fanconi hope

newsletter

Fanconi Hope Charitable Trust | Charity Reg No. 1126894 | December 2017

Our Fanconi Hope Conference and Family Fun Day Oct 17

An update from Bob Dalgleish, Fanconi Hope Secretary

As many of you will be acutely aware, a Fanconi Hope Newsletter is long overdue, particularly as there have been a number of changes and developments in the Charity in recent times. In this newsletter you will find more information on the topics below.

Firstly, I would like to say a big thank you to all those who attended the **Conference and Family Day** at Twycross Zoo in October this year. This was a very significant event, with a record number of families present, and was very well received, so our intention is to hold these every two years, with smaller social events in between.

We have made some changes to our **Board of Trustees**, mainly because two of our number have retired or moved on (Thomas Carroll our Chairman and Dr Mary Morgan), but also to ensure we have the right mix of medical specialisms relevant to FA as well as the right geographic spread, since the centre of gravity to date has been more Northern-based. These changes will be formalised at our AGM in the Spring, and in my own case this will formalise my new role as Chairman of the Charity.

We are extremely grateful to have the continued support of our Patron, **the Duchess of Devonshire** and who, this year, has been instrumental in us receiving an £8000 grant from the Duke of Devonshire's Charitable Trust to support one of our research projects.

Our **Fanconi Hope Website** has been undergoing a significant update to reflect the need for increased security, the ability to use it on mobile devices and to make information easier to find. Amine Touati, the partner of one of the Haematology Nurses at St Mary's Paddington, was inspired by the events at the recent Conference to kindly offer his services, as a professional web designer to update our website free of charge. It is now in the final testing stages and should go live in the next few weeks.

Our close collaborative relationship with the US-based **Fanconi Anemia Research Fund** continues, with our co-organised and co-funded International FA Gene Therapy Working Group meeting for the 8th year running, helping to push forwards the use of Gene Therapy for FA, and with trials in 3 countries now under way in varying degrees of maturity.

Our most significant project to date however, is our Fanconi Hope-funded **Study into the Long Term Effects of FA** led by Dr Stefan Meyer in Manchester and Dr Marc Tischkowitz in Cambridge. This study includes the part-time role of **Beth Lee** in an FA Research Liaison role, which involves her interacting as much as possible with FA families in the UK and Ireland.

With our increased financial commitments to the Long Term Study, the International FA Working Group and Family Events, we do now need to step up our fundraising efforts fairly significantly, so I would urge you all to take up the challenge of organising some fundraising events in your local area.

My personal contribution in this regard is a sponsored climb of Mount Kilimanjaro in late January 2018. Climbing and hillwalking have always been my passions, but I am a long way past 'peak' fitness so it will be a significant challenge for me. If anyone wishes to sponsor me, you can do so at www.justgiving.com/bobdalgleish. Thanks!

And finally, a big thank you to all our volunteers, trustees, supporters and fundraisers who have assisted our work through fundraising in 2017. I along with our other Trustees would like to wish you Seasons Greetings and a very Merry Christmas and hope that in 2018 you manage to cope with the many challenges that FA presents.

Bob Dalgleish, December 2017

Fanconi Hope Conference and Family Fun Day October 17

This was a very significant event, with the largest gathering of FA families ever seen in the UK - 110 people in all, including families, helpers, clinicians, researchers and nurses. The feedback we received was overwhelmingly positive and we have taken note of the comments so that we can tailor the event to meet your expectations as closely as possible next time round.

