



friends[®] Together

Vol 9, Issue 1 Spring 2026

Rare Disease Day 2026

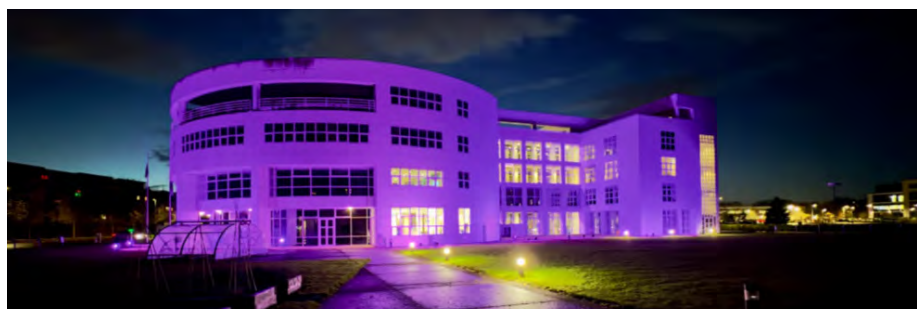
As the rarest month of the year, February sees families, patient organisations, healthcare professionals, and researchers stand together to raise awareness for the 1 in 17 people living with a rare condition. Rare Disease Day is recognised around the world, offering an important opportunity to shine a light on the resilience of those living with rare conditions while highlighting the urgent need for continued research.

FOP Friends marked the occasion by arranging to have Siemens' flagship UK office illuminated in purple as part of the Global Chain of Lights. Oliver visited the offices to meet with Sue and tell her about his life with FOP, while Siemens shared vital facts about both rare diseases and FOP with their staff throughout the day. We were also grateful to receive a generous donation from their charity fund too.

Earlier in the week, Helen and Andrew had the privilege of attending the Westminster Rare Disease Day Parliamentary Reception. Hosted by Genetic Alliance UK, the event was a wonderful opportunity for patient advocacy groups, health representatives, and policymakers to discuss the ongoing challenges and goals for those living with rare conditions.

We were also thrilled to have the opportunity to 'take over' the social media channels of Rare Revolution Magazine, sharing facts about FOP and POH with a wider global audience.

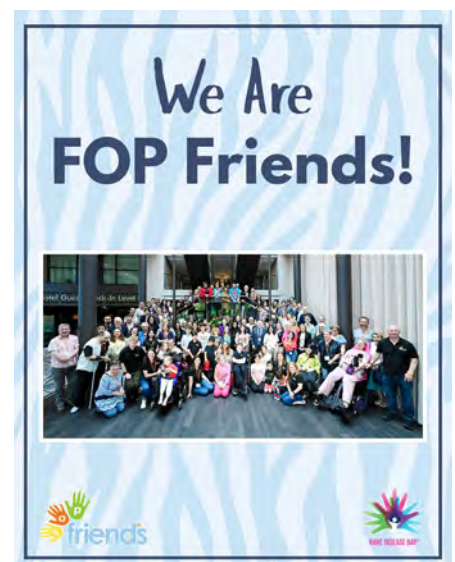
We are thankful for every opportunity to share our stories and will continue to advocate until we have a treatment and a cure for both FOP.



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.

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A word from Fiona



Welcome to another fabulous issue of FOP Friends Together. We are stepping into that beautiful season of lighter mornings, longer days, and carpets of crocuses, daffodils and snowdrops, a time that brings with it a renewed sense of hope and the promise of new beginnings.

I am delighted to share a few words as we look ahead with excitement to our upcoming FOP Friends Conference and Family Gathering at the Radisson Blu Hotel, Manchester Airport, this May. Opportunities for families to come together are so important to learn about the latest advances in research and potential treatments, while also enjoying evenings filled with conversation, laughter and fun. Creating that balance of knowledge, connection and joy is at the very heart of FOP Friends.

Our community continues to grow stronger, especially as we warmly welcome new families into the FOP Friends network. That shared support is vital as we work together to drive forward research towards effective treatments and ultimately a cure for FOP. This biennial event is always a particular highlight for me, as I am once again fortunate to spend time with our incredible children in the FOP Friendzone. Each year may bring new challenges, but I always leave feeling inspired and deeply moved by the resilience, humour and determination shown by every child and family as they navigate life with such grace.

