fop friends®

Together

Vol. 8 Iss. 3 Autumn 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

A Weekend on the Wild Side

This September, we were delighted to host a Family Safari Weekend, bringing together many of our FOP and POH families. Thanks to a generous grant from The Russell Lang Charitable Trust, we were able to provide an unforgettable three-day event in Kidderminster for both new and returning families.

The weekend began on Friday evening with an informal drinks reception at the Premier Inn. It was a lively start, filled with conversation, crafting, laughter, and an energy that quickly made families feel at home. A good night's sleep was then in order, ready for our weekend ahead…

A word from Alison

With it recently being Trustees' Week, I've taken a moment to reflect on what it means to me to be a trustee: the highs, the lows, and everything in between.

If I'm honest, I wish I didn't need to be a trustee. I wish my sister and her boys didn't have to live with the relentless pain and challenges that FOP brings every single day. I wish they didn't have to fight so hard for the most basic of needs or the smallest bits of support that so many of us take for granted. The constant advocating, the endless forms, the conversations that shouldn't have to be had - it's exhausting, and it's unfair.

So being a trustee is, in part, my way of channelling that frustration into something positive. It's about doing something, however small, to help make things better, not just for my family, but for all the FOP families living this same reality. There are many moments that make this worthwhile. The biggest high for me is the community, as you'll read further on about our safari weekend. Being part of a group of people who get it is something special. And we do have a laugh along the way!

Together, as trustees, we're able to make a difference, even if it's just in small steps. Knowing that we're helping families feel less alone, that we're contributing in some way to improving lives, raising awareness, and inching ever closer to that longed-for cure - that's what keeps me going.

It's a privilege to serve alongside such passionate people; all the trustees of your charity, past and present, inspire me!

Alison, FOP Friends Trustee

Research Round-Up

Exciting News! Anticipation has been building since both Regeneron's OPTIMA and the STOPFOP clinical trials completed this past summer. And last month, the FOP community received some incredibly positive preliminary news…

In September, Regeneron announced very positive results from the Phase 3 OPTIMA trial in adults (18 and over). This study involved an Activin A monoclonal antibody called garetosmab.

The OPTIMA trial was a pivotal trial. This means it was the final major study required by regulatory agencies like the US FDA and UK MHRA before a drug can be approved for marketing.

OPTIMA has been described as "showing garetosmab to be the first and only experimental therapy to bring a dramatic reduction in the number and volume of abnormal bone formation (HO) in people living with FOP".

You can read the full press release from Regeneron here: **newsroom.regeneron.com/news-releases/news-release-details/regeneron-announces-positive-phase-3-trial-adults-ultra-rare**

In summary:

- Two doses (3 and 10 mg/kg) of garetosmab have proven to be highly effective in reducing the number of new bone lesions by greater than 90% compared to placebo.
- It achieved a reduction of more than 99% of the total volume of new bone injuries.
- The higher dose of 10 mg/kg also reduced painful flare-ups by 89%.
- The positive impact was such that the Independent Data Monitoring Committee recommended that patients who were treated with placebo on the clinical trial switch to garetosmab as soon as possible to obtain benefit.

Safety and next steps…

The reported adverse events were similar to those seen previously with garetosmab, including nosebleeds, as well as skin and soft tissue infections, which were greater at the higher dose. However, none have been yet reported as serious.

Regeneron plans to submit this data to the FDA by the end of 2025 to seek US marketing approval, with submissions outside of the US following later in 2026. We do not yet know the timings to seek marketing approval in the UK with MHRA.

A critical Phase 3 study of garetosmab in adolescents and children with FOP, called OPTIMA 2, is also planned for 2026. Early intervention in children is critical to avoid the severe progressive impact of FOP from a young age, making this trial paramount.

While we should remain optimistic, we must be cautious as Regeneron has only posted highlights. The regulatory agencies still need to review the full data in order to make a decision on marketing approval.

In other news…

We now look forward to learning the outcome of STOPFOP, hopefully before the end of 2025. This study treated patients with saracatinib, a different type of drug known as an activin kinase inhibitor. Another positive result with this different mechanism would be a real bonus.

Two other clinical studies with similar activin kinase inhibitors (PROGRESS and FALKON) remain active and ongoing. Finally, the ANDECAL clinical trial studying andecaliximab, a humanised antibody that specifically inhibits MMP-9, is still ongoing in the USA only.

We look forward to bringing you further updates in due course as things progress.

