

Welcome to our
Patient Information Day!

Introduction:
Alex Johnson &
Emily Crossley
Co-Founders/CEOs



**“80% of
success is
just showing
up”**



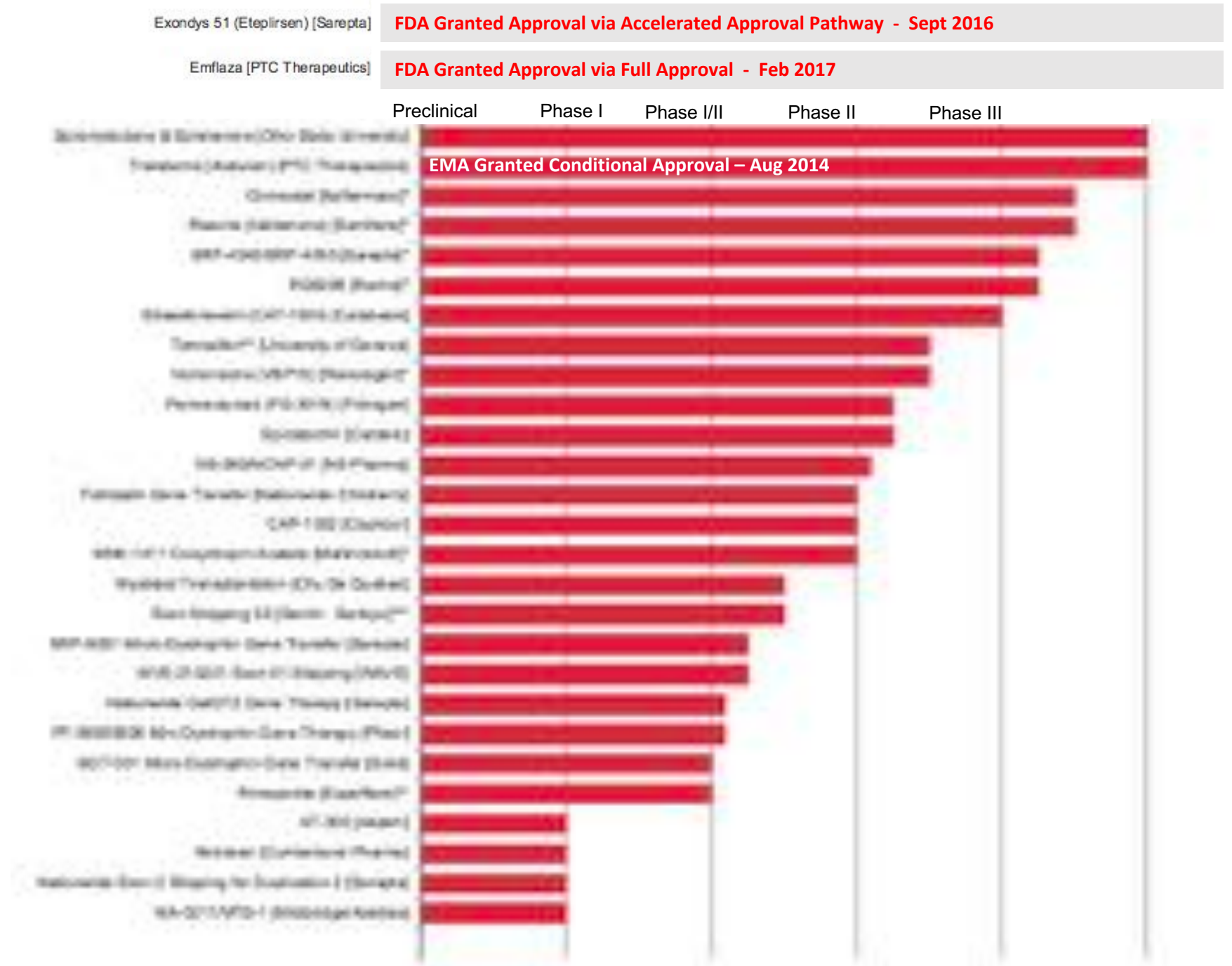
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TODAY IS
ABOUT HOPE



