



# PROGRESS Report

APRIL 2021

## Gene Therapy Research Takes Exciting First Steps

In Pursuit of a Cure  ifopa

Exciting progress is already being made as gene therapy research begins thanks to the success of the *In Pursuit of a Cure Campaign* in 2020.

The FOP community came together to support the IFOPA's first-ever targeted research grant in gene therapy, allowing the recipients – Drs. Guangping Gao and Jae-Hyuck Shim of the University of Massachusetts – to take important initial steps.

Gene therapy represents a promising new frontier for treating a number of rare genetic diseases, and the IFOPA is grateful for the opportunity to launch research efforts in this direction.

"The IFOPA has organized a group of gene therapy experts along with FOP scientists to leverage their collective expertise and provide guidance and support on the research, said IFOPA Executive Director Michelle Davis.

"Drs. Shim and Gao have made remarkable progress over these past nine months."

**In collaboration with FOP specialists and their own team of researchers, Drs. Shim and Gao have focused the work on three different approaches to treating FOP. This includes gene silencing to reduce the number of copies of the FOP receptor protein, gene addition which increases the number of copies of the healthy receptor protein, and gene replacement to reduce the FOP receptor protein and increase the healthy receptor protein simultaneously.**

### SO FAR, THE TEAM HAS:

- » Generated adeno-associated virus (AAV) vectors for the three different gene therapy approaches. AAV vectors are delivery mechanisms to carry the new genetic material to the target cell.
- » Tested to make sure the AAV vectors can target the right cells and tissues in FOP.
- » Tested the gene therapies in human FOP cells.
- » Begun to test gene therapy in adult FOP mice to see if this approach can reduce extra bone.

With these significant developments, there is an ambitious plan as they finish the first year of their grant. The research team hopes to generate AAV vectors to correct the FOP mutation (gene editing), examine gene therapy in juvenile FOP mice, and generate AAV vectors carrying genetic materials that may suppress extra bone formation in FOP mice in response to a flare-up.

The IFOPA is committed to exploring every avenue of research toward a cure for FOP. In addition to drug development and clinical trials, gene therapy offers another path forward and there's hope it will benefit the FOP community over time.

"We are so pleased to be working with the IFOPA to aggressively pursue curative approaches to treating FOP," Dr. Shim said.

"While there is still a long road ahead, with many challenges to tackle, we are laser-focused on developing a novel gene therapy for FOP."

**THANK YOU**  
to the incredible group  
of researchers and  
experts moving this  
project forward!

Drs. Guangping Gao, Jae-Hyuck Shim (Lead Investigator), Jun Xie, Yeon-Suk Yang, Jungming Kim and Oksun Lee of the University of Massachusetts

Drs. Fred Kaplan and Eileen Shore of the University of Pennsylvania

Dr. Jeffrey Chamberlain of the University of Washington

Dr. David Goldhamer of the University of Connecticut

Dr. Benjamin Levi of the University of Texas Southwestern Medical Center

Dr. Paul Yu of Harvard Medical School and Brigham and Women's Hospital



**LEARN MORE**

about gene therapy and  
In Pursuit of a Cure at [ifopa.org/curefop](https://ifopa.org/curefop)

# In Pursuit of a Cure Fundraisers Bring Hope to the FOP Community



## In Pursuit of a Cure

### CO-CHAIRS

Rory & Eric Otto  
Rebecca Wallace & Kyle Wallace

**Thank you to all *In Pursuit of a Cure* fundraisers!**

### TOP 10 FUNDRAISERS

Rory Otto	\$77,822
Rebecca Wallace & Kyle Wallace	\$14,058
Candace Hixson	\$2,555
Amy Gordon	\$2,439
Sue Dupre	\$2,256
Lawanda Maryland	\$1,903
Felicia Wray	\$1,573
Stephanie Burgess	\$1,534
Cassie Eckart	\$1,170
Michelle Davis	\$1,076



Photo left: Sue Dupre (far right) with nephew Gabe, niece Angela and great niece Alex who lives with FOP; Photo right: Lawanda Maryland with son Quinton who was recently diagnosed with FOP

In August 2020, the IFOPA launched *In Pursuit of a Cure* – a research campaign that started with an educational webinar on gene therapy and culminated in a virtual global event to raise funds to support the first-ever FOP research grant in gene therapy at the University of Massachusetts, as well as ACT for FOP drug development grants. The community came together in a huge way raising nearly \$490,000.

The incredible success of the *In Pursuit of a Cure* Campaign inspired hope and gratitude in the FOP community. It was only possible because of the FOP community's incredible generosity and the dedication of 24 fundraisers who supported the campaign.

Individuals with FOP, family members and friends went above and beyond to fund new research. We can't thank them enough.

Lawanda Maryland saw the "Inside Edition" feature from the 2019 Family Gathering and realized her son, Quinton, had FOP. After Quinton, age 3, was diagnosed, she wanted to generate support for research. She used text messages and Facebook to contact anyone who might help.

"*In Pursuit of a Cure* is meaningful to me, in that it gives us hope a cure is on the horizon. We want to participate in every effort to find a cure for FOP in Quinton's lifetime," Maryland said.

"This also offered the opportunity to share our son's diagnosis with everyone in a way that gave them a chance to digest the news in their own space and time."

Sue Dupre got involved to support her niece, Alex, who is 14 and lives with FOP. She took advantage of having a large network of friends across the country that could be reached on Facebook and sent emails about the campaign. She exceeded her fundraising goal and was moved by how many people wanted to offer support.

"I see the need for this research every day in Facebook posts from Alex and her mom who posts as Alex's Army. I'm happy to help and will continue to stay informed and make contributions as I'm able," said Dupre.

"Also, I'm astounded at the rate at which FOP research is moving, thanks to the IFOPA. So much has happened in the seven or eight years I've known about FOP and the more money we funnel into this research, the sooner babies born with FOP will face a healthier future."



