



FARA NEWS

Friedreichs Ataxia Research Association of Australasia newsletter

Issue 1 April 2008
www.fara.org.au

FROM THE PRESIDENT

Friedreich Ataxia sufferers and families may have wondered why our information to them has not been updated in the past year. However, we are aware that you grow frustrated at hearing about what is happening in laboratories when what you want to know is when all this research is going to be relevant to my condition and when can I expect to hear about drug trials with humans. Frustration is a natural consequence of having to wait upon drug development because time taken seems to be endless. We just have to understand that novel drugs have to be trialled initially upon healthy volunteers and animals subsequent to them being found to have validity through laboratory trials with mice. This process can take several years and then has to pass through a bureaucracy that ensures the safety of individual sufferers who participate in the eventual trials. Well, the time has arrived when we can claim that the research that has been laboriously undertaken over the past decade is at last beginning to translate into practice.

Contents

FROM THE PRESIDENT	1
Scripps Research Team Reverses Friedreich's Ataxia Defect in Cell Culture	2
FARA(A) Friedreich Ataxia National Research Review Meeting And FARA(A) AGM	4
Prenatal Diagnoses of Friedreich Ataxia	7
A note from the Treasurer:	7
Dennis Family Homes donates proceeds to FARA(A)	9
GO THE TAN FEBRUARY 2008	10
GOLFFFA GOLF DAY DECEMBER 2007	10
YAFFA BALL 2006.....	11
OTHER FUNDRAISERS	11
DONATIONS.....	12
2006 – 2007 Financial year	12
DONATIONS.....	13
2007 – 2008 Financial year	13
FARA Scientific Advisory Committee	14
Contact FARA	15

You will see from the newsletter that the time has arrived when we can claim that that the research that has been undertaken laboriously over the past decade is at last beginning to translate into practice. The Friedreich Ataxia Research Association (Australasia), has been diligently pursuing funding and applying this to

relevant research in Australia and New Zealand. Each year in November the association, through its Scientific Advisory Committee, commits significant funding to research groups that are heavily involved in work with antioxidants, iron chelators, stem cell research and gene therapy. While we have met with disappointments in some areas, particularly where human trials have been delayed or abandoned, the research that is occurring here and throughout the world gives us cause for great optimism. We can say with confidence that the therapeutic period for the treatment of Friedreich Ataxia is now with us. A mere twenty years back the number of researchers into FA could be counted on one hand. The giant leap forward in the numbers now involved is a reflection of the interest that the disorder has become and the significance it could well have for other degenerative diseases.

We have attempted in this edition to present the most up-to-date research worldwide. Readers will note the variety of this work including that being undertaken in Australia. The latter is in no small measure due to the wonderful response from our many donors who are listed here. The executive of our organization has worked diligently over the past several years since the establishment of our Australasian concept to ensure that our sufferers have reason for optimism about the future. The principal driving force has been our Executive Director, Varlli Beetham, who has worked tirelessly and successfully to raise the funds which have made our local research possible. We are also fortunate that in the Friedreich Ataxia Clinic in Melbourne we have probably the most sophisticated support system in the world.

We continue to rely on the support of all families if we are to maintain our present momentum. Through your

contacts we would hope that our fundraising continues to prosper and that the relevant therapies now being investigated become a reality through appropriately funded human drug trials.

*Peter Rousch AM
Emeritus Professor*

Scripps Research Team Reverses Friedreich's Ataxia Defect in Cell Culture

Newly Developed Compounds Activate Silenced Gene Responsible for Debilitating Disease

LA JOLLA, CA, August 18, 2006

Embargoed until Sunday, August 20, 2006, 1 PM Eastern Standard Time –

A team from The Scripps Research Institute and the University of California School of Medicine has developed compounds that reactivate the gene responsible for the neurodegenerative disease Friedreich's ataxia, offering hope for an effective treatment for this devastating and often deadly condition.

The results of the research are being published on August 20 in an advanced, online version of the journal *Nature Chemical Biology*.

