friends® Together
Vol. 8 Issue 1 Spring 2025

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

Inside

DATE: 7/02/2025
PAY: FOP
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L-R Marry Barrett, Dell Ford, Phyllis Brosnan and Patricia Donnelly.

Kilburn Ceili Warms a Winter's Night

February can be a cold, dark affair, but the chill was held firmly at bay in Kilburn, London, at the Quex Road Ceili. There was nothing but warmth indoors as friends and dancers gathered at the fundraising event, in honour of Isla and her friends with FOP.

The Ceili was organised by our long-time supporter Phyllis, with friends Patricia and Dell. There was laughter, lively music, and leaping as people danced the night away, under the guidance of Brian Deveraux and Tom Kelleher.

Live music and vocal performances, provided by the talented young members of the Parish Ceili Band, really made the evening special, while a grand raffle added to the fun and excitement.

Thank you to everyone, especially Anna and Mags for the use of the room, for their tireless work in creating such a wonderful evening. And a special thanks to all our dancers for their generosity and enthusiasm! The event raised an amazing £1937.

A word from Rachel

Hello 2025! January and February may have felt endless, but I'm happy to report I've noticed many signs of spring on my recent runs, giving me hope that winter is finally behind us. You might be wondering, "Are you running for a reason?" Well, yes, I am!

In a moment of madness last year, I signed up again for the Manchester Marathon in honour of Oliver. Setting up my fundraising page and taking on some longer runs has given me the opportunity to reflect on how far we've come as a charity and community, raising awareness of the condition and helping the families affected. The training period and longer distances feel like a metaphor for our race to find a treatment and cure for FOP and for the work the charity has carried out over the last 12+ years. It's a long road and sometimes the goal feels like it's getting closer, sometimes we feel like it's still far off in the distance. What doesn't change is the determination that I, the other trustees and the community feel to keep going, and keep pushing on, no matter how hard, to find that treatment and get the support the community needs. Fundraising is tough right now and everyone is feeling the pinch but I do urge you to keep trying to find ways to raise funds so we can continue in our work to support families and research. And if you happen to be in Manchester at the end of April, please do come and cheer me on!

Rachel, FOP Friends Trustee

Research Roundup

Dr Andrew Rankin

In the previous two newsletters I highlighted the report, published early in 2024, around the unique FOP patient who was almost devoid of symptoms and bone formation in his early 20s, seemingly shielded by the lack of an inflammatory trigger.

It turned out that an important inflammatory enzyme produced by inflammatory cells was extremely low in this individual. That protein was MMP-9, an enzyme that is produced by inflammatory cells; and it turned genetic sequencing discovered that the patient had a mutation in the gene controlling MMP9, in addition to the FOP mutation in ACVR1. Blocking MMP9 pharmacologically was then demonstrated to prevent heterotopic bone formation in the FOP mouse model and all this information led to Ashibio, a California based start-up Biotech, planning a new Phase 2 clinical trial of a humanized antibody that specifically inhibits MMP-9, Andecaliximab, in patients living with FOP.

The good news is that this ANDECAL clinical trial is now recruiting patients at Mayo Clinic, Minnesota (Bob Pignolo) and UCSF, California (Ed Hsiao) with two other sites in the US open but not yet recruiting. ANDECAL is targeting recruitment of 92 patients and predicts completion in 2029 so we have a while to wait to see if this entirely novel approach can offer an alternative therapeutic possibility to the current agents in clinical trials targeting the ALK2 receptor.

There is also a small (11 patient) preliminary study in patients with severe FOP to observe if the off-label use of anti-IL1 therapies as rescue therapy, such as anakinra or canakinumab, can block flare activity and heterotopic ossification in FOP. Anti-interleukin-1 (IL-1) therapy is a treatment that blocks the activity of IL-1, a cytokine that causes inflammation and has been shown to be effective for treating conditions such as rheumatoid arthritis and gout. This study at UCLA is estimated to complete in 2027 and if successful could lead to a formal Ph2 clinical trial.

All other interventional clinical trials, ongoing but not yet completed, target different elements of the mutant Activin Receptor-like kinase 2 (ALK2) encoded by Activin A receptor type I gene, ACVR1 (Table).