**BE SURE YOU'RE SIGNED UP FOR FOP CONNECTION**, the IFOPA's monthly eNewsletter, to receive updates on our gene therapy and drug development grant programs. Sign up at [ifopa.org/connect](https://ifopa.org/connect)

# FOP Awareness Day Goes Global



For a list of all countries served by FOP organizations, visit [ifopa.org/fop\\_international\\_organizations](https://ifopa.org/fop_international_organizations)

For the first time, FOP Awareness Day is a truly international event. Organizations worldwide are coming together in a new and powerful way to coordinate efforts with "Uniting to Cure FOP" as the theme for 2021.

Resources for this first-ever Global FOP Awareness Day have been available online at [ifopa.org/fop\\_awareness\\_day](https://ifopa.org/fop_awareness_day), and members of the FOP community from around the world planned awareness activities for their countries and with the IFOPA to help bring attention to FOP and the search for a cure.

"With the motto of FOP Germany for 2021 being to 'Focus on Possibilities,' we are thrilled to be a part of the Global FOP Awareness Day campaign to raise as much awareness as possible. This is vital because we are all in this together and can do so much more with joint forces," said Nadine Grossmann, co-chair of FOP e.V.

Individuals with FOP, family members, caregivers and friends, as well as researchers, clinicians and pharmaceutical companies have been active in raising awareness. From framing their Facebook profile picture to sharing FOP facts and videos as part of the 23 Days of FOP Awareness Challenges to making a donation in honor of FOP Awareness Day, there have been lots of ways to get involved. Donations made through April 30 will be matched dollar for dollar (up to \$40,000).

New FOP awareness shirts are also available to help increase support and spread the word. **Short and long-sleeve shirts, tanks and sweatshirts are available in unisex, women's and children's sizes.** International orders are welcome.



## APRIL 23 FOP AWARENESS DAY

celebrates the day it was announced that the FOP gene, ACVR1, had been discovered.

**15 YEARS** 2021 marks the 15th anniversary of the gene discovery.



**GRAB YOUR SHIRT**  
**AND RAISE AWARENESS ALL YEAR LONG!**

Visit [ifopa.org/fop\\_awareness\\_day](https://ifopa.org/fop_awareness_day)





# Six Grants Awarded to Advance Promising Research

Thanks to the incredibly generous support of the FOP community, the IFOPA recently awarded six grants to fund vital research – four ACT (Accelerating Cures and Treatments) for FOP Grants and two through the Million Dollar Bike Ride (MDBR) Pilot Grant Program.

**Since 2015, 22 ACT for FOP Grants have provided more than \$1.2 million in funding to scientists for the research and development of safe, transformative therapies for FOP through a competitive application process.** We deeply appreciate all who've donated to this important program, especially our 2020 funding partners FOP Australia, FOP Friends®, FOP Italia and Joshua's Future of Promises.

Our partnership with the Penn Orphan Disease Center (ODC) at the University of Pennsylvania helps fund additional research through the MDBR. We're very grateful for the Team #cureFOP riders whose fundraising efforts, and a donation from the IFOPA, raised \$50,000 which was matched with \$30,000 from the ODC making two new FOP research grants a reality. **Since the IFOPA started participating in the Million Dollar Bike Ride in 2018, we have funded more than \$235,000 across six research grants.**



## 2020 ACT FOR FOP GRANT RECIPIENTS

### Combined Effects of an IL-1 $\beta$ Inhibitor and Microbiota Ablation on Heterotopic Ossification in FOP Mice



#### PRINCIPAL INVESTIGATORS:

(Left) Daniel Perrien, PhD; (Right) Ed Hsiao, MD, PhD

**INSTITUTIONS:** Emory University;

University of California, San Francisco

**COUNTRY:** United States

**AWARD AMOUNT:** \$50,000

**DESCRIPTION:** FOP flares are highly inflammatory and published evidence suggests immune cells from FOP patients are more sensitive to inflammatory activity. We and others have proposed there may be anti-inflammatory approaches to reduce heterotopic ossification (HO) and flare intensity and/or frequency. Microbiota, the bacteria and viruses that live on or in our bodies, have recently been recognized as a major regulator of systemic inflammation, including levels of the inflammatory protein IL-1 $\beta$ . Our preliminary data show that removal of normal gut bacteria reduced injury-induced HO in FOP mice by about 50%. A published case report also suggests use of an IL-1 $\beta$  inhibitory drug reduced ongoing and future flares in multiple FOP patients. Therefore, this project will test the combined effects of blocking IL-1 $\beta$  and removing gut bacteria

on flare progression and HO formation in FOP mice. The goals are to generate key initial data needed to determine if manipulation of gut microbiota combined with anti-IL-1 $\beta$  therapy holds the potential to reduce inflammation and HO in FOP. If our hypotheses are proven correct, the data may support a future interventional clinical trial in FOP patients and collaborative applications for National Institutes of Health (NIH) funding.

### Efficacy and Safety of anti-IL-1 Treatment in Children, Adolescents and Young Adults with FOP



#### PRINCIPAL INVESTIGATOR:

Ruby Haviv, MD

**INSTITUTION:**

Meir Medical Center

**COUNTRY:** Israel

**AWARD AMOUNT:** \$69,144

**DESCRIPTION:** FOP is a result of ongoing intra-cellular signaling through the bone morphogenic protein (BMP) pathway. Interleukin-1 (IL-1) has been linked to the mineralization of human and mice bone marrow mesenchymal cells. Investigators hypothesized that treating FOP patients with anti-IL-1 agents will help improve life with this disease, including minimizing FOP flares. Dr. Haviv currently treats three FOP patients with canakinumab, an anti-IL-1 agent. To date, encouraging responses are being observed with flare rates and heterotopic ossification progression. This grant is to support a small pilot study to expand our understanding of IL-1 inhibition in FOP and its role as a potential treatment against flares.