There is so much to look forward to this year. Recently, we proudly took part in the Global Chain of Lights for Rare Disease Day, with Siemens illuminating their flagship UK office in Manchester in the official Rare Disease Day colours of pink, green, blue and purple. We also look ahead to FOP Awareness Day on Thursday 23rd April, a significant date marking the discovery of the ACVR1 gene, which causes FOP.

It is a genuine privilege to work alongside such inspiring individuals and families. Together, we continue to build hope, strength and progress for everyone affected by FOP.

Fiona, FOP Friends Trustee

Research Roundup

In our last newsletter, we shared the very pleasing progress from Regeneron's adult trial, a major milestone for our community. However, the journey of medical research often involves navigating both successes and setbacks. While we are all buoyed by the recent positive results from Regeneron, the reality of drug development means that not every path leads to the outcome we hope for. This edition, we bring you updates from Ipsen's fidrisertib as well as Regeneron's garetosmab.

Update on the Ipsen FALKON Study

As many of you may have seen from the December press release, Ipsen has decided to stop the Ph2 FALKON study of their ALK2 kinase inhibitor, fidrisertib. A follow-up letter was released on 9th January 2026. It can be found on our website by scanning the QR code below.

In medical research, even when early lab results look promising, a drug must prove it is both safe and effective in patients to move forward. It is important to know that the decision to continue or stop a trial isn't just up to the pharmaceutical company.

A totally independent Data Monitoring Committee (DMC), made up of safety physicians, clinical trial experts, statisticians, and, crucially, two FOP specialist physicians, monitored the data on a quarterly basis. Because they are independent, they are 'unblinded,' meaning they know exactly which patient has taken the drug and which has taken the placebo, while the pharmaceutical company team remains completely 'blinded' to that knowledge.

At the one-year mark, the DMC performed a 'futility test' to see if the drug reached a high enough threshold of benefit to justify continuing.



Unfortunately, fidrisertib showed less than a 20% reduction in new bone volume compared to the placebo. This meant it failed the futility target. It also showed no meaningful reduction in flare-ups or pain.

Because of this, the DMC endorsed the decision to terminate the study. This decision appears final, which means that even for individual patients who felt they saw personal benefit, access to this experimental agent will now stop.

The positive news, and one of the learnings which came out of the fidrisertib trial, was that no significant safety issues were observed with the drug. This is encouraging for the wider class of ALK2 kinase inhibitors. We now await with optimism the data from the StopFOP (saracatinib) trial, which completed late last year.

Update on Regeneron

On a brighter note, we have major news regarding Regeneron's garetosmab. On February 19th, 2026, the US FDA accepted their application for Priority Review. A decision on its approval in the USA is expected by August 2026. Regeneron also submitted garetosmab for marketing approval in the European Union (EMA) in February, with a decision expected in the second half of 2026.

So, what does this mean for patients in the UK? FOP Friends are in communication with Regeneron and are keen to support the company's submission for marketing approval with the UK regulatory agency (MHRA) and ultimately reimbursement through the NICE process; the timing however is currently unclear.

We are, however, particularly hopeful because the UK has recently 'radically improved' how it handles orphan drugs (medicines for rare diseases):

- **Accelerated Pathways:** The MHRA and NICE (who decide on NHS funding) have launched new 'Aligned Pathways' designed to speed up access. This can cut months off the usual wait time by reviewing the drug for safety and cost-effectiveness at the same time
- **International Recognition:** The UK now has a process to 'recognise' approvals from trusted partners like the FDA. If the FDA approves garetosmab in August, it could potentially smooth the path for the MHRA approving garetosmab here in the UK.

- **The Innovative Medicines Fund (IMF):** This is a ring-fenced fund specifically designed to give patients early access to promising non-cancer drugs while the final long-term data is being collected.

FOP Friends trustees are actively engaged with NICE and the MHRA through workshops and consultancy to ensure the FOP voice is at the heart of these initiatives.

