

IFOPA Hosted the First-Ever FOP Drug Development Forum in November 2014

December 5, 2014

The IFOPA hosted the first-ever FOP Drug Development Forum in Boston, Massachusetts on November 21 and 22, 2014. The Forum was a groundbreaking event that brought together researchers from universities and biopharmaceutical companies from around the world to discuss the important questions and challenges of developing a treatment for FOP.

It is an exciting, dynamic time in the FOP community. Incredible progress is being made in many directions that could lead to a treatment for FOP, ranging from Clementia's development of palovarotene, to Novartis' biomarkers study, to the many ideas and possibilities for potential therapies being evaluated in laboratories and biopharmaceutical companies around the world. Given all the progress and growth on so many fronts, the IFOPA leadership recognized that it was time to bring the research community together.

"One of the most important things a patient organization can do is bring people together," said Betsy Bogard, Global Research Development Director at the IFOPA. "The FOP Drug Development Forum convened some of the best and brightest research minds under one roof to share the latest thinking about FOP therapeutic development."

The two-day meeting was designed to support the IFOPA's vision for a safe and transformative therapy for FOP. The goals of the meeting were to address questions and knowledge gaps that exist in FOP drug development; stimulate new ideas to help advance development of potential therapies as quickly and efficiently as possible; and facilitate

dialogue, foster collaboration, and form connections among interested researchers.

The patient voice is critical in the drug development process and was a key part of the meeting, with a small number of patient representatives playing an active role in the event.

"The Forum was a long-awaited dream of many community members where industry and academia came together in a spirit of collaboration, focused on identifying gaps in knowledge and forging relationships that will secure future treatments for FOP," said Amanda Cali, IFOPA Research Committee Co-Chair and Forum Coordinator.

Attendees

The majority of individuals invited to participate in the meeting were scientists and doctors from leading universities and biopharmaceutical companies worldwide. The IFOPA also extended invitations to several government experts, a few investment firms, the International President's Council, and a small number of FOP patient and parent advocates. The composition of the attendee list was intended to maximize dialogue and information exchange amongst researchers and foster as much progress and collaboration in drug development as possible.

A total of 128 individuals attended the meeting from 16 different countries around the world, as follows:

- 12 FOP clinicians from 11 countries
- 37 university representatives from 22 institutions worldwide
- 35 representatives from nine biopharmaceutical companies and three investment firms
- 5 participants who currently work or previously worked in the US or European government
- 5 individuals living with FOP
- 9 IFOPA Board and Research Committee members
- 4 International President's Council members
- 21 family members, volunteers, professionals, and other guests

Agenda

The meeting agenda covered four general topics in FOP drug development, organized into five "sessions" of short talks: clinical trial design and biomarkers for FOP (Session 1), natural history of FOP (Session 2), tools and models for FOP drug screening and development (Session 3), and therapeutic approaches to FOP (Sessions 4 and 5). Each of these sessions consisted of a series of four or five short "FOP Talks"—12-minute talks modeled after TED talks to highlight innovative ideas and areas where discussion is needed in FOP drug development.

In addition to these five sessions, the IFOPA was pleased to welcome speakers each morning with experience working in US or European government to discuss considerations around government approval and commercialization of therapies.

The agenda incorporated the patient speakers in three places. Two patient representatives, Ian Cali and Rory Otto, gave the opening and closing talks, respectively, of the conference. In this way, patients had the first and last word at the meeting. In addition, the agenda included a one-hour patient panel at the end of the first

day. During this panel, facilitated by Julie Collins, International President's Council representative from Australia, five patient representatives answered questions submitted in advance by researchers and drug developers. The panel was chosen to represent the age spectrum in FOP: young children, teenagers, and adults.

All five patient representatives at the meeting provided powerful perspective to the research community in attendance, with some attendees saying that the patient panel was the best part of the meeting.