## In Vivo Gene-Editing for FOP Based on Transgene Reconstitution



### PRINCIPAL INVESTIGATORS:

(Left to right) Shailesh Agarwal, MD; Vicki Rosen, PhD; Yuji Mishina, PhD

**INSTITUTIONS:** Brigham and Women's Hospital, Harvard Dental School, University of Michigan

**COUNTRY:** United States

**AWARD AMOUNT:** \$87,000

**DESCRIPTION:** In this study, investigators will develop a gene therapy which empowers the patients' cells to express an inhibitor of Activin A. As a result, upon injury, we expect that patients' own cells will automatically protect against the development of FOP lesions. The project draws on previous experience studying FOP and will make use of the ACVR1 R206H mouse model. This approach will include in vitro studies to validate the gene therapy and in vivo studies to quantify ectopic bone volume using microCT imaging. Our goal is to develop a gene therapy which has the potential for clinical evaluation.

## In Vivo Proof-of-Concept Analysis of Antibodies to Mutant ALK2 and a Next-Generation Bispecific anti-ALK2-Inflammatory Pathway Inhibitor



### PRINCIPAL INVESTIGATOR:

Nicholas Nicolaides, PhD

**COMPANY:** Navrogen

**COUNTRY:** United States

**AWARD AMOUNT:** \$37,180

**DESCRIPTION:** Navrogen's research team has developed a novel approach to treating FOP by developing immune-based agents that target the FOP receptor and an immune component believed to contribute to inflammatory flare-ups associated with the underlying cause of heterotopic ossification. One of the benefits of this approach is the preferential targeting of the mutant FOP receptor and local inflammatory suppression in FOP lesions. They have formatted these agents to be suitable for future human testing. Grant funds will enable the further development and preclinical testing of these agents in vivo for efficacy and safety as the next step towards possible human testing.

## MILLION DOLLAR BIKE RIDE GRANT AWARDEES



Team  
#curefop

## Influence of Microbiota on Innate Immune Responses and Heterotopic Ossification in FOP



### PRINCIPAL INVESTIGATORS:

(Left) Daniel Perrien, PhD  
(Right) Ed Hsiao, MD, PhD

### INSTITUTIONS:

Emory University; University of California, San Francisco

**COUNTRY:** United States

**AWARD AMOUNT:** \$40,000

**DESCRIPTION:** FOP is a currently untreatable genetic disease in which skeletal muscle repair is misdirected to endochondral bone formation known as heterotopic ossification. Despite the monogenetic cause of FOP (gain-of-function point mutations in ACVR1/ALK2), disease severity and progression vary widely among patients with the same mutation. This suggests additional factors such as background genetics, environmental or nutritional influences can modify the course of disease. Based on exciting preliminary data demonstrating that removal of microbiota in FOP mice reduces injury-induced EHO, this project will determine whether introduction of specific anti-inflammatory bacteria to the gut microbiota can regulate the severity of injury-induced flares in FOP mice. Unlike commercially available supplements, the probiotics in these studies will include highly potent live bacteria specifically selected for their newly discovered roles in regulating musculoskeletal diseases. If our hypothesis is proven correct, these studies may form the foundation for a clinical trial in FOP patients and multiple applications for NIH funding.

## Role of the GM-CSF Pathway in Heterotopic Ossification Associated with FOP and Novel Therapeutic Strategies to Suppress the Inflammatory Response

### PRINCIPAL INVESTIGATORS:

(Left) Eileen Shore, PhD; (Right)

Nicholas Nicolaides, PhD

### INSTITUTION/COMPANY:

University of Pennsylvania, Navrogen

**COUNTRY:** United States

**AWARD AMOUNT:** \$40,000



**DESCRIPTION:** FOP is caused by a mutation of the ACVR1 gene, however, poor understanding of the cellular mechanisms that induce HO has limited new drug development and treatments. It is clinically established that flare-ups precede HO in patients and that inflammatory cells are abundant in FOP lesion biopsies. This proposal will examine whether GM-CSF-responsive cells support progression of FOP HO lesions and whether neutralizing antibodies targeting GM-CSF prevent HO. Our proposal is based on our findings, generated through a previous MDDR grant, that hyperproliferative cells in HO lesions express high levels of GM-CSF receptor, and that anti-GM-CSF antibodies reduced HO in pilot experiments. Confirmation that the GM-CSF pathway plays a direct or indirect role in HO will better define the specific cellular and inflammatory pathways influencing HO in FOP and offer new strategies to treat and/or manage the disease, including near-term clinical trials using existing anti-GM-CSF monoclonal antibodies that have already been used in human clinical trials.

# 2020 Virtual Family Gathering Increases Participation and Accessibility



## CONNECT

Coming together to connect as a community was a highlight for participants of all ages and backgrounds.

**NEARLY  
650  
PEOPLE  
REGISTERED**



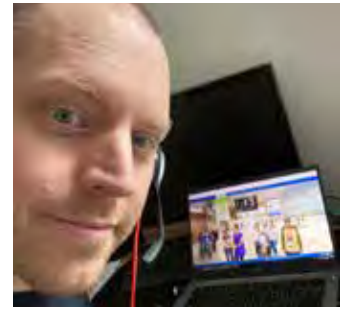
**251  
FAMILIES**



**120 ADULTS WITH FOP  
20 CHILDREN WITH FOP  
24 LOCAL HEALTH  
CARE PROVIDERS**

**47  
COUNTRIES  
REPRESENTED**

**LIVE AUDIO  
TRANSLATIONS IN  
17 LANGUAGES**



**T**he pandemic brought many challenges, but also the opportunity to create a Virtual FOP Family Gathering with unique resources and more accessibility than ever before.

Thanks to amazing sponsors, the FOP community worldwide participated in the event at no charge. The free, online gathering allowed us to:

- » Eliminate barriers such as cost or travel difficulties that kept some people from past in-person events
- » Offer access to materials and presentations for a full year following the event
- » Expand to include a greater international focus in presentations and community discussions
- » Share resources from FOP national organizations around the world, IFOPA programs and the FOP Family Fun Zone in virtual exhibit hall booths

"Not being able to travel long distances, I haven't attended the Family Gathering in 20 years. The virtual format made it possible," Sharon Kantanie, from the United States, said.

"My family greatly appreciated the chance to learn new things and socialize with other families...There's just no substitute for special opportunities like this."

