FOP Awareness Day

23 April 2019 #FunFeet4FOP

fop friends together

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www.fopfriends.com

A speech to remember

In December, Oliver and his dad Chris were invited to speak at Regeneron's Christmas Conference. They were asked to "inspire" – and they most certainly did! It was Oliver's first solo presentation, entitled 'FOP and Me', and he was quite simply amazing! The room was moved to tears and he received a standing ovation for his speech. He did himself and the charity proud, raising awareness of both the condition and the charity.

Regeneron is still recruiting participants for their LUMINA-1 study, which is investigating if the drug anti-Activin A antibody (REGN2477) can slow or stop bone growth in patients with FOP (as it did in mice) and if the drug is well-tolerated.

Visit www.fopfriends.com/regeneron to find out more information.

INSIDE

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 Fiona

Welcome to yet another fabulous issue of 'FOP Friends Together'. I am delighted to be writing a short word from myself, having just returned from our second FOP Friends Family Gathering at Center Parcs in Nottinghamshire. Before heading off, I had wonderful memories of the Gathering in 2017 come flooding back, reminding me of the fun that was had. I'm so please to say that our second Gathering did not disappoint! It is so important for families to have the opportunity to come together, relax and share stories; this is a fundamental aim of FOP Friends. Our community is strong, and with new families becoming a part of our community, we are able to grow even stronger.

Recently, we have begun to share a 'Supporters Spotlight' across social media. This has enabled us to say a huge thank you and show our appreciation for the support we have had, and still continue to receive, from a large number of people. This support remains crucial in facilitating research to ensure a treatment and cure for FOP. It is an exciting time with trials in progress.

Having been a trustee for a number of years, it has been incredible to watch the charity grow. The support and opportunities it provides has been invaluable; it has made a big difference to so many people. Long may it continue.

Clementia seek FDA approval

Clementia completed their recruitment for their Phase 3 MOVE trial four months ahead of schedule and have recently announced that they are in the process of filing the first-ever application seeking FDA (Food and Drug Administration) regulatory approval for their FOP drug, palovarotene.

clementia

Q&A with Dr Alex Bullock

As you may know, most of the money raised by our supporters goes towards funding our Oxford University Research Team. We caught up the team's leading professor, Dr Alex Bullock:

Dr Jong Fu Wong, Dr Alex Bullock, Dr Roslin Adamson, Miss Elizabeth Brown

Over the last five years, what advancements have been made by the Oxford Team?

Our most significant finding has been the clinical drug candidate saracatinib (also known as AZD0530), which we identified as a highly effective inhibitor of the FOP causative protein ACVR1 (also known as ALK2). As we've reported previously, saracatinib was first developed by AstraZeneca for the treatment of ovarian cancer. While it did not show sufficient benefit for drug approval in this condition, it has been made available by AstraZeneca for testing in other diseases. Importantly, saracatinib has now been tested in over 700 people and was shown to be well tolerated when taken as a daily tablet. We are currently completing the necessary legal paperwork to start a new trial using saracatinib in FOP later this year. As soon as we have more information we will communicate this through FOP Friends.

In addition, we have been studying the root cause of FOP at the molecular level. All cases result from tiny changes in the ACVR1 gene (typically one erroneous letter – A, C, G or T in DNA – out of 1530 letters in the coding sequence). As a result, a faulty protein is made with one erroneous amino acid out of 509. We've been able to solve the 3D structure of one of these faulty proteins and have produced several others for functional analyses.

It has been hypothesized that the faulty protein may simply have broken its shackles (e.g. tight binding to the FKBP12 protein) like a rocket detaching from a launch pad. Our experiments confirm this effect, but also suggest that the FOP rocket has an extra engine. Fortunately, drugs such as saracatinib can bind to the FOP protein to switch the rocket off again.

What are your short- and long-term plans for future research?

With a current IFOPA project grant we are conducting a feasibility study to test whether other more unique drug binding pockets can be utilised on the FOP protein. Targeting such sites with small molecule inhibitors would allow the longer term development of a next generation drug with the hope of it having the fewest side effects. We are also exploring potential synergies between drug development for FOP and the brain cancer DIPG.

To read more about the team's future research plans, to gain an insight into the Oxford Team, and to hear what Dr Eleanor Williams gets up to, in addition to her FOP research role, visit www.fopfriends.com

The Oxford team are always happy to be contacted by anyone in the FOP community, who is interested in seeing the laboratory and learning what they do. Email info@fopfriends.com to get involved.

Accelerating Cures and Treatment

We're delighted to announce FOP Friends was one of the leading donors to contribute to the 2018 ACT grant program, where four grants studying FOP were awarded a total of $207,310.