Finally, Regeneron are planning to open a Global Paediatric Clinical Trial in 2026. This is an exciting development as it is a critical step in treating the condition before it takes a significant hold. Again, the RNOH will be the UK's site for this trial once it is available, and the team will approach patients and their families if they are considered eligible for the trial. We look forward to bringing you further updates in due course, and at our conference in May.

If you have any questions about clinical trials in the UK, please reach out: andrew@fopfriends.com

I look forward to meeting up and chatting with many of you in a few weeks.



To the trial participants: Thank You

Choosing to join a clinical trial is a brave, personal decision, and one which has the potential to change the lives of many. We know that many people on the fidrisertib trial were incredibly disappointed, frustrated, and even angry, to lose access to this drug, as they felt it was helping them personally.

We would like to take this opportunity to thank each and every person who committed to this trial. Please know that even though this trial did not produce the outcomes we were all desperately hoping for, your participation further advances the understanding of how treatments work in the body, improves the design of all future FOP trials, and brings us closer to the eventual breakthrough we are all working toward. Because of you, the global FOP community is further ahead than we were before. Thank you for your bravery and for helping us find the answers we need.



Congratulations Oliver!

After a five-month wait for his provisional license due to DVLA delays, followed by months of practice chaffering his family everywhere, we are delighted to share that Oliver has passed his driving test!

Passing your driving test is a major milestone for many 17-year olds, but for someone living with a medical condition, the independence and freedom it brings are immense. Oliver was never able to have a bike to "pop round and see his friends", but now that he has his license and his own wheels, there's no stopping him!

Wishing him many years of happy travels and adventures.

We have gathered some helpful information for our families regarding all things Motability. Scan the QR code below to learn more:



A Cracker of a Christmas!

Christmas may seem a distant memory now as we all prepare for Easter, but at FOP Friends we are still glowing from the generosity of our friends and supporters over Christmas.

Led by our new Fundraising Coordinator, Cathryn Roys, we launched our first Christmas Appeal. Families and friends were invited to light a bauble on our virtual Christmas tree and leave a message of hope and support for someone they love. We were completely overwhelmed with everyone's generosity. Our tree looked magnificent, and the messages left were simply heartwarming.

In total, our tree raised an incredible £3,500! Thank you to everyone who donated.



Beautiful Baubles

The incredibly talented Heather donated her talents once more this Christmas by painting commissioned baubles of our friends' favourite furry friends! Helen was so impressed with the baubles last year that she ordered two for the "fur babies" in our family: Alison's Quito and Rachel's Buddy. We were all blown away by how beautifully Heather captured these pampered pooches. Thank you so much, Heather, for creating such precious keepsakes and for donating your time and talent, which has raised an amazing £468!



Regeneron

Oliver and Helen were delighted to attend Regeneron UK's Christmas event in Uxbridge.

Oliver gave an inspiring update about life with FOP, once again stealing the show, while Helen shared the story of how the charity came to be. After the presentations, they enjoyed a lovely Christmas dinner with the team, followed by a virtual catch-up with the Development Team, joined by colleagues from both the UK and the USA. It was a whirlwind trip but a truly wonderful experience for them both!



Christmas at Arval UK

Christmas, we were honoured to be nominated as one of Arval UK's Christmas Charities for 2025.

As part of the festive celebrations, Helen and Oliver visited the Manchester office in December to sell tickets for their Grand Raffle. Following the success of the event, Oliver was delighted to return to the office this month to receive a cheque for the final proceeds.

A huge thank you to Arval UK, Helen Molyneux, and all the staff for such a generous donation and an amazing Christmas gift!



Our very own Secret Santas!

We were also delighted to receive a number of generous donations through the Christmas period.

We are so thankful people chose to donate to us, either in lieu of cards, in memory of someone special, or simply as a way of spreading joy and hope.

We also received some anonymous 'Secret Santa' donations, so if that was you, please know your kindness brightened our day.



A Conversation with Nadine: Living Well Through the Pursuit of Knowledge.

Last year, we shared our commitment to Metabolic Support UK's 'Living Well' movement, highlighting stories from our community, both in the UK and beyond, about the ways they endeavour to live well with FOP.

As part of this ongoing movement, our wonderful blogger, Isabel, met with Nadine to hear how she strives to live her best life and navigate the unique challenges of the condition with such positivity:

"I admire how everyone deals with FOP in their own, unique ways."