The Speakers covered a range of relevant topics, and included the following:-

- **Dr Stefan Meyer** from the Royal Manchester Children's Hospital with an Introduction to Fanconi Anaemia, clinical management and long term health of patients.
- A primer on the Genetics of FA by **Dr Marc Tischkowitz,** of Addenbrooke's Hospital
- **Dr Philip Ancliff**, currently involved in the first UK FA Gene Therapy Trials programme on Developments in Gene Therapy for FA.
- **Dr Nigel Jones** from the University of Liverpool covering the biological aspects of FA
- **Dr Josu de la Fuente** of St Mary's Hospital, Paddington, on Bone Marrow Transplantation
- A double act from **Dr Eunike Velleuer,** Heinrich Heine University and **Ralf Dietrich,** Executive Director and Family Support Coordinator, German FA Support Group on their Early Cancer Detection Study.
- **Beth Lee,** FA Nurse and Fanconi Hope Research Liaison, Royal Manchester Children's Hospital on Family issues / late effects and how to interact with her and the Late Effects Study
- **Louise Parry**, Treasurer, Fanconi Hope and FA Parent, and daughter **Amy Parry** on living with FA

Many of the Presentations can now be found on our website at www.fanconihope.org/home/families-and-affected-individuals

Throughout the event there was the opportunity for those affected by FA to take part in Dr Eunike Velleuer and Ralf Dietrich's study through giving mouth swab samples.

The zoo was clearly a big hit with the children, as were the indoor and outdoor play areas. For those able to stay overnight, there was ample opportunity to continue to make new friends throughout the evening meal at the hotel nearby.









Changes in the Organisation of the Charity

Regretfully we have had to say farewell to our long term Chairman, Thomas Carroll, due to the challenges he faces with his own family's health on top of with his demanding as a neurosurgeon in Sheffield. Thomas was a key driving force in the creation of the Charity, the UK Standards of Care document and the International Gene Therapy Working Group, so he leaves behind him an impressive legacy.

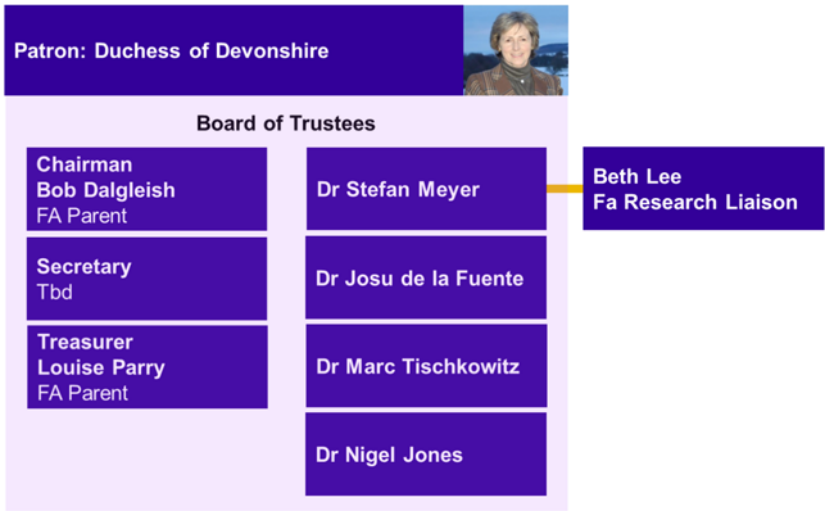
Dr Mary Morgan, our Haematologist Trustee from Southampton retired to live in Germany earlier this year and we wish her well.

To reflect the breadth of medical skills required for such a complex condition as Fanconi Anaemia, and to widen our geographic representation, we have made some changes to our Board of Trustees. One of our other founder Trustees of the Charity, Alan Gillespie, based in Sheffield, has stepped down and we have brought in Dr Josu de la Fuente, a haematologist from London, Dr Nigel Jones, a Biologist from Liverpool and Dr Marc Tischkowitz, a geneticist from Cambridge. We thank Alan for his expertise and wise counsel in particular during the preparation of the UK FA Standards of Care document.

Louise Parry, an FA parent from Manchester remains as Treasurer, whilst Bob Dalgleish from Portsmouth changes role from Secretary to Chairman.

These changes will be formalised at the next AGM in Spring 2018

The structure of the Charity is now as shown below. Later in the Newsletter you can find out more about our Trustees and helpers.