Dinner Reception

After the first day of the Forum, the IFOPA hosted a dinner reception for all meeting participants. Although the event was intended simply to allow guests to relax and have fun, it provided an opportunity for several special moments. First, Marilyn Hair, Chair of the IFOPA Board of Directors, welcomed the group and shared the IFOPA's history and vision. Second, we were pleased to issue three awards to individuals who have, over the years, been consistent leaders and contributors to FOP research activities: Nick Bogard, Amanda Cali, and Jen Snow. Third, in preparation for the Forum, the IFOPA created a short video, using video clips sent in by community members, to help motivate and thank researchers for their work. We premiered this video at the dinner reception and it is now available on our YouTube Channel, FOP News. To view the video, called "Change My Life," please click here: <http://youtu.be/CxvznUlxyk>

Outcomes

The IFOPA wanted to ensure that we had practical outcomes from this amazing gathering of scientists, doctors, and community leaders. The last part of the meeting was dedicated to group discussions of two questions: (1) What gaps in knowledge or

data do we need to address to enable development of a safe and transformative therapy? and (2) What is the one thing that the community can do *collaboratively* to enable FOP therapeutic development?

Meeting participants divided into eight groups of about 10-12 individuals per group to discuss these questions over a working lunch. Each group's input was summarized and shared in a summary led by Robert Pignolo, MD, PhD from the University of Pennsylvania. Here are the top answers that came back from the discussion groups:

Q: What gaps in knowledge or data do we need to address to enable development of a safe and transformative therapy?

1. Identification of biomarkers
2. Better understanding the variable phenotype of FOP
3. Advancing the understanding of FOP natural history
4. Better understanding the systemic nature of FOP pathology

Q: What is the one thing that the community can do *collaboratively* to enable FOP therapeutic development?

1. Share animal models, patient samples, reagents & data
2. Encourage trial participation
3. Hold Forum annually
4. Develop the registry
5. Fund collaborative grants with an open process

We are currently bringing all the ideas back to the Board of Directors for discussion and strategic planning. The outcomes of this meeting will shape the IFOPA's plans and activities for 2015 and beyond.

Dr. Pignolo closed out the discussion with wonderful words of inspiration that beautifully articulated our wish for collaboration:

"Competition should be directed toward collaboration. Be first to collaborate."

Sponsorship

The FOP Drug Development Forum would not have been possible without the generous contributions of the FOP families who have been fundraising day after day, year after year to support research and enable IFOPA operations. Their efforts are the bedrock of all that we do. In addition, we were thrilled to have sponsorship directed specifically to the Drug Development Forum from the following families and organizations:

- The Bogard Family and Friends
- Clementia Pharmaceuticals, Inc.
- Genzyme, a Sanofi company
- The Henrotay Family and Friends
- Novartis Institutes for Biomedical Research

Event Planning & Operations

Betsy Bogard, Amanda Cali, and Lori Henrotay led preparations for the Forum, with direction and approval from the IFOPA Board of Directors.

We received input and guidance from the University of Pennsylvania team, to whom we are grateful for helping us develop the preliminary agenda. We also received input and feedback from various participants along the way to ensure the agenda would meet their needs. Their collective expertise helped make the Forum the great success that it was.

We were fortunate to have the skills, talents, and time of a small army of volunteers to assist with the event. The volunteers helped with everything from graphics layouts of our materials, to staffing the registration desk, to distributing microphones during question and answer sessions. The team of volunteers

included IFOPA board members, patient advocates, International President's Council members, Research Committee members, and friends of the FOP community. The Forum would not have been successful without their extraordinary contributions.

We thank the IFOPA staff at the Florida office, Denise Vietti, Victoria Mandracken, and Sue Weidner, for their tireless work in support of the Forum and our quest to find a therapy for FOP.

The Venue

The meeting was held at the historic Fairmont Copley Plaza Hotel in Boston, Massachusetts. This location was chosen for its proximity to Cambridge, Massachusetts, one of the most vibrant cities for biotechnology, the pharmaceutical industry, and drug discovery. The Fairmont Copley Plaza is a short drive from the Boston airport, a major hub for air travel, making it accessible to those traveling from far away.