Investigational Drug	Company	Mechanism of Action	Trial Name	Phase	Estimated Completion Date
Garetosmab	Regeneron	Activin A mAb	OPTIMA	3	2025
Zilurgisertib	Incyte	Kinase inhibitor	PROGRESS	2	2028
Fidrisurtib	Ipsen	Kinase inhibitor	FALKON	2	2029
Saracatinib	Amsterdam UMC	Kinase inhibitor	STOPFOP	2	2025

The Bone-a-Fide Group

With FOP being so very rare, it's unlikely there is someone nearby who also has FOP and truly understands what it means to live with the condition. It can also be tough when getting out is difficult.

It can sometimes feel very isolating. Hamish felt like he wanted to connect more with others, so had the great idea of setting up a group exclusively for adults 21+ living with FOP in the UK. So, without further ado, we present…The Bone-Afide Club. The aim of the club is to create a friendly and support space where people can connect and share experiences. The group is all about fun, friendship, and everything in between – the frightening and the fabulous!

If you would like the link to join, please just get in touch!

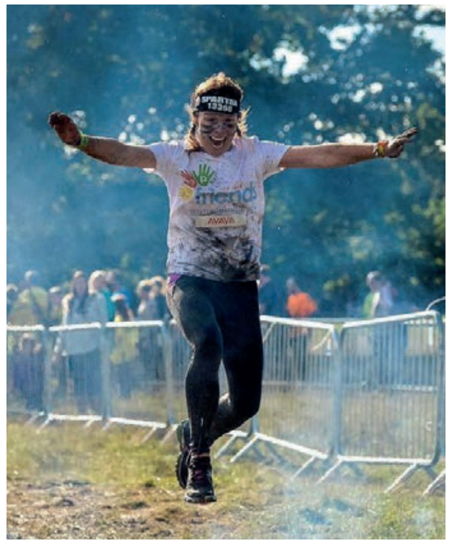
Thank you Nicky

It is with sadness and gratitude we announce that Nicky has stepped down from her role as a trustee at FOP Friends. Nicky made the difficult but necessary decision to focus her time and energy where it matters most: her gorgeous family.

Nicky has been a trustee since 2015. Ever since her daughter, Isla, was diagnosed, Nicky has been a tireless advocate for everyone living with this rare and incredibly challenging condition. Her tireless efforts to raise awareness, fund vital research, and support families affected by FOP have truly made a lasting impact.

Over the years, Nicky has been such a key part of everything we have done at FOP Friends. She's assisted with conferences, family weekends, and countless other events, offering support, sharing her experiences, and connecting with other families who understand what it's like to live with FOP.

Nicky has also been a powerful voice, sharing her story of the challenges and realities of living with FOP in the news and on tv, from newspaper articles and webinars to sitting on the famous couch of This Moring. Nicky has been open and honest, not only highlighting the challenges that FOP brings but also celebrating Isla's many incredible achievements and milestones. Her willingness and courage in sharing both the highs and lows has inspired countless people and helped to highlight the importance of our cause. She and Isla have both been true inspirations.



We also want to give a shout out and our thanks to Nicky's wonderfully supportive network of friends and family. Their generosity and commitment through Friends of Isla have raised over £200,000—an extraordinary achievement that has helped to fund critical research and made a real difference to FOP research and support programs. There have been parties, clothes swaps, cycles, walks, runs and more!

While we're definitely going to miss Nicky on the board, we're so happy that she, Isla, and her wonderful family will always be part of the FOP Friends family and community. We look forward to continuing to share in Isla's adventures and celebrating her many successes in the years ahead.

From everyone at FOP Friends and from all those living with FOP, thank you, Nicky, for everything you've done and continue to do. You've made a difference that will never be forgotten.

8 August 2014

Community News

Gym users in a spin to support baby Isla

Fundraisers help charity that supports children with rare conditio

By Fergus McEwan

Rencontrez nos amis!

FOP Friends and FOP France have long been friends and supported each other. FOP France has made many donations to the University of Oxford's research team through us, so today we shine a light on one of our partner patient organisations. We chatted with Marie-Emmeline, President of FOP France, and Laurent Gouy, Treasurer of FOP France to find out more about our friends across the Channel…

How and when was FOP France founded?

FOP France was founded in 2012 by Marie-Emmeline and Antoine Lagoutte, after their son Alexandre was diagnosed with FOP. They wanted to create an organisation to support others as well as research into a treatment for their son. There was no organisation in France at the time, so FOP France was created. Over the years, other families affected by FOP have been eager to get involved and contributed to the success of the association. Their aims are to unite families, raise awareness of FOP, and support innovative research and treatments.

