



f friends together

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www.fopfriends.com



Putting our best feet forward

This Easter saw 22 fearless fundraisers walk on hot coals to help raise funds and awareness for FOP. Those taking on our first-ever charity Firewalk included FOP Friends Chairman Chris, FOP parent Rob, FOP sibling Ross, Trafford councillors Amy Whyte and Steve Longden, and our very own FOP Nurse Jackie, who drove

all the way up from Stanmore to Manchester to take on the challenge. The night was an overwhelming success, with over £6000 raised for FOP research – an unbelievable amount and the charity's biggest one-off event to date!

To view photos from the exciting evening, visit page 4.

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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 is a Registered Charity in England and Wales 1147704



A word from Rachel



Welcome to another edition of 'Together'. It always delights me to see so much good news.

We're particularly excited to share the great time our families had at our second Family Weekend. We are also thrilled to tell those who haven't heard already about our first publication 'Supporting a child with FOP: a practical guide to their learning journey'.

We have been overwhelmed by the positive response to this book, both in the UK and internationally. However, these are just two examples of how we support those who are living with FOP; as a charity we exist to do so much more than just fundraise.

Whilst a weekend away and a book are impressive demonstrations of our support, behind the scenes we are there for our families on a daily and weekly basis. Currently, we are working to raise awareness of FOP within the medical community to improve the level of care they receive, but also to hopefully prevent future misdiagnoses.

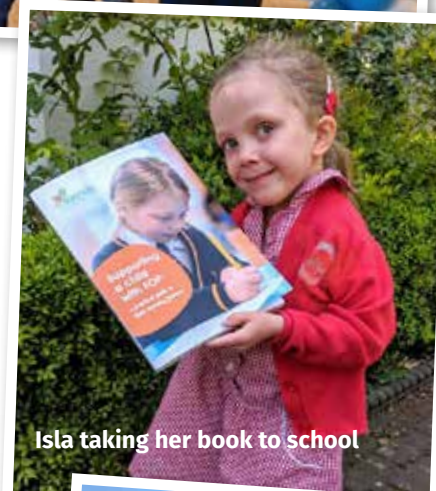
We have compiled databases of other charities that can provide the practical support our families desperately need. We are also there to provide friendship, advice, and listening ear for our families as they navigate this most challenging of journeys. I, on behalf of all the trustees, would like to thank all those who support the charity as that allows us to continue to support our families.



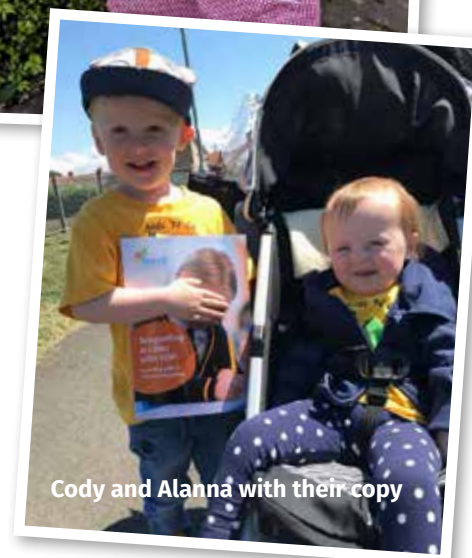
Our FOP children receiving their books at a Regeneron meet-up in May

Supporting a child with FOP

We were thrilled to launch our first publication 'Supporting a child with FOP: a practical guide to their learning journey' in May. This book was made possible thanks to a generous grant from Regeneron Pharmaceuticals. This book is an essential and informative guide for anyone supporting a child with FOP. It details FOP in a reader-friendly manner, while providing invaluable advice. It covers advocating for the child, medical considerations, family support and practical tips – from helping parents at the very beginning of their child's learning journey to providing useful age-appropriate insights and considerations as the child ages. We hope the guide will provide information, support, and encouragement to enable our FOP children to achieve their potential. Thank you to all the parents, people with FOP, medical advisors, teachers, schools, advisors, children and friends who shared their experiences and insights.



Isla taking her book to school



Cody and Alanna with their copy

Blueprint secures Fast Track Designation

Blueprint Medicines' Phase 1 trial of BLU-782 in healthy volunteers began at the start of this year, and they recently announced that the FDA has granted Fast Track Designation to BLU-782 for the treatment of FOP. Fast Track Designation means that early and frequent communication between the FDA and a drug company is encouraged. As a result of Fast Track Designation,

accelerated approval is also common, making new effective drugs available as quickly as possible.