Collaboration with the International President's Council helped draw attendees and presenters from around the globe and the general sessions included written and audio translations in 17 languages. This brought new perspectives to talks including the Ability Toolbox Community Member panel and the International FOP Medical Experts panel.

"The online event was incredibly inclusive and provided opportunities for the FOP community globally to access talks and meet other people

who are in similar situations. It was quite an experience for such an isolating disease," said Suki Turvey from the United Kingdom.

An important part of every Family Gathering is the time spent with people who understand what you're going through. Small group discussions were held within the event to give specific groups a place to form friendships and share experiences.

"What I appreciate most about the Family Gathering is the connectivity of the FOP community, especially all the wonderful moms. I'm so honored to have been part of the moms' discussion group. It means the world to me," Lori Danzer, from the United States, said.

Connections were fostered through a variety of engaging games and activities as well.

"Playing Bingo was phenomenal, fun and allowed me to connect with the community.

I saw many FOP community members demonstrate their incredible creativity in the talent show. With being a virtual event, the IFOPA maintained the family atmosphere that exists in face-to-face gatherings," said Ariana Romero from the United States.

Building on the success of the 2020 Virtual FOP Family Gathering, the IFOPA will host an online event again this year on November 19-21, 2021.

"While we miss seeing members of the community in person, we look forward to welcoming everyone on our virtual platform in 2021," said IFOPA Family Services Manager Hope Newport.

"We hope to have more community members involved in presentations, and more opportunities for connection outside of the general sessions to make the most of this time we have together!"



(Top) Sienna Otto participates in the Garbage Man Challenge FOP Fun Zone activity and (Bottom) Jaxon Hamilton shows off the #cureFOP beanie he won participating in Cotton Ball Soccer

# Virtual Family Gathering Creates Connections for Kids

Children with FOP need the unique support of friends who understand the challenges of living with mobility restrictions and the precautions often taken to prevent flare-ups and new bone growth. It's rare to find that in person, so the Family Gathering makes space for kids to connect. By moving the event online in 2020, it was possible for even more kids to feel like they're a part of the FOP community.

Within the virtual exhibit hall, the Fun Zone booth offered lots of fun activities and great resources. The booth was staffed by Lexi Gonzales, a certified therapeutic recreation specialist and sister of 10-year-old AJ who has FOP. Lexi provided everything from mindfulness moments and story times to recipes – all tested and approved by AJ!

There were also two Kids Contest events held on zoom during the Family Gathering which showcased how activities can be both fun and practical for disease management.

Kids raced each other in the Garbage Man Challenge to see who could pick up all their "trash" first without using their hands. They enjoyed competing against each other while using tools from around the house, such as kitchen tongs or a reaching stick, to grab small items and place them in bins or containers. Adaptive equipment was never so fun.

No one stayed on the sidelines during Cotton Ball Soccer. Using straws, kids blew their cotton soccer balls into a goal with only one breath. Each attempt started from a further distance away until there was a winner. Everyone got silly with the challenge and performed deep breathing exercises to improve lung capacity and pulmonary health.

Youth attendees were also great participants in the community social events that took place throughout the Virtual Family Gathering weekend.

"Sienna, who is 10, loved staying up late to play Bingo and watch the Talent Show during the Family Gathering, and she had a blast with both Kids Contests and the Fun Zone," said Rory Otto, Sienna's mom.

"It was really nice for her to see friends with FOP and have the chance to interact in a fun and casual setting. She also loved seeing staff who are like family to her now."

Some kids had been involved in past events, but other participants were interacting with the IFOPA and FOP community for the first time. They all received prizes and made friends – a win-win!

## LEARN

Families increased their knowledge of disease management and well-being in a variety of topics and settings.

15

GENERAL  
SESSION  
PRESENTATIONS



17

SMALL GROUP  
DISCUSSIONS FOR  
COMMUNITY MEMBERS  
BY ZOOM



6

TOPICAL GROUP  
DISCUSSIONS BY ZOOM

29 FOP COMMUNITY  
MEMBER FACILITATORS



20 COMMUNITY RESOURCE  
EXHIBIT BOOTHS

8 FUN-FOCUSED  
COMMUNITY  
SOCIAL EVENTS



(Examples include: Happy Hour, Bingo, Journaling Workshop, Talent Show)

## SMALL GROUP MEETINGS WERE HELD FOR:

- » Adult Men with FOP (ages 18 and over)
- » Adult Women with FOP (ages 18 and over)
- » Spouses and Significant Others of Individuals with FOP
- » Mothers of Individuals with FOP
- » Fathers of Individuals with FOP
- » Siblings, Extended Family and Support Network of Individuals with FOP
- » Bereaved Family Members of Individuals with FOP
- » English-speaking attendees (Individuals with FOP)
- » English-speaking attendees (Family and Support Network)
- » Spanish-speaking attendees
- » Portuguese-speaking attendees
- » French-speaking attendees
- » German-speaking attendees
- » Mandarin-speaking attendees
- » Polish-speaking attendees





## GROW

Participants shared what they are most grateful for and how they have grown through this experience.

## THANK YOU to our 2020 Family Gathering sponsors!

### VISIONARY PARTNERS



**REGENERON**  
*science to medicine®*

### LEADERSHIP PARTNER



### GATHERING SUPPORTERS



Additional Sponsorship from Keros Therapeutics

“

I think it's great that so much information has been collected, and that there is a network all over the world. My little grandson was only diagnosed in August 2020. A lot of things are very new to us and it's great to see this community. Thank you very much for that!

“

I enjoyed meeting several new moms, and it was fun to reconnect with others. It gives me strength to talk with them and share feelings that no one else understands.

”

“

We loved the Fun Zone activities and the chance to see other children with FOP.

”

“

The Virtual Family Gathering was our first since travel is so difficult. It was amazing to put names to faces and have all the current information.

”

“

There is so much information being presented that will help in every way of life!!! It was nice how everyone was able to connect with each other!

”

“

I loved the ability to connect with others around the world.

”

“

I am grateful for the ability to attend from home. There are many of us in the late stages of the disease that cannot travel or cannot travel for other reasons. I believe it's important to allow everyone the same opportunity to view exhibits, learn from researchers and meet others battling the same war.