For more information on current clinical trials, and for research updates, visit: **fopfriends.com/research/** or scan the QR code.

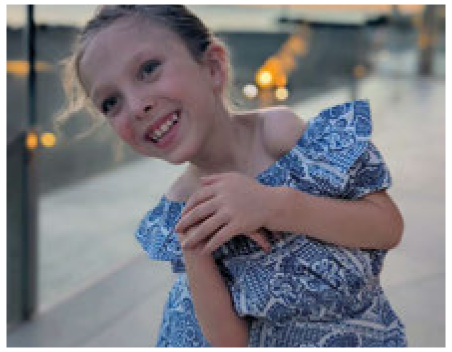
If you have any questions, please reach out ✉ **info@fopfriends.com**

Inspirational Isla

We are absolutely delighted to share that Isla Williams has been honoured with the prestigious Emerging Young Leader Award in this year's IFOPA Jeannie Peeper Awards.

We have watched Isla blossom from a determined toddler into a remarkable advocate for everyone living with FOP. She truly lives by example, embracing every opportunity she is given… we all remember her incredible gymnastics routine! She enjoys a wide range of activities, from swimming, dancing, and horse riding, to quieter moments spent crafting and writing. These days, she is kept very busy training her puppy, Ralph, who will become her companion and assistance dog.

Isla and her friends have been tireless fundraisers for FOP Friends, generating much-needed funds and vital awareness. Additionally, Isla has taken her story to the famous couch on This Morning to share her experiences and will also be appearing on BBC Newsround in the coming months. Her determination hasn't gone unnoticed.



Last year alone, she received the Quest Riding for the Disabled Zac Galt Memorial Trophy for her perseverance, and the Junior Windlesham Parish Council Community Award for her awareness efforts.

Isla's message is clear: **"I want people to be kind, curious, and supportive, not just to me, but to everyone who is living with FOP."**

Anyone who has had the privilege of meeting Isla knows she is always the brightest star in the room. Congratulations on your well-deserved award, you make your family and friends incredibly proud.

To find out more about all the incredibly worthy 2025 winners, visit: **www.ifopa.org/2025-winners**

Chris Attends StopFOP Stakeholder Meeting

Last month, Chris flew to the Netherlands for a meeting hosted by FOP Stichting for StopFOP stakeholders.

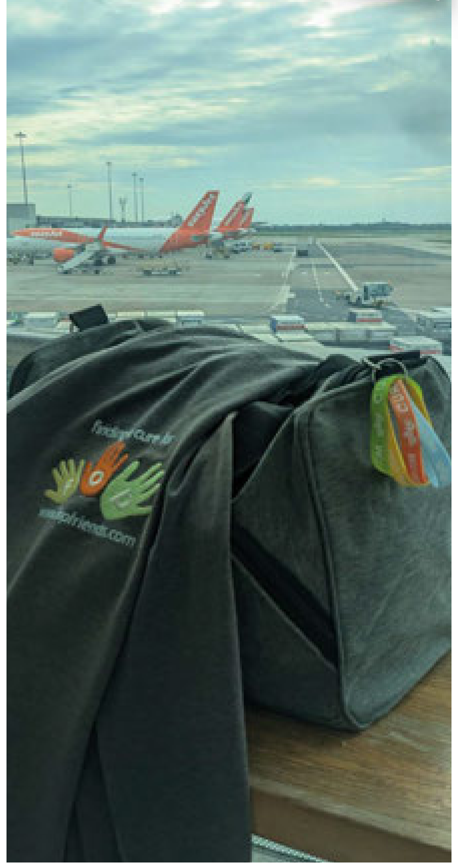
Researchers gave updates on the latest developments regarding the Saracatinib drug trial, which is the potential treatment discovered by Dr Eleanor Williams at the University of Oxford.

Chris was pleased to hear about these exciting and hopeful times for the FOP community as we continue to seek an effective treatment for FOP.

The research into Saracatinib was funded in part by families, so it should be noted that this progress wouldn't be happening without the commitment of those families, along with the fundraisers, researchers, and doctors who have made it happen.

Chris also enjoyed meeting up with other FOP patient groups, advocates, and medical professionals. The opportunity to network with others on the same journey is always invaluable and leads to closer connections.

We thank FOP Stichting for hosting the event, as every meeting takes us one step closer to a treatment and cure for FOP.



Oscar's 100th Park Run

Oscar, who is good friends with Harry, has been a keen runner for many years, enjoying taking part in Park Runs on a Saturday morning.