Tell us a little bit about how you support the FOP community in France:

FOP France holds a biennial conference to bring together FOP patients and their families for a day filled with activities and a friendly atmosphere. There are presentations by researchers and medical professionals; workshops; and advice, tools and tips for living with FOP.

FOP France sends out an annual journal called 'Les Echos', which keeps members and supporters up to date with the year's events.

Through fundraising efforts, FOP France is delighted to have supported travel costs for patients to take part in the STOPFOP trial in Amsterdam.

FOP France also hosts online groups to keep families connected, and offering video calls to help people feel less isolated. We have also worked on a number of projects to keep our families informed and supported including a children's story book 'La Prophétie de Dysplasia'; a guidebook for young adults; a video to raise awareness of FOP and our organisation on social media; and a feature film for adults entitled 'Mes Frères'.

Who are your FOP specialists in France?

We have two leading FOP doctors in France. Dr Geneviève Baujat is our paediatrician, and also a member of the ICC for FOP, with Professor Thomas Funck Brentano leading on adult FOP carer. We are also fortunate to have Dr Lisa Friedlander as our FOP specialist dentist.

As supporters of the FOP team at the University of Oxford, we're really thankful for your donation. What inspired you to support the team there?

We're delighted to support the Oxford University team, primarily because of their central position within the international FOP research community. The guidance of their mentors, Professors Jim Triffitt and Alex Bullock, to whom we express our sincere appreciation gives us great confidence that their work represents a truly impactful and reliable way to advance research in this important area.

Are there any particular moments that have stood out for you?

A defining moment for the FOP France association was our first seminar organised in 2016. This gathering, a mixture of apprehension and excitement, was an essential step in building a supportive community, united by an extraordinary human adventure and shared vision.

How do you see the future of FOP support and research evolving?

We are going through an important and exciting time for research, with a number of clinical trials in progress. In light of the massive investments being made by pharmaceutical companies, it is essential that FOP France alongside them, clearly defining its actions to complement and effectively support the research and the patients involved.

With this in mind, the association has taken a major step forward with the creation of its Scientific Council in 2022.

What's next for your organisation? Do you have any exciting plans on the horizon?

During 2025, FOP France hopes to be a sponsor of the Transat Café L'Or (formerly the Transat Jacques Vabre), the longest and most demanding transatlantic yachting race! We are hoping to support a team of two skippers with the aim of raising public awareness of FOP on an international scale!

We are also organising our next conference in Paris. We are also hopeful that France will be considered as a host country for the DDF in the coming years.

Lastly, do you have a message you'd like to share with the global FOP community?

The Palovarotene experience has shown the need for a united international community to make health organisations understand the vital urgency of obtaining a first cure as quickly as possible.

It is up to us to speak up for patients!

How can our supporters follow you?

Facebook / Instagram / Twitch (in development) and www.fopfrance.fr

If you could sum up your organisation in three words, what would they be?

Hope, support, research

Andrew at Oundle School

Through a connection at Oundle School, Northamptonshire, FOP Friends trustee Dr Andrew Rankin was invited to speak about his experiences in developing therapies for people living with rare diseases to the school's Sir Peter Scott Society in November 2024.

The Scott Society is a pupil led society focusing on the STEM subjects, with a committee of pupils being responsible for inviting and organising speakers throughout the academic year. The school has been governed by the Worshipful Company of Grocers of the City of London since its foundation by Sir William Laxton in 1556.

The school accommodates more than 1100 pupils, generally between the ages of 11 and 18.

It is the third-largest boarding school in England after Eton and Millfield.

It's alumni – known as Old Oundelians – include entrepreneurs, scientists, politicians, military figures and sports people.

The invitation offered the opportunity to educate potential future leaders about the science and medicine of rare disease, but more importantly to raise awareness around the issues facing the rare disease community in the UK and, in particular, people living with FOP.

The audience consisted of around 50 students in the 5th and mainly 6th forms, both from the UK and around the globe. The students were very welcoming, engaged and demonstrated interest with multiple questions, both during the presentation and afterwards in a small group.

Andrew found this a rewarding experience and challenging to develop a presentation and speak with a smart group of young people as opposed to his usual audience of university students, clinicians and scientists; it is always good to get the message out there to new audiences and Andrew hopes there may be other opportunities to raise awareness particularly with young people at school and colleges.

Everyone's Best Friend!

