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® Together

Vol. 9 Iss. 2 summer 2026

Our Sixth Family and Friends Gathering

After many months of anticipation and planning, our sixth Family Gathering in Manchester finally arrived.

We welcomed friends from across the globe for a wonderful weekend of reunions, friendship, laughter and the chance to create new memories together. Families, friends, medical professionals, researchers and FOP Friends organisers came together to share experiences, learn from one another, and celebrate the strength of our FOP and POH community.

Throughout the weekend, attendees enjoyed a packed programme of presentations and discussions.

Medical professionals and scientists provided updates on the latest research, clinical trials and emerging treatments, while other sessions explored many aspects of living with FOP and POH.

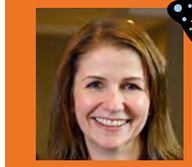
We also shared updates on FOP Friends' advocacy work and the progress being made to support those affected by these conditions.

Once again, we were incredibly fortunate to be joined by a panel of leading international experts in FOP and POH. Their dedication and incredible generosity helped make the weekend truly exceptional.

Our FriendZone provided a safe and welcoming space for our children and young people and, by popular demand, we welcomed back our furrier friends from Noah's ART. Excitingly, new for 2026 was the Adaptive Gaming crew which, as you might expect, was a huge success with young and old alike!

Saturday evening saw our ever-popular quiz return during our Family Dinner, which was all love and laughter. The weekend is always packed with information and emotions, so we were delighted to be able to offer our families time to pause on Sunday morning with a reflective Kintsugi session before we bid farewell for another year.

Inside



A word from Rachel

As an event planner by trade, organising an event like the FOP Friends Family Conference is never straightforward.

There are many moving parts, careful considerations and countless small details involved in bringing everyone together safely and successfully. It can feel complex and demanding, but seeing the end result makes it entirely worthwhile.

For a relatively small community, there was a huge sense of connection and true friendship throughout the conference. Whether it was families – new and old - meeting others who understand the realities of living with FOP, conversations between researchers and attendees, or simply spending time together, the value of being together was clear to see. We heard updates from those who continue to work hard to improve understanding of FOP and explore future treatments. Some of the most meaningful moments came through informal conversations, shared experiences and the support people offered one another.

Thank you to everyone who attended, contributed and helped make the conference such a positive experience. For a rare condition such as FOP, coming together matters. Sharing experiences, building connections and ensuring that nobody feels alone is at the heart of what this charity exists to do. We are only as strong as the community we build together, and I hope we can continue to support one another in the months and years ahead.

Rachel, FOP Friends Trustee

Andrew's Antics!

Andrew has only been a trustee with us for a short while, but already he has made a huge impact for FOP Friends. His contributions span supporting the daily running of the charity, hosting webinars, and serving on the Steering Group for the Adult Rare Bone Disease network and other committees. On top of all that, he regularly represents FOP Friends on the national and international stage. We hardly give him time to enjoy his retirement – he's non-stop!

We are so thankful for his time and expertise. We are also grateful for the grants and stipends that allow him to travel to many of these events.

So, without further ado, let's hear what he's been up to recently...

Research Round-Up

The end of 2025 and the first half of 2026 have seen especially exciting news for two potential therapeutics treatments for FOP in late-stage clinical development. Both have now reached their first submissions for marketing authorisation: the ALK2 kinase inhibitor, zilurgisertib, and the Activin A monoclonal antibody, garetosmab.

In May, we reported on the announcement that Mirum Pharmaceuticals has entered into an exclusive licence of worldwide rights to zilurgisertib from Incyte. As we explained, this is not unusual for Biotechs and small pharma companies. Chris and I have spoken with senior members of the Mirum team earlier in the summer, and there is every indication that they will aggressively continue the global development of this exciting potential therapy for people living with FOP. We understand that Incyte will continue to lead the discussions with US regulator FDA with support from Mirum, who would take over marketing authorisation if and when approval for adults and adolescents in the US is confirmed, likely in September 2026. Mirum will increasingly be involved in further clinical development in younger people living with FOP, currently led by Incyte. FOP Friends offered their support to Mirum and we hope to see their participation at future Manchester and global conferences. The positive partnership between the two companies was demonstrated by co-sponsorship of two presentations of very positive data from the Ph3 PROGRESS trial at ENDO (Endocrine Society Annual Scientific Meeting; June, Chicago, IL, USA), the lead author being our friend Prof. Bob Pignolo from the Mayo Clinic who held clinics and presented at our Manchester Conference.