Q&A with Clarissa Desjardins, Founder, President, CEO and Director of Clementia, an Ipsen Company

To start with, we'd love to hear a brief history of Clementia

I founded Clementia after reading a 2011 Nature Medicine article which showed that palovarotene, a drug that had been under development by Roche for a different indication, could potentially block new bone formation in animal models of FOP, a disease I had not heard of before that time. As a scientist, and a mother, I was impressed by the data and compelled to do something for those living with this disease to alleviate their suffering. While I could not have predicted exactly where we would be today, the strong efficacy data in relevant animal models and the large safety database from Roche's prior development program gave us confidence that we could rapidly advance in clinical trials towards a potential approval. We have learned so much about the disease over these past few years that we could not have predicted at the start, and this learning will benefit the entire FOP community in future research programs.

For those new to the FOP community, who may not be aware of palovarotene, would you be able to give an overview of the drug and how it works?

The mutated ALK2 receptor, which is the cause of FOP, leads to excess BMP signalling that results in new bone formation. Palovarotene is an oral once-daily pill, which binds and

activates the retinoic acid receptor gamma (RAR γ), and potentially inhibits BMP signalling by reducing the abundance of the second messengers responsible for BMP signalling. Palovarotene was initially developed by Roche for COPD and emphysema, and tested in over 450 patients who were dosed for up to two years. Since we acquired palovarotene from Roche we have tested palovarotene extensively in FOP in our Phase 2 trials, and in 2018 we completed enrolment of over 100 individuals with FOP in our Phase 3 trial (the MOVE Trial) which is evaluating a new dosing regimen for FOP. In September 2018 at ASBMR we presented updated data showing a >70% reduction in new bone formation after a flare up for those individuals treated with palovarotene.

It was announced at the start of this year that Clementia was seeking FDA approval for palovarotene – how is the process to get this approval going? Can you confirm any timescales?

We met with the FDA in October of 2018 and asked if our Phase 2 data could form the basis for a New Drug Application (NDA) for palovarotene. The FDA agreed that our Phase 2 data could form the basis of an NDA submission, and following that meeting we announced that we would be submitting the NDA for palovarotene to the FDA by the end of 2019. Since that time, we have had multiple meetings

with the agency and continue to be on track for an NDA submission by year end. Based on those timelines, we could be approved and on the U.S. market in mid-2020. International regulatory filings (ex-US) are expected to begin in 2020.

Shortly after this, it was also announced that Clementia would be merging with Ipsen Pharmaceuticals – what does this merger mean for the future of palovarotene?

Our clinical trials and regulatory plans are unchanged and the entire Clementia team is now part of Ipsen. Ipsen has a shared patient-centric culture and their global commercial presence and capabilities will expedite our shared vision of bringing palovarotene to patients.

Thank you for taking the time to answer our questions. Is there anything else you would like to add?

I would just like to thank the entire community of those living with FOP for the life lessons they have taught us. We feel privileged to have worked side by side with you and the dedicated healthcare professionals to advance palovarotene towards potential approval, and we look forward to continuing on this collaborative journey.

Ipsen Pharmaceuticals acquire Clementia

Ipsen is a leading biopharmaceutical group that specialises in developing medicines for rare diseases. They are excited to have acquired Clementia, and their drug Palovarotene to enable

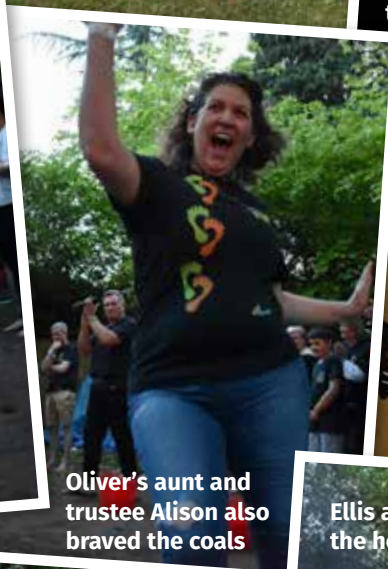
them to move forward with improving the lives of those affected by FOP. They are delighted to be able to continue the excellent work of Clementia. To read more, visit www.ipсен.com