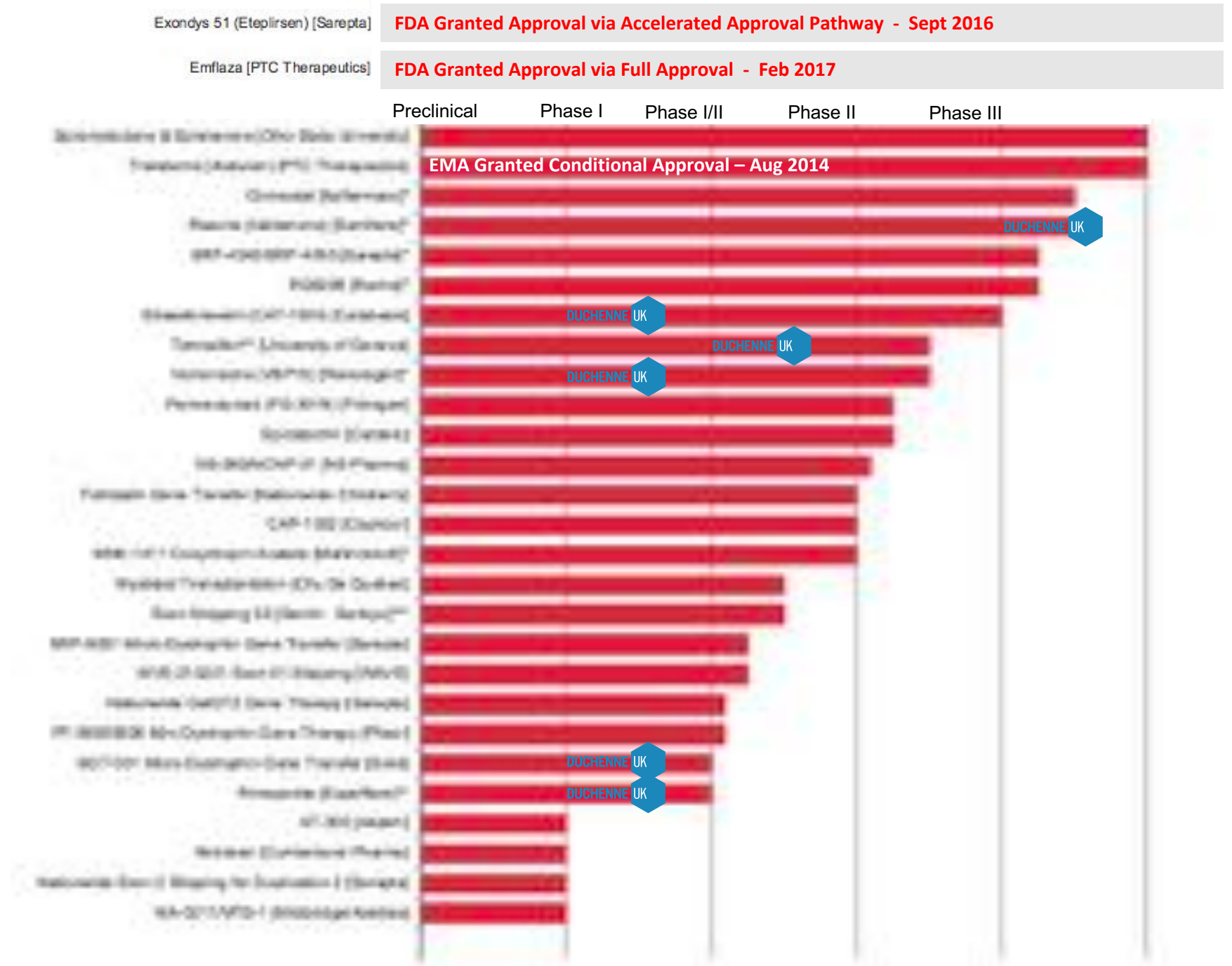
Duchenne Drug Development Pipeline 2019

* = will recruit/recruiting globally
 ** = will recruit/recruiting EU only
 *** – will recruit/recruiting Japan only



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**ACCELERATING
RESEARCH:
DMD HUB, PROJECT
HERCULES & WORK
FUNDED BY
DUCHENNE UK**

**TRIAL UPDATES:
GENE THERAPY AND
STEROID
ALTERNATIVES**

**DUCHENNE
UK**

**BREAKOUT
SESSIONS: GET HELP
FROM NICK CATLIN
FOR EHCP & SCHOOL
SUPPORT**

**WEARABLE SUPPORT
WITH ASSISTIVE
DEVICES**

Duchenne Information Day



THE SOLID SUIT – Assistive Device



STRETCH

Ankle stretch-assist



MOBILITY

Middle body support



REACH

Support and mobilise
upper body parts

Other services we fund



WHAT DO YOU – AS PARENTS WANT
TO KNOW!