Fanconi Hope Website and other Social Media

Our website, www.fanconihope.org is currently undergoing an evolution rather than a revolution, to make the site more secure, make it mobile friendly and make it more easily navigable. These changes are being done in the background and can't yet be seen, but we expect the site to go live on the next few weeks. Please note that this is not a race with the new FARF website! It's purely coincidental that both sites are undergoing updates at the moment. The website will be more of a permanent information repository whereas our open Facebook page will contain more dynamic content.

Facebook: We have a Facebook page: www.facebook.com/fanconihope for general announcements and posts. For more private discussions about particular health matters and to allow FA families to get to know each other better, it is better to use our closed group: **Fanconi Hope Family Support Group**. Just type this name in your Facebook Search box and request permission to join the group.

You can also find us on **Twitter:** @fanconihope and on **Instagram**: fanconi_hope

Fanconi Hope-funded Study into the Long Term Effects of FA

There are potentially around 300 individuals in the UK with Fanconi Anaemia. It is evolving from a rare and serious paediatric condition to a condition affecting a considerable number of younger and increasingly older adults. Not much is known about what happens in the long term and how problems are managed. We therefore want to collect data about the genetic problem and clinical course. From this data we will get a much better idea of the problems affecting individuals with FA during their lifetime and how best to manage them

This study, led by Dr Stefan Meyer in Manchester and Dr Marc Tischkowitz in Cambridge, which is fully funded by Fanconi Hope, based on your fundraising efforts, seeks to track the treatments and progress of patients with FA in the UK over the long term, so we can better establish what treatments are most effective, and to provide a solid evidence base on which these treatments can be justified, and, more importantly, funded. Through this study a Registry of patients with FA is being created, something which has been a long term aspiration of Fanconi Hope, since to date there has been no way of knowing how many families are affected by the condition in the UK.

Beth Lee; Fanconi Hope Research Liaison

The funding within the study also funds a part-time role for Beth Lee, a highly experienced FA nurse, in an FA Research Liaison role, which involves her interacting as much as possible with FA families in the UK and Ireland.

The Study is necessarily patient-led and required patients and parents to communicate with Beth on a regular basis so she can understand what treatments people are on, how their health is progressing and what lifestyles they are adopting. This will slowly build up a picture of what works and what doesn't and this will be fed into the study so the bigger picture can also be understood.

Get to know Beth!

Whatever stage you are at with FA please get in touch with Beth at beth.lee@fanconihope.org, She has a wealth of knowledge about FA and she will be happy to discuss anything related to FA, including questions such as:

- What regular long term screening should be taking place,
- What vaccinations should I be having?

She is also used to dealing with some of the softer issues, such as feelings of guilt and loneliness which may occur from time to time.

How do I join the Study? Just let Beth know you would like to be part of it. All patients with diagnosis of FA can be included in the study, but patients or parents will need to sign a consent form. All you then need to do is keep in contact from time to time and let Beth know your progress. She will contact you 6 monthly to yearly over several years to document changes and keep the records up to date.

The success of our study relies on strong interaction with you and your families, so please get in touch with Beth at beth.lee@fanconihope.org. She will delighted to talk to you by phone or by email.

If you would like her to give you a call to discuss any aspects of FA at all, please email her and she will arrange a mutually convenient time to call you.

Fundraising

Over the 8 years of our existence families, relatives and supporters have raised over £250,000.

Fundraising efforts in recent years have resulted in average annual incomes of around £20,000, but with our increased financial commitments to the Long Term Study, the International FA Working Group and Family Events, we do now need to step up our fundraising efforts fairly significantly, to raise an average of around £30,000 per annum. Our Long Term Study for example costs £20,000, (which includes the part-time funding of Beth Lee in her Research Liaison role), and the International FA Gene Therapy Working Group meetings typically cost us between £6000 and £8000 per annum, so you can see that our existence and ability to fund Family Events and Research depends critically on your fundraising efforts and donations.

I would therefore urge you all to consider taking up the challenge to organise fundraising events in your local area. Even small amounts of money add up and make a difference.