Amid a Summer filled with hectic deadlines and research commitments, Nadine was able to find a momentary pause within her busy schedule to sit down and share her perspective of what it means to Live Well. Her words are motivated her desire to give back and support the FOP community that has been central to her practise of Living Well.

"I try to enjoy every day to its fullest," she tells me. "To find joy and beauty in the little things, and to not postpone them, but to do them as soon as I can." This philosophy wasn't instant, it developed over time. What helps most, she adds, is "knowing that there is a community who always has my back." It's that sense of belonging that fuels her commitment to contribute something meaningful to celebrate them."



The Art of Listening to One's Body

For Nadine, the Living Well Movement coexists as something collective but also deeply personal.

"Although we share a community, Living Well has to be nuanced for each person," she reflects. "FOP presents differently in everyone, and so does life."

She puts a few spaces before adding,

"I look up to every individual living with FOP. I admire how every one deals with it in their own, unique way."

Her own practise of Living Well is rooted in her mantra for self-love.

"I don't need to be able to keep up with others. On those days, I try to be especially kind to myself and not push too hard. Sometimes I just call it a day, go back to bed, or give myself some me-time."

It's a gentle reminder that listening to your body is not a weakness - it's your strength.

Unlocking the Wider Picture

Before her diagnosis, Nadine's ambitions lay elsewhere.

"I actually wanted to study something with languages," she relates, "but the jobs related to languages didn't sound very appealing to me. That's why I started studying biology."

It was around her early studies where she experienced her first flare ups of FOP, though her diagnosis hadn't yet been confirmed. When she later began her PhD, she found herself drawn more deeply into the world of FOP research.

"What really motivated me," she says, "was out amazing community. Getting to know other individuals living with FOP, the researchers, the doctors—all of them inspired me. I wanted to contribute at least one puzzle piece to the big picture, to our shared quest for treatments and ultimately, a cure."

The Motivation Behind her Research

When asked whether her own experience with FOP has shaped her approach to research, Nadine responds thoughtfully.

"I try to keep in mind which research question would best serve individuals living with FOP - to improve their quality of life. In challenging times, it helps to remember who I'm doing all this for."

Her work reflects that sense of purpose.

"I hope my research can lay the foundation for new treatment approaches," she says, "not just for FOP, but for the broader rare disease field. Maybe other researchers can apply some of my findings to their own questions."



Nadine's Wisdom On Living Well

When I ask Nadine about the advice, she would give to young people with rare conditions who are trying to balance their goals with their health, her response is both proactive and uplifting:

"Talk to peers and others who have pursued their goals. Listen to your body and don't push beyond your limits—it will backfire at some point. And try things out. There is no shame in failing. Sometimes we need to fail to learn."

Her motivation comes from the community around her—and the hope that research and community-led movements, will make things easier for future generations.

"I wish for the next generation of FOPers to have a carefree childhood and life."

Closing Reflections

As we wrap up, Nadine leaves me with a message that perfectly captures the heart of the Living Well Movement:

"We are more than our condition, and I wish people would see us for who we are—people."

For students navigating university life with FOP, what she recommends is simple but vital:

"Connect with other disabled people and staff at your university. Learn what support you're entitled to have. You need to actively ask for help—so don't be shy and stand up for yourself."

Most importantly Nadine reminds us that Living Well isn't about perfection; it's about connection, curiosity and kindness—both to others and ourselves.

We're delighted to announce that Nadine will be joining us in Manchester for our Conference as one of our keynote speakers.

Lights, Camera, Action



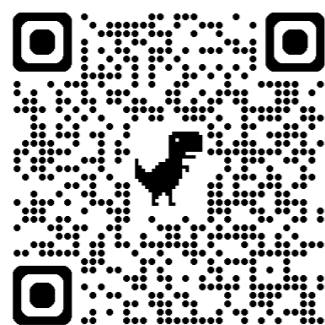
In January, Helen and Chris welcomed the IFOPA's camera crew, Dana and Dave, to capture a very special story.

On 23rd April, FOP Awareness Day, our global community marks 20 years since the discovery of the FOP gene. To honour this milestone, the IFOPA is launching an inspiring new video series: Journey to Discovery: A Breakthrough Built on Hope.

