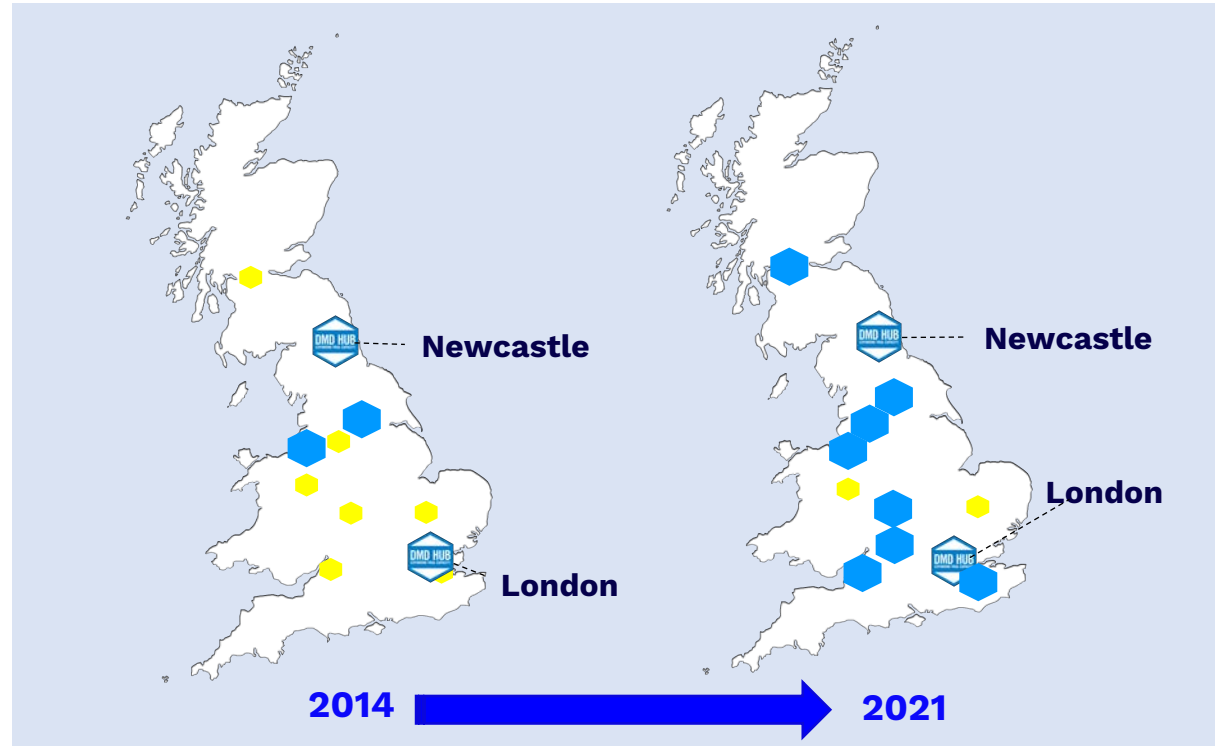


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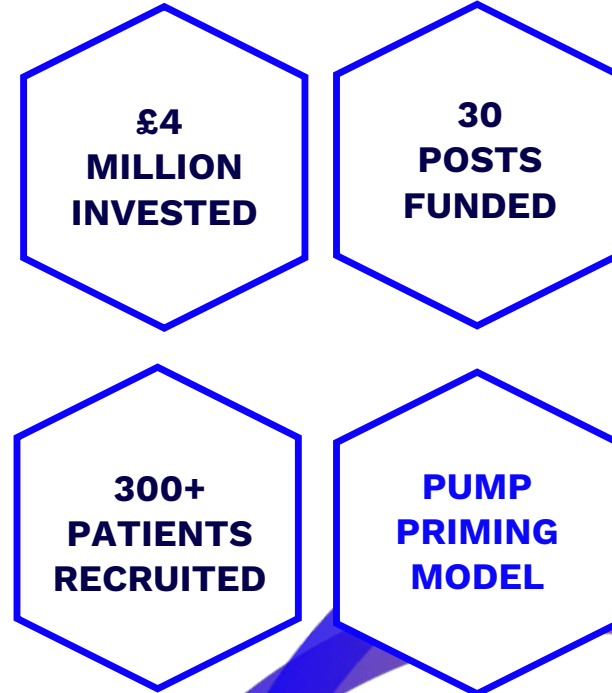




# Achievement to date: clinical research infrastructure



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# A useful resource for the community



[Home](#) / [Clinical Trial Finder](#)

[Patients](#) [DMD Hub Sites](#) [Industry](#) 

[JOIN THE HUB](#)

[SEARCH FOR CLINICAL TRIALS](#)

[DONATE](#)

## CLINICAL TRIAL FINDER

The DMD Hub's Clinical Trial Finder brings together trustworthy and reliable information on all existing and upcoming trials for Duchenne Muscular Dystrophy in the UK.