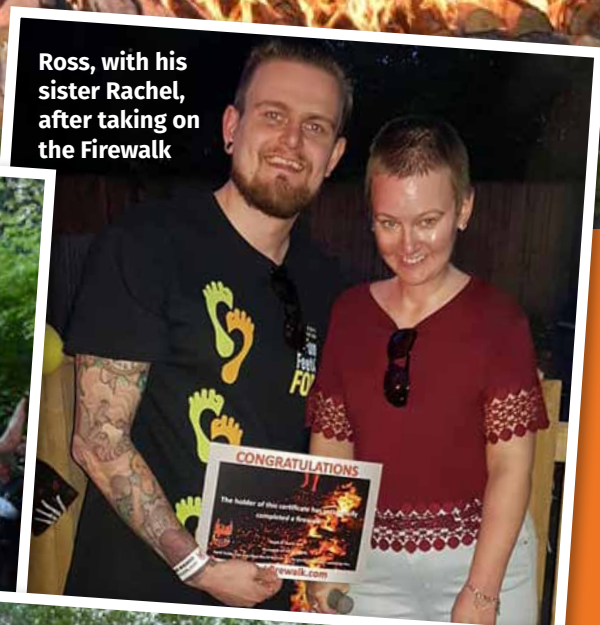
Firewalk continued...



Nurse Jackie marching for all her FOP friends



Oliver's aunt and trustee Alison also braved the coals



Ross, with his sister Rachel, after taking on the Firewalk



Ellis' dad Rob taking on the challenge



Ellis and Oliver lighting the hot coals

Christmas comes early in the office!

Summer may be in full swing but we're already getting into the festive spirit – our new Christmas card has been sent to print!

Last Christmas, our FOP community all came together in a brilliant joint effort to raise awareness of FOP. We can't wait to outdo last year.



Attending Oxford University's Vice-Chancellor's Dinner

Helen and Chris were delighted to represent FOP Friends at Oxford University's Vice-Chancellor's Dinner in May. They received the prestigious invitation in recognition of the charity's contribution to their excellent FOP research team, led by Dr Alex Bullock.

After a welcome by Vice-Chancellor Louise Richardson, there was a panel discussion on the impact of Artificial Intelligence (AI) on our everyday lives. Professor Charlotte Deane gave a particularly interesting speech on how AI could be a real game changer for



drug development, with its potential to make drug discovery quicker, cheaper and more effective.

To read more, visit www.ox.ac.uk/ai

Other Fun Feats

Our fourth #FunFeet4FOP was bigger and better than ever. Thank you to everyone who supported us with our campaign this year. We had some brilliant photos and so many shares on our social posts, raising loads of vital awareness for FOP. Along with our Firewalk, check out some other fun feats below...



Silly socks at school

We were so delighted to hear that Oliver's primary school held a 'Silly Sock' day to celebrate #FunFeet4FOP and help us raise as much awareness as possible. They not only shared some brilliant photos but surprised us by announcing they'd raised over £330 for FOP research. Thank you to all the staff, students and parents at Tyntesfield Primary for being so thoughtful and generous!

Get involved next year! Most schools decide on their charity fundraising dates early in the academic year, so get in touch with your children's teachers and save the date.



Some well needed R&R

FOP Friends of Isla enjoyed a Charity Pamper Evening, raising £370 for FOP research. The school mums were treated to a facial tutorial and massages from Tiffany Choat Beauty and received gorgeous gifts from Aurelia Probiotic Skincare.



Cakes and competitions at RNOH

Not only did Nurse Jackie walk on hot coals for FOP but she also organised an FOP Awareness day at the Royal National Orthopaedic Hospital. There was a teddy naming competition and cake sale, which raised an impressive £220. Thank you to Jackie for all your efforts and to all the patients, relatives and staff for their incredible support.



Want to sing with a friend, just duet!

The community in Framlingham put on their best dancing shoes and shouted from the rooftops with their wonderful Karaoke night to help raise awareness of FOP. The night was an overwhelming success, bringing friends and families together in style. Local businesses also generously donated their services/goods to help with the charity raffle on the night. Thank you to Darren, Gemma and everyone who took the time to get involved. An unbelievable £912 was raised!



Garden Centre helps grow awareness

Thank you to Sally and Graham at Garthowen Garden Centre, who very kindly helped us raise awareness by featuring one of our collection pots at their tills, in aid of FOP Friends of Jamie. They've always been so supportive in the past and their efforts mean so much to our small but special charity.