We are registered with both Just Giving and Virgin Money Giving if you wish to undertake Sponsored events.

One painless way for you and your friends and relatives to fundraise for us is to shop online using **EasyFundraising.** There are over 3,100 shops and sites all ready to give Fanconi Hope a free donation when you shop, including Amazon, John Lewis, B&Q, The Trainline and loads more. Simply go to www.easyfundraising.org.uk, search for Fanconi Hope and then set up an account. There's no catch or hidden costs, just an easy way to raise extra money for Fanconi Hope

To give you an idea what our supporters have been up to here are just a few examples of Fundraising events this year:

- Christine Bruce has a JustGiving Page for donations from her work team in lieu of giving each other Secret Santa Presents. This has currently raised almost £300 including GiftAid. (see picture above)
- Steve Moore ran the London Marathon in April and raised over £1000
- Simon Bartle undertook a Fanconi Hope Bike Ride and Pub Crawl in the Chilterns, raising over £300.

Roger McLean lost weight for Fanconi Hope in January 17 raising £25

Team Lee raised over £350 from their English Half Marathon in Warrington in September 17

Tyler Picton, who with his Dad **Mark** raised £500 with a sponsored Martial Arts Day in December 17

This is just a small selection of our fundraisers but a big **THANK YOU** to ***all*** the people who have fundraised for us or donated directly to us during the year!

THANK YOU!

Some other fundraising ideas - Collect money directly with our Collection Boxes and Buckets or nominate Fanconi Hope as your Company Charity of the Year.

We have a number of items to support your fundraising efforts – please see our website: www.fanconihope.org/giving-and-fundraising/resources

Please also consider giving monthly by Standing Order or Direct Debit. We have a number of donors who give anything from £5 to £20 per month and together these all mount up over the year.

Support you can call on from Other Charities

Fanconi Hope is a small charity with very limited (and voluntary) resources. Fortunately, there are a number of other charities ready and willing to support you. It is also always worth asking at your hospital if there is a Charity Coordinator to talk to, who can advise on more local charities.

Here are just a few:

- **Together for Short Lives:** Support & advice for families with seriously ill children in the UK. www.togetherforshortlives.org.uk
- **Contact (was Contact a Family):** the charity for families with disabled children and genetic conditions. We support families, bring families together and help families take action for others. www.contact.org.uk
- **The Rainbow Trust Children's Charity** provides emotional and practical support to families who have a child with a life threatening or terminal illness. (www.rainbowtrust.org.uk)

It is well worth reviewing the range of services these charities have to offer as you may not need them now but may need to call on them in the future.

