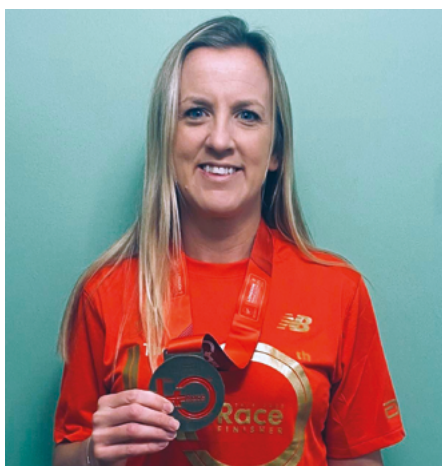




FOP Friends exists to **help find a treatment and a cure** for the rare genetic condition fibrodysplasia ossificans progressiva (FOP), and to **support the families** affected by it

These Girls Can!



We are incredibly thankful to these inspirational supporters who have taken on personal challenges to raise money and awareness for FOP.

Although their challenges took place across the country, as a team they have **raised over £7300** to support the research into a treatment and a cure for FOP.

See page 4 to read the stories behind their successes.

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A word from Nicky

Hello and welcome to the final issue of "FOP Friends Together" for 2020. And what a year it has been! For myself as an FOP parent, I must admit thinking at times, "welcome to my world" as suddenly no one is able to take health for granted. Despite living through these times of uncertainty, I am thankful the pandemic has highlighted the importance of kindness and living for each day.

As a Trustee, I am seeing first-hand the impact on charities like ours. Almost all events cancelled, and charity fundraising is limited to digital efforts (Facebook fundraisers, amazon giving etc) but also inspiring individuals like Becky, Fiona and Jane who took part in a virtual London Marathon.

We were excited to participate in the IFOPA's Family Gathering, which was the most global and inclusive FOP event yet. The Gathering, which is normally held in the US, went online so we were excited to be able to join as a family, meet with other families and learn the latest developments from the world's experts.

Thank you for your continued support, we could not do it without you. I hope you stay safe and well and wish you a Merry Christmas and New Year.

Nicky
FOP Friends Trustee

PS. Isla assures me that despite the pandemic, Santa has all his elves on track for the big day!

Clinical Trial Update

In early November, Regeneron put a hold on their Phase II Clinical trial for Garatasmab LUMINA-1 Trial. The company released a statement which can be read here: fopfriends.com/regeneron-update-november-2020/

The STOPFOP Trial has now starting recruiting in Amsterdam, and is hoping to start taking patients at the London site in early 2021. **Read the 'Life in Lockdown at Oxford University' article on page 6 to learn more.**

REGENERON
science to medicine®



Thanks to The Window Co

We were thrilled to be picked as The Window Company's Charity of the Year. However, fundraising didn't quite go as planned due to the lockdown! Thank you for all your support.



Lights, Camera Action!

Continuing the charity's goal of raising awareness of FOP, earlier this month, Chris took part in an educational teaching presentation as part of Peer Voice 'Let Their Feet Lead the Way'. Chris was filmed, along with Professor Keen and Dr Cheung (Canada).

The video covered the early identification of FOP, and the logistics of how patients are tested and referred to FOP specialists. The video, podcast and learning content will be distributed to 250,000 medical professionals, including endocrinologists, orthopaedists, and paediatricians, worldwide.



Thank you to the Rotary Club of Uxbridge



We were delighted to receive a donation from Uxbridge Rotary Club for £500 as part of their annual donation drive.

The Club has been a supporter of FOP Friends for many years now, after learning about the charity through one of their members who knows Isla's grandparents.

The Club enjoy holding fundraising events, principally an annual Autoshow which has been raising between £20,000- 30,000 pa but sadly could not take place this summer. They support local community and youth good causes as well as some abroad including donating to the Rotary worldwide effort to eradicate Polio. They are looking forward to getting back to a 'new normal' next year when they can resume their fundraising efforts. We are most grateful to the members for their continued support.



Jake Goes Virtual

This year's pandemic meant that our long-time supporter Jake was unable to hold his annual yard-sale to sell his unwanted toys, books and games to raise money for FOP Friends. However, Covid wasn't a match to stop Jake raising money for the charity that has been close to his heart for so many years. Instead, 14 year old Jake created his own virtual 'race' to support our race to find a treatment for FOP. Jake challenged himself to run 300 miles over the course of the lockdown, from his home in Surrey to 'Paris'. This impressive sporting achievement raised £100 for FOP Friends. Thank you so much for taking on this challenge! Merci beaucoup de tous nos Amis!