”



**JOIN US**  
for another year of connecting  
virtually as a global community

**NOVEMBER 19-21, 2021**

If you would like information on donating to or sponsoring this important community event, please contact Michelle Davis, Executive Director, at [michelle.davis@ifopa.org](mailto:michelle.davis@ifopa.org).

# Friendship Over Pages Leads to More Online Community Events

The Friendship Over Pages (FOP) Book Club started in February 2020 and became a great way for FOP families to connect as social distancing became a way of life. There were seven book clubs throughout 2020, and participants voted on each book, read it at their own pace over a month or two and then discussed it on Zoom video conferencing.

"It has been fun and interesting to meet and talk about books I might not otherwise read," Annalena Josefsson, mom to Augusta from Sweden, said.

"What we have in common is of course FOP, but otherwise we're different ages and come from completely different backgrounds, cultures and countries. This has given me new perspectives, but also a sense of community. Overall, it's been great to have met new people in this way."

Building on the momentum of the FOP Book Club, the IFOPA created a Zoom account for the FOP community so people could take the initiative to plan their own events. There have been online get-togethers such as happy hours, crafting sessions and game nights as a result.

A highlight of the year was seeing enthusiastic participation in the social events during the Virtual FOP Family Gathering which accommodated multiple time zones and languages. Bingo, Happy Hour, a Caregiver Campfire Chat, Kids Contests and a Talent Show all drew rave reviews. The feedback is guiding planning for more online social events in 2021.

"The FOP community has always found strength and resilience in the knowledge shared by its members," said IFOPA Family Services Manager Hope Newport.

"With the virtual social events and many opportunities for fun, laughter and connection coming this year, individuals will see that the love and joy of the community is a vital part of its support as well."



"What we have in common is of course FOP, but otherwise we're different ages and come from completely different backgrounds, cultures and countries. This has given me new perspectives, but also a sense of community. Overall, it's been great to have met new people in this way."

— Annalena Josefsson, mom to Augusta from Sweden



**SEE THE TENTATIVE SCHEDULE** for this year's events and learn about participating on the video conferencing platform at [ifopa.org/community\\_social\\_events](https://ifopa.org/community_social_events)

## MARCH

Self-care and Meditation Variations

## APRIL

Friendship Over Pages Book Club Meeting

## MAY

Family Game Night

## JUNE

Friendship Over Pages Book Club Meeting

## JULY

Crafting with a Purpose

## AUGUST

Friendship Over Pages Book Club Meeting

## SEPTEMBER

Trivia Night

## OCTOBER

Friendship Over Pages Book Club Meeting

## NOVEMBER

Onstage Entertainment

## DECEMBER

Friendship Over Pages Book Club Meeting

# COVID-19 Sparks Creativity in Community Fundraising

It simply wasn't possible to hold traditional in-person community fundraising events in 2020 that typically provide significant revenue for the IFOPA's mission-critical programs and services. The FOP community embraced the need, quickly pivoted with several existing events and even created a new one to raise more than \$385,000!



The popular **MIDNIGHT SUN COLOR RUN** took on new life as a virtual 5K event with a scavenger hunt to engage the local community. People shared photos on social media and generated even more interest in the fundraiser.

"This year's virtual event allowed those living elsewhere to participate. It was very heartwarming and eye-opening. We'll definitely plan ways to include out of town supporters going forward," said organizer Lucy McConnell.



In the midst of the pandemic, the Segal, Maracic and Martucci families weren't sure if they should host the **FOP ASHLEY'S CURE GALA**. They felt it was important to provide supporters with an update on the impact of their donations, so they took the event online hoping it might reach more people and raise more awareness.

"We could not have anticipated the magnitude of generosity, love, support and the positivity that resulted in the first-ever FOP Ashley's Cure Virtual Gala. For that we are humbled and grateful," they said.



Wendy Henke had a similar experience with her **B.A.D. (BIKE ACROSS DELAWARE) TO THE BONE** event. When the 15-mile ride from one side of the state to the other was canceled, she set up a virtual event for people to bike 15 miles anywhere on their own over one month.

"We appreciated everyone who participated in B.A.D. to the Bone. We thought it was going to be a bare bones event, but it turned out to be a good way for people to donate to the IFOPA and enjoy getting outside no matter where they live," Henke said.



The hugely successful **ZIPPERO BBQ THROWDOWN** shifted from an in-person competition to a BBQ dinner donation event that raised money for the IFOPA and gave food to local nonprofits and first-responder agencies.

"We wanted to keep the momentum of ZipperQ alive. Going virtual was a great option for the BBQ teams to maintain their involvement with the event. Adding other organizations to receive the food and keep the community connected was an extra bonus," said Amy Gordon.



Tiffanie Williams hosted **GAMING FOR A CURE**, the first-ever video game tournament fundraiser. Her family got the idea while quarantined at home watching kids play games together online.

"Gaming for a Cure had almost 165 gamers playing Super Smash Bros on Nintendo Switch while watching FOP Youth Ambassador videos to learn about FOP and make donations," said Williams.

"It was a fun way to use the virtual world for our benefit, and it felt great to get creative and do something exciting and safe for everyone!"

We're thankful for the commitment and creativity that went into virtual and socially-distanced fundraising this past year. They showed the strength and resilience of the FOP community.

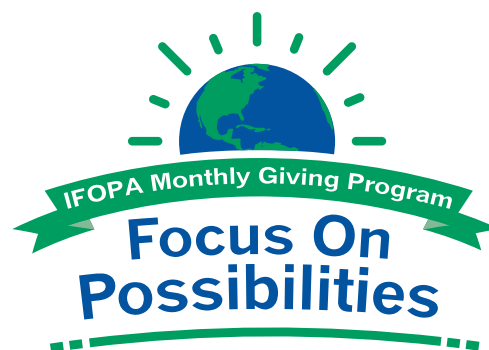
"The pandemic reinforces how critical our mission is to find treatments and a cure and to provide support programs so no one feels isolated," said Community Fundraising Manager Cathryn Roys.

