Killowen Fundraising Group

Reg. Charity Ref. No. XT 34854

for

Duchenne Muscular Dystrophy

making muscle wasting history

James and Matthew "Looking towards a Future"

www.killowenfundraising.com

A Race Against Time

ACTION TODAY. HOPE FOR TOMORROW.

How can we help?

Please continue to support our various annual events:

- Christmas Fayre at Annett's Garden Centre
- Annual Easter "Miles for Muscles" Run/Walk @ Kilbroney Park
- Fashion Show
- Raffle for Premium Football Final tickets
- Annual Street Collection
- Christmas Raffle
- Sponsored Cycle
- Sponsored football

Donations eligible for Gift Aid Scheme
If you have any fundraising ideas please contact any of the members listed below.

Government Lobby

Members

Brian McAnulty (Chairperson)	Rostrevor
Kathleen Grant (Vice-chair)	Hilltown
Roger Morgan (Secretary)	Warrenpoint
Michael and Aislinn Grant (Joint Treasurer)	Killowen
Ian and Eileen Ryan (Joint Treasurer)	Killowen
John and Tracey Rice (P.R.O.)	Rostrevor
Michael, Bronagh and Martin Grant	Killowen
Niamh Thornton	Warrenpoint
Aidan Murdock	Newry
Helen McEvoy	Hilltown
Margaret McManus	Kilkeel

Approximately 250 boys and 1 girl are living with Duchenne in Ireland today

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Front photo by Sean McAleenan (Macscape Photography) 079 2962 3088

About Killowen Fundraising Group

Update July 2012

In the summer of 2005 the two young Grant brothers from the small community in Killowen Village, on the shores of Carlingford Lough, were diagnosed with Duchenne Muscular Dystrophy.

A small group consisting of friends, neighbours and relatives were formed to raise funds for research into this devastating condition.

Visit: www.killowenfundraising.com

What is Duchenne?

Duchenne is the most common of approximately 60 types of Muscular Dystrophy.

It is a severe genetic muscle wasting disease for which there is (as yet) no cure.

The condition is usually diagnosed in early childhood and affects mainly boys, with rare instances of girls developing it. Children are often in a wheelchair, sometime after 10 years. Muscles continue to gradually deteriorate thereafter, because of lack of dystrophin.

Life expectancy has improved considerably over years due to treatments but mainly because internationally accepted best practice standards of care are being implemented by GP's and local hospitals.

Further information available from info@actionduchenne.org

Aims of K.F.G

- To raise funds which go directly to researchers and clinicians that we believe have the best chance of developing improved therapies for this generation.
- To subsidise areas of essential treatments, equipment, drugs, etc not covered adequately by the Health Board.
- To continue to raise awareness of Duchenne at local and government levels through our website, information pamphlets and lobbying.
- In association with Action Duchenne and Duchenne Ireland, to urge the government to simplify the means of clinical trials for genetic medicines that will bring treatments to market much more quickly.
- To ensure that international standards of care are maintained and that young people with Duchenne have a quality of life and longevity that does not suffer because of lack of funding.

Killowen Fundraising Group Finances

In late 2011 the amount raised by K.F.G for research passed the £300,000 mark.
These funds were allocated as follows:

(I) £200,000 to personalised research into exon-skipping being conducted at the University of Western Australia, Perth
(II) £83,000 to MDEX research into exon-skipping at the Royal Holloway University, London
(III) £24,000 to Action Duchenne (Charity No. 1101971)

Mr John McGinn (John McMahon Accountants) provides annual accounts on a voluntary basis.

K.F.G. are now a registered charity. Donations are eligible for Gift Aid (Tax) Scheme.

Some funds in future will be set aside for essential treatments equipment, drugs etc not covered adequately by Health Board.

Making Muscle Wasting History

Research

Ten years ago very little research into treatments for Duchenne was taking place.
Until recently the only treatment for the condition has been steroids, which can help delay the progression of the muscle damage.

In 2010 there were:

- 4 pre-clinical trials
- 87 animal model experiments
- 10 cell experiments

Many more have been started since. **Our recent funds have been forwarded directly to the James and Matthew Grant Foundation at U.W.A, Perth.** The research has been monitored on behalf of K.F.G by Prof. Kate Bushby (Newcastle-On-Tyne). With her help and funding from Duchenne Ireland, cell strains, including those of James and Matthew, have been sent to the laboratory in Perth.

**Work on assessing the viability of skipping our set of exons is almost completed.
Exon-skipping could be of benefit to 60-80% of boys to a variable degree and drugs for 10-14% of these could be available in next few years but others will take longer. Delays due mainly to the cost of producing drugs and goverment's lack of urgency in fast tracking clinical trials for genetic medicines.**

Hopes for the future

Phase 2 trials are under way with Halo Therapeutic HT-100. This has great potential and may prevent inflammation that leads to muscle damage and promotes healthy muscle regeneration.

Some of the most likely trials to produce treatment in the next few years are Exon-skipping, PTC Therapeutics and Utophin.

It is hoped that a "cocktail" of these will slow down the progress of muscle wasting and continue to extend life expectancy.

If progress at a similar rate as has happened over the last 10 years continues then it is our hope that a cure will be found (probably via stem cell treatment research) and that many with the condition at present will survive to see this day.

Stem cell transplant in mice has proved successful in Italy when healthy muscle cells were made and symptoms of the Duchenne type were alleviated. Much more research is needed in order to apply this approach to humans.

ALSO

A pioneering project at Royal Holloway University of London (Professors I.T. Foster and George Dickson) is looking at a system to produce full length dystrophin. **If this is successful it will treat ALL patients with the condition.** Currently most of the other treatments mentioned above are only suitable for a sub-set of patients.
Nick Catlin at Action Duchenne says "This is the first treatment that could be called a cure... but will take a number of years to bring to market." (March 2012)

We are hopeful that many with the condition at present will survive to see the day when the inevitable cure will be realized.

**At present many Duchenne patients in Denmark have a life expentancy of early 40's.
Hospitals and Health Boards need to be continually reminded of this.**