Amazing Amy

This November, Isla's friend Amy gave a presentation to her class as part of their Thought for the Week.

Amy and her family have long been supporters of FOP Friends. Amy explained FOP to her classmates and how it had impacted Isla's life yet never stopped Isla making the most of every day. Amy reflected on the fun times she has with her friend, and talked about how Isla is always there to cheer her on when she does dance competitions and shows like the 'The Wizard of Oz' with Surrey Youth Ballet. Amy's favourite thing to do though, is to make up dances with Isla - anywhere and everywhere! Amy explained to her friends that her friendship with Isla keeps her grounded and makes sure she makes the most of every day. Amy donated her pocket money to buy some wristbands so she could give them to her classmates at the end of her presentation. Thank you, Amy, for being such an amazing ambassador for Isla and FOP Friends.



Supporting our families: Putting an Ambulance Flag on your address

Did you know, if you have a complex medical condition, you can notify your ambulance service and have a flag put on your address?

If you suffer from any complex condition, such as a severe allergy or a rare condition, you can let your ambulance service know in advance what measure they need to take -or avoid - in order to give you the best possible medical care in the event of an emergency. Every Ambulance Trust has a different process, so we have produced a leaflet to explain the process for each Trust. Visit our website to download the leaflet, and get everything put in place before it's too late. There are also details on our website about how to inform the Fire Service if you have mobility restrictions that may need additional consideration in the event of an emergency evacuation.

Visit: www.fopfriends.com/supporting-families



These Girls Can!

After the fabulous achievements of the children in our last newsletter, these girls all stepped up to raise desperately needed funds for FOP research, but also enjoyed their own personal successes!

Sarah and Sinead's Virtual Bike Ride to Paris



Sinead writes, "On Saturday 19th September, Sarah and I set off on our 140 mile cycle to Winchester and back for FOP Friends!

Although disappointed that we didn't get to complete our London to Paris ride as planned with our colleagues, we were delighted to have the opportunity to complete a cycle to roughly match the distance from London to Paris.

We had initially hoped our fellow teachers would join us on the ride, but now with a limit of 6 people, Sarah and I decided to go it alone. Although we are both experienced cyclists, this was our biggest cycle to date.

We set off at 9am from Brixton, decked out in our finest bib shorts, clip-in shoes, laden with all sorts of cycling gadgets. We stuffed our bags with Trek bars and set off out of London. We worked our way through Clapham, Wandsworth and skirted around the wonderful sights of Richmond.

One dodgy turn however and we suddenly found ourselves whizzing down the hard shoulder of a motorway, desperate for some sort of sign to Staines! We veered left, did a few loops around an estate that we happened across, and then back onto the motorway! No amount of pleading with the Satnav seemed to work.

We pulled over to inhale a Trek bar and set off again - vans, trucks, cars, motorbikes all letting their thoughts be known to us as we trundled along the M4!

Soon enough, we got to Staines and I had never been so delighted to see my old stomping ground - the Debenhams cafe! I had spent many a Saturday frying breakfasts and chips in there, and now it was like some sort of mirage, we were nearly halfway to Winchester!

At the halfway point we were still feeling pretty good. Stopping for a loo break in a fairly hostile Lidl, we feasted on another Trek bar and set off for the next half. The last 10 miles were the toughest, so close yet so many hills to climb!!

We got to Winchester unscathed, and celebrated with a 0% Peroni - we also decided we would do Dry September (because we felt like we weren't being punished enough!).

The following day we set off, knowing the first part would be the most challenging - not only was the wind against us, but the big hills were waving us out of Winchester. Sarah had told me that there was a pretty hefty hill in the first 10 miles, but decided against telling me as we were approaching it - I have a tendency to throw hissy fits (and the bike) attempting pretty gruelling hills!! I was all the glad for it - at the impressive average speed of 4 miles an hour, we hoiked ourselves up the biggest hill of the ride. Cher, obviously blasting all the way! We took a quick snap at the top for the family, puffy and red-faced and made our way back home.

Weaving our way through Surrey, we were delighted as we edged closer to the Big Smoke. Never have we been so delighted to catch a glimpse of the concrete jungle A40! It wasn't the most scenic ride, alongside the A & B roads to Heathrow Airport, cycling amongst broken bumpers and road kill, but we were nearly home!

Having been fairly upbeat the whole way (Sarah had a bit of a meltdown outside the Medstead Co-Op earlier on) it was the last half mile that did me in. I seized up at the bottom of our road, and point blank refused to get back in the saddle - the agony!