TOP FIVE NEW HAPPENINGS

WHAT TREATMENTS ARE COMING
DOWN THE TRACK FOR MY SON

- 1) MICRO-DYSTROPHIN – GENE THERAPY
- 2) TAMOXIFEN – RESULTS DUE 2021
- 3) STEROID ALTERNATIVES: VAMOROLONE & EDASALONEXENT
- 4) EXON SKIPPING DRUGS – WAVE, SAREPTA & NS PHARMA
- 5) RAXONE – WITH THE EMA AGAIN FOR CONDITIONAL APPROVAL – 79 patients accessing it through EAMS



Patients

DMD Hub Sites

Industry



DONATE

SEARCH FOR CLINICAL TRIALS

Join the DMD Hub to
stay up-to-date with
clinical trials in the UK



[Home](#)[About the Hub](#)[News](#)[Donate](#)[Search for Clinical Trials](#)[DUCHENNE UK](#)

CLINICAL TRIAL FINDER

The DMD Hub's Clinical Trial Finder brings together trustworthy and reliable information on all ongoing 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 different measures, inclusion criteria and an easy to understand lay summary. You can download a fact sheet for each trial and use search filters to find trials that are relevant to you. 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.

Read about what clinical trials are about, and why they matter.

And join The DMD Hub, to stay up to date with the latest DMD clinical trial updates.

The DMD Hub is a non-profit making charity with charitable status in England. The charity does not have a charitable status in Scotland or Wales.



Catabasis- POLARIS DMD

Phase 3 POLARIS DMD Trial

Full Summary

POLARIS DMD is a global, placebo-controlled, Phase 3 trial for edasutonexant (CAI-3004). Edasutonexant is an NF- κ B inhibitor which could provide an alternative to steroids. Edasutonexant has been shown to preserve muscle function and substantially slow Duchenne disease progression in the MoveDMD trial.

This trial will evaluate the efficacy and safety of edasutonexant in patients with DMD, and is intended to support an application for commercial licensing of edasutonexant.

Study Number: not on clinicaltrials.gov.uk #

Description by Catabasis Pharmaceuticals

The POLARIS DMD study is a global Phase 3 study to evaluate the efficacy and safety of edasutonexant (CAI-3004) in boys 4 to 7 years-old affected by DMD.

Two boys will receive edasutonexant for each boy that receives placebo and after 12 months, all boys are expected to receive edasutonexant in an open-label extension. Edasutonexant is an oral therapy.

Edasutonexant is a potential foundational therapy that is being developed for all patients affected by DMD. Edasutonexant inhibits NF- κ B, which drives inflammation, fibrosis and muscle degeneration and suppresses muscle regeneration.



Site Status
Not participating

Dr. Longwell
London, United Kingdom
London, United Kingdom
United Kingdom
United Kingdom

