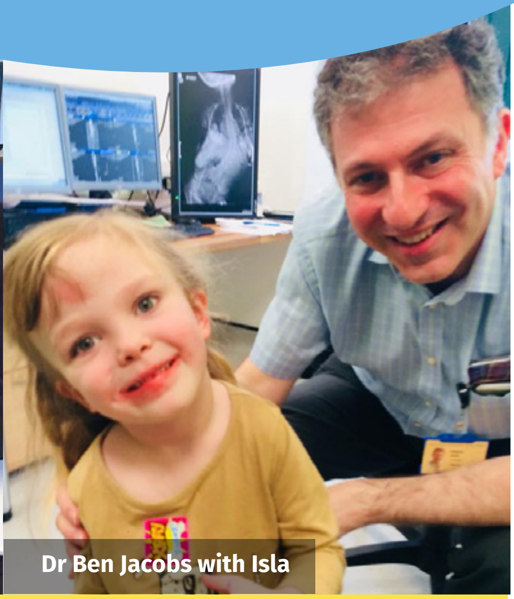
fop friends®
together
Vol 2 Issue 2 | April 2018
www.fopfriends.com


Jackie Vinton, Dr Richard Keen, Andy Symonds with Millie


Dr Ben Jacobs with Isla

A very special FOP support team

Usually, when we talk about our supporters, we're talking about the fantastic people of all ages who raise money and awareness to support FOP research. But we'd like to highlight another team that plays an important role in the lives of a number of FOP families in the UK, as well as a critical role in working to find a cure for FOP. They are the knowledgeable and fantastic team of specialists at the Royal National Orthopaedic Hospital (RNOH) in London.

Besides being involved in the care of many of the UK's FOP patients, these dedicated individuals are also running the MOVE Phase 3 trial for palovarotene in the UK and are a site for the Phase 2 Regeneron study. (More on these over the page).

As a community we're fortunate to be able to access this support team which includes: Consultant in Metabolic Bone Diseases, Dr Richard Keen; Paediatric Consultant, Dr Benjamin Jacobs; Rheumatology Consultant, Dr Judith Bubbear; Clinical Nurse Specialist in Metabolic Bone Disease, Jacqueline Vinton; Research Associates, Amit Patel and Andy Symonds; and Pharmacist, Gaurang Purohit.

We're fortunate to have this world class team on our doorstep and not a day goes by when the UK's FOPers and their families aren't grateful for their dedication and support. On behalf of all our families, we THANK YOU!

INSIDE

Two new clinical trials 2
Oxford research update 2
Meet Paul Sands 3
Q&A with Dr Judith Bubbear 4

FOP Friends exists to help find a treatment and a cure for the rare genetic condition fibrodysplasia ossificans progressiva (FOP), and to support the families affected by it.

FOP Friends is a Registered Charity
in England and Wales 1147704

A word from Nicky

It's an exciting time for FOP Friends and I could not be prouder of what the charity achieves for such a rare disease with so little resource. Not only does every single penny raised for the charity go directly to advance research for FOP, the charity also tirelessly seeks other funding avenues to help maintain the invaluable support network for our FOP community.

Soon after my daughter was diagnosed with FOP we were able to join the 2014 UK FOP Family Gathering. Isla was just a few months old, and understandably I was incredibly anxious ahead of the meet. As soon as we arrived we were put at ease by the wonderful welcoming families, our dedicated doctors who had travelled from far and wide, and most of all by those with FOP and their sparkling spirits. We were no longer alone on this journey.

Now, just four years later, with advances in research, potential treatments, and two active drug trials, there is a new level of hope for us all. I am incredibly excited to be part of this journey and can't wait to meet with our special friends new and old this May at the 2018 FOP Family Gathering.

With huge thanks for your continued support.

Nicky x

We are delighted to announce that, thanks to the generous grants from **The Boshier-Hinton Foundation** and **The February Foundation**, we are able to produce the newsletter for another year.

Two new clinical trials recruiting those with FOP

Created by Freepik

There is much excitement in the FOP community with not one, but two clinical trials now recruiting in the UK. From having no potential treatments five years ago to this, is quite astounding.

Palovarotene in Phase 3

For the FOP community, 2017 ended on a note of hope with positive news from US pharmaceutical company Clementia.

The results from the Phase 2 clinical study of palovarotene showed that a continuous daily dose of the drug, with a higher dose at the first sign of a flare-up, reduced the formation of new bone volume by 95% at 12 weeks when compared to those not taking the drug.