Many of our friends and supporters have a furry friend as part of their family, but for those living with a disability, they are much more than just a friend.

Service animals play a vital role in the lives of many people with disabilities, providing invaluable assistance and promoting independence, with a bond so deep it's impossible to quantify. These furry friends are companions, confidence and cherished members of the family.

Some families welcome a dog or cat, simply as a loving addition to the home. But for others, they may decide that a dog will bring other benefits to the family and support someone to live more independently. This is a big decision and it's important to make sure you do plenty of research before taking on this responsibility. We have gathered together some information as a starting point for any of our families who may be considering it as an option. **Visit: fopfriends.com/assistance-dogs/**

We asked some of our families to share what made their furry friend so special…

Isla and Ralph

Ralph is a 3-month old Maltipoo. He is being trained to be Isla's assistance and therapy dog. He is only three months old but his training to support Isla is already in full swing! We have taken him to cafes, the pubs, and for walks around the village. He seems to be taking everything in his stride and is enjoying all the attention he gets! Ralph gives the best snuggles and cuddles and his companionship has already made a world of difference. We're so excited to have him.

Brodie and Winnie

My name is Brodie and My Dog is called Whinnie. Whinnie is 2 years old. She is a miniature sausage dog. Whinnie cheers me up and makes me so happy. She loves to sit on my lap and give me lots of cuddles. She's is crazy and so funny barking at the postman every day. She is my best friend!

Rachel and Vada

Vada, my two-year-old Imperial Shih Tzu pup, has been by my side since she was tiny. I named her after Vada from My Girl—one of my all-time favourite movies. She's truly one of my best friends, and I miss her terribly whenever we're apart. We love going for walks together (though she's not a fan of the rain!), and she especially enjoys our trips to Wales in the caravan. Cuddling up with her is the best—she's wonderful company and always makes me laugh.

Alanna and Blaze

My name is Alanna and my cat is called Blaze. She makes me happy. She is a great comfort when I am sad. She loves cuddles and she loves to play. I have enjoyed watching Blaze grow from a kitten. I have learned how to care for her, feed her and clean her litter box. Blaze is the calmest cat, with the loudest purr. Blaze keeps me calm and makes me happy when I am sad. She is the most patient and cuddliest cat with the softest fur coat. I love my cat.

Louis and Henri

Louis and Henri are the pampered pooches of Liam. They are both cockerpoos. Louis is 6 years old and Henri is 2. Liam has had both his dogs from being puppies, although Henri was actually a rescue pup. Louis and Henri are much loved members of the family and are real bundles of energy! They adore adventuring the outdoors in the British weather with friends.

Luciana and Spangle

Hi, I'm Luciana. My special friend was Spangle, a beautiful English Springer Spaniel. Sadly, Spangle went over the rainbow bridge in February. She was my constant companion and loved beyond measure. She fully understood my needs and was always so gentle. She understood I couldn't bend, so learned to drop her ball on the sofa so we could still play - she even knew to stand her front paws on the bottom step of the stairs so I could tickle her ears or put her 'necklace' on. She was able to pick things up for me too. Spangle had a way of always making feel better with her sixth sense of knowing when I wasn't feeling great. She loved cuddles and was an absolute blessing when I felt anxious. I will miss my gorgeous girl, forever in my heart.

Charlie and Duke

Charlie's best friend was Duke, a gorgeous black poodle. Charlie had got Duke when he was just a seven-week-old ball of fluff and curls. They were inseparable, going everywhere together. They played, went for walks, and Duke gave the best cuddles - always knowing when Charlie needed one. They were always up to no good together. It took Duke over a year to adjust to life without Charlie, and he still misses him to this day.

Welcome Kyle

FOP Friends, along with FOP France, are delighted to be able to support research into FOP at the University of Oxford. The team, led by Professor Alex Bullock, continues to evolve over time as staff and students graduate to new training positions and career promotions.

From March 2025, the team will comprise of two PhD students, Will Seaton-Burn (3rd Year) and Kyle Ferguson (1st Year), both supported by an Oxford-The Simcox Family Scholarship. The students are supervised by Professor Bullock and Dr Ellie Williams.

Postdoctoral researcher Lap Hang Tse ("Carrie") left the team at Christmas for a new job back in her home of Hong Kong. Postdoctoral researcher Julien Cros is also leaving the team on 25th Feb for a new job in his home country of France. They have been valued team members over the past two years or more, contributing both to their own research and to the training of our PhD students and will be greatly missed.