In the new study, the researchers tested a variety of compounds that inhibited a class of enzymes known as histone deacetylases in a cell line derived from blood cells from a Friedreich's ataxia sufferer. One of these inhibitors had the effect of reactivating the frataxin gene, which is silenced in those with the disease. The researchers then went on to improve on this molecule by synthesis of novel derivatives, identifying compounds that would reactivate the frataxin gene in blood cells taken from 13 Friedreich's ataxia patients. In fact, one of the compounds the researchers tested produced what amounted to full

reactivation of the frataxin gene in 100 percent of cells tested.

"This is marvelous," said Joel Gottesfeld, Ph.D., a professor in the Scripps Research Department of Molecular Biology and leader of the project. "I've met the parents of many children affected with the disease and some of the patients and it would be just a dream to be able to help them." "Dr. Gottesfeld's work holds tremendous promise of real therapeutic benefit for Friedreich's ataxia patients," said Ron Bartek, president of Friedreich's Ataxia Research Alliance (FARA). "This discovery appears to be our only near-term prospect for significantly increasing transcription of the frataxin gene. FARA is pleased to have been able to support this important work."

A New Theory

Researchers are still working to understand the reasons the triplet repeats prevent transcription of the frataxin gene, although the gene itself remains intact. Although other theories have been proposed, the new research supports an explanation known as the "histone code theory."

Histones are proteins that are the chief constituent of the nucleosomes around which DNA is wrapped in cells. The new theory suggests that histones must contain certain chemical cues, including acetyl groups, for nucleosomes to assume the formation that allows the genes they package to be expressed.

One idea suggested by the paper's authors is that the triplets cause an unusual DNA structure that attracts proteins such as histone deacetylases (HDACs), removing critical acetyl groups from the histones, packaging the histones in an inactive form called heterochromatin, and ultimately leading to silencing of the frataxin gene.

Based on this theory, Gottesfeld and his colleagues began looking for compounds that might block the

HDACs with the goal of reactivating frataxin production. The researchers were able to draw from a range of commercially available products because many HDAC inhibitors have been developed as tools for molecular biology research and as potential cancer treatments.

Though Friedreich's ataxia impacts neuronal and muscle cells, these are not readily available for research. So, the group instead worked with white blood cells, or lymphocytes, which are easily obtained from blood samples and can be prevented from dividing, making them a suitable proxy. Experiments revealed that one HDAC inhibitor, called BML-210, did in fact reverse the heterochromatin formation in cultured lymphocytes from Friedreich's ataxia patients and increased the production of frataxin messenger RNA (mRNA), a precursor to production of the protein, although not sufficiently to bring protein production to normal.

Next, the researchers chemically modified BML-210 to produce a variety of analogs whose effects on the cells were then tested. One class of analogs produced a two to three-fold increase in frataxin transcription amounting to full reactivation of the frataxin gene in an astonishing 100 percent of cells from 13 Friedreich's ataxia sufferers. "They never failed," said Gottesfeld. Such therapeutic reactivation of a silenced gene has only been achieved for a handful of other diseases. Importantly, the team's HDAC inhibitors have also proven uniformly non-toxic to the lymphocytes and do not significantly affect cell growth rates.

Ongoing animal studies also have not revealed any toxicity. If the results of animal testing remain positive, said Gottesfeld, the HDAC inhibitors could enter human trials as a Friedreich's ataxia treatment in as soon as 18 months' time.

"FARA is truly excited about rallying additional support," Bartek noted, "so

as to move Dr. Gottesfeld's compounds through preclinical drug development and into clinical trials just as quickly as possible."

Other Friedreich's ataxia treatments under development are largely aimed at better treating symptoms of the disease, rather than grappling with the root cause of low frataxin production. Additional compounds that increase expression of frataxin protein have also been developed, but are likely too toxic for therapeutic use. Gene therapies or stem cell treatments may eventually be available to increase frataxin production, but such options are probably many years off. "Our small molecules offer a therapeutic approach to pursue in the near term," said Gottesfeld.

In addition to Gottesfeld, authors of the new study, entitled "Histone deacetylase inhibitors reverse gene silencing in Friedreich's ataxia," are David Herman, Kai Jenssen, Ryan Burnett and Elisabetta Soragni, also from Scripps Research, and Susan Perlman from the University of California School of Medicine, Los Angeles.

In addition to funding from FARA, the study was supported by grants from the National Institute of Neurological Diseases and Stroke of the National Institutes of Health.