The International FA Gene Therapy Working Group

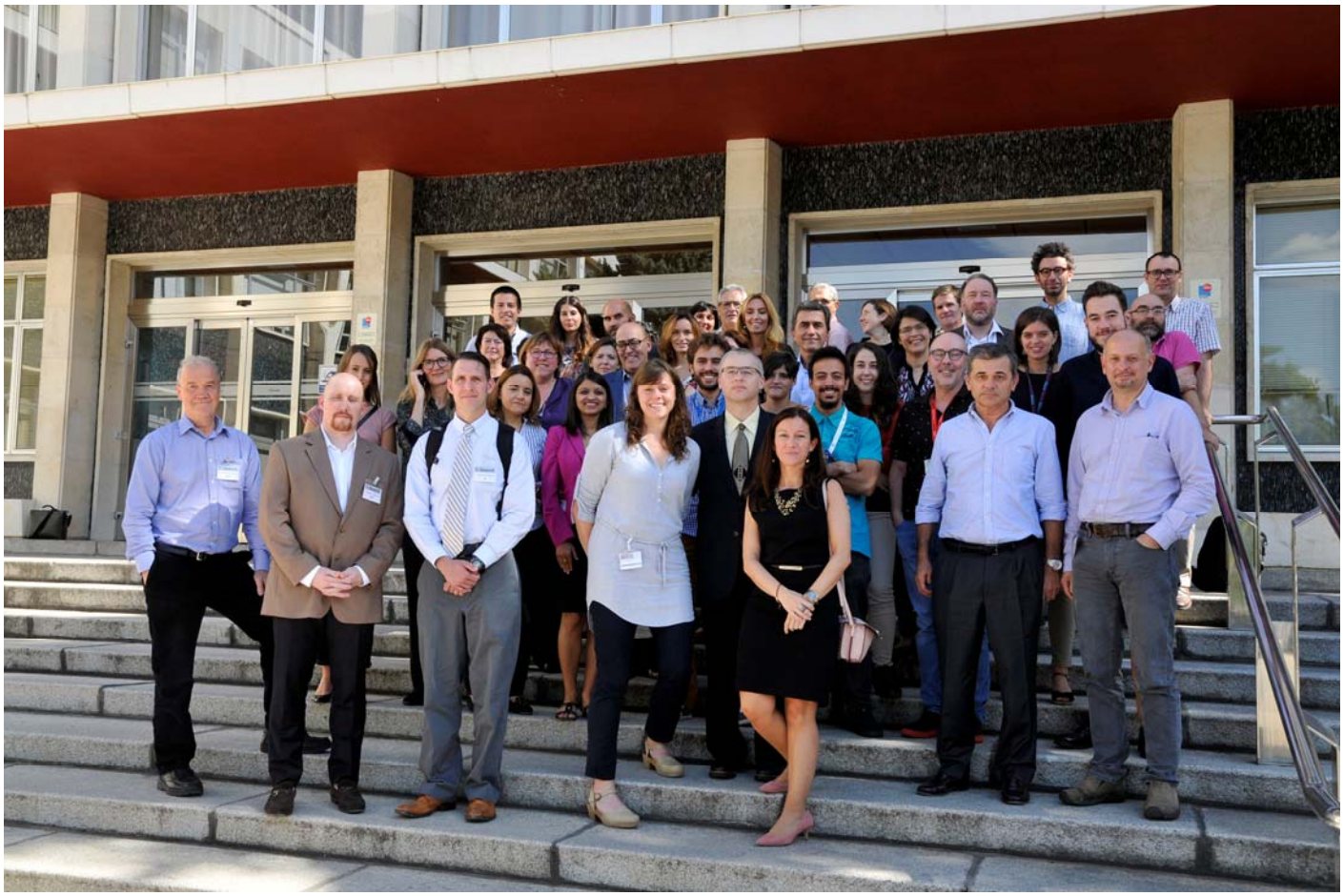
The International Fanconi Anaemia Gene Therapy Working Group, organised and funded jointly by the Fanconi Hope Charitable Trust and the Fanconi Anemia Research Fund (FARF) was set up in 2008 to bring together leading Gene Therapy and Fanconi Anaemia experts from across the globe to create an action plan for gene therapy trials in Fanconi Anaemia and thereby accelerating the process which had its beginnings in the early 90's. The goal of gene therapy in Fanconi Anaemia is to develop a safe treatment for bone marrow failure, while preventing leukaemia and reducing the risk for subsequent squamous cell carcinomas.

The group, which meets annually, is chaired by **Prof Jakub Tolar**, now Dean of the University of Minnesota Medical School, and is now in its 8th year of existence, and helped by the progress in the Group, trials are now underway, at various stages of maturity in the US, Spain and the UK.

In an exciting recent development, Rocket Pharma in the US has chosen Fanconi Anaemia as a candidate condition for the commercial development of gene therapy. Convincing pharmaceutical companies to invest in what is a very rare condition has always been seen as one of the stumbling blocks to commercial development of gene therapy cures, so this is a great step forwards.

What is remarkable about the Working Group meetings is that researchers are willing to share the results of their work freely within the Group prior to publication, which means that the various teams working in UK, the rest of Europe and the US can take advantage of the very latest knowledge being gained by each team in order to revise their own research programmes. This level of knowledge sharing before the official publication of results is very rare in the research world, and is to be applauded, as it demonstrates the commitment by all those involved to make maximum progress for the sake of those affected by FA.