Our Clinical Trial Finder has been designed for patients and caregivers, to be as accessible and comprehensive as possible. Every trial has information on outcome measures, inclusion criteria and an easy to understand lay summary. You can use search filters to find trials that are relevant to you and download a fact sheet for each trial. The information on each trial has been sourced directly from industry and hospitals and is verified by Duchenne UK and the DMD Hub management team.

# Trial search example

## SEARCH FOR TRIALS

Fully recruited 

☐

Recruiting 

☐

Enrolling by invitation 

☐

Not yet recruiting 

☐

Trial Complete 

☐

Trial terminated 

☐

All

☒

## SEARCH BY LOCATION

All locations



## FILTER BY

Age 

## Treatment type

Mutation specific therapies

☐

## Pfizer - CIFFREO



**A Phase 3 Study to Evaluate the Safety and Efficacy of PF-06939926 for the Treatment of Duchenne Muscular Dystrophy**

### HUB SUMMARY

This study is a phase 3 trial testing the safety and efficacy of Pfizer's gene therapy construct, PF-06939926. It is delivered using an adeno-associated virus, AAV, and carries a shortened version of the dystrophin gene (mini-dystrophin). The treatment will be given by an intravenous infusion.

Two-thirds of the participants will receive the treatment. One-third will be randomly allocated to the placebo arm, but will be able to receive the treatment in the second year, so long as it remains safe to do so.

Please note that patients will need to be on daily steroids for 3 months before screening, to be eligible. They will also be able to be recruited to the trial up until their 8th birthday. For more information about the recruitment process for gene therapy trials, please click [here](#).

**Overall Trial Status**  
Recruiting



**UK Locations**

 [London - GOSH](#)

 [Alder Hey](#)

 [Newcastle](#)



**Trial Sponsor**

Pfizer Inc



**Age**

4-7



**Mutation Specific**

All treatment types



**Muscle Biopsy**

Muscle Biopsy Required



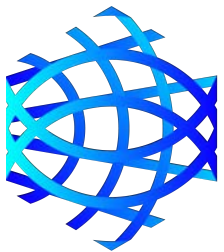
**Ambulation**

Ambulant





**DMD  
CARE UK**  
Best care for all

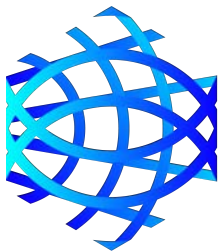
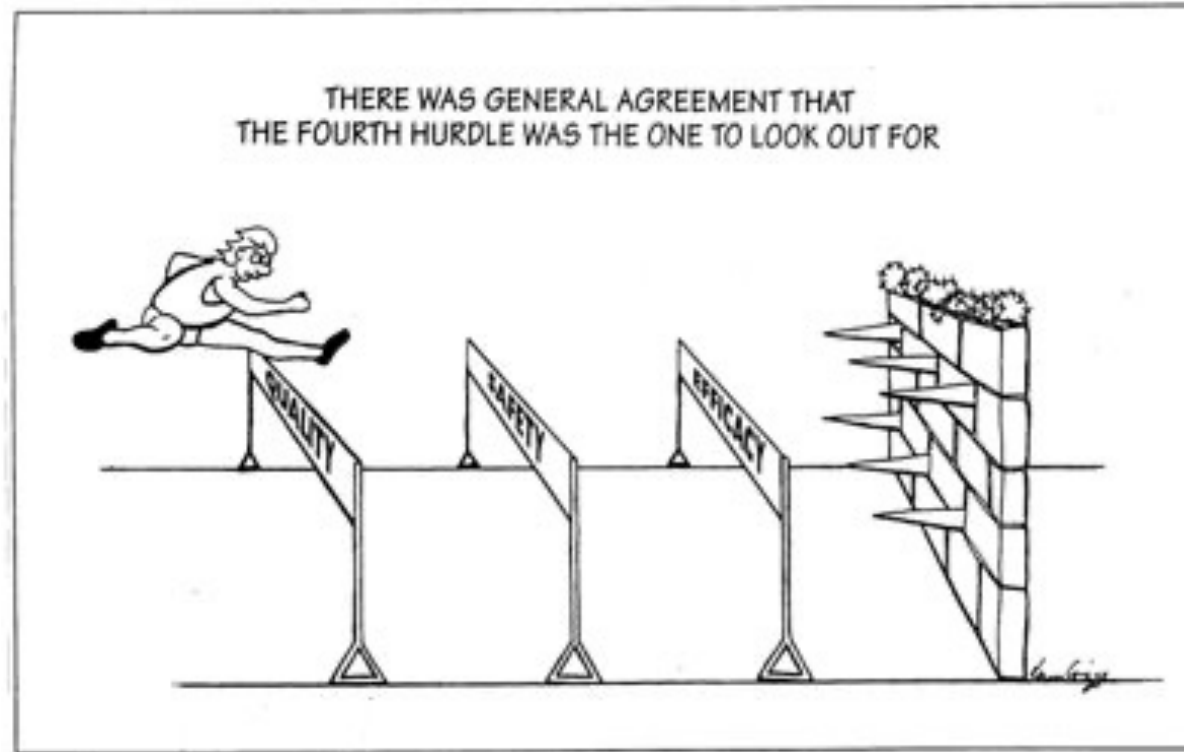