Sarah pulled out every motivational quote in the book to get me going again, and with the promise of a curry takeaway, I eventually agreed to keep going. We clipped back into the pedals and hit the (very short!) road for the last bit of the ride. 140 miles, and £3,370 for FOP Friends, we did it!"

London Marathon Ladies!



Becky's story

Becky tells us, "I had a place in the London Marathon in April, which was postponed to October and then changed to a virtual event.

I completed a solo (my first) marathon on the original marathon date in April and then signed up for the Virtual London Marathon in the middle of August - leaving only 6 weeks to train!

I was determined to do both to raise as much as awareness and much needed funds as possible. I'm pleased to say I've raised over £3000.

I enjoyed the April one, but absolutely loved the October event (despite the horrendous weather). It was great having family out on the route to support and friends to run with.

Roll on the London Marathon in October 2021!"



Fiona's story

Fiona says, "For some crazy reason I entered the ballot for the London Marathon and was thrilled when I got a place in the ballot.

I hadn't run more than a couple of miles since I was pregnant with my eldest 9 years ago so I knew it would be a huge physical challenge as well as a logistical one, squeezing the training around our busy family life. But...as well as being one of the things on my to-do list, I wanted to use the opportunity to raise money for my friend's daughter Isla...and it was this little girl who gave me the inspiration and motivation to just do it!"



Jane's story

"Having taken up running a few years ago, I'd really caught the bug. I'd already done a half marathon for FOP Friends so had set my sights on a new challenge. And when I got the letter telling me I'd got a coveted place in the iconic London Marathon, I knew I wanted to race for a cure! For Oliver and all his friends. It was going to be my first FULL marathon! Eek! That's 26.2 miles! I knew I wasn't going to beat Kipchoge and his sub 2 hour record, but I could help the race to get a cure for FOP, which affects my good friend Helen's son, Oliver.

Oliver was born in the same hospital as our daughter Jessica just a few days earlier and is the 1 in 2 million child who is born with this genetic condition. Pretty tough luck, and makes you realise that any one of us could be affected by something like this at any time.

I was disappointed when the actual race was (rightly) postponed. Instead, I ran through my neighbourhood, doing laps of my local streets to make up the miles. It made the event even more special and memorable, having family and friends standing on the roadside, cheering me on, it really did make all the difference. I can't tell you the feelings of elation to have my mum at the window and all my supporters (socially distanced of course!) celebrating as I 'crossed' the finish line on my doorstep! Bring on 2022 when take to the streets of London to do the real thing!"



FOP on the TV

Tin Soldiers continues to be shown across the world, raising awareness of FOP and reaching out to the undiagnosed with hope of connecting them with medical professionals who can help to care for them.

There are many factual videos about FOP, where inspirational members of our community have shared their stories to help increase the profile of FOP. Visit www.fopfriends.com/video-library to check them out.

And then there are the TV shows where FOP has featured as part of the storyline, which always generates curiosity and questions. How many have you seen?

Casualty, 'Flutterby', S30 e8

The Good Witch, 'Surprise Me', S2 e5

Game of Thrones, (Greyscale is inspired by FOP)

Saving Hope, 'This Little Piggy', S2 e2

Grey's Anatomy, 'My Favorite Mistake', S3 e19

Bones, 'The Devil is in the Detail', S5 e14

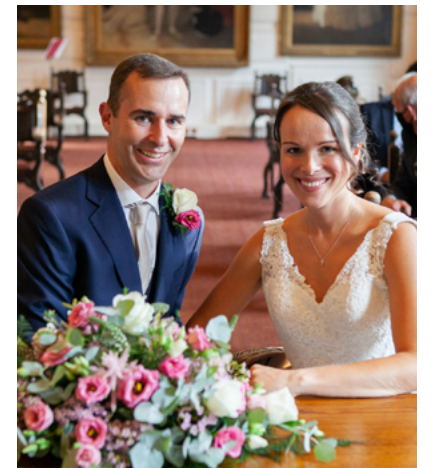
Congratulations to Mr and Mrs Muller

On 18th September, trustee Nicky married James Muller in Windsor

The pandemic meant that their original civil ceremony in April and wedding in May in Slovenia, had to be postponed. However, the day finally arrived and Nicky and James were able to tie the knot with close family in attendance in September, in a beautiful ceremony at Windsor Registry Office.