ENDO 2026 also saw the presentation of the very positive data Ph3 OPTIMA trial of garetosmab in adults living with FOP, this time the lead author and presenter was the UK's Prof Richard Keen, another good friend of FOP Friends who many of you know personally and also held clinics and presented at our Manchester Conference. Regeneron have submitted for marketing authorisation to both the FDA in the US and European Medicines Agency (EMA), again with an outcome on approval expected by September 2026.

No further clinical trial updates from other companies have been reported; nor have additional marketing authorisations in other regulatory jurisdictions, including the UK MHRA, been submitted but FOP Friends will continue to encourage and support these critical activities.

In other news, our friends at the Canadian FOP Network support research carried out by Dr. Toshifumi Yokota and his colleagues at the University of Alberta. They have recently published a paper about their most recent findings in the journal Molecular Therapy - Nucleic Acids. Dr Yokota studied an experimental RNA-targeting approach called antisense "gapmers". These are short synthetic pieces of genetic material designed to bind to a specific RNA message inside cells; their goal was to design gapmers that would recognise and reduce the mutant ACVR1 message while leaving the normal version of the gene as unaffected as possible. Introduction into these gapmers of only a single-base modification in cells from people with FOP, and in engineered muscle cells carrying the FOP mutation, led to a selective reduction in the mutant ACVR1 signal at both the RNA and protein levels.

Furthermore, treatment reduced signs of osteogenic differentiation, which is the process by which cells begin turning toward bone formation such as heterotopic ossification in FOP. While very early days, it's good to see completely new ideas and potential avenues for therapeutic development; the research community are not resting on their laurels!

Buongiorno Livorno!

In April, Andrew headed off to Livorno, Italy, to join our friends for the 18th FOP Italia Meeting where he was able to discuss research advances and important updates from industry representatives.

Professor Alex Bullock was in attendance and presented an update on the exciting FOP Research taking place in Oxford, alongside their collaborator Dr Paul Yu from Massachusetts General Hospital, Boston USA. Work by the Oxford FOP Research Team, which was made possible by FOP Friends' fundraising efforts, led to the discovery of Saracatinib as an ALK2 inhibitor. Pre-clinical studies suggested Saracatinib may be a potential therapy for FOP and led to the European STOPFOP clinical trial. Professor Marliese Eekhoff provided an update on the STOPFOP clinical trial, which Alex Bullock and Paul Yu's research labs are also a part of. Nadine also presented her doctoral thesis project. In her research, she is investigating the impact of age on FOP disease progression.

iDR26

Ahead of the International Drug Repurposing Conference (iDR26) in May, Andrew and Dr Sarah Dixon collaborated to produce a poster for presentation at the event in Brussels. iDR26 brought together the global drug repurposing community with a focus on rare diseases, policy reforms, funding and incentives, community initiatives and AI-driven discovery. It hosted a varied audience including patients, patient groups, and policy makers.

Drug repurposing identifies new uses for existing approved drugs or drugs that have already undergone clinical trials for another purpose. These drugs already have recorded safety data, which can enable faster, more cost-effective drug development, something that is especially important for rare diseases, like FOP.

The poster aimed to showcase the discovery of Saracatinib, an orally bioavailable drug developed for cancer, as an ALK2 inhibitor, as an example of a drug repurposing case study.

Andrew did a great job presenting the poster and delivered an impressive flash talk, which earned recognition of the poster as runner-up in the Poster Competition!

For more information on clinical trial, and updates, visit https://fopfriends.com/pharmaceutical-news-update/

Connections in Canada

As someone who is half-Canadian, this trip was particularly impactful for Andrew. In June, Andrew made a flying visit (literally and metaphorically!) to Montreal for the International Conference on Children's Bone Health, organised by the ISCBH, of which FOP Friends is a proud Patient Group Member. There were a number of patient organisations in attendance, along with leading doctors and researchers from across the world, with the shared goal of improving the lives of young people with bone conditions. There were two posters presented on FOP, one looking at a potential new therapy for treating FOP, and another with results from the ANDECAL trial. Andrew discussed new therapeutic opportunities with researchers from both academia and industry but his prime activity was taking part in a panel along with representatives from other rare paediatric bone charities. The discussion was around 'Patients as Partners: How Can We Collaborate?' Andrew presented examples of FOP Friends contribution to the Adult Rare Bone Disease Collaborative Network Steering Committee. The panel then invited questions from the audience leading to a lively discussion and endorsement of why patient participation is so important in all elements of care, research, therapeutic regulation and access.

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Repositioning is similar but uses potential drugs that have undergone clinical development in other diseases/conditions but not taken forward for marketing approval, such as saracatinib. repositioned for FOP in the STOP-FOP clinical trial.