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# REIMBURSEMENT AND ACCESS

The fourth hurdle in drug development



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# HERCULES

DMD: Health Research Collaboration United in Leading Evidence Synthesis

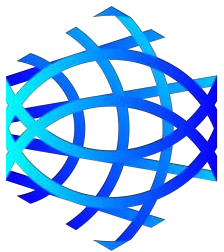


**FLEUR CHANDLER**



**JOSIE GODFREY**

A ground-breaking international collaboration set up  
by Duchenne UK to develop tools and evidence to support Health  
Technology Assessments and reimbursement decisions for new  
treatments for DMD

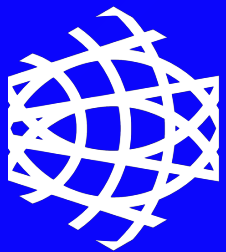


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**THANK YOU**

**FOR TAKING PART IN SURVEYS**



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**If something sounds too good to be true, it usually is**

## The miracle cure

DMD Hub Clinical Trial Finder - all verified clinical trials will be listed on here. Trials must also be listed on [clinicaltrials.gov](https://clinicaltrials.gov)

You should never be asked to pay to take part in any research

You should be advised and should be able to discuss any treatments with your doctor



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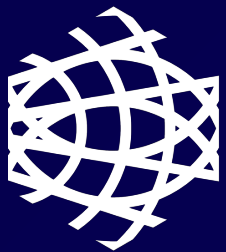
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**SUPPORT**

**NEW FRIENDS**

**COMMUNITY**

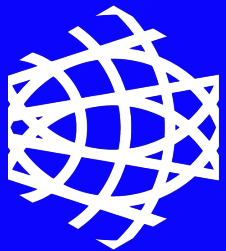
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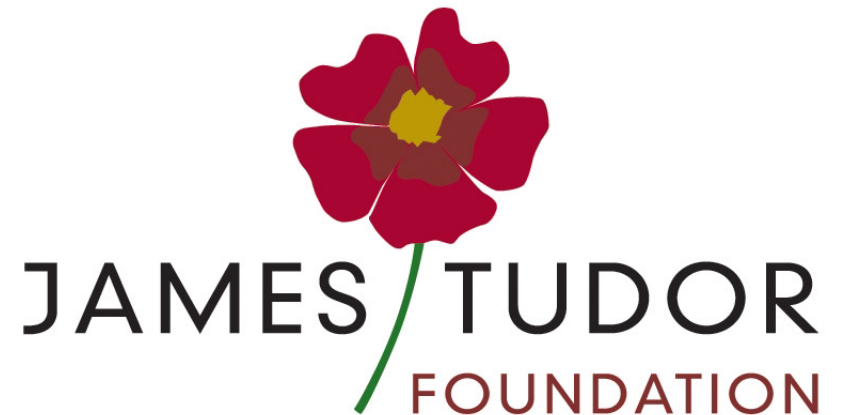
**Duchenne  
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THANK YOU TO THE  
JAMES TUDOR FOUNDATION