Nicky's father, Harvey, walked her down the aisle, with Isla and Hettie as her flower girls. James' sons Oliver and Theo served as his Best Men. The newly-weds had a simple 'honeymoon' in Rhodes with their daughters, but are looking forward to celebrating their wedding in style in Slovenia in 2021 with all their family and friends.

We wish them all every happiness as they start their new life together.



Life in Lockdown at the University of Oxford: An update from Prof. Alex Bullock



The University of Oxford research team is led by Professor Alex Bullock who works alongside Dr Eleanor Williams, Dr Jong Fu Wong, and with PhD student Liz Brown. We were delighted to catch up with Professor Alex and his team to learn about their work and the current impact of the Covid-19 pandemic. In July, Alex presented a webinar on the STOPFOP phase 2 clinical trial which is investigating ACVR1/ALK2 inhibition by saracatinib as a new treatment for FOP. A video recording of the webinar is still available to watch on their trial website at en.stopfop.com/

Professor Bullock, are patients able to enrol on the trial yet?

We are pleased to announce that patient enrolment has now commenced at the clinical site in Amsterdam, although opening of the London site is likely to be delayed until early next year due to Covid-19. A third site should open shortly in Germany at Garmisch-Partenkirchen. The investigational drug saracatinib has not been tested in children before. For this reason, we must limit our first trial to adults to demonstrate safety and efficacy before we can consider treating children. Families can make enrolment enquiries via the STOPFOP website.



How has FOP Friends made a difference to the discovery of saracatinib?

Our discovery of saracatinib as an ACVR1/ALK2 inhibitor was led by Dr Ellie Williams who has been supported by the kind donations from FOP Friends, as well as FOP France and the Associazione Noi ci Siamo (Switzerland). The rationale for our work is simple: if ACVR1/ALK2 is the faulty gene component responsible for FOP, it forms an obvious point to intervene with a drug to rebalance its biological activity to prevent FOP. In the webinar, we likened this to trying to put a gag in the mouth of the protein to stop it sending too many signals to make new bone. Ellie is also researching how the faulty protein becomes too talkative to help us to better understand FOP and to make the smartest drugs. There are often multiple ways of stopping a signal when it is unwanted. Consider switching a radio off at the power button or disconnecting the speakers – both stop the sound being produced but in different ways. Ellie has been solving the 3D structure of the FOP-causing protein ACVR1/ALK2 showing how drug fragments can bind to regions other than the mouth, which is bound by saracatinib (a scientific technique called fragment screening).

The motivation for this is to explore the potential for second-generation drugs with the highest level of safety.

Dr Williams, how has lockdown affected your work?

Over the first lockdown, the labs were temporarily closed to all work unrelated to Covid-19. This didn't mean a stop to the FOP work for me though, as I was working on analysing a lot of data I'd collected before the lockdown on a project looking for an 'allosteric site'.

What that means is that I've been hunting for a second off switch – if we imagine our radio analogy again, the main on/off switch is the same as our main medicine binding site and that's the one we've been mostly working on.

However, we can also think about the cables at the back of the radio as a second potential 'off' switch. I've been working on trying to identify molecules that will bind to the back of the protein and switch it off from there – because this is a new approach we don't have much of an idea what shape molecules we need to bind to the back of the protein. Instead what we've done is try and bind lots of very tiny bits of molecules to the protein to see what sticks and from that we can try and build something bigger that works more like a medicine. This is a bit like taking lots of small Lego bricks that fit in the gap and linking them together to make the perfect shape. It's not something that I've studied before so there's a lot of ground work to do on it but it's looking quite exciting.

Dr Wong, what role do you play in the team?

I am the newest member and joined the team in Oxford from Singapore as a senior researcher, working on developing new drugs against the ACVR1/ALK2 protein, and researching the wider implications for conditions other than FOP

So the research into FOP helps to further knowledge and understanding into other conditions?

Yes! As well as FOP, drugs against this protein are now considered a promising approach to fight the lethal childhood brain cancer, Diffuse Intrinsic Pontine Glioma (DIPG). I have received funding from The Brain Tumour Charity in the UK to develop ACVR1/ALK2 inhibitors that can cross into the brain and therefore may be of therapeutic value for both FOP and DIPG.

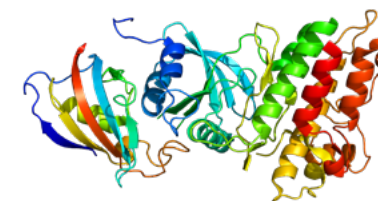
Working in an international alliance, I have tested over 400 new drug-like molecules. Dozens of potent and selective ALK2 inhibitors were identified. In conjunction with in vitro and in vivo pharmacokinetic studies, the alliance has selected two of these as lead molecules for further development towards a clinical drug. Both compounds have excellent blood-brain-barrier penetrance, oral bioavailability and other drug-like characteristics. They also show some promising effects in killing some subgroups of DIPG patient-derived cells.

