IFOPA: Supporting the FOP Research Community

US Family Gathering
December 1-3, 2017

Confidential – For IFOPA Internal Use Only
The IFOPA is building research infrastructure to enable and accelerate drug development

**FOP Drug Development Forum**
The ONLY meeting where clinicians and researchers can come together to share data and discuss advances in FOP research

**FOP Connection Registry**
The largest, global clinical dataset of people with FOP through a patient-centered approach in order to contribute to better understand the natural progression of FOP, FOP treatment guidelines and new FOP treatments

**Competitive Grant Program**
Support FOP investigators with research funds, which support and enable the development of new therapeutic approaches for treating FOP

**FOP Biorepository**
A centralized biomaterial repository -- contributed to by academic researchers, industry and by people with FOP -- to provide researchers access to difficult to obtain human samples to enable and expedite basic research in FOP

**FOP Mouse Model**
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• IFOPA’s 3rd FOP DDF, held in Sardinia Italy from Oct 13-14
• 180 researchers, clinicians, (7) biotech/pharma companies, regulators, IPC leaders, and FOP families
• Over 30 scientific talks ranging from clinical trials to basic research
• 2 panels sessions (PwF and Drug Development)
• Unique opportunity for the FOP research community to come together to share data and network

“
It’s definitely my favorite conference to attend. I come back to the lab motivated and determined. The DDF was really interesting and perfectly organized. We find our energy and ideas in this type of exchanges.”
2017 Drug Development Forum Agenda

FRIDAY, OCTOBER 13, 2017
7 to 8:15 am  Breakfast and Networking
8:30 to 9:20 am  Welcome and Opening  
Salons de la Infanta and Reina – Ground Floor
9:20 to 11 am  FOP Talks 1: Clinical Investigation of FOP
11 to 11:30 am  Coffee Break and Networking
11:30 am to 1 pm  FOP Talks 2: Receptor Targeting in FOP  
Salons de la Infanta and Reina – Ground Floor
1 to 2:15 pm  Lunch and Networking  
Restaurant Imperial – 2nd Floor
2:15 pm to 4:05 pm  FOP Talks 3: Building the Foundation for Clinical Advancement  
Salons de la Infanta and Reina – Ground Floor
4:05 to 4:40 pm  Updates from International Councils and Consortiums  
Salons de la Infanta and Reina – Ground Floor
4:40 to 5:10 pm  Coffee Break and Networking
5:10 to 6:10 pm  Patient Panel: Perspectives on Drug Development  
Salons de la Infanta and Reina – Ground Floor
6:10 to 6:20 pm  Day One Closing
7:30 to 9:30 pm  Drug Development Forum Dinner  
Restaurant Imperial – 2nd Floor

SATURDAY, OCTOBER 14, 2017
7 to 8:15 am  Breakfast and Networking
8:30 to 8:40 am  Welcome Back and Opening Remarks  
Salons de la Infanta and Reina – Ground Floor
8:40 to 10 am  FOP Talks 4: New Therapeutic Approaches in FOP
10 to 10:15 am  2017 Competitive Research Grant Awards
10:25 to 10:55 am  Coffee Break and Networking
10:55 am to 12:50 pm  Disease Mechanisms with Future Impact for Drug Development  
Salons de la Infanta and Reina – Ground Floor
12:50 to 2:05 pm  Lunch and Networking  
Restaurant Imperial – 2nd Floor
2:05 to 3:05 pm  Drug Development Panel Discussion  
Salons de la Infanta and Reina – Ground Floor
3:05 to 3:50 pm  Insights from Other Rare Bone Disorders
3:50 to 4:15 pm  Forum Closing
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IFOPA Competitive Review Grant (CRG) Program

• The IFOPA CRG program awards funding to international investigators interested in researching FOP

• The purpose of the IFOPA CRG is to help enable development of safe and transformative therapies for the disease

• Requests for Proposals for research are focused on discovery and advancement of new therapeutic approaches to FOP with preference towards approaches likely to have near-term clinical or translational relevance

• Blinded applications are reviewed by an independent Scientific Advisory Board, free of conflict of interest and with relevant and appropriate expertise

• A three year old program that has awarded over $475,000 in research funds

• Generous support from the IFOPA, FOP Friends, FOP Australia, Canadian FOP Network, Joshua's Future of Promises Foundation, and the Brinkman family.

Scientific Advisory Board

Vicki Rosen, PhD, Chair
Department of Developmental Biology
Harvard School of Dental Medicine

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Department of Molecular, Cell and Developmental Biology UCLA/ Orthopaedic Hospital Department of Orthopaedic Surgery

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Department of Orthopaedic Surgery
University of Michigan

Michael Whyte, MD
Division of Bone & Mineral Diseases
Washington University School of Medicine

Michael Zasloff, MD, PhD
Department of Surgery Georgetown University Medical Center
The 2017 IFOPA CRG Award Recipients!

2017 Grant Recipients

Modes of treating FOP with clinically relevant Alk inhibitory compounds
Principal Investigator: Daniel S. Perrien, Ph.D.
Institution: Vanderbilt University Medical Center

H-SAADDs: Hypoxia-Selective ALK2 Allosteric Destabilizers & Degraders for FOP Prophylaxis
Principal Investigator: Jay C. Groppe, Ph.D.
Institution: Texas A&M University College of Dentistry

Interrogating new therapeutic targets for human FOP
Co-Principal Investigators: Pamela Yelick, Ph.D.; Edward Hsiao, M.D., Ph.D.
Institutions: Tufts University; University of California, San Francisco

Validation of novel muscle regenerating prophylaxis for FOP related heterotopic ossification
Co-Principal Investigators: Yuji Mishina, Ph.D.; Benjamin Levi, M.D.
Institutions: University of Michigan; University of Michigan School of Dentistry
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Biorespositories are collections of human biological materials (and data) for the purposes of conducting future research.

**FOP BIOREPOSITORY VISION:** A centralized biomaterial repository -- contributed to by academic researchers, industry and by people with FOP -- will readily provide researchers access to biosamples, which enable and expedite basic research in FOP.
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FOP Mouse Model

- The IFOPA will manage the distribution of an “open access” FOP mouse model to researchers interested in studying FOP.
- A mouse model of FOP that has the same ACVR1[R206H] mutation as ~97% of people who have FOP. This mutation can be turned on experimentally to start the process of HO.
- Enable studies to assess new therapeutic compounds and/or to further our knowledge about FOP.
- Collaboration in progress with Vanderbilt University and La Jolla Pharmaceuticals.
- Available to researchers starting in 1h 2018.
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Thank you!!

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