FARA(A) Friedreich Ataxia National Research Review Meeting And FARA(A) AGM

Monday 26th November, 2007
Dean's Ganglion, University of Melbourne Faculty of Medicine
4th Floor, 766 Elizabeth St, Melbourne

FARA held its 3rd annual scientific national research review meeting and AGM in Melbourne on Monday 26th November, 2007.

The one day meeting seeks to serve the following purposes:

- A. All FA-related research groups in Australia to meet and hear progress reports on current research projects and findings.
- B. The FARA(A) Scientific Advisory Panel (SAP) members to meet all groups and enable questions to be answered to better consider grant applications for research, student or trial support.
- C. To facilitate discussion between clinicians, scientists, patients and support group members.
- D. To enable both the opportunity for discussion from a broader opinion group, and for any potential new research opportunities / collaborations, particularly those that involve Australia-wide or international collaborations, to be discussed.

Researchers attended from Queensland Institute Medical Research, University of Sydney, Murdoch Childrens Research Institute and University of Melbourne. Members of FARA(A)'s Scientific Advisory Committee were present, as were board members from FARA(A), FA Association (Victoria) and FAN (Qld), also FA patient representatives from South Australia, Victoria, NSW, Queensland and New Zealand.

FARA(A) President Emeritus Professor Peter Rousch opened the meeting and introduced Professor Bob Williamson as Chair of the meeting while also representing FARA(A)'s Scientific Advisory Committee (SAC).

Progress reports were received from:

1) University of Sydney, Prof Des Richardson

The FA research team have 2 main objectives, firstly to develop iron chelators for the removal of iron overload in the mitochondria, which causes oxidative stress, protein

damage and results in cardiomyopathy. Secondly, to better understand how frataxin works.

In the search for appropriate iron chelators, Prof Richardson has found that PCIH iron chelators are relatively lipophilic (high permeability, leading to oral effectiveness), effective at extracting iron from cells & inhibiting iron uptake, and are orally effective in animals. They believe that it shows potential as an iron chelator for the treatment of FA.

They are using a frataxin conditional knockout mouse which closely resembles the cardiomyopathy in human FA. There is a manifestation of FA pathology beginning at 6 weeks of age, failure to gain weight and an early death at about 10 weeks of age. The post-mortem examination shows cardiomyopathy, myocardial fibrosis and myocardial iron loading.

Future plans are to test the chelators on a neurological mouse model.

2) **Murdoch Childrens Research Institute, Associate Professor Martin Delatycki**

Martin spoke of the work being conducted at the FA Clinic, Monash Medical Centre.

Martin also highlighted the clinical trials that are being conducted or planned locally and internationally. These overseas trials included:

Idebenone Phase I completed – promising results
Phase II planned
– US, Europe

EPO Phase I/II open label completed – promising results

Deferiprone Phase I/II open label completed – promising results

Phase II/III planned – US, Europe

In Australia, a trial is being planned for EPO: multi centre – sites in Melbourne and Rochester (New York), and perhaps other sites? placebo controlled phase II/III trial

2 doses - EPO vs placebo
12 month study with a 6 month interim analysis

Ofunding from Edison Pharmaceuticals and FARA (US)
Current status of the trial: protocol or trial design is being finalised

paperwork is being prepared for submissions to the regulatory authorities and ethics committees

Murdoch Childrens Research Institute, Dr Joe Sarsero

Joe spoke of his laboratory team's work on pharmacological therapy for FA.

This is based on the premise that there is an overall correlation between GAA expansion length and transcript levels, amount of residual frataxin, age of onset and severity of the disease.

Based also on the fact that heterozygous carriers with approximately 50% frataxin levels are asymptomatic, the hypothesis is that any increase in frataxin levels may be beneficial to patients, and several fold increases may slow or stop disease progression. It's believed pharmacological approaches could be adopted to

achieve higher expression levels.

High throughput screening has identified 18 compounds out of 2000 (0.9%) that elicited a greater than 3 fold increase in EGFP expression and were identified as primary screening hits.

The plan is for EGFP to be measured by flow cytometry. Then tests will be conducted on lymphoblast and fibroblast cell lines derived from FA patients. An assessment in a cell culture model of FA will then assess the ability to functionally compliment overall defects inherent in FA. A preclinical evaluation of lead compounds will take place in FA mouse models. Then human trials will be possible.