And finally, Liz, tell us a little about your role:

My role in the team is generously supported by an Oxford-The Simcox Family Graduate Scholarship.

You may well know about the genetic code captured in our DNA, but there is also a more recently described epigenetic code. This is determined by proteins in our cells that can change whether genes are tightly packed away and inactive, or open and active (similar to a book either being open and read, or hidden away in a bookcase).

This second code may contribute to the variation seen in how FOP may develop in different individuals. It is also forms a further biological system to consider for drug development.



How does this link into the research by others in the team?

I am studying whether this strategy could benefit both FOP patients and those with the childhood brainstem tumour DIPG. I tested 60 drug-like compounds in the laboratory and found a small subset that can kill DIPG cancer cells that have the same faulty ACVR1/ALK2 gene that is found in FOP, even at low doses.

I'm planning to investigate these further in FOP cells and to test directly whether they block the messages we think cause the unwanted bone formation.

Professor Bullock, is there anything else you would like to add?

We are fortunate to be able to continue our research work under the new lockdown with only minor disruption, but like everyone we wish for a very different 2021.

A big thank you again to everyone across the UK and beyond who continues to support FOP Friends and the research community. A safe and happy holiday season to all.

What is FOP?

It may be easy for us to assume that because you read our newsletter and follow us on social media, that you know all about FOP.

However, it is a complex condition which has more questions than answers. We have produced an easy to read guide 'FOP Fibrodysplasia Ossificans Progressiva: the essential facts' and you can download it for free from the website.

It is a useful resource to share with others, to explain the condition. Visit www.fopfriends.com/supporting-families and check it out.



Our contribution to fighting Covid-19

As you may have heard in the news, the University of Oxford has been heavily engaged in the fight against Covid-19 by developing a vaccine, in phase 3 clinical trials, antibody testing kits, which identify if someone has the virus – as performed now at Heathrow airport – as well as other drug strategies for Covid-19 treatment. The development of these approaches has required help across the University. Jong Fu and Ellie explain, "We worked as volunteers in the production of the SARS-CoV-2 spike protein that is used in serological tests to identify the presence of antibody against the novel coronavirus. Serological tests are vital in understanding the prevalence of past infections and the persistence of antibody production in the body after recovering from infection. The effort requires a lot of spike protein material to do all the tests that were needed so it was all hands on deck, to make enough of the spike protein to cover demand. Although not trained as virologists or immunologists, we helped to improve the spike protein production efficiency and workflow by utilising our expertise in cellular biology and structural biology."

Christmas is Coming!

After perhaps the most challenging year most of us can remember, Christmas is nearly upon us!

We still have no idea what Christmas is going to 'look like' but there are still ways you can support FOP Friends without leaving your home!

Tell us your story

If you have a story to share about your life with FOP please get in touch. We'd love to hear from you.

We would also like to shout about any of your achievements and challenges you've done for FOP Friends.

Thank you to the following organisations for their generous support in funding the FOP Friends Together newsletter:

The February Foundation

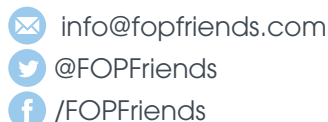
The Manchester Guardian Society Charitable Trust

The Sobell Foundation



FOP Friends

The Cabin, 1 Cumberland Road,
Sale, Cheshire, M33 3FR.



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

Be a #XmasPartyHero

Has Covid cancelled your Christmas party? Why not ask your employer to donate part or all of the cost of the party to FOP Friends and become a #XmasPartyHero?

Visit www.xmaspartyhero.co.uk for more information.



Sending a Christmas greeting



Our Christmas cards are now on sale. You can buy them through eBay, or get in touch. Packs are £5 for two packs ten cards, with free postage.



Don't want to send a card? No problem! You can send a Christmas greeting through Facebook and make a donation through our Virgin Money Giving page.

Visit our website:
www.fopfriends.com/christmas-2020

Let your Christmas shopping benefit FOP Friends



If you aren't able to go out to your high street and have to shop online, please consider using easyfundraising.co.uk and choosing FOP Friends as your charity to benefit from your purchases?

We are also registered as a charity with Amazon Smile so please remember us when you click to buy your Black Friday and Christmas bargains.

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