Welcome to our Parent Information Day

Saturday 2\textsuperscript{nd} October 2021
An introduction to newly diagnosed and first-time parents

Alex Johnson & Emily Crossley
Co-Founders of Duchenne UK, DMD mums
Thank you for coming!

Eighty percent of success is showing up.

Woody Allen
Alex Johnson & Emily Crossley
Alex Johnson, Co-Founder
Emily Crossley, Co-Founder & CEO
Format of the day

Morning

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

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

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

Pie & Ale drinks reception
Balancing life after DIAGNOSIS
How do you prioritise information?
First steps

TAKE CARE OF TODAY

TAKE A BREAK

TAKE CARE OF YOURSELF

WHERE IS THE CARE?
You are your son's best advocate
YOU WILL FIND HERE

SUPPORT

NEW FRIENDS

COMMUNITY

KNOWLEDGE & UNDERSTANDING
Family and Friends Funds - 42
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
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

Collect or order today for FREE
A LOT HAS HAPPENED
HOPE IS HERE
THANK YOU!

Any questions?
Welcome to our Parent Information Day

Saturday 2nd October 2021
Introduction and updates from Duchenne UK

Alex Johnson & Emily Crossley
Co-Founders of Duchenne UK
TODAY IS ABOUT

HOPE AND
COMMUNITY
Alex Johnson & Emily Crossley
FURTHER, FASTER, TOGETHER

RESEARCH - funding groundbreaking medical research

SUPPORT – DMD Care UK, Family folders and materials

ADVANCE – Project HERCULES, DMD Hub, Policy
Ways Duchenne UK can support 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

• Pre-order: Transitions folder for advice on adolescence and adulthood

Collect or order today for FREE
Learning, behaviour and wellbeing in DMD: Working with school and college breakout session

Nick Catlin
Director/ SEN Advisor

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
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

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

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

Funding the best projects

Collaboration & partnerships

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

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

The first UK patient ever dosed with a gene-therapy

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.
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

*Duchenne UK*
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
Achievement to date: clinical research infrastructure

- £4 million invested
- 30 posts funded
- 300+ patients recruited
- PUMP priming model
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.
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.
REIMBURSEMENT AND ACCESS

The fourth hurdle in drug development
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.
THANK YOU FOR TAKING PART IN SURVEYS
The miracle cure

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

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

DMD Hub Clinical Trial Finder - all verified clinical trials will be listed on here. Trials must also be listed on clinicaltrials.gov.

You should be advised and should be able to discuss any treatments with your doctor.
YOU WILL FIND HERE

SUPPORT

NEW FRIENDS

COMMUNITY

KNOWLEDGE & UNDERSTANDING

Duchenne UK
THANK YOU TO THE JAMES TUDOR FOUNDATION FOR SUPPORTING THIS EVENT
Thank you to our Family and Friends Funds - 42
THANK YOU!

Any questions?
Speed introductions
Learning, behaviour and wellbeing in DMD:
Working with school and college breakout session

Nick Catlin
Director/ SEN Advisor

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Associate Professor UEL

Duchenne
UK

DECIPHA
Dr Jon Rey-Hastie
CEO Pathfinder’s
Neuromuscular Alliance