#MakingMemories

One of the most amazing things about our conference is seeing the smiles from families who know they are with friends, people who truly understand what their daily life looks like.

We are thankful to each and every person who comes along to share a little of themselves with others, to make the event so personal and meaningful to everyone who attends.

We have selected a few photos and a few words from guests, which capture just some of the special moments from our weekend…

We would like to thank our sponsors Big Lottery Community Fund, Incyte, Ipsen, Regeneron, and The Zochonis Charitable Trust for their generous support.

Thank you SO much for your hard work. Just know that you are so appreciated and loved! Big hug to each single one of you!

As parent to a teenager with FOP, meeting others who have FOP or are also parents was heartening. Not to have to go over the painful details again and again was great.

Conference in numbers

15 countries
150 attendees
28 families
8 new families
25 Healthcare professionals
Too many giftbags to count!
1 dog and
1 bearded dragon!

So much was put into this conference, it couldn't have been better.

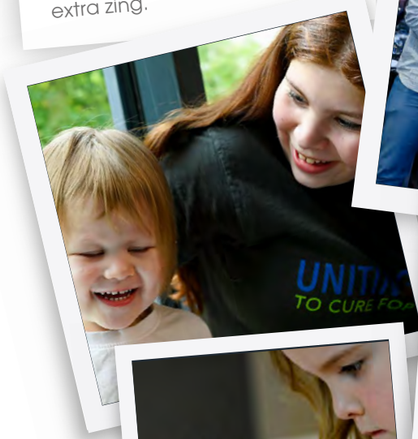
As someone older, it's heartening that I can give something back, whether that's chatting to the parent of a newly diagnosed person or simply catching up with old friends. It's nothing short of amazing and incredibly giving of you all.

Knowing we are not alone with this condition gives us hope.

I am going home no longer feeling isolated.

I left feeling more confident towards the future.

Every conference has been good but this one felt particularly welcoming and had excellent vibes. I'm not sure why but it just had that extra zing.



Remarkable.

COMMUNITY FUND · Incyte · IPSEN Innovation for patient care · REGENERON SCIENCE TO MEDICINE · The Zochonis Charitable Trust

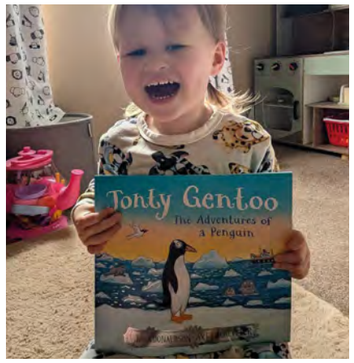
Books…

One of our favourite projects of the year is the Alma Triffitt Book Award.

Jim's wife, Alma, was beside him every step of the way. Alma would accompany Jim around the world on his research trips, with her kindness and support felt by all those she encountered, and she was a beloved friend to FOP Friends.

In loving memory of his late wife, Jim bestowed a generous grant to our children and young people who are living with FOP or POH, to share Alma's passion for reading. Each year in June they receive a hand-picked book, selected by the staff at Oxford's Blackwell's - Jim's favourite bookstore.

A huge thank you to Jim and the all the staff at Blackwells.



…Books…

Harry's friend Susan Kempster continued with her charity bookshop at the local Notcutts garden centre where she works, to raise awareness and funds in honour of her little friend.

She has proudly displayed information about FOP Friends, and avid readers have made generous donations for books. Susan's second collection raised £97.36. Thank you to Susan, the staff at Notcutts, and the wonderful customers who have made their donations grow to a blooming marvellous total of £184!

…And more books!

When you are navigating living with a complex health condition such as FOP or POH, every day can be a challenge. There is a wealth of support out there, but a quick search can leave families feeling overwhelmed and unsure where to start. Inspired by the Rainbow Guide produced by the Choice Wellbeing charity, we compiled our own directory to signpost families to services that can make a real difference. Our goal is to make it easier to discover available resources, connect with partner organisations, and access vital support.

We are very thankful to Lauren Packer, who volunteered with us for the project, and who worked tirelessly to collate all the relevant organisations and design the guide. Thanks to a generous grant from The Russell Lang Charitable Trust, we have posted physical copies to our registered families. If you would like to receive one, please get in touch and we will gladly send a copy your way.

RachFest26 – A Family Affair!

Anyone who knows Rachel knows just how much she loves to party and live her best life! This summer was no different, as she attended the inaugural RachFest, an event held in honour of… her!

Organised by Rachel's Uncle Ian, alongside close family and friends in Devon, the event was a true celebration of family and friendship. Rachel and her mum, Julie, set off on a road trip to Torrington for the festival, joined by Auntie Shelly and Uncle Dave.