The crew's journey took them from Sale, Cheshire, to the University of Oxford to meet Professor Jim Triffitt, who contributed to the original discovery, and Professor Alex Bullock, whose team leads today's cutting-edge science. In London, they met Professor Matt Brown, who played a crucial role in mapping the genetic markers that bridged the gap between clinical care and genomic sequencing. They then flew on to Germany to meet with Nadine.

This series tells the story behind the science, celebrating the patients, families, and researchers whose collaboration made this progress possible. Helen and Chris were honoured to share their story as both parents and founders of FOP Friends, reflecting on their part in this huge international effort. We can't wait to share the finished series with you.

You can see our timeline From Discovery to Today... here:



Costello's Cakes!

Staff at Costello Medical recently turned their Manchester and Bristol offices into bustling bakeries to mark Rare Disease Day.

Their teams whipped up a spread of home-baked goodies so impressive that The Great British Bake Off should definitely take note.

Beyond the tasty treats, the event was an important opportunity to raise awareness of the unique equity issues faced by those living with rare conditions every day.



We were honoured to receive a fantastic £192.75 from the proceeds. A massive thank you to everyone who baked, bought, and supported their mission to help those living with FOP and other rare conditions.

What a Great Grandad!

We were delighted to receive a donation just before Christmas from little Lexi's grandad, Graham Lowe.

In Lexi's hometown, Grandad Graham has distributed collection pots in the local pubs, golf club and football club. He oversees the pots and then regularly collects the donations in due course. Thanks to generous friends, families and locals, Graham sent an amazing £2,100 to FOP Friends in honour of his granddaughter. When Graham isn't being a collector extraordinaire, he likes nothing more than to go to the park and play on the swings or in the sandpit with his little Lexi. It's the simple things!

From everyone living with FOP, we thank you.



Global Awareness Day 2026



www.fopfriends.com/shop

Update from the University of Oxford FOP Research Team

Our PhD students, Will Seaton-Burn and Kyle Ferguson, continue working hard to develop better new treatments for FOP. Will is now in his final year and continues to enjoy climbing, while Kyle is entering his 2nd year and has become a keen rower.



Proteins are specialised molecular machines carrying out essential work to keep cells functioning. Our cells (skin cells, nerve cells, muscle cells etc) need to communicate with one another to coordinate when specific cells should grow, move, multiply, or change from stem cells into specialised tissues. Central to this communication are specialised receptor proteins, such as ACVR1, which sit on the cell surface. These receptors act as molecular relays: they receive new signals from outside the cell and transmit them as instructions inside, to direct how the cell behaves.

In FOP, the ACVR1 receptor protein (also known as ALK2) contains one faulty piece that makes it hyperactive, causing it to transmit more messages than it should and allowing instructions for bone to be built in the wrong places.

Our current clinical trial, STOPFOP, is testing a drug called saracatinib. This drug works like a bung to block the activity of the faulty ACVR1 and stop its transmissions. The results of the STOPFOP trial will be known soon. Meanwhile, Will and Kyle are looking at strategies for the future to make an even more effective bung.

Most medicines travel through the bloodstream before reaching cells. They are designed to recognise specific proteins and attach to them,

rather like sticky toffee also fall off (you may have seen Paddington Bear using toffee apples to stick to the ceiling to hang upside in the film Paddington 2 but then fall off). However, just as toffee can eventually come unstuck, drug molecules can detach and stop working. They are also gradually broken down and excreted from the body. So, researchers are developing strategies to make medicines longer-lasting. In the past year, Will and Kyle have been testing two kinds of drug design approaches that aim to achieve this.

New drug design strategy #1 (Kyle)

The first strategy involves a new type of drug that has two separate sticky surfaces. One surface sticks to the faulty ACVR1 protein, as before. The second surface sticks to the waste disposal machinery (a shredder) that recycles old proteins inside our cells. This design brings ACVR1 to the cell's "shredder", directing its irreversible breakdown. The building blocks ("amino acids") that make up the ACVR1 protein can then be reused to make more ACVR1, but a drug can shred it again. This is a new and promising way to develop future medicines that are even more effective against FOP. The prototype work to date has been developed in collaboration with another PhD student at the University of Strathclyde and forms part of our ongoing collaboration with the "Medicines 4 Kids" (M4K Pharma) initiative, which is run by a charitable foundation. Several more years of research would be needed to develop a potential future medicine.