However, because the Working Group meetings involve reports of results from the various teams before publication it is not possible for us to publish detailed notes from the meetings themselves. We therefore leave this to the Chairman of the Group, Prof Tolar, to report in FARF newsletters and presentations as he feels appropriate.



Record Numbers at the Working Group Meeting in Madrid in 2016

Meet Our Board of Trustees

Dr Stefan Meyer

Stefan works as an Honorary Consultant Paediatric Oncologist at the Royal Manchester Children's Hospital, where he also leads the multidisciplinary care for children with Fanconi Anaemia, and the Young Oncology unit at the Christie Hospital

Graduated in Medicine in Hamburg, Germany. MD University of Hamburg. Basic Paediatric training in Lubeck, Germany, and Manchester. MRCP/MRCPH(UK) 1995. Higher Specialist Training in Paediatrics and Paediatric Oncology in Manchester. Leukaemia Lymphoma Research Clinical Research Fellow 2000 – 2004, Paediatric Oncology, Manchester. PhD 2004, University of Manchester. CRUK Clinician Scientist 2005 -2010, University of Manchester. Since 2010 Senior Lecturer / Honorary Consultant Paediatric Oncologist, Stem Cell and Leukaemia Proteomics Laboratory and clinically in the Department of Paediatric and Adolescent Oncology of the University of Manchester at Royal Manchester Children's and Christie Hospitals.

Dr Marc Tischkowitz

Marc is a University Reader and Honorary Consultant in the Department of Medical Genetics at Cambridge. He completed his medical degree in 1993 and trained in Medical Oncology before completing his training in Medical Genetics. From 1999-2002 he undertook a PhD researching the role of Fanconi Anaemia gene mutations in the development of sporadic acute myeloid leukaemia. He held a Consultant post at Great Ormond Street Hospital before moving to McGill University, Montreal in 2005 where he worked on Faculty for six years before coming to Cambridge. Much of his research has been on the Fanconi Anaemia genes and hereditary breast cancer predisposition but his interests cover all areas of hereditary cancer and translating the recent advances in genomic technology into clinical practice.

Dr Josu de la Fuente

Dr Josu de la Fuente is a leading Consultant Paediatric Haematologist working both in the NHS and the private sector in London. He is the Clinical Lead for Paediatric Specialties at Imperial College Healthcare NHS Trust, where he directs the Blood and Marrow Transplantation Programme and leads the Service of Paediatric Haematology. He is a Senior Lecturer in Haematology at Imperial College London.

Dr Josu de la Fuente is a Fellow of the Royal College of Physicians of London, the Royal College of Physicians of Ireland, the Royal College of Paediatrics and Child Health and the Royal College of Pathologist, reflecting his expertise in haematology and paediatrics. He is a member of the British Society for Haematology, the American Society of Haematology, the American Society of Paediatric Haematology/Oncology, the American Society of Blood and Marrow Transplantation, the European Haematology Association and European Society for Blood and Marrow Transplantation.

Dr Nigel Jones

Nigel is a Senior Lecturer in Genetics at the Institute of Integrative Biology at the University of Liverpool. He has a BSc in Genetics/Microbiology, University of Wales Swansea (1982). PhD in cellular and molecular biology at the MRC Radiobiology Unit, Harwell (1987). Postdoctoral fellow at Lawrence Livermore National Laboratory, California (USA) working on human DNA repair genes (1987-1988) and postdoctoral fellow at Swansea University working on DNA repair mechanisms, genetic toxicology and human biomonitoring (1989-1995). Since joining the University of Liverpool in 1995 his research on DNA damage responses has focused on the role of the Fanconi anaemia proteins in DNA repair and cancer predisposition.

Bob Dalgleish – Chairman designate

Based in Portsmouth, Bob has recently retired from a business development role in electronics research, allowing him to spend more time with the Charity. Bob is one of the founders of the Charity, having recognised the need for a family support organisation following the diagnosis of his daughter Louise, with Fanconi Anaemia in 2007. Louise is now more than 5 years post Bone Marrow Transplant and is currently studying for A levels.