Team Isla exceeds target

Isla's FOP Fight fundraising page that was set up just over 4 years ago recently exceeded its target of £60,000! This achievement is thanks to the collective effort of all the members of Team Isla, who constantly go above and beyond with a variety of different events to raise awareness of FOP.

Giving Tuesday £1,031.25

Did you know the Tuesday after #BlackFriday in November is called #GivingTuesday? Every year this day is the chance for individuals to give back to charities. We were so lucky that Sophos Plc kindly fund-matched every donation made on the day to Team Isla's Giving Tuesday page.

Gig for Isla £2079

In its 5th year, Gig for Isla was another tremendous success with the Isle of Wight community again coming together in style. The L&M Band performed all night and a lot of fun was had by everyone in attendance.

Jake & Amy's Annual Toy & Book Sale £85

Jake and Amy, two of our youngest fundraisers, always hold an annual toy and book sale to raise awareness and funds for FOP.

Beetle Drive £342.15

Also over on the Isle of Wight, Debs, Debbie and Magot held their annual Calbourne Beetle Drive evening, where a great time was had by all.

Masonic Ball £2845

Thanks to Sue Woodard who organized such a special evening in aid of FOP Friends.

Lambrook School £1122.86

Isla's super cousin Toby, along with his wonderful mum, raised over £1000 at school to help Isla & her FOP friends find a cure for FOP and also did an assembly speech to raise awareness. Simply awesome.

Swap4FOP £800

Simply take a bunch of friends, each bringing a few unwanted items. Add an entry fee, a raffle and some fizz, and you've got a fun night! Recycle, take away some treasure, and raise much needed funds for FOP Friends. Isla's godmother did just that but with 20 friends and raised a whopping amount! Get in touch info@fopfriends.com to host your own.

Afternoon Tea £406.25

Clair and Mary hosted a fabulous afternoon tea, raising an amazing amount. Showing how a small, fun event can make. such a difference to our small but special charity.

100km Thames Path Challenge £902.50

Imagine running 100k? This is just what Josie has done for us! Josie and her daughter Stella met Isla and her mum in 2014 when both girls were 6 weeks old, a couple of months before Isla was diagnosed with FOP.

Heather & her twin sister Paula's creative efforts £260

Heather's a keen hobby painter, and her twin sister is an avid sewing and knitting creator of lovely things! They were both keen to see if they could sell their work at craft fairs to benefit FOP Friends and they've definitely been successful.

Boxing Day fundraiser £974

On Boxing Day (Wren Day) this wonderful group got dressed up and visited 35 pubs Irish dancing whilst raising money for charity.

Our first-ever Christmas card campaign

This year we revealed our first ever old-school Christmas card to help raise awareness of FOP and to help continue funding the work of our Oxford University Research Team and supporting our UK FOP families. We challenged all our FOP families to take part and see who could sell the most cards. 23 families got on board and a staggering £1479.60 was raised, with 10,000 cards distributed across the country. Ultimately, the O'Shaugnessy family came out on top, selling an impressive 125 packs of cards. Who will take the crown this year?

Office interns

We were so lucky to be joined in the office before Christmas by Nicola and Keane, two Manchester University students, who completed a 30-hour placement as part of their Psychology course. They were the masterminds behind our '12 Days of Christmas' social media campaign.

YPI girls make it to final

A group of inspiring girls took part in the Scottish Youth Philanthropy Initiative representing FOP Friends. After holding multiple fundraising events, including a raffle and a Buttery morning serving lots of lovely treats, the programme culminated with the girls getting on stage and presenting about why FOP Friends should win a £3000 donation. The girls were disappointed not to win, but we were overwhelmed with their efforts and so very grateful for all the time they spent on the project and the awareness that they raised for FOP.

#FunFeet4FOP 2019

We're thrilled to announce that this year we're collaborating with the IFOPA on our #FunFeet4FOP campaign. Make sure you post photos of your #FunFeet from April 1st, along with our #FunFeet logo and hashtag. Tag your friends to take up the challenge and help spread FOP awareness!

Did you know...

96%

of all money fundraised goes directly to supporting our Oxford Research Team. Our other projects (including our newsletter, our biennial conference, and our family respite weekend) are all solely funded by grants and trusts.

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

Thanks to the generous grants from **The Boshier-Hinton Foundation** and **The February Foundation**, we were able to produce this newsletter.

We hope you enjoy receiving our newsletter. However, if you no longer wish to be on our mailing list, please drop us an email: info@fopfriends.com

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Give with confidence

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