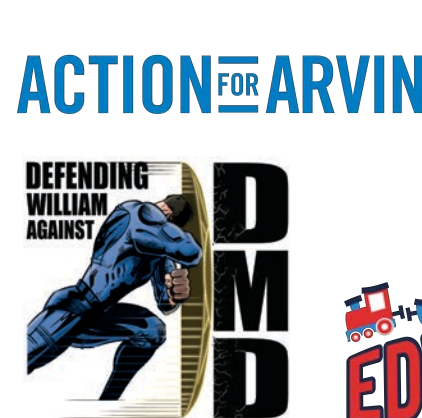
FOR SUPPORTING THIS EVENT




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# Thank you to our Family and Friends Funds - 42




**ACTION FOR ARVIN**




DUCHENNE UK



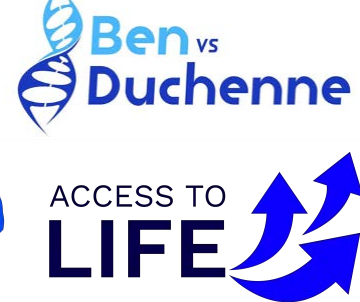
**ACTION FOR ZACH**




Archie's Army  
Helping Fight Muscular Dystrophy




**BACKING JACK**  
to help us beat Duchenne Muscular Dystrophy




Ben vs Duchenne




**CURE4GEORGE**  
Fighting Duchenne Muscular Dystrophy




**HELPING HAYDEN**




**EDWARD STEAM TEAM**




Elliot's Endeavour to End Duchenne




**FOR FELIX**




**FOR FELIX**




DUCHENNE UK  
Join us to end Duchenne  
Help Harry  
Registered Charity No: 114094




**CHASING CONNOR'S CURE**




HENRY'S HURDLES




**HOPE FOR GABRIEL**  
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**STRENGTH FOR STANLEY**




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**Duchenne UK**  
**TEAM DEX**  
FUNDING RESEARCH INTO DMD



team felix



# THANK YOU!

Any questions?