Louise Parry – Treasurer

Louise lives with their family near Manchester. Her daughter Amy, with FA, is now more than 5 years post Bone Marrow Transplant and leads a busy and active life. Louise is a Trustee and the Treasurer of Fanconi Hope.

Not Trustees but still a vital part of the organisation…

Beth Lee – Fanconi Hope Research Liaison/Coordinator

Beth is a dedicated nurse working at The Royal Manchester Children's Hospital with over thirty years' experience of Haematology and Oncology. Beth especially enjoys supporting and caring for Fanconi patients and their families. She has a good understanding of the many physical, psychological and genetic issues facing families and is passionate to assist families in any way she can. Beth started working for Fanconi Hope in May 2016 as Research and Liaison Coordinator.

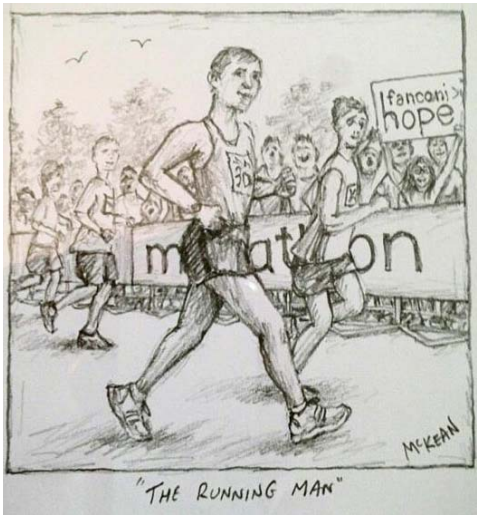
Jeannie Dalgleish – Family Coordinator

Jeannie, whose daughter Louise has FA has been an active supporter of the charity since it was founded. This has included speaking to many parents, families and professionals. She has organised many events and has promoted the charity in the press. Until recently she guided the Emmerdale storyline around the character with FA - Sarah Dingle.

Louise Dalgleish – Media Support

Louise is 17 and has FA. She received a bone marrow transplant in 2012 and is now doing well. Her role in the charity is to help with social media updates. This involves working with various platforms including Facebook, Twitter, and Instagram. Louise is studying graphics as one of her A-levels, meaning she has a good knowledge of design which is relevant to her role in the charity.

How You Can Help Us



We really need your support in helping to raise money to keep the Charity finances healthy.

We are a relatively small community, so it's vital that as many families, friends, relatives and supporters as possible participate in the fundraising effort.

Please also consider donating directly via our website or at **www.justgiving.com/fanconihope**.

If you have fundraising ideas, please share them via our Facebook page **www.facebook.com/fanconihope** and fell free to contact us to discuss your ideas further and see what support we are able to provide you with.

(Please see contact details at the foot of the page).

If you would like to actively help with our fundraising programme please contact Bob Dalgleish at rad@fanconihope.org.

Other ways you can help

As you are probably aware it is critical that FA patients have well-matched donors for bone marrow transplants.

Please help by:-

- Becoming a donor
- Persuading others to do so too
- Offering your services to Nolan Recruitment Drives

You can find further details at ***www.anthonynolan.org***

In the Future ….

This is the first newsletter for a long, long time, and our intention is that it will now be produced annually. We would like to have more family-oriented material included, so we would love to publish your stories if you are willing to share them.

Our Family Meetings will be every two years due to the amount of work involved on organising them, but we hope to arrange smaller meetings in between times.

How to Contact Us

By email: info@fanconihope.org (general matters) or Beth Lee at beth.lee@fanconihope.org for FA Support

By Phone: 0300 330 1410

By Post: Bob Dalgleish, PO Box 905 Southsea, PO1 9JG

Please note that all Fanconi Hope Trustees give their time voluntarily, and all but one lead busy working lives, so please bear with us if we take a little time to respond to you.