3) Queensland Institute Medical Research, Dr Nuri Gueven

Dr Nuri Gueven and Martin Lavin have recently been awarded funding from FARA (US) to undertake a study using CTMIO. This is being conducted in partnership with Mark Pook (London). Humanised GAA repeat mouse models are being used (Pook) to evaluate the protection of FRDA cells by CTMIO. The study is for a 3 – 6 month duration. The rotarod will be used to measure neuro behaviour. Measurement of oxidative stress in the cerebellum and heart will be established using DNA damage response markers.

Future plans are to develop and test CTMIO based compounds with equal or better activity and

commercial pharmaceutical interest.

4) University Melbourne, Dr Mirella Dottori

Mirella explained that a curious characteristic of FRDA is that certain cell types are more vulnerable to the disorder. For example, within the nervous system only specific neurons, such as sensory neurons, degenerate. The basis for this selective vulnerability is still unknown. Data that aid our understanding of the molecular pathways within specific cell types that are known to be affected by the disorder, especially in human cells that carry the trinucleotide repeat expansion within the *FXN* gene, will help to explain why sensory neurons are affected to a greater extent than other neurons.

The aim of this research is to establish a robust and well-characterized differentiation protocol to derive peripheral sensory neurons (of the type found in dorsal root ganglia, DRG) from hESC.

Setting up a DRG culture system will be of value to establish *in vitro* models of FRDA, and to investigate possible cell replacement therapies for treating FRDA.

Following the completion of the presentations, a discussion on upcoming clinical trials took place. Parents of FA patients clearly identified their need for faster access to clinical trials, and identified their desire to support the trials to get them happening quicker. Parents identified their desire to be closer to the Ethics Committees during times of critical decision making.

Nuri Gueven noted that this was a feeling shared amongst parents of AT patients that he closely deals with.

At the completion of the meeting there was a feeling that real progress is being made by all research groups, and that there is real cause for hope that we are getting closer to treatments for our patients. Collaboration between the groups has begun and is producing results.

Members of FARA(A)'s Scientific Advisory Board commented positively on all research groups and recommended favourably that FARA(A) continue to support these groups financially.

Prenatal Diagnoses of Friedreich Ataxia

We all know that Friedreich ataxia is often not diagnosed until an affected child is a teenager, and by then many couples either cannot have more children, or have decided that their family is complete. However, in a few cases couples who want children may know they are at risk because they have had a child with FA when still young themselves, or because they have relatives with FA and have been tested. At present, the FA prenatal gene test is available using chorion villus sampling at about eleven weeks of pregnancy, and if the child will be affected the couple face the hard choice of whether to continue with the pregnancy. Some couples choose not to have the test, and leave the outcome to chance.

I am writing this note to mention that there is a new test available where IVF is used to create several embryos (using eggs from the mother and sperm from her partner), and DNA from each embryo is tested to see if the embryo is affected by FA. Only

embryos that are unaffected are placed in the womb, guaranteeing that any child that is born will not be affected by Friedreich ataxia. Embryos that would be affected can either be discarded or used for research into treatment for the disease.

This test is available in Australia for many inherited diseases, such as cystic fibrosis and thalassaemia, and has now been worked up for FA by at least one centre. The procedure has the disadvantages of IVF (both the eggs and the sperm have to be collected, the embryos have to be grown and one replaced after the test, the couples have to use barrier contraception during the IVF cycles and there may be some time before a pregnancy is achieved). However, it guarantees that any successful pregnancy will give a child who will not be affected by FA, without the trauma of an abortion. Unfortunately, it is only available through the private IVF clinics, and costs about \$3000!

I know this is not for everyone; it is a personal decision which couples have to take for themselves. However, if you are a couple who is at risk of having a child affected by FA, or know such a couple, you can talk to your genetic counsellor about preimplantation genetic diagnosis (PGD), or to Martin Delatycki at the clinic, or drop me an Email (r.williamson@unimelb.edu.au) if it is more convenient.

Bob Williamson, 5th February 2007

A note from the Treasurer:

I often come across the perception that FARA is a large organisation and that its executives and board members must be well remunerated, maintaining an office in Melbourne's CBD, jumping on planes and staying in flash hotels to attend meetings etc.