"These fundraising events enable us to keep pushing forward and make sure people are connecting with others."



**NEED ASSISTANCE** with your virtual or socially-distanced event?  
Check out ideas at [ifopa.org/virtual\\_events](https://ifopa.org/virtual_events) and email [cathryn.roys@ifopa.org](mailto:cathryn.roys@ifopa.org).





# Monthly Donors Make a Difference

**Together, we work to #cureFOP.  
Together, we are committed to hope.  
Together, we Focus On Possibilities.**

**W**e're very excited to be well on the way to 100 inaugural monthly donors for our Focus On Possibilities team which started in February 2019. There are currently 92 Partners in Possibilities who make convenient donations every month to support the IFOPA's critical programs and resources.

Anyone can join the Focus On Possibilities team. The average monthly gift is around \$50, but gifts range from \$5 to \$260. This includes several contributions from people who give through a workplace matching program, so their gift is doubled.

"The combined monthly amount donated is almost \$4,500, and that's a significant annual total," said IFOPA Community Fundraising Manager Cathryn Roys.

"Together, these gifts represent nearly \$54,000 a year – a level of giving some couldn't reach as individual donors. Having these gifts to count on each month makes it easier for the IFOPA to make commitments to research and support family services and awareness programs."

The IFOPA is extremely grateful for all of our generous monthly donors. If you'd like to join them and be part of the first 100, sign up today at [ifopa.org/monthly\\_giving](https://ifopa.org/monthly_giving).

"Being a part of the monthly donor program means that I have made supporting the International FOP Association a part of my lifestyle," Erin G said. "It means that, even though I don't have FOP, I am with those who do. It means I love this community, its hope and its resilience and I'm willing to pledge financial support – no matter how small – indefinitely."



**IF YOU'RE NOT A  
MONTHLY DONOR,**

**sign up today at [ifopa.org/monthly\\_giving](https://ifopa.org/monthly_giving)  
and Partner in Possibilities!**

## THANK YOU to our monthly donors!

Anonymous Donors (21)	Chang Jae Lee
Emma Albee	Ann Lothrop
Monica Anderson	Joe Martucci
Patty & Dwayne Atherton	Abbie & Gary McGuire
Joe Barbera	Beth Miller
Bedford-Gay Family	Katy Miller
Sarah Benish	LaVerne Miller
Molly Bernier	Molly Miller
Paul Brinkman	Steven Miller
Bernard Buckley	Hope & Nick Newport
Paula & Brian Callahan	Cory Nickerson
Doug Coleman	& Mike Williams
Jan & Wedge Crouch	Family of Heather Niles
Charles Curry	Megan Olsen
Manuela & Hendrik Jan Danser	Otto Family
Michelle Davis	Natalie & Halsey Otto
Pam Emery	Dan, Kelli & Maddy Perrien
Beth Ann & Jeff Filippi	Patty Pinkham & Larry Albee
Flexer Family	Cassandra & Quentin Porter
Fransen Family	Christina Porter
Danielle Fraser	Jamal Porter
Bethany & Don Gibson	Lindsey Quinn
Lisa & Pat Gillooly	Ann Rhodes
Gonzales Family	Frank & Barb Rickabaugh
Amy & Jacob Gordon	Cathryn & John Roys
EMG & TLG Grimm	Nancy Sando and Family
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Shannon Halford	Shim Family
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Evan Harris	Chris & Paul Surovich
Peggy & Tim Hoene	and Family
Joe, Suzanne	Brianna Tanner
& Joseph Hollywood	Alicia & Josh Vera
Claudia & Kerry Hueston	Rebecca Wallace
Reina Karunaratne	Ilene Weiss
Sammi Kile	Liz Wolensky-Gadea
Karen Kirchhoff	Wray Family
Adrienne Klouse	Nichole Yco

*\*As of April 2, 2021*

# FOP Collaborative Research Project Annual Report Now Available

**"The community is deeply grateful to Dr. Kaplan for his vision in starting the report and his commitment to providing this roadmap toward a treatment."**

– Amanda Cali

The 28th Annual Report of the FOP Collaborative Research Project from The Center for Research in FOP & Related Disorders at The University of Pennsylvania is coming soon. Each year, this report captures progress achieved in the search for treatments and a cure, and looks forward to the possibilities of future research.

"The Annual Report has been a guiding light for the FOP community for the past 28 years. It's been a beacon for research and drug development, shedding light on the quest for answers to FOP," said Amanda Cali, FOP mother, Advisor to the Tin Soldiers Program, Trustee of the Radiant Hope Foundation & Executive Associate International Clinical Council on FOP.

"The metaphors that are customary in the Annual Report bring an understanding of complex concepts and provide a depth of insight to the reader, whether that person is familiar with science or not," she continued.

In addition to providing key updates, the report features "The Last Word" section to highlight the needs, experiences and hopes of people within the FOP community. It offers the chance to view research through the perspective of those living with FOP.

"The last word belongs not to the donors and benefactors, not to the physicians, scientists, researchers, journalists or historians – but to the patients – who struggle valiantly and who look to us for a better way. The patient's voice is always the most important voice in the room," said Dr. Fred Kaplan, co-director of The Center.



**TO READ THE REPORT ONLINE,** visit [ifopa.org/28th\\_fop\\_annual\\_report](https://ifopa.org/28th_fop_annual_report).  
To order a print version, please email [together@ifopa.org](mailto:together@ifopa.org).

## IFOPA Staff Share Programs and Services Worldwide

In 2020, FOP programs and services were published in journals and featured in virtual conferences around the world.

### MAY

The most recent publication from the FOP Registry was released in the Journal BONE further expanding what is known in the scientific community about FOP. Publications from the Registry are made possible by the ongoing contributions of FOP patients and their caregivers when they complete Enrollment and Follow-up Surveys.

### JUNE

Former IFOPA Research Director Adam Sherman spoke at the Global Genes Rare Drug Development Symposium on the topic "How to Be a Great Research Partner."

### SEPTEMBER

The IFOPA was pleased to showcase our FOP Registry – along with important emerging data – in a poster at the 2020 American Society of Bone & Mineral Research Annual Meeting.