As he was approaching his 100th run, he decided to make it count even more – by running in honour of Harry! Not only did Oscar complete his 100th 5K, he got a shoutout over the tannoy, and he raised £314. We all need a friend like Oscar!

Safari, Surprises, and Celebrations

Saturday was all about the animals at West Midlands Safari Park! After a hearty breakfast, our families headed off to West Midlands Safari Park. We gathered at the gloriously eccentric safari-themed Spring Grove House, with its safari-themed décor which also served as a quieter hub for hot drinks, crafts, and friendly conversation.

The safari drive was a great fun (with the giraffes definitely stealing the show!). After a delicious buffet lunch, we managed to sneak in a surprise birthday celebration for trustee Andrew who had chosen to celebrate his birthday with us! After lunch, families explored the walking safari attractions, enjoyed the antics of the lorikeets and on-form hippos, before bravely tackling the theme park rides. That evening, families dressed up for a truly memorable reception at Stone Manor. It was a wonderful celebration complete with dancing, eating, teddy-bear making, balloons, and what felt like the world's longest (but most exciting!) raffle. Watching the children play together was the absolute highlight.

Sunday brought a calming, beautiful end to the weekend with a gentle stroll around Arley House and Arboretum. The beautiful golden autumn leaves provided a picture-perfect backdrop. Families enjoyed the late summer sun on the patio as the children played on the lush grass. Some of our younger guests even braved the dungeons - their new fluffy friends by their sides!

A light lunch of sandwiches, cakes, and scones brought the weekend to a close. Everyone left with tired feet, but hearts full of new friendships and happy memories.

I liked the lions roaring. I loved making my stuffed teddy, Liza the Lion, I sleep with him every night. I really loved the dungeons too!

Harry

Thank you for an absolutely unforgettable weekend. Everyone has been so welcoming and kind sharing their stories with us… We felt so understood as all the parents there have had the exact same feelings we've had and get it which is so refreshing.

Parent

I really enjoyed seeing everyone again after a year's break. I had a fun time at the safari park. I particularly liked the raffle because I won loads of prizes. I really appreciate everyone who has put the time and effort into organising it. Thank you so much.

Ellis

It was really fun to play with my new friend Harry.

Jacob

I got to go on a fairground ride for the first time!

Child with FOP

It was so interesting to speak to others and hear their stories. We learnt so much and we now have those connections to reach out if we have questions.

Parent

Our daughter was allowed to socialise in a safe environment where everyone was conscious of her needs even the littlies were inclusive and able to understand. Having children show so much care and love towards her is something that fills us with so much joy

Parent

I really enjoyed hanging our with Rion and his family, we had a lot of laughs. I also enjoyed driving the safari drive – that was cool!

Oliver

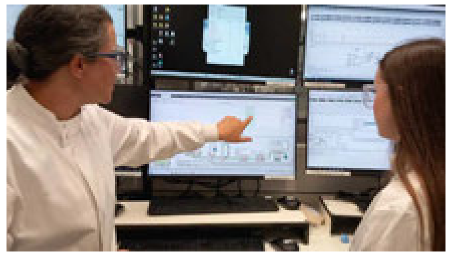
The entire weekend left me feeling far more connected to our small but mighty community. Where everyone could not only thoroughly enjoy themselves but also significantly strengthen bonds, both new and old. This weekend reinforced that we are united. Whether the emotion shared was grief or hope, a strong link runs between us all, and this wonderful weekend served to strengthen that unity.

Hamish

The Next TOP FOP Scientist?

The Deane family has been a long-time champion of FOP Friends. This summer, daughter Jessica, an aspiring Biomedical Sciences student, visited the cutting-edge FOP research lab at Oxford University. Jessica was delighted to receive a VIP tour from Dr. Eleanor Williams, who has been passionate about finding a treatment for FOP for many years. We're delighted Jessica was able to take time out of her studies to share her highlights:

Hi I'm Jessica! A FOP friend and prospective Biomedical Sciences student. I'm applying to Oxford University this year for October 2026 entry. I am going into year 13 this year and I am currently studying A-level Biology, Chemistry, and Theology, Philosophy and Ethics. Also, I am completing an EPQ this year which is focused on neurological disease and disorders. Whilst studying A-level Biology, I developed a particular interest in human Biology, and more specifically disease and treatment of disorders, especially regarding genetics. How and why such small biological molecules, substances and particles control our identity and health is such an interesting area to me, especially in new and developing genetics and disorders research, which has become a huge focus of medical research and innovation in the past few years.