In fact, FARA has one paid employee, Varlli Beetham, who, for many years,

was virtually a full-time but **unpaid** employee. Our office in Melbourne, in addition to phones, postage and general office support, continues to be provided by the Beetham family at no cost.

The various board members who so readily jump on planes to attend meetings generally do so at their own expense. FARA reimburses the costs for scientific researchers to attend these important meetings, but most of the board members pay their own way. Even our Scientific Advisory Panel members, such as Prof Bob Williamson, who are so generous with their time, often refuse to accept reimbursement of their costs.

The board members are all parents of FA'ers, so in addition to the daily challenges that FA brings for any family, they also have to take time off work and are seriously 'out-of-pocket' for their work for FARA.

It is a credit to Varlli and everyone else involved that FARA gives the impression of being a large corporate organisation. It allows us to "punch above our weight" when it comes to research, and ensures that every dollar that is donated is put to the best use possible.

FARA is focused on finding effective treatments or a cure for Friedreich's Ataxia and by necessity must be single-minded in its approach. We will always try to help with fundraising activities, but FARA can't always meet the expectations of families looking for support (even with fundraising activities, our support is fairly limited and we have to rely on whoever is organising the activity to be largely self-sufficient).

We are very fortunate to receive so much support from so many people and organisations, and are grateful to the many families and friends of FA'ers

who contribute so much. I especially want to thank the board members and their families for their contributions which come in many forms.

Mike Dwyer
Treasurer



Dennis Family Homes donates proceeds to FARA(A)

In a major push to support research funds for Friedreich Ataxia, Dennis Family Homes have donated the proceeds from the construction and sale of a double storey, four bedroom home to FARA(A).

“We are thrilled that through the sale of this magnificent double storey home at Manor Lakes we are able to donate \$34,693 to FARA to help find treatments and hopefully a cure for this devastating disease,” says Ms Adele Levinge, Executive Director, Dennis Family Corporation.

The FARA charity home was constructed with the generous support of not only the Dennis Family but many leading building industry suppliers who donated products and time to this special project.

FARA(A) would like to sincerely thank the following suppliers for their generous support of the FARA charity home:

A&L Windows	Funnel Design	Truss-Rite
AM Electrics	Galintel	Universal Safety
Avro Steel Roofing	Impact Blinds	Systems
Bonaire/Vulcan	J & J Heating	Wattyl Paint
Boral Bricks	Kefton Ceramics	Westwood First National
Boral Roof Tiles	Kimmark Electrics	Zanette Home
Bowens	Meridian Pest	Improvements
Buildsafe Australia	Management	Canterbury
Carpet Call	Peuker & Alexander	Windows&Doors
Central Scaffolding	Smorgon Steel	ColSmith Garden &
Clean Cut Site Clean	Steel-line Garage Doors	Building Supplies
Craig Gowling Stairs	Timberlite	Systems Management
Crystal Interior Concepts	Tradelink Melbourne	Print Solutions

GO THE TAN FEBRUARY 2008

GO THE TAN was held at the famous Tan track in Melbourne on 1st and 2nd February, 2008, for its fourth consecutive year. With the continued support of major sponsor Australia Post, and unprecedented media coverage, a fantastic turnout of supporters over the 2 days helped to generate \$20,000 for FA research.

This year almost 200 Australia Post staff entered the Australia Post challenge, while media support in the lead up to the event was unprecedented. The event was again expertly MC'd by sports reporter Michael Roberts and the 2008 Celebrity Challenge included Nova 100's Dave Hughes, triple brownlow medallist Bob Skilton, Melbourne Storm premiership player Robbie Kearns, AFL legend Glenn Archer, sports reporter Tiffany Cherry, Channel 9's Andi Lew, Totally Wild's Natalie Hunter, Jockey Stephen Baster and former AFL players Ang Christou and Ang Lekkis.

A highlight of this year's event was 9 year old newly diagnosed FA sufferer Emma Jones attending with her entire class from St Vincent de Paul Primary School. Emma's Grade 5 class did a lap of the Tan and all finished together in an emotional show of support for their class mate.

GO THE TAN 2008 created enormous media exposure for FA. The event featured on the 6 o'clock news on Channel 9 and 7, while a live broadcast interview with Michael Roberts and Glenn Archer was run by radio station SEN, while Nova 100 aired a live interview with Dave Hughes at the completion of the race. The Age and the Herald Sun both published articles on the event.