### SEPTEMBER

IFOPA Executive Director Michelle Davis spoke at Global Genes LIVE: A RARE Patient Advocacy (un)Summit on the topic "Global Rare Disease Advocacy & Cross-Border Collaborations." Among other topics, her talk featured the International President's Council which was founded in 2007.

### OCTOBER

A poster highlighting the implementation and growth of the IFOPA's Resilient Living Program was featured at the NORD Rare Diseases and Orphan Products Breakthrough Summit. The poster was authored by Hope Newport, IFOPA Family Services Manager.

### OCTOBER

FOP Registry Project Manager Sammi Kile spoke at the European Calcified Tissue Society Annual Meeting on the topic "Patient-Driven Data in Rare Diseases."

# New Web Page Links FOP Community to Research Studies & Clinical Trials

A new page on the IFOPA website creates greater awareness and understanding of the drug development process and connects families with current research studies and clinical trials.

With an easy to remember URL – [ifopa.org/about-clinical-trials](http://ifopa.org/about-clinical-trials) – the page offers a helpful overview of trials. It also gives people in the FOP community resources for learning more about how trials are conducted and considerations for participation; there's even a glossary of terms.

"A vital role of patient advocacy organizations is keeping their community informed about research studies and clinical trials," said IFOPA Research Committee Chair Betsy Bogard.

"The IFOPA created this site to provide access to the details on all FOP studies and trials in one place. It also has great videos for families to learn more about the drug development process and clinical trials."

Clinical trials are essential to developing potential treatments. Without trials, and without patients to participate in them, drugs to treat FOP will never be considered for approval. At [ifopa.org/about-clinical-trials](http://ifopa.org/about-clinical-trials) you'll find a list of interventional trials under the "Ongoing Clinical Trials in FOP" link and a list of observational studies (where it's not required to take a drug to participate) under the "Participating in Other Research" link.



## FOP Registry

Patient-Driven Data for a Cure

The FOP Registry is the largest and most detailed collection of clinical and medical information about FOP and those who are living with the disease.

The Registry collects information on how FOP affects people to help clinicians and researchers better understand FOP in order to improve clinical care, and speed the development and availability of disease-changing treatments and, one day, a cure.

The best part about this database is that it's growing every day. That's because FOP families from around the world, and their FOP physicians, are contributing to it.



**LEARN HOW TO PARTICIPATE**  
at [ifopa.org/fopregistry](http://ifopa.org/fopregistry).



**PATIENT PORTAL  
&  
MEDICAL PORTAL**



**FOP-PROMPT  
DAILY SYMPTOM  
TRACKER**



**COLLECTING  
66-MONTH  
DATA**



**12 DATA  
REQUESTS**

**7  
LANGUAGES**



**LAUNCHED  
JULY 2015**



**69  
COUNTRIES  
PARTICIPATING**

**LONGITUDINAL DATA  
MADE POSSIBLE BY**



**6-MONTH  
FOLLOW-UP  
SURVEYS**



**IOS  
MOBILE  
APP**

**498  
REGISTERED**



**343  
COMPLETED  
ENROLLMENT**

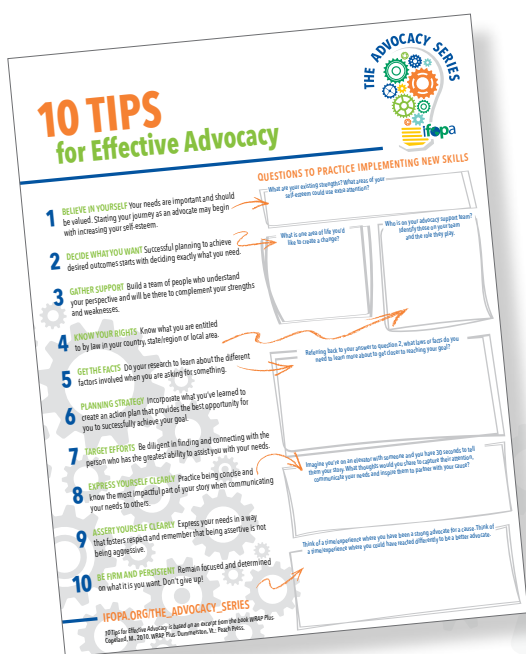




# The Advocacy Series Empowers Our Community

## Quarterly topics for The Advocacy Series in 2021

- Advocacy: You Can Make A Difference
- Advocacy at All Ages
- Advocating in a Medical Setting
- Advocating for Mobility and Independence



The IFOPA Family Services staff just launched The Advocacy Series to connect families to resources within the FOP community and beyond. The goal is to help more people with FOP and their families overcome challenges by giving them a larger support network and the knowledge needed to advocate for themselves.

"Many families share a common need for assistance in navigating and accessing resources," said Family Services Manager Hope Newport.

"From insurance approval to emergency medical preparedness, we want to empower all families to feel confident knowing they have the ability to take action and create change."

The Advocacy Series will focus on one topic every quarter, and there are three ways members of the FOP community can participate.

- » **INTRODUCTORY PODCASTS:** Staff and community members will present the topic and highlight specific challenges to address, as well as content and resources that will be covered that quarter.
- » **COMMUNITY PANELS:** Individuals with FOP and families from around the world will have the chance to connect to share experiences and learn from each other. Meetings will take place on Zoom allowing all participants to speak and ask questions. There will also be group exercises.
- » **RESOURCE WEBINARS:** Attendees will hear a subject-matter expert present on the quarterly topic and then have the opportunity to ask questions.

The Introductory Podcast will be available on YouTube so listeners can access translated sub-titles. The Community Panel and Resource Webinar will be hosted on Zoom video conferencing and simultaneous translation will be provided in the following languages: Arabic, Bengali, Chinese (Simplified), Chinese (Traditional), Dutch, English, French, German, Hindi, Italian, Japanese, Korean, Polish, Portuguese, Russian, Spanish, Tamil and Vietnamese. To learn more about Zoom and the translation resources, visit [ifopa.org/the\\_advocacy\\_series](https://ifopa.org/the_advocacy_series)

At the end of each of the quarterly periods, resources from the past three months' podcasts and Zoom meetings will be combined into a workbook style resource for families to use. There are plans to continue the series in 2022 to explore topics such as insurance, career, school and housing.