The FOP research is now based in the Nuffield Department of Medicine Research Building, located close to the Churchill Hospital and Nuffield Orthopaedic Hospital.

Currently Kyle is working on finding new treatment options for FOP. He is studying a type of drug called bi-functional degraders, which are designed to do two things: to block the harmful protein, ACVR1 that causes FOP, but also help the body get rid of it completely. This is different from current FOP drugs which mainly just stop the protein from working. This new pathway is important because it offers a different and potentially more powerful way to manage FOP.

We invited Kyle to introduce himself:

> Growing up in the Cheshire countryside, ideally situated between three of the UK's best national parks, I developed a love for outdoor adventures- especially wild camping in the Lake District, where I have slept on mountain tops, tiny islands, crowded bothies, and even caves. Since Oxford is not the most mountainous region, my free time is now spent going to the gym five days a week (only to undo all my progress with wine on the weekends). Embracing the Oxford culture, I've even taken up rowing, a sport that demands very early mornings and has quickly reminded me of my past neglect of cardio.
>
> Before joining Oxford, I completed a Bachelor of Science in Biochemistry at Imperial College London, graduating in 2023, and then pursued a Master's by Research in Structural Biology there the following year, focusing on substructures within the mitochondria and drug discovery. Now, as a DPhil student on the FOP team at the University of Oxford, my research aims to explore and validate the use of bi-functional degraders as a potential therapeutic approach for FOP. Bi-functional degraders, such as PROTACs and LYTACs, aim not only to inhibit the FOP-causative protein ACVR1 but also to target it for destruction by the body's own cellular machinery. This dual-action approach could potentially offer advantages over existing FOP drugs, which primarily work by blocking the protein's activity. By actively degrading ACVR1, bi-functional degraders may provide a more effective and sustained method to manage the disease.
>
> Last February, I had the opportunity to attend the Medics4RareDiseases event in London, where I got to hear inspiring stories from the FOP Friends charity and gained valuable perspectives from patients, researchers, and clinicians alike. Hearing these voices reinforced my commitment to this research and to finding innovative therapeutic options that could genuinely improve the lives of those affected by FOP. Finally, I am especially grateful for the funding I receive from a Simcox Family Scholarship, which makes my DPhil studentship possible.

Christmas Light Spectacular

The festive spirit is shone brightly on Chestnut Drive in Sale, Cheshire, thanks to the incredible Christmas lights display which has become a beloved local tradition! This year's switch-on was a truly special occasion, with Mayor Amy Whyte doing the honours and illuminating the street with festive sparkle.

What began as a few houses with their twinkling lights has become a truly magnificent spectacle, which many people drive miles to enjoy.

The switch-on event was a busy affair, filled with laughter and festive cheer. A special thank you to the Sale Rotary Club for bringing along the jolly man himself, Santa Claus! Even the Grinch couldn't resist the festive atmosphere and, we're happy to report, was on his best behaviour and quite the amiable fellow, even posing for photos when asked!

The wonderful residents of Chestnut Drive put in all this effort not only to brighten the dark winter nights but also to support some incredibly worthy local causes. Visitors enjoying the festivities are invited to make a small donation, with proceeds this year going to one of the Mayor's chosen charities. And, as you know, we are absolutely deLIGHTed to be one of her charities!

A huge, heartfelt thank you to all the residents of Chestnut Drive. Your generosity and willingness to go the extra mile to bring sparkle to the season and support those in need is truly amazing! You are all superstars!

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

A Creative Christmas

Our talented supporter Heather has been turning her creative talents towards creating gorgeous Christmas keepsakes.

In the run up to Christmas, Heather was busily painting portraits of furry family members onto baubles and cushions. In lieu of payment, Heather invited her customers to make a donation to FOP Friends in honour of Isla.

Hemel Hampers!

A huge thank you to Dave and Emily Boggins, and their daughter Steph, for organising a fantastic hamper event across Hemel Hempstead football club and their two pubs, the Greenacres Taverns in Hemel Hempstead.

Emily created three wonderful hampers filled with delicious treats, which were raffled off at the three venues. An incredible £884 was raised for FOP Friends in honour of Lexi. The Boggins family are longtime friends of Lexi's grandad, and their pubs have hosted numerous events to raise money and awareness for FOP Friends. We are so grateful to the Boggins family, their customers, and supporters for their continued friendship and generosity.

Lexi with her grandad and Sarah