The Sponsor
Catabasis Pharmaceuticals

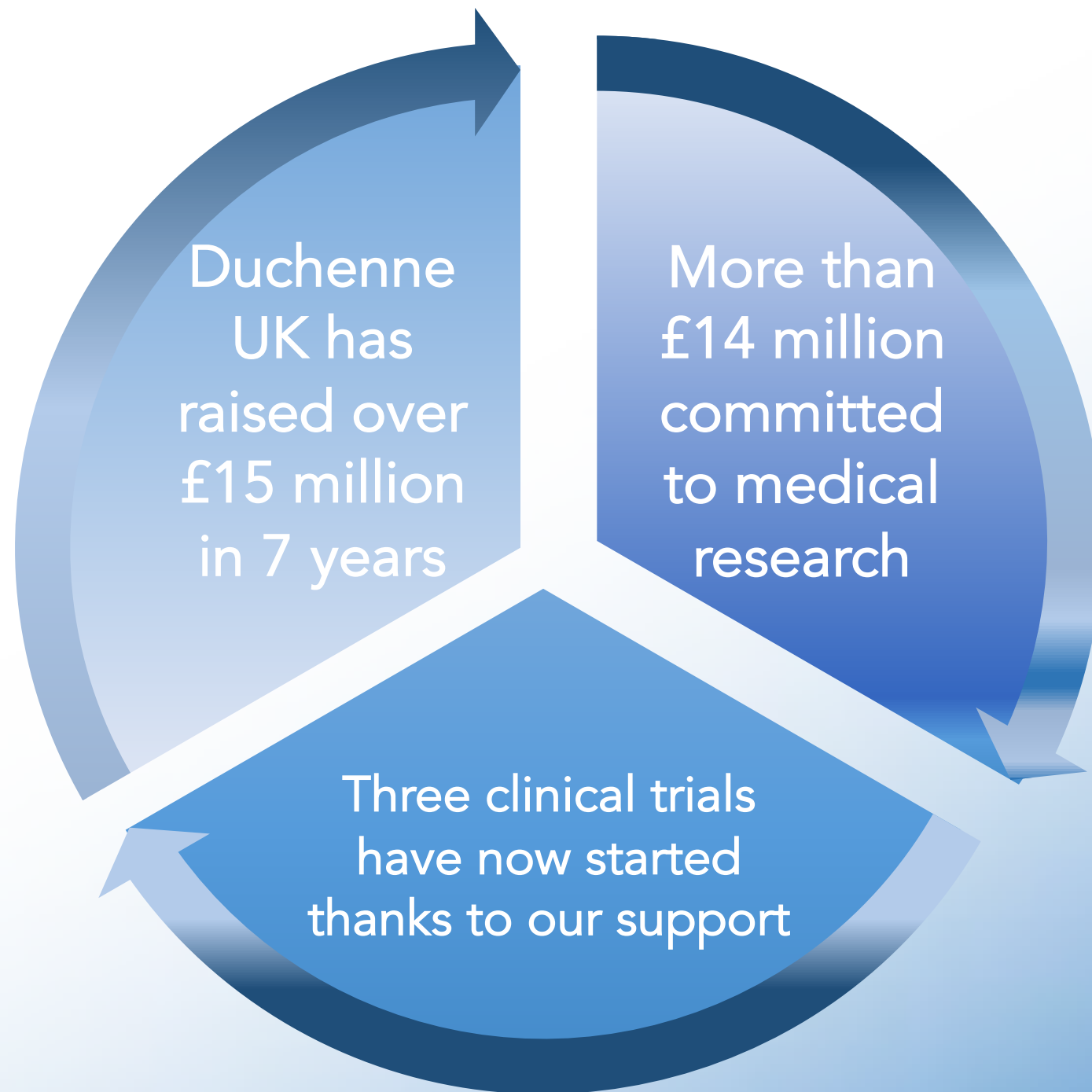
Age
4-7 years

Mutation Specific
Non-mutation specific therapy

Music Therapy
No Music Therapy needed

Site
N/A

Phase





Duchenne UK

INNOVATIVE THINKING TO ADVANCE RESEARCH: EVIDENCE BASED, RESULTS DRIVEN





SOLID
BIOSCIENCES

We helped make Gene Therapy get into the clinic
for Duchenne Muscular Dystrophy!

2014 committed \$5million to Solid Biosciences

2018 First Patient Dosed

DUCHENNE UK



CLINICAL TRIALS & TRIAL SUPPORT

TRIALS:

- REVERAGEN - VAMOROLONE
- SOLID – SGT-001
- TAMOXIFEN TRIAL - TAM DMD

SUPPORT:

- DMD HUB – EXPANDING TRIAL CAPACITY
- ACTIMYO
- APARITO

How else can we have impact?

THE ATTRACTION OF REPURPOSING

- Testing drugs already approved in one condition for efficacy in DMD
- Drugs already approved for use – shortened development timeline compared to a novel compound
- Drugs are often cheap and readily available
- Industry unlikely to fund these trials so we have to

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& TRIAL SUPPORT

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Clinical experience with Tamoxifen for DMD

A compassionate use programme

Dr Talya Dor

Neuropaediatric unit

Hadassah Medical Centre- Jerusalem

17 boys enrolled, 14 still participating



First 3 patients treated with tamoxifen

- Good safety profile over 19 months
- Stable motor function
- Stable or reduced CPK



Repurposing: Tamoxifen as a treatment for DMD



Tamoxifen Trial

- Duchenne UK has committed over £1.5 million to the Tamoxifen trial
- First patient dosed in June 2018
- 45 patients now recruited
- Holders of ODD for Tamoxifen as a treatment for DMD

Duchenne UK invests £1.25 million into new research of treatments for DMD

4 PROJECTS ANNOUNCED



DMD INSPIRE

2019 MAJOR GRANT CALL

- The University of Minnesota was awarded **\$945,654** to support pre-clinical work on **regenerating muscle** in DMD patients with **stem cells**. THE AIM IS TO GET THIS PROJECT INTO CLINICAL TRIALS.
- AGADA Biosciences were awarded **£235,200** to test the impact of two already approved drugs on **protecting muscle** in DMD. AGAIN THE AIM IS TO GET INTO CLINICAL TRIALS.
- Leiden University was awarded **£73,500** to test if **Vitamin B3** (Nicotinamide Riboside) can improve **muscle regeneration** and help retain muscle tissue.
- The University of Geneva was awarded **£199,245** to investigate the potential of repurposed compounds to **regenerate muscle** and **prevent fibrosis**.