"Our hope is that the collective knowledge of individuals with FOP, their families and their caregivers will create a powerful opportunity for connection and growth for all participants," Newport said.

"We believe that for many issues and challenges, the FOP community already has the answer – but individuals may need some help in finding it."



**LEARN MORE AND GET THE SCHEDULE**  
at [ifopa.org/the\\_advocacy\\_series](https://ifopa.org/the_advocacy_series)

# Ability Toolbox Expands Personal and Online Support

The Ability Toolbox program, launched in June 2019, empowers individuals with FOP and supports caregivers by promoting independence through the use of tools and home adaptations. The program is growing rapidly and now provides support from IFOPA staff and the FOP community, as well as an Online Guidebook to help individuals with FOP live as independently as possible.

The **Tool Ambassadors** program recently launched to connect people with practical advice. Ambassadors are individuals with first-hand experience with various tools and the positive effect they have on improving confidence and overall quality of life. Members of the FOP community can request to talk with an Ambassador to hear how one of their peers handled a situation that required creative thinking to maintain independence after a loss of mobility.

"We have Tool Ambassador volunteers from various backgrounds and demographics who can speak to a wide range of needs. They're located across the United States, and we have one in the United Kingdom and one in Australia. We hope to add more international Ambassadors in 2021," said Family Services Coordinator Karen Kirchhoff.

Homemade tools tend to be of interest because they can be more cost-effective and easier to modify than commercial products. Amie Specht attended the Ability Toolbox small group discussion at the Virtual FOP Family Gathering and enjoys sharing the homemade tools that help her as she lives with FOP.

"I've learned that homemade tools are a better experience because having FOP makes my body different, not just to an ordinary person but others with FOP too. Having handmade tools lets us customize them for individual needs," said Specht.

During the Family Gathering, attendees shared devices to help with bed mobility and tools for make-up application, reaching and scratching. There were also discussions on eating and bathroom routines, durable materials and sanitizing items.



The new **Online Guidebook** is a handy database of adaptive tools, equipment, home modifications and tips for the FOP community to access as a resource for independent living. It's now available at [guidebook.ifopa.org](http://guidebook.ifopa.org) and will be a key resource for years to come as community members continue to add to it.

**Toolkits for Medical Professionals** are also in development with prototypes going to a few doctors in early 2021. These kits will offer information about the IFOPA along with a sample of common tools that can be tried out by people recently diagnosed with FOP or those asking about adaptive equipment. Doctors can email [karen.kirchhoff@ifopa.org](mailto:karen.kirchhoff@ifopa.org) to request a kit.

"We keep looking for all the ways we can provide the FOP community with more exposure to tools and more opportunities to learn how tools might improve their lives as they overcome the challenges of FOP," said Kirchhoff.



**DO YOU HAVE COOL, HELPFUL TOOLS?**  
Send pictures, videos and explanations of homemade tools to [karen.kirchhoff@ifopa.org](mailto:karen.kirchhoff@ifopa.org). They may be shared in the new Online Guidebook and inspire others to make their own!

**ACCESS THE NEW ABILITY TOOLBOX ONLINE GUIDEBOOK** at [guidebook.ifopa.org](http://guidebook.ifopa.org)  
Get information on useful tools for more independence in daily life.



## Welcome our New Tool Ambassadors:

Emma Albee  
Oliver "Ollie" Collins  
Stephie Edwards  
Steve Eichner  
Kathy and Chuck Ford  
Kristi Gonzales  
Sharon Kantanie  
Whitney Weldon



**REGENERON**  
science to medicine®



The IFOPA is able to offer Ability Toolbox: Everyday Tools for Independent Living thanks to generous support from the Radiant Hope Foundation, as well as Regeneron Pharmaceuticals and Ipsen.

# FOP Biobank Reopens After a Brief Pause Due to COVID-19



After a brief pause due to COVID-19, we are pleased to announce that the IFOPA's FOP Biobank has reopened and is accepting donations. Qualified scientists can use these samples for research that looks into factors that affect treatment and disease outcomes. These important samples are provided to researchers all over the world.

## FOP BIOBANK:

- » Collects blood, urine, saliva (DNA) and tissue (from "baby teeth") from people with FOP
- » Needs first-time donations from additional people with FOP
- » Accepts repeat donations from people with FOP (these are some of the most important donations)

Due to the need to process donations in a timely manner, the FOP Biobank can only accept donations from individuals living with FOP in the United States.

 **VISIT** [ifopa.org/biobank](https://ifopa.org/biobank) to learn more and start the donation process.

**IF YOU'RE A RESEARCHER** interested in securing samples for FOP research, visit [ifopa.org/biobank\\_samples](https://ifopa.org/biobank_samples)

## WATCH FOR THESE 2021 EVENTS:

**Every Friday & Saturday in April //**  
Gaming for a Cure: Jackbox Games Style  
(Organized by Andrew Davis)

**April 23 //** Global FOP Awareness Day  
(15th Anniversary of the announcement of the gene discovery)

**April 24 //** Riding for Tyler - #cureFOP  
(Organized in honor of Tyler Hanf)

**June 12 //** Million Dollar Bike Ride  
(Offered virtually again in 2021)

**November 19-21 //** Virtual FOP Family Gathering



**WATCH** [ifopa.org/calendar\\_of\\_events](https://ifopa.org/calendar_of_events) for fundraising events, FOP webinars and virtual social events added throughout the year.

## IFOPA STAFF

**Michelle Davis**  
Executive Director

**Danielle Kerkovich, PhD**  
Director of Research  
Development & Partnerships

**Hope Newport**  
Family Services Manager

**Cathryn Roys**  
Community Fundraising Manager

**Sammi Kile**  
FOP Registry Project Manager

**Karen Kirchhoff**  
Family Services Coordinator

**Brianna Tanner**  
Donor Relations & Digital Coordinator

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