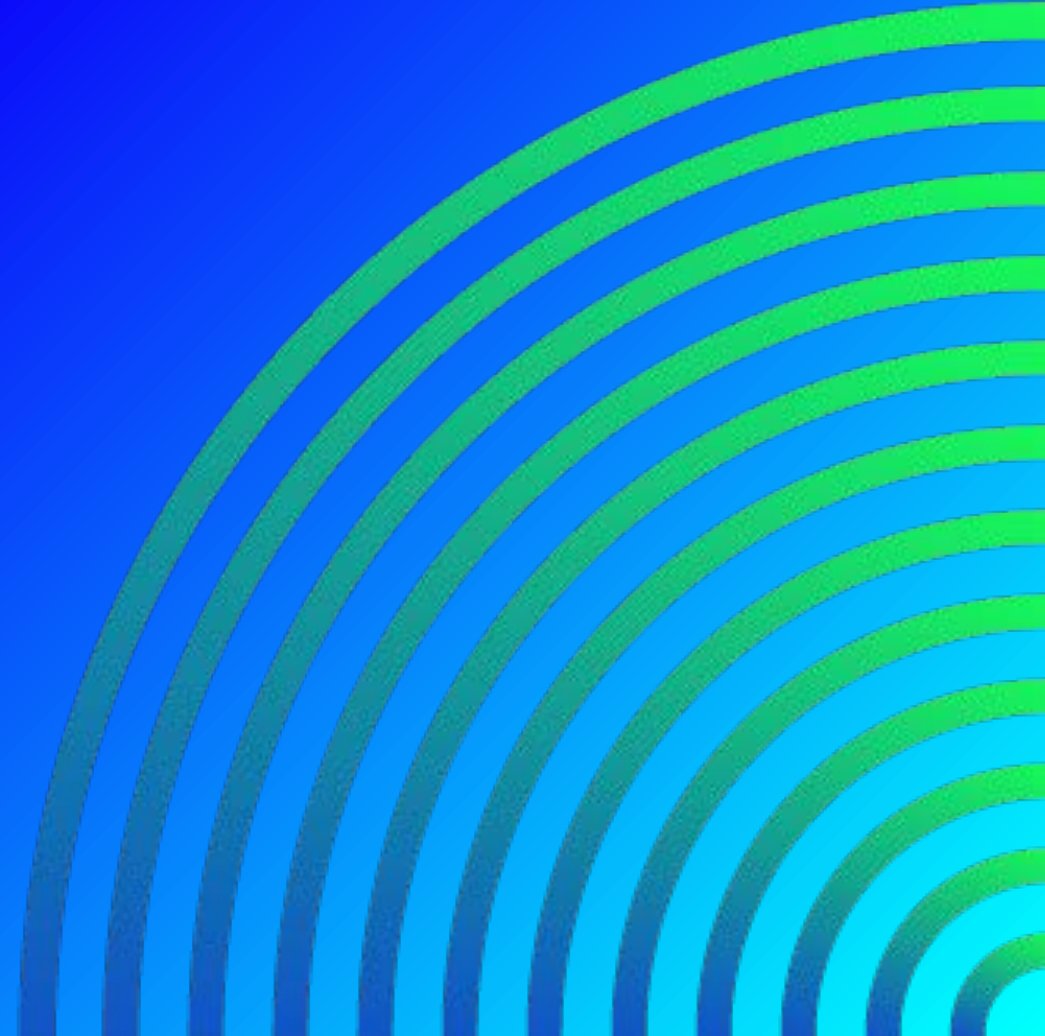
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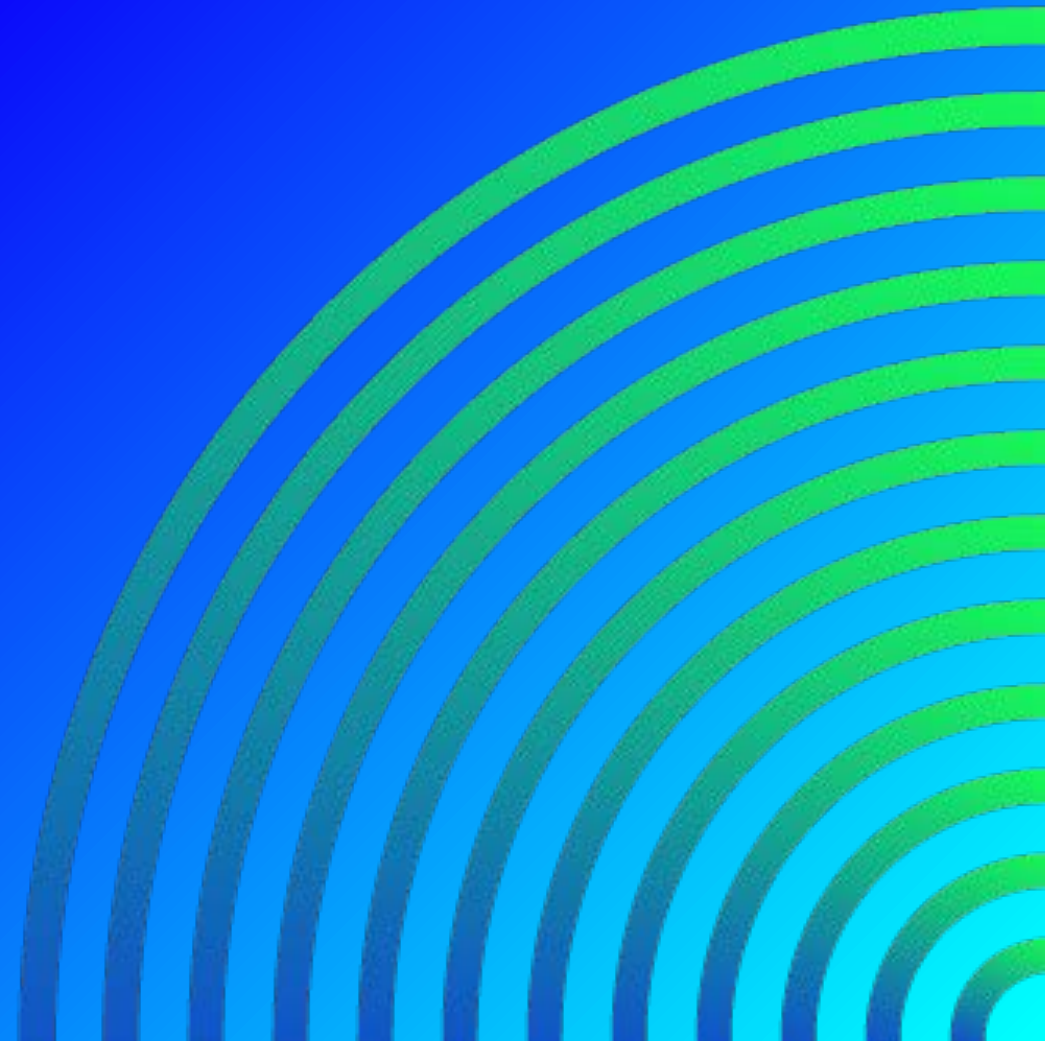
Speed introductions