We would like to thank our sponsors Australia Post (in particular Managing Director Graeme John) and Start to Finish Event Management (Directors Terry and Joan O'Halloran) for their continued support of FA research through Go The Tan. In particular we'd like to thank Mick Coffey (Treasurer and Secretary FA Vic), father of Tanya who has FA, for his enormous efforts in co-ordinating almost 200 Australia Post participants. The event would not be the same without this crucial support. We would also like to thank:

Transworld Freight
Services
City of Melbourne
YAFFA

Michael Roberts
Dynamic Duo PR &
Events
Runners World magazine

Fosters Group
Mossimo
St Vincent de Paul
Primary

GOLFFFA GOLF DAY DECEMBER 2007

The first Monday in December has become the traditional FA golf day for most of Melbourne's corporate elite. On Monday 10th December at the Commonwealth Golf Club, 34 teams of 4 joined to play as GOLFFers Fighting Friedreich Ataxia at the 9th Annual Charity Golf Club. Sponsored by Ernst & Young and supported by BHP Billiton, the event raised a record \$120,000 for FA research, becoming our largest fundraiser to date.

We would like to sincerely thank Ernst & Young and BHP Billiton (Chris Lynch) for their overwhelming continued support of FA research in support of their friend Tamara Curran who has FA, and the Commonwealth Golf Club for their event sponsorship. We would also like to thank all of the organisations who participate in the event:

ANZ Bank
Australia Post
BNP Paribas
Bank of Tokyo

Mitsubishi
Capgemini
Caterpillar
Commonwealth Bank

Computershare Ltd
CSC
David Bristow & Assoc
Deloitte Touche

Tohmatsu
Deutsche Bank
Dupont
EDS
Ernst & Young
Goldman Sachs
JBW
JP Morgan Australia Ltd
Korn/Ferry International

KPMG
Macquarie Bank Ltd
Merrill Lynch Int
Australia nab
PricewaterhouseCoopers
UBS Warburg Australia
Ltd
UBS Equities
Unisys

Wishlist
Skilled Engineering
Mitsubishi
Nufarm
Renfrew
Fosters
Murdoch Childrens

YAFFA BALL 2006

In June 2006, the annual YAFFA Ball was held at Atlantic South Wharf, Melbourne. With 500 guests in attendance, the event raised a record \$50,000 for FA research. The night was MC'd by Channel 9's Postcards presenters Bridget McIntyre and Laurence Mooney, with entertainment supplied by DJ Nick Rutherford and live band Big Deal.

We would like to thank our major sponsors LAN Airlines and Tempo Holidays who donated our major raffle prize of a trip for 2 to South America, Amunuca Island Resort for their donated auction prize of 7 nights accommodation for 4 people in Fiji and Clive Peeters for their donated LCD TV.

We'd also like to thank the following people and organisations who donated prizes for the live auction, raffle or silent auction:

AFL
Horizon Sailmakers
Tooheys
VOK
Duncan's
Merricks General Store
Mash restaurant & bar
Cobram Estate
Peppers Moonah Links
Dr LeWinn's
Minimink

Ishka
Maxwell Williams
Global Ballooning
Melbourne Aquarium
Diving HQ
Selkirk
Collins Simms
The Ducor Group
Excessories
Cedar Meat
IMG

IBO clothing
The Lyall
Maggie T
818 Dental
Pevonia
Auspack
Elan Hair
Kazuri Hair
Books to Wear
BMW

OTHER FUNDRAISERS

FARA receives the proceeds from many small fundraising initiatives throughout the year. This support from family and friends of FA'ers is enormously appreciated and together goes a long way towards enabling funding for research projects that otherwise may not be possible. Every bit helps!

White Suede sale

Australian fashion label White Suede conducted their 2007 Christmas sale so that proceeds could go to FARA. Owner and designer Jacqui Demkiw created the FARA fundraiser due to a close family friend suffering from the disease. \$1600 was raised from the sale and donated to FARA.

The Vineologist lunch

Social group The Vineologist conducted a luncheon fundraiser for FA and raised \$2500 for FARA, as friends of YAFFA.