New drug design strategy #2 (Will)

The second group of drug molecules emerged from a collaboration with researchers at the University of Tübingen in Germany. These molecules are designed to recognise the faulty ACVR1 in a similar way to other drugs but because of their special chemistry, once they stick they cannot let go, acting more like superglue instead of sticky toffee. They become bound forever to the kinase domain and switch off its activity. The cell will still naturally make new ACVR1 protein, but giving the drug again can turn it off again. One advantage of a molecule that cannot let go is that you may achieve more robust protection against FOP, a key consideration for enabling potential surgical procedures.

In the past, developing drugs like these has been challenging because they can sometimes stick to other places where they are not supposed to and this causes complications. The design of the molecules was guided by 3D structural maps gathered by our research group to show the precise shape of the ACVR1 protein. These maps made it possible to carefully optimise the drug molecule design on the computer before testing it in the laboratory.

To our delight, the first early molecule designs showed activity. However, they behaved in a subtly different way from our initial expectation. Surprises like this are not uncommon in research and often lead to new insights. So, through small careful changes we are now working to understand these drug molecules better and make them as effective as possible.

See you soon!

We are incredibly grateful for your continued support, which makes this work possible. We look forward to sharing more updates with you at the Manchester FOP Family Gathering.

We also warmly welcome families who would like to visit our laboratories in Oxford. Please do get in touch directly or via FOP Friends.

Our Family Gathering!

Our 6th Conference in Manchester is nearly here! We're excited to welcome families to the Radisson Blu from 15th-17th May 2026.

To ensure the event is accessible, we can now fully fund attendance for FOP & POH patients and immediate families, thanks to secured grants.

Enjoy an incredible line-up of FOP experts, clinical appointments, and workshops. For younger guests, the FriendZone returns with Noah's Art animals and new Adaptive Gaming! There's plenty of time to connect, from our Friday Meet & Greet to our family quiz dinner and Sunday's mindful Kintsugi workshop.

To download the programme and book, visit www.fopfriends.com or scan the QR code. We can't wait to see you there!



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



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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

Be a part of #TeamFOPFriends at the Great North Run 2026!

Once again, we have been able to secure places for the world's biggest and best half marathon: the Great North Run!

Join 60,000 runners cross the iconic Tyne Bridge. Watch as the Red Arrows roar across the sky to paint it in red, white, and blue. From the start line to South Shields – there is no other race with an atmosphere like it!

This is your chance to be part of the world's greatest finish line. Don't just watch the sea of runners on your TV this year... lace up your trainers and become one of them. And use your race to help us win our race to find a treatment for FOP.

For more information, visit: <https://fopfriends.com/gnr/>



A Small Bookcase with a Big Mission!

If you've ever had the pleasure of meeting little Harry, you'll understand why he isn't short of friends! Long-time family friend, Susan, wanted to help when she heard of his diagnosis. She tells us:

"Harry's mum, Harriet, was my daughter's bridesmaid, and we've known Harry since the day he was born. Watching him grow up has been a joy; he is such a character, always making everyone laugh at our family BBQs.

When my daughter told me about Harry's diagnosis, I was deeply moved by the incredible way Harriet and Phil coped with such humbling strength. I donated when Harriet did her run, and we were there to cheer her on. I've never seen anyone look so happy and full of energy that close to the finish line.

Last year, I started a charity bookcase at Notcutts Garden Centre where I work. As our third collection box was nearing capacity, I asked my manager if we could support Harry next. Because of the personal connection, he thought it was a lovely idea.

Since sharing 'Harry's Story,' the bookcase has been more popular than ever. Most people haven't heard of FOP and are genuinely interested in learning more. I've been inundated with book donations and contributions. I truly believe that lots of small gestures add up to something significant for such a special boy."

We thank Susan for her kindness, raising vital awareness and funds for Harry. Every event - no matter the size - makes a real difference. We are thankful for you all.