What has the hub achieved?

£2M
committed
so far

6 new
HUBsites

19 posts
funded

“ No studies have been turned away since this award. Actually having the staff in place has allowed us to expedite many preparatory processes of assessing new studies for adoption whereas in the preceding two years the team had turned down 10 novel experimental approaches.

Professor Francesco Mutoni Director of the Dubowitz Neuromuscular Centre, Great Ormond Street Hospital for Children.

”

HUB Sites



“

I think the most important development to increase trial capacity in the UK for patients with muscular dystrophy, especially Duchenne muscular dystrophy (DMD) is the DMD Hub which is funded by Duchenne UK. The DMD Hub is a collaborative project between the major DMD clinical trial sites, the NIHR and Duchenne UK.

Professor Volker Straub, Harold Macmillan Professor of Medicine for the Institute of Genetic Medicine at Newcastle University

”

DMD Family Folder

Providing support and guidance to help parents navigate a devastating DMD diagnosis
 500 distributed to families, healthcare professional and teachers



Work is now underway for a folder
 about transitions into adolescence
 and adulthood

Whizz-kidz

move a life forward

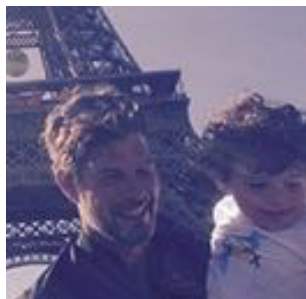
Collaborating with Whizz-Kidz
and the University of Edinburgh
to Develop a DREAM CHAIR

We are a parent led charity...



Patient Advisory Board!

Alastair
Robertson



Alex & Justine
Latham



Ana Chadwick



Andy
Haycock



Anna Clark



Divyesh Popat



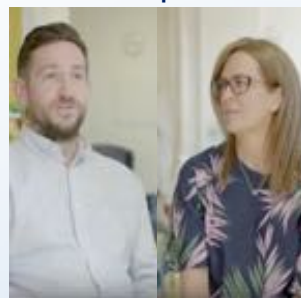
Emma Hallam



Fleur Chandler



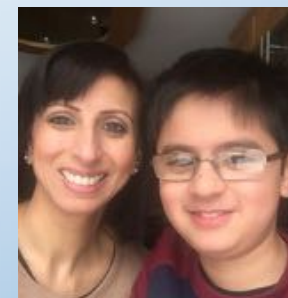
Gareth & Rachel
Halpin



Janet Hoskin



Jaspal Mann



Kerry Shippey



Patient Advisory Board!

Katrina Ruthven



Nick & Kirsty
Ohly



Matt Crawford



Laura Wright



Lisa Kuhwald



Maggie Wellington



Nick Catlin



Pauline Clark



Rebecca Burnett



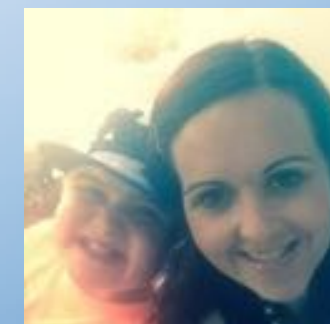
Sejal Thakrar



Tony Levene



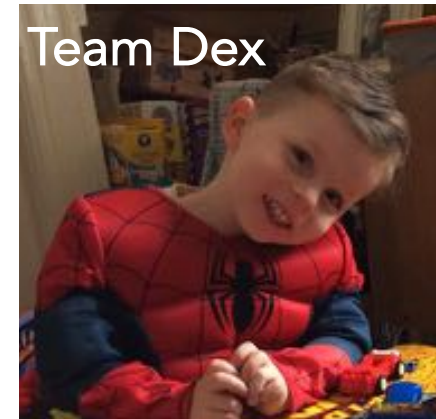
Vici Richardson







ARCHIE'S
MARCH



Team Dex



The Lygo Family Fund



Partner Charities



THANK
YOU

DUCHENNE UK