Having been granted the status of a Breakthrough Therapy in the US, the company is now taking the drug into Phase 3 clinical trials.

The site for the MOVE Phase 3 trial for palovarotene at the Royal National Orthopaedic Hospital (RNOH) in London opened this month. The trial is recruiting patients with FOP, aged 4 and over and is expected to run for 24 months. Already, the first participants have attended RNOH for their initial screening, with some patients receiving their first dose.

For more information, go to: www.fopfriends.com/clementia.

Anti-Activin A antibody in Phase 2

In June 2016, following on from the promising studies in mice, US biotechnology company Regeneron ran a Phase 1 clinical trial of anti-Activin A antibody (REGN2477). **Their study in healthy volunteers is now complete, and based on the positive findings, Regeneron has launched the Phase 2 trial for adults.**

The trial, known as the LUMINA-1 study, will investigate if the drug can slow or stop bone growth in patients with FOP (as it did in mice), and if the drug is well-tolerated.

There are two UK clinical trial sites for this study, the RNOH in London and the Manchester Royal Infirmary. They are looking to recruit 40 individuals between the ages of 18 and 60 to take part in the 19-month trial.

For more information go to: www.fopfriends.com/regeneron.

Oxford Research Update – third trial on the way?

As you may know, most of the money raised by our supporters goes towards funding FOP research, specifically the work of the University of Oxford's FOP Research Team.

Over the last year, the team at Oxford has been continuing to prepare for clinical trials of saracatinib, an investigational drug from AstraZeneca that has showed promise as a possible treatment for FOP. They've also been collaborating with other academics and companies to advance other drug-like molecules. This has included collaborating with experts from the cancer drug discovery field.

UNIVERSITY OF OXFORD

Meet
Paul Sands

This quarter's FOP friend is 54-year-old Paul Sands from Hadleigh in Essex. Diagnosed with FOP when he was three, Paul's had a tough year FOP-wise, but has kept busy with his many hobbies.

When did you receive your FOP diagnosis?

I was diagnosed when I was about 3 years old. When I was younger, I used to go to Great Ormond Street Hospital and University College London Hospitals until I was transferred to the Nuffield OP centre in Oxford, although I've not been there for many years as my FOP has been stable. However, at the moment I'm going through a rough time as I had a fall and broke my left wrist. My wrist was pulled back into position and put in plaster for two months which has made things very difficult for me recently.

I need to have most things done for me e.g. washing, dressing, feeding, drinking, toileting and showering. I also need assistance with getting in and out of my van. I enjoy going shopping, visiting friends, socialising at the pub, visiting the cinema and dining out. For the past 18 years I've lived in a sheltered accommodation unit with a full-time carer.

How do you get around?

I drive a VW Transporter with rear-entry tail lift. I can drive myself using an adapted van seat, although recently I've not been driving as often as I used to. The furthest north I've been is Birmingham, but I have covered most of Wales and the east and west of England. I've also travelled abroad to Florida, Disneyland Paris, Portugal, Malta, Cyprus, France, Belgium, Holland, Germany, Spain, Jersey and Guernsey.

When and how did you become of aware of a FOP community?

I've been aware of the FOP community for some years, although I've never met any fellow FOPers in person. I first made contact with FOP Friends about a year ago and hope to meet up with some FOPers in the UK one day.

What's the one thing you can't get through the day without?

My desktop computer and internet.

What's your favourite way to spend your time?

I spend most of my time Facebooking and buying and selling stuff on eBay. I love playing games on Facebook — Zynga poker (not for real money), Words with Friends, Sweet Road, Pet Rescue Saga, Diamond Dash and Buggle are some of my favourites.

I also enjoy chatting to friends and relatives. I like watching films, anything from Sci-fi, action and war, to dramas. I love music and I was a DJ for a few years. I was the chairman of our local Phab club for nearly 20 years, until I had to give up for personal reasons. I do like books, but don't read them because I can't find a comfortable position to hold or see them.

I'll admit to being a bit of a hoarder. I love my clutter and stuff. I have hundreds of CDs and DVDs and even old VHS tapes. I also have thousands of photos that aren't on the computer yet – one day I'll get around to it!

Anything else you'd like to share with others in the FOP Friends community?

Making friends as an adult can be difficult for anyone, let alone when you are living with a condition like FOP. We are all at various stages of this painful and hideous disease. We need to enjoy life to the full!

What song would you pick as the soundtrack to a great day?

Proud by M People. I have other options, just depends what I feel at the time.