I had the pleasure of visiting the FOP research lab in Oxford during this summer. I met with Ellie the FOP researcher, along with many PhD students conducting projects and working in the lab. I was able to see the cutting-edge technology that was being used by the team in their latest research.

Seeing the samples and experiments being conducted was truly invigorating and inspiring. I learnt about the new, promising technology and strategies that are being trialled for new treatment strategies for FOP. The protein inhibitors and other molecules being harnessed in treatment in ways that were creative and tactical was eye opening to just how far the medicinal treatment engineering field can go and how much potential it holds to revolutionise treatment options for those who live with FOP.

Whilst touring the facilities of the lab, I saw how intricate and delicate some of the equipment, such as the pipettes were, and how thorough the process of the experiments was, such as producing, harvesting and observing crystallised proteins in order to assess the effectiveness of a particular inhibitor was successful for a specific protein. At the same time, a large expanse of machine systems and controlled environment rooms were set up to analyse data and study results.

I learnt that the lab team was looking for new breakthroughs from every possible angle. I was amazed to learn that even the Didcot particle accelerator is involved in the research at the Oxford FOP lab for treatment discovery.

Seeing how determined and hardworking all the staff were at the lab was truly inspiring. I hope to work in research when I have completed my degree, to study and work with genetic diseases and find new breakthroughs that can change and benefit the lives of many who may live with these disorders.

It was truly energising to be at the heart of innovation for medical research. There were countless other research laboratories surrounding the FOP lab also working on several different projects in medical innovation which made me inspired and hopeful for the future of medical discovery. I am so excited for the promising future of FOP research and medical research in Oxford which I hope to be a part of.

Please join us in wishing Jessica every success in both her application to the University of Oxford, and in her upcoming exams. Her passion for genetics and disease research is inspirational, and we have no doubt that wherever she studies, she is destined to be a brilliant asset to the future of biomedical science.

Brilliant Brooklands

Earlier this month, Christine Kay organised an American Tournament at Brooklands Tennis Club to raise funds for FOP Friends, a charity very close to her heart… her next-door neighbour is Oliver!

The dry but foggy Sunday saw an amiable yet competitive spirit. The intense event concluded with a truly tense final.

Congratulations to champions Steve Kay and Richard Ratcliffe, who finished victorious over runners-up Ian Hughes and Charles Hickson. Oliver was there to congratulate the players and present their prizes.

A huge thank you to Christine for organising and a special mention goes to Judy Taylor for helping on the day.
Christine announced an ace amount was raised: £1,130!

Out and About

We've had an incredibly busy and productive few months, getting out and about to raise awareness of both rare disease and FOP.

1

Helen at Sheffield w/ M4RD

Last month, Helen delivered a presentation to first-year doctors at North General Hospital, Sheffield, for the amazing organisation, Medics for Rare Disease. Helen has long been inspired by this incredible charity and was delighted to be able to help deliver the first session of their Rare Aware training.

It was an important presentation for the next generation of doctors, introducing the importance of being aware of the challenges faced by those living with a rare diagnosis. These challenges range from the diagnostic odyssey to get the initial diagnosis and the difficulties of managing and juggling appointments, to the lack of available treatments, and more.

A big thank you to Medics for Rare Disease who are working tirelessly to raise the profile of rare disease with the next generation of doctors.

2

Raising FOP Awareness with Occupational Therapists

Helen was recently invited by Vicky Yeomans to deliver a presentation to a group of Occupational Therapists (OTs) in Tameside. Helen met Vicky through the Salford University project. The session was focused on raising crucial awareness of FOP within the profession.

Occupational Therapists play a vital role in helping patients maintain independence and quality of life. Helen's talk educated the OTs on the realities of living with FOP, providing them with practical insights into how they could best support a patient with the condition if they came across one in their practice.

This targeted training helps ensure that professionals across various healthcare fields understand the unique needs of the FOP community, leading to better and more informed patient care.

