FOP Registry:  
Connecting the FOP Research Community  

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How one person can make an impact
Outline for our discussion

• What is a registry?
• What is the value of the FOP Connection Registry?
• How do you participate in the FOP Connection Registry?
• Questions and discussion
What is a registry?

“...a patient registry is an organized system that uses observational study methods to collect uniform data (clinical and other) to evaluate specified outcomes for a population defined by a particular disease, condition, or exposure, and that serves a predetermined scientific, clinical, or policy purpose(s).”


AHRQ Publication No. 07-EHC001-1
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A voluntary collection of medical information to help researchers, doctors, drug developers, and regulators
There are many ways of studying diseases and treatments

Studies that assess the safety and/or effectiveness of a drug (or procedure)
There are many ways of studying diseases and treatments

- Clinical Trials: Studies that assess the safety and/or effectiveness of a drug (or procedure)
- Natural History Studies: Studies that look at the natural course (e.g. no drug or procedure) of a disease
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Disease Registries: A voluntary collection of medical information to help researchers, doctors, drug developers, and regulators
There are many ways of studying diseases and treatments

A voluntary collection of medical information to help researchers, doctors, and drug developers

- “Real World” data (vs controlled)
- Everyone with the disease can participate
- Global representation
- Data collection at home
- Indefinite timeline (long-term)
The Challenges of Studying Rare Diseases

- Small Population
- Incomplete Information About the Disease
- Differences in the disease expression
- No Roadmap for Conducting Studies
- Having the right clinical outcomes to study
How rare disease registries overcome these challenges

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Increases the understanding of the disease
Informs researchers
Informs drug developers
Increases understanding of treatment outcomes
Improves quality of care and outcomes
The IFOPA has one overarching vision

“One Registry” .... that contains the most robust, complete and global data on FOP, accelerating the development, approval and access to new therapies

People With FOP

- Researchers
- Pharma/Biotech
- Payers/HTAs
- Regulators
- Clinicians
The Patient Portal Has Had a Successful Start

Participation in FOP Connection Registry Patient Portal

Notes:
Four translations have been implemented, which will increase overall numbers (German, Italian, Spanish, Portuguese). Russian in process.

iPhone app in development.
45 Countries are represented in the FOP Registry.
FOP Connection Registry: Contributing data to the Research Community

Presentations of Data

- FOP Italia, April 2016
- FOP Friends UK, May 2016
- European Conference on Rare Diseases, May 2016
- FOP Australia, June 2016
- European Bone Research Society, June/July 2016
- FOP France, October 2016
- FOP Drug Development Forum, October 2016, 2017
- FOP Connection Newsletters
How does the Registry’s patient portal work?
Simplifying participation in the Registry’s patient portal

**STEP 1**
REGISTER AND COMPLETE ENROLLMENT SURVEY
1 to 2 hours to complete, depending on how much information about your FOP you provide

**STEP 2**
FOLLOW-UP SURVEY
(15 to 30 minutes every 6 months)
FOP Connection Registry

An international, observational registry by and for the FOP community to help advance FOP therapeutic development.

Click here to learn more, or visit the IFOPA website.

Not Registered? Join Now!

organize

Having a strong, vibrant registry helps our community be better networked for clinical trials and helps researchers plan better for clinical trials. We need your participation to succeed. Join now!

empower

Information is power, and a community-owned registry puts the power of data collection into our own hands. Whether you are a patient or a doctor, sharing your experience with FOP will help make us a stronger community and enable more and better therapeutic development. Join now!

educate

There are many unanswered questions about FOP. Help us all learn more about FOP from each other, educate those outside our community, and ultimately enable better and more efficient development of treatments. Join now!

Sponsored by the International FOP Association

Contact Us
How do you participate in the Registry?
1. Register and provide informed consent.
How do you participate in the Registry?
2. Enter your past information one time.
How do you participate in the Registry?
3. Update your information every six months.
Work has begun to develop the FOP Connection Registry’s medical portal.

The medical portal will allow clinicians to enter clinical data on the FOP patients under their care.
FOP Connection Registry

Participate and You Could Win

WIN
Because you make such a difference in the success of the FOP Registry, we want to thank you with the chance to win an iPhone and other prizes.

(over for details on two opportunities to win)

2018 FOP Registry Raffle – Your chance to win a iPhone
January – July 2018

REGISTER now at fopconnection.org

STEP 1
REGISTER AND COMPLETE YOUR ENROLLMENT SURVEY
1 to 2 hours to complete, depending on how much historical information about your FOP you provide. You can take a break and come back later to complete the survey.

STEP 2
COMPLETE YOUR FOLLOW-UP SURVEY
15 to 30 minutes every 6 months

COMING SOON!
The new FOP Registry iPhone App
The future of the FOP Connection Registry

- Continue to expand registry access around the world
- Improve registry access through portable devices
- Enhance reporting features for registry participants
- Support the FOP Community
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