THANK YOU FOR YOUR FEEDBACK

Earlier in the year, we asked you for feedback on our newsletter. To those of you who returned our survey, a very warm thank you. The feedback received was overwhelmingly positive. We are pleased that you enjoy hearing about the work and achievements of our supporters and the work of the charity. However, some of you said you wanted more updates on the progress with research and clinical trials. As we're sure you can appreciate, the nature of the research means that breakthroughs don't happen as quickly as we'd all like. We always share the latest developments on our Facebook page, so if you aren't following us, now is the time to Like us! Excitingly, we've had more research news in the last few months than we have had for a while, so we hope you all particularly enjoy reading this issue! In response to the feedback, we have added another page on our website, where you can go and find the latest news and developments. Visit: www.fopfriends/clinical-trials to read more.

Q&A with Dr Judith Bubbear

Dr Judith Bubbear joined the team as a rheumatology consultant at the Royal National Orthopaedic Hospital last year. She is London born and bred, and has worked on and off at the RNOH since she first started her medical training.

Where were you before you joined RNOH?

I was a consultant at Whipps Cross Hospital in East London for seven years where I led the osteoporosis clinic.

When did you first become aware of FOP?

It was during one of my six-month placements as part of my medical training. I was a senior house officer and had the good fortune of being placed under Dr Keen at the RNOH. I remember it clearly. During a clinic, Dr Keen looked at his patient list and said: "You should see this person, they have something very rare." I met with the patient and they were very kind in telling me about their FOP. After they'd left I asked: "How do we treat this?" And he said something like: "We can't. We are as nice and as helpful as we can be, but sadly there is no treatment right now."

It was during this placement that I knew I wanted to become a rheumatologist and specialise in rare bone diseases. I was able to spend another two and half years at RNOH as a clinical research fellow, focusing on osteoporosis, before I had to move on.

Almost 15 years later and I'm back where my interest first began. In some ways it feels like I've come home, and I'm so excited to say that there is a real possibility that soon the answer Dr Keen gave me back in 2003 will no longer be true.

What other conditions do you specialise in?

As well as seeing patients with rare bone conditions such as FOP, brittle bone disease and fibrous dysplasia, I also work with patients who are suffering from osteoporosis.

Also, I actively try to educate other medical professionals about FOP and other rare bone diseases. I'm part of a group that is setting up a London-wide rare bone disease network of rheumatologists, geneticists, endocrinologists, paediatricians and other specialists. Our aim is to pool our knowledge to try and improve patient care.

Are you directly involved in any FOP research projects? What is your role?

Yes, I'm involved in seeing people who will be enrolled in both the Regeneron study and Clementia's palovarotene study. I'm what's known as a "sub-investigator" on these trials.

What's your favourite way to spend any free time you have?

With my family. I have two children (aged 11 and 8) and I'm married to a deputy head teacher. I also love running and music.

What's the one thing you can't get through the day without?

That's the easiest question I've had to answer. Tea.

What three things would you take with you to a desert island?

My piano (you never mentioned a weight restriction!), my running trainers, and my Bible.

Will this be your last newsletter? Let's stay friends!

We hope this won't be your last newsletter...but unless you fill out and return the enclosed form, we won't send you any further issues. This is to ensure the charity is compliant with the new General Data Protection Regulations (GDPR) which come into force on 25 May 2018. You can read our Privacy Notice here: www.fopfriends.com/privacy-notice. Alternatively, visit our website and complete the online form.

About FOP

Fibrodysplasia ossificans progressiva, or FOP, is one of the rarest and most disabling genetic conditions known to medicine. In people with FOP, bridges of bone develop across the joints, progressively forming a second skeleton that imprisons the body in bone. Currently, there is no known treatment or cure for FOP.

A cure for the disease could also benefit people living with osteoporosis, arthritis and heart disease, as well as those affected by heterotopic ossification (a major complication in hip replacement surgeries and in sports and military injuries), and other more common bone and muscle disorders.

FOP Friends
The Cabin, 1 Cumberland Road
Sale, Cheshire M33 3FR

www.fopfriends.com

info@fopfriends.com

@FOPFriends

www.facebook.com/fopfriends

Thank you to the following trusts and foundations for their kind support in funding the FOP Friends Together Newsletter:

The February Foundation

Manchester Guardian Society Charitable Trust

The Astor Foundation

The Sobell Foundation

Registered with FUNDRAISING REGULATOR

Give with confidence

FOP Friends is registered with the Fundraising Regulator, an independent self-regulatory body for UK fundraising that encourages commitment and compliance with best practice in fundraising.

Designed by Helen Joubert Design