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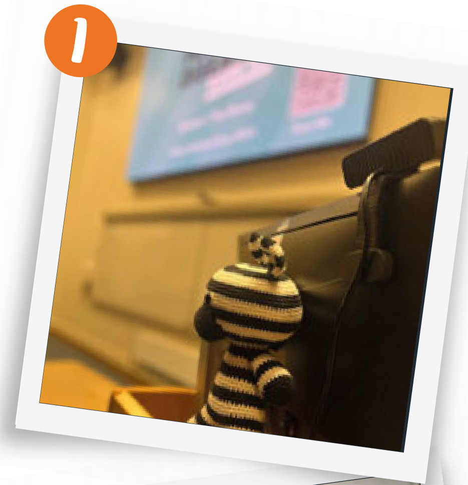

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CamRare Summit 2025

Andrew and Helen attended the CamRARE RAREsummit at the Wellcome Genome Campus in Cambridge earlier this month. The summit, which celebrated the 10th anniversary of the Cambridge Rare Disease Network (CamRARE), successfully brought together over 300 experts, researchers, industry leaders, and patient advocates to collaborate and debate challenges in the rare disease space. Their attendance at this flagship event allowed them to engage in discussions on key themes, including patient-led research, innovation through the Rare Disease Research Network (RDRN), and the impact of technology on care. We were also pleased to have the opportunity to showcase a science poster at the event, raising further awareness of FOP. In addition to the presentations, it was a great opportunity for networking with other organisations who are key in driving improvement forward to improve the lives of everyone living with a rare condition.

4

Manchester Rare Disease network

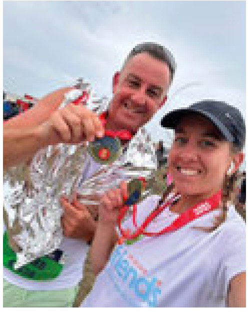
In his first official role as a member of the MRCC's advisory panel, Chris attended the MRCC Strategic Delivery Plan Workshop at City Labs 1.0 in Manchester. The session focused on the achievements of the MRCC and included round table discussions on how to move towards the MRCC's goals for the coming years. The MRCC's mission, since its establishment in 2022, is succinctly "to improve the lives of people and families living with rare conditions."

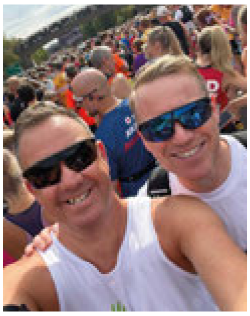
Since its formation, the MRCC has evolved into an effective platform to link and coordinate Rare Condition-related work in the region. It aims to break silos, facilitate cross-institutional, cross-disciplinary, and cross-theme connections, and to enable the sharing of best practices. It is well-positioned to improve the lives of those affected by Rare Conditions.

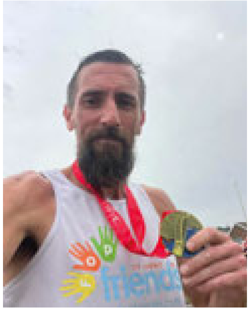
Great North Run

We were lucky to have another team of incredible runners, ready to lace up their shoes and take on the iconic Great North Run for a loved one. Jane, Rebecca, Richard and Simon, joined Mick who was running for his son Lenny, and Hope who was running for her brother Ellis.

> The day was nothing short of incredible: the atmosphere was electric, and the sense of community amazing with complete strangers pushing and willing each runner forward over 13.1 miles of sheer determination.
>
> Was it tough? Absolutely!!! But with every mile, I thought about the daily challenges faced by those living with FOP and the incredible strength and resilience they show every single day.
>
> I've already booked my hotel for next year because I know, firsthand, just how vital these funds are. Together, we run for every FOP warrior who inspires us to keep moving forward.
>
> **Hope, Ellis' big sister**







Our amazing team raised £3486!

If you would like the opportunity to take on this iconic race in 2026, then register your interest by scanning the QR code now!

This newsletter has been made possible thanks to a grant from **The Zochonis Charitable Trust.**

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Ain't No Mountain High Enough...

A huge congratulations to Joe and his friends who successfully summited Snowdon to raise funds and awareness for FOP Friends, in honour of his daughter, Monroe.

The team faced a foggy day but their spirits were not dampened! Joe absolutely smashed his fundraising target, thanks to generous sponsors and additional collections from his friends, making a massive difference to everyone affected by FOP. Thank you to everyone who supported this huge challenge. We hope they enjoyed a celebratory drink back at base camp!

Cheers for Millie!

Please join us in thanking the lovely team at The Railway Inn, Framingham.

They hosted a fantastic beer festival, where the community spirit and lovely sunny weather shone brightly throughout the day. The event successfully raised an amazing £238 in honour of Millie, with proceeds supporting FOP Friends, as well as EACH, and the East Anglian Air Ambulance. A huge thank you to everyone who bought raffle tickets, enjoyed the live music, and to all the generous local companies whose donations made the day a great success.