Maggie T Christmas in store promotion

All Victorian Maggie T stores ran an awareness campaign about FA and FARA throughout the busy Christmas and summer period during 07 / 08. Information was placed at registers and donations accepted. This was done in support of Kaye Beetham, a Maggie T staff member and FA parent.

The Rotary Club of Boroondara / YAFFA Trivia Night

Boroondara Rotary Club together with YAFFA conducted a very successful trivia night in May 2007, raising over \$5000. This was due to the hard work of a very successful team who gathered many silent auction prizes that were quickly snapped up during the night.

YAFFA Queensland

YAFFA was proudly extended to Queensland in 2007 under FA sufferers Jamie Lee and Samantha Dwyer and their classmates. They joined a committee and conducted a local fundraising event at their school, raising over \$5000 for FARA, a fantastic achievement especially for their first event.

St Vincent de Paul Primary School Parents Association ran a live auction at a Fashion Parade night, donating \$3000 to FARA in support of their student Emma Jones who has recently been diagnosed with FA.

St Paul's Anglican Grammar. Each year the leaving year 12's raise money on their final day for the charity of their choice. They decided this year to support their classmate Amith Murthy who has FA. The money raised for FARA has more than doubled the money raised in any other years for any other cause. When it was announced at the Valedictory dinner how much was raised for FARA, there was not a dry eye in the house.

Di Stipkovich, mother of FA sufferer, together with her mother, make patchwork quilts to raise funds for FARA. Di's mother sells the quilts at local markets or to her friends in her nursing home to raise funds to support her grandson.

City to Surf Jo Gilbert – YAFFA member – ran the Sydney City to Surf in 2007 in support of FARA and her friend Carrie Beetham who has FA. Jo's efforts raised over \$500.

Dubbo College and Dubbo South Public School – in honour of the Thompson family, both of these schools undertook fundraising activities to support their friends Jenna and Henry who have FA. Together they raised almost \$1800 for FARA.

Angelo Pepe – Angelo is a consistent financial supporter of FARA. Angelo is the father of Veronica, who has FA. The Pepe family are of Italian heritage but live in Hong Kong.

DONATIONS - 2006 - 2007 Financial year

GRANTS

Goldin Foundation	\$20,000
Kirby Foundation	\$5000
Freehills Small Community Grants program	\$5000

\$1000 - \$4999

Education & Culture
Australia (Rousch family)
The Vineologist
Adtrans Truck Centre
Foam Tape Sales – Tom Stark
Murthy family
Air Cargo Club Australia
RM & CM Glascodine
RA & K Bray
ANZ
BNP Paribas

Bank Tokyo Mitsubishi
Caterpillar
Commonwealth Bank
Computershare Ltd
CSC
Deloitte Touche
Tohmatsu
Deutsche Bank
Dupont
EDS
Goldman Sachs JBW
JP Morgan

Korn / Ferry International
KPMG
Macquarie Bank Ltd
NABPriceWaterhouseCoopers
UBS Warburg Australia Ltd
UBS Equities
Unisys
Skilled Engineering
Mitsubishi
Nufarm

\$5000 - \$9,999

Socofar Ltd

Dwyer Family

Ernst & Young

\$10,000 - \$25,000

Renfrew

BHP Billiton

Australia Post

\$25,000 - \$49,999

Dennis Family Corporation

Lord Family

\$50,000 +

Gleeson Family

DONATIONS - 2007 – 2008 Financial year

GRANTS

Paul Newman Foundation	\$50,000
Kirby Foundation	\$5000

\$1000 - \$4999

Curran Family funeral
St Vincent de Paul
Primary School
White Suede
Skilled Engineering
Merrill Lynch
Mitsubishi
Australia Post

ANZ Bank
BNP Paribas
Bank Tokyo Mitsubishi
CSC
Deloitte Touche
Tohmatsu
EDS
Korn/Ferry International

KPMG
Macquarie Bank Ltd
PriceWaterhouseCoopers
UBS Warburg Australia Ltd
Unisys
Peter and Anna Rousch
RA & K Bray