The Fairmont is a highly sought-after venue. It would not have been possible for us to host the meeting at this wonderful location without the willingness of the exceptional team at the Fairmont to accommodate our non-profit budget. Thanks to their support of our cause, we were able to host the meeting in a world-class hotel while still maintaining our modest event budget.

Feedback from Participants

Immediately following the Forum, we sent out a survey to ask for feedback on the event, which so far has been overwhelmingly positive. Our email inboxes have been filled with words of appreciation from attendees. Here are some of the comments we have received to date:

"The Drug Development Forum was an exceptional milestone in the amazing history of the FOP community.... It was an absolutely magnificent, first class event. I was proud to be there. You all should be very proud of what you accomplished."

—Frederick Kaplan, MD, University of Pennsylvania

"I would like to extend my sincerest thanks for what you put together for the FOP community. I attend and participate in many conferences/meetings/symposia, big and small, and I can tell you that the FOP2014 forum was one of the best that I have ever attended. But, frankly, no words can match what I would really like to say – the Forum that you put together was simply exceptional."

—Aris Economides, PhD, Regeneron Pharmaceuticals

"The symposium last weekend was one of the best symposium I've ever attended in every possible way: everything was so smooth; the wonderful mix of people, right from patients, families, regulatory, clinicians, academicians and industry....Brilliant job!! On this Thanksgiving, I thank you and your team for organizing this meet. I'm back to the lab, continuing FOP research with a stronger drive, motivation and inspiration. Thank you."

—Davaveena Dey, PhD, Brigham and Women's Hospital

"I can say honestly it was the most inspiring and informative meeting I have ever attended without question. It was truly transformative for me as a person, surgeon and scientist. To meet and get to talk to the patients was fun, educating and inspiring.... I can't wait now to get back into the laboratory and start making new discoveries to help people [with FOP]."

—Benjamin Levi, MD, University of Michigan

"I wanted to thank you again for organizing a truly inspiring forum. The organization was

impeccable, and the content was incredibly valuable. Being an academic scientist is a great privilege, but it does come with many pressures. In such an inspiring way, the Forum reminded us all why we work so hard every day. Even so, the patients and you have given us far more than we can return."

—David Goldhamer, PhD, University of Connecticut

"[Your] excellent work and organization... made the Forum such a great success. Everything was perfectly arranged and the mix of talks and patient contact was exceptional. Everyone present was moved by the need to urgently find a therapeutic agent that will at least ameliorate the symptoms of FOP that result in progressive immobility. We all return to our laboratories inspired to focus our efforts and work even harder to achieve this goal."

—James Triffitt, PhD, University of Oxford

"Thank you for a wonderful meeting. It exceeded my expectations, and it was heartening to see so much interest from the industry."

—Charles Hong, MD, PhD, Vanderbilt University

"Thank you very much for your kind invitation for the fruitful and outstanding FOP 2014 Drug Development Forum.... I learned many, many things concerning FOP at the forum from you, patients, clinicians and scientists. I do hope that I will be able to contribute a little to the development new treatments for FOP in the near future."

—Takenobu Katagiri, PhD, Saitama Medical University

"I wanted to sincerely thank you for what turned out to be an amazing experience at the FOP meeting; it far exceeded my expectation.... What you and your group are doing is a lesson for all of us in the "rare" disease field and I look forward to additional opportunities of collaborating with you."

—Adam Resnick, PhD, Children's Hospital of Philadelphia

More Information

More information about the Forum, including the agenda, photos, and the list of organizations and institutions in attendance, will be available on the Drug Development Forum page on the IFOPA website soon. To view a video summary of the Drug Development Forum, please click here: <http://youtu.be/cKgjl7DVhpU>

For any questions related to the Forum or IFOPA's efforts to support FOP research and drug development, please email questions@ifopa.org.