The Torrington Town Hall was bedecked in vibrant pink - Rachel's favourite colour! In this glorious summer weather we have all been enjoying, guests gathered to sing the night away to classics from Johnny Cash, Rod Stewart, and more. Ian and his musical friends provided the soundtrack, jamming on guitars, drums, and even spoons, with a highlight of the evening being a special set of Rachel's personal favourite songs. Friends were treated to a truly heartwarming performance from eight-year-old Olivia, who stole the show playing her guitar and singing alongside her Grandad Ian. The event helped raise awareness for FOP and brought in a fantastic £135, proving that even the smallest of gigs can end on a high note.

Run Sandringham 24

More wonderful #TeamFOPFriends runners took on challenges in June. Isla's friends Rachel and Christian, completed the Sandringham 24 Trail Race.

They were inspired by their wonderful friend whom they had followed for years, for her determination to fight every challenge she faced.

The event took place around the Sandringham Estate, the winter residence of the Royal Family, with participants racing against the clock to complete as many laps as possible within 24 hours. **Their efforts raised a phenomenal £1,570 for FOP Friends!**

"The run was definitely one of the hardest things I've ever done! It was fine until 10pm but overnight was really hard. We managed to complete 12 laps - 60 miles each, so 24 laps - 120 miles in total! We also won the mixed pairs race too, so got a nice little trophy along with the medal."

In case you were wondering, 120 miles is around 4.6 full marathons! A huge congratulations to them both for their achievement and thank you for raising so much for FOP Friends.

Mick's Manchester Marathon

Lenny's dad, Mick, headed 'Up North' to take part in the Manchester Marathon. We're always excited when supporters run in the city because FOP Friends gets to put on a cheer squad!

It was a gorgeous spring day to tackle 26.2 miles, and the good people of Manchester lined the streets to encourage everyone. Mick was confident about his time and all his hard training paid off. Helen and the team managed to surprise him at several points along the route with custom signs to boost his spirits.

Mick pledged to run until his wheels fell off – and they very nearly did! He crossed the finish line in an impressive 3 hours and 27 minutes!

Even more incredible was his fundraising total: thanks to his generous family and friends, he raised a fantastic £900. Mick really did do his son Lenny proud.

Oxford Luncheon

Chris and Helen were invited once again to attend the University of Oxford's Vice-Chancellor's luncheon.

This year's event was to celebrate the opening of the Schwarzman Centre for the Humanities. We were invited in recognition of FOP Friends' generous donation to the FOP research team and our continued collaboration. Chris and Helen had a wonderful day, exploring this remarkable new library and resource for all students at the University. It was also a great opportunity to network and share the work of FOP Friends with others.

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

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

info@fopfriends.com
@FOPFriends
/FOPFriends

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What next? Webinar

Following promising results from Regeneron and Mirum's phase 2 trials, we recently hosted a webinar presented by Professor Richard Keen.

Recapping his presentation from our annual conference, Professor Keen walked us through the journey of moving a drug from clinical trials into everyday clinical care for our families.

Professor Keen explained the MHRA and NICE processes and outlined what families will need to do in the coming months to support the applications. These are exciting times for our families, and FOP Friends has already made a start behind the scenes.

To watch the presentation, scan the QR code.

London Landmarks Success

You can forget Harry Styles – our Harry is way more popular! A group of his dedicated friends proudly crossed the finish line after completing the London Landmarks Half Marathon in his honour!

Through months of training and determination, the team not only achieved a personal milestone while raising vital funds.

The event brought together friends, family, and cheering crowds along the route. Harry and his sister Phoebe were right there, holding up signs and giving out sweets to all the runners.

Reflecting on why they took part, Christina and James shared, "I've known Harry's mum since high school, and it was heartbreaking to see everything the family were facing. Despite so much adversity, Harry remained the happiest and bravest little boy, and his family showed incredible strength throughout. We wanted to do something that could make a difference, however small, so we decided to raise money and awareness through the half marathon. We ran because we wanted to support Harry and his family, who continue to inspire everyone around them."

Scarlet, who also took on the challenge, explained, "Running the LLMH a second time meant I could support an amazing family in my community with FOP.

Harriet is an amazing teacher, kind and extremely caring. Having known her in the teaching community I wanted to give back to her family and support them through this difficult period, like she has always supported me with her kindness. She is an outstanding mum with a massive heart and running for her family was a privilege."

The five-strong team – Scarlet, Angela, Christina, James D and James B - **raised a showstopping £4,882.00.** And if you think that deserves an encore, #TeamHarry2 will be taking on the Royal Parks in October!