\$5000 - \$9999

Rotary Club Boroondara
Transworld Freight Services

YAFFA Qld
Ernst & Young
Goldman Sachs JBW

JP Morgan

\$10,000 - \$25,000

BHP Billiton
Computershare Ltd
Australia Post

\$25,000 - \$49,999

Paul Newman Foundation

\$50,000 +

Gleeson Family

FARA Scientific Advisory Committee

FARA has a Scientific Advisory Committee (SAC) that advises annually on all funding applications with the prime objective of maximizing research potential that will lead to treatments and cures. The SAC was implemented to ensure all funding allocations are not subject to bias or influence and are the result of expert opinion and long term planning. Every dollar will deliver its best possible outcome. It is also intended to provide expert direction on future funding allocations. FARA's SAC comprises:

Professor Bob Williamson – former Director of the Murdoch Childrens Research Institute (MCRI) and member of the World Health Organization (WHO) Committee on Ethics and Genetics, who currently chairs the OECD Committee on Pharmacogenetics. Professor Williamson was involved in the identification of the genes for Friedreich Ataxia, Cystic Fibrosis and Alzheimer's disease. He is currently a Fellow of the Australian Academy of Science.

Professor Edward Byrne - Executive Dean of the Faculty of Biomedical Sciences and Head of the Medical School at University College London (UCL). Until March 2007, Professor Byrne was the Dean of Medicine at Monash University. He is the Founding Director of both the Melbourne Neuromuscular Research Institute and the Centre for Neuroscience. He is Professor of Experimental Neurology at

the University of Melbourne. As Director of the Centre for Neuroscience, he played a major role in driving the establishment of Neurosciences Victoria and Neurosciences Australia. His research group has made significant contributions in muscular dystrophy and mitochondrial research.

Dr Bronya Keats - Professor and Head of the Department of Genetics and Director of the Molecular and Human Genetics Center of Excellence, Louisiana State University Health Sciences Center. She is also founding Director of the Center of Acadiana Genetics and Hereditary Health Care. Dr. Keats has long been a leading scientific investigator in the search for genetic markers for hereditary diseases. She has made significant contributions to such searches in FA, Usher syndrome, Tay Sachs disease, and various hearing disorders.

Professor Kathryn North - Professor North is Douglas Burrows Professor of Paediatrics and Child Health at the Children's Hospital at Westmead, Sydney, Head of the Discipline of Paediatrics and Child Health and Associate Dean. She also heads the hospital's Neurogenetics Research Unit and Deputy Head of the Institute for Neuromuscular Research. Professor North's laboratory research interests focus on the molecular basis of inherited muscle disorders – particularly muscular dystrophies and congenital myopathies. At the Children's Hospital, Professor North has established the Clinical Neurogenetics Service which currently cares for more than 1,500 patients and their families.

Dr Elizabeth Coulson – Dr Coulson is the Laboratory Head of the Nerve Cell Survival Lab in the Neurogenesis, Mental and Neurological disorders department, Queensland Brain Institute, University of Queensland. The research interest of the stem cell

laboratory is to characterise and understand the molecular mechanisms controlling neuronal cell survival during neurodegenerative disease or injury.

Crescent
Melbourne, VIC 3004

Contact FARA

PETER ROUSCH – PRESIDENT, NSW

PHONE 02 4225 1745
FAX 02 4225 2779
EMAIL prousch@fara.org.au
ADDRESS 19/2 Harbour St
Wollongong, NSW 2500

STEVE BEETHAM– SECRETARY, VIC

PHONE 03 9882 8103
EMAIL sbeetham@fara.org.au
ADDRESS 78 Victoria Rd
Hawthorn, VIC 3122

MIKE DWYER – TREASURER,

Queensland & ALL DONATIONS
PHONE 07 5528 9018
EMAIL mdwyer@fara.org.au
ADDRESS 32 Karema Cres
Runaway Bay Qld 4217

RODGER ALEXANDER – New Zealand

PHONE 3 960 5988
EMAIL ralexander@fara.org.au
ADDRESS 41 Aylmer Street
Somerville
Christchurch 8002
New Zealand

TIM CURRAN – Victoria

PHONE
EMAIL timcurran@bigpond.com
ADDRESS 7 Strathallyn Rd
Ringwood VIC 3134

VARLLI BEETHAM– EXECUTIVE DIRECTOR

PHONE 03 9867 1910
EMAIL varlli@fara.org.au
ADDRESS Ground Floor 3 Bowen