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Frank Robertson  
Whizz-Kidz  
The DreamChair





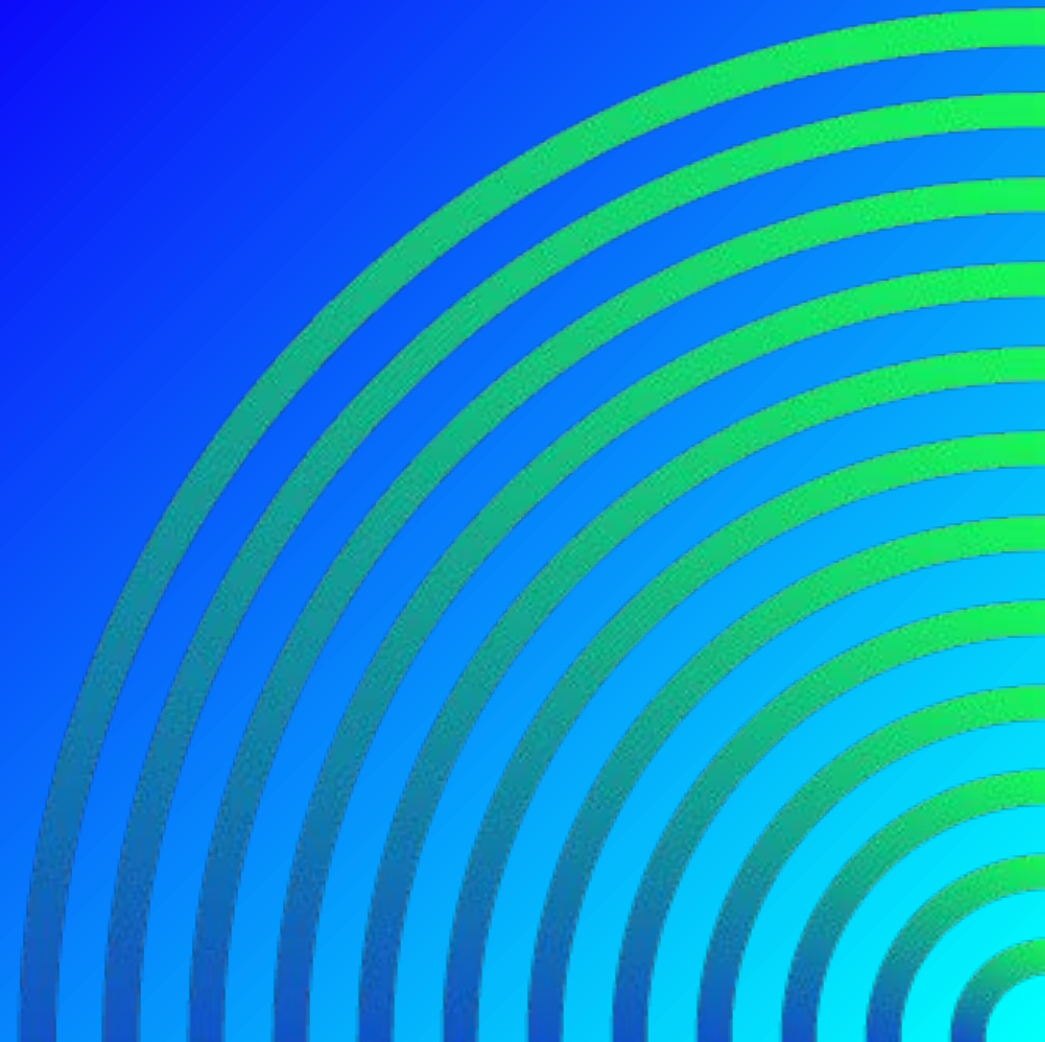
■ PEOPLE'S ■  
POSTCODE  
■ LOTTERY ■





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Frank Robertson  
Whizz-Kidz  
The DreamChair

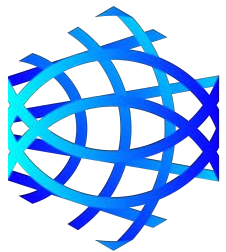


# Learning, behaviour and wellbeing in DMD: Working with school and college breakout session



Nick Catlin

Director/ SEN Advisor



**Duchenne  
UK**



Janet Hoskin

Associate Professor UEL





Duchenne  
UK

Dr Jon Rey-Hastie  
CEO Pathfinder's  
Neuromuscular Alliance