"It was an amazing weekend. Meeting up and being able to have fun with other FOP families makes us feel like we are not alone." FOP parent



Friends, families and a whole lot of fun

Friday 15th March saw 13 of our FOP families descend on Center Parcs, Sherwood Forest for a fun-filled weekend, surrounded by nature, friends and adventure.



The weekend began with an informal Welcome Evening on the Friday for the families to come along to, once they had arrived at the venue. This was a very relaxed event and all the families enjoyed having the opportunity to catch up with each other, and to comment on how all the children had grown!

We organised a family bowling session for the Saturday lunch where all the families chose their own teams and all the children chose who they wanted to play with. The bumpers along the edges and the bowling ramps meant all children could join in and there were many laughs.

On the Saturday evening, we held our family buffet dinner. Seating was open and the children and families all sat with their friends. There was also a children's entertainer who had a range of games and activities that

the children could all join in with, at their own level. Everyone had a blast, dancing, plate spinning and there was even some limbo!

Each child also had the chance to choose an activity of their choice to complete over the weekend, examples included den building, teddy bear making, archery, laser clay pigeon shooting, aqua jetting and horse riding. They had the rest of the weekend to spend in the pool, hang out in the woods, or take time out in the lodge.

The feedback we received highlighted how valuable the weekend is to our families. Many are already looking forward to our next get together! Since its inception in 2012, FOP Friends have made great strides to help fantastic friendships form amongst our FOP children and their siblings and we want to continue to build on this. We do have





"[We loved] making memories this weekend with the FOP Families that hold a very special place in our hearts and make this journey so much easier & brighter." FOP parent

a treatment for FOP on the horizon but for that to be fully effective, we need to nurture the children's mental wellbeing too. Thank you to everyone who came along and fully immersed themselves in the weekend. Special thanks to The Adamson Trust, Genetics Disorders UK, The Percy Hedley Foundation, and The Bruce Wake Foundation for their generous grants – without their support the weekend would not have been possible. Finally, thank you to trustee Helen whose dedication and hard work has allowed for this amazing opportunity to connect with other FOP Families.

"I made lots of new friends this time, and I enjoyed meeting up with my old friends." FOP child



SAVE
THE
DATE



Conference and Family Gathering 2020

The date has been set for the 2020 FOP Friends Conference and Family Gathering. The venue will be the same Manchester Radisson Airport Hotel. We're in the early stages of planning but if any families would like some ballpark figures to help to budget for the weekend, please contact us at info@fopfriends.com.

Our Awesome Athletes



Harry's first 5k

Congratulations to the amazing Harry, who completed his first-ever 5k run, at just 6-years-old! He ran the whole 5k without stopping and even managed to sprint for the finish. Not only that but he more than doubled his fundraising target – raising over £1000! Thank you so much, Harry. You're a superstar.



21 km in 2hrs 25 minutes and 10 seconds

After meeting Oliver in December at Regeneron's Christmas party, Amy was inspired to take on the Cambridge Half Marathon to raise awareness of FOP. She completed the race in an impressive time and tripled her fundraising target, raising an unbelievable £912.50. An amazing achievement!



Manchester University Boat Club row in the rain

We were so delighted when MUBC got in touch with us to tell us that they had decided once again to take on a 24-hour row in aid of FOP Friends. The team rowed through the night and through torrential Manchester rain, but their spirit never faltered. They are such a thoughtful University society and their efforts meant a lot to us all.



Julian's second cycle

Julian took on the Aberdovey Bike Ride for the second time, cycling over 105 miles in an impressive 7 hours and 35 minutes. This was no easy feat, but Julian managed to complete the cycle 6 minutes faster than last time. Congrats Julian and thank you for all the support!



About FOP

Fibrodysplasia ossificans progressiva, or FOP, is one of the rarest and most disabling genetic conditions known to medicine. In people with FOP, bridges of bone develop across the joints, progressively forming a second skeleton that imprisons the body in bone. Currently, there is no known treatment or cure for FOP.

A cure for the disease could also benefit people living with osteoporosis, arthritis and heart disease, as well as those affected by heterotopic ossification (a major complication in hip replacement surgeries and in sports and military injuries), and other more common bone and muscle disorders.

FOP Friends

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Thanks to the generous grant from **The February Foundation**, we were able to produce this newsletter.

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



Give with confidence

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