FOP Registry:
Connecting the FOP Research Community

Adam Sherman
Research Director, IFOPA

US Family Gathering
December 3, 2017
San Francisco, CA

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).”

Registries for Evaluating Patient Outcomes: A User’s Guide.

AHRQ Publication No. 07-EHC001-1

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).”

Registries for Evaluating Patient Outcomes: A User’s Guide.

AHRQ Publication No. 07-EHC001-1

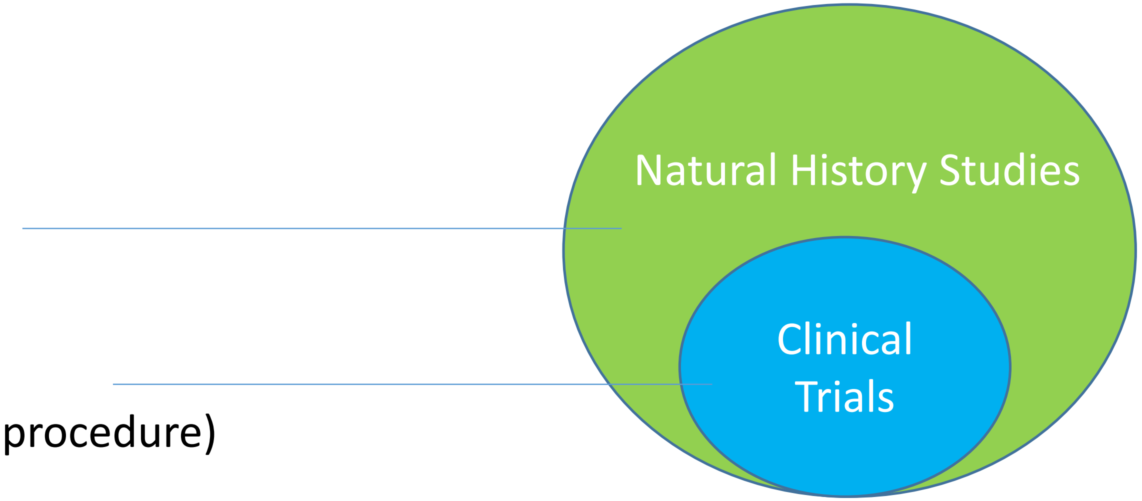
A voluntary collection of medical information to help researchers, doctors, drug developers, and regulators

There are many ways of studying diseases and treatments

Studies that assess the safety
and/or effectiveness of a drug (or procedure)

Clinical Trials

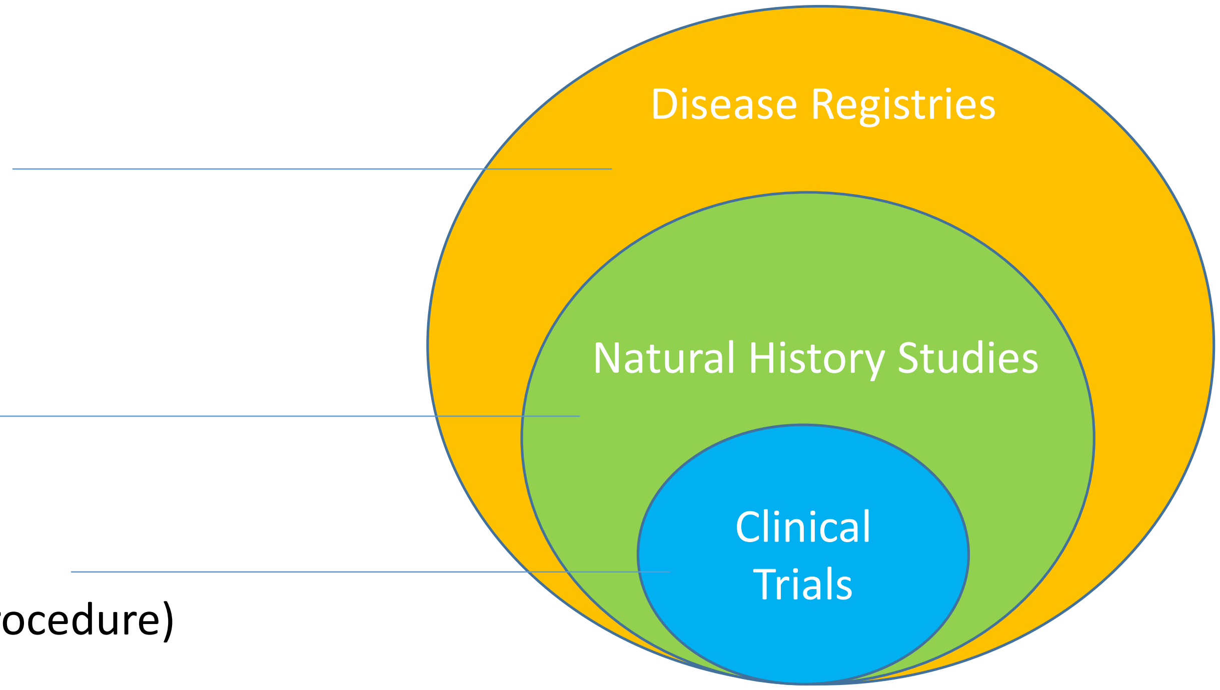
There are many ways of studying diseases and treatments



Studies that look at the natural course
(e.g. no drug or procedure) of a disease

Studies that assess the safety
and/or effectiveness of a drug (or procedure)

There are many ways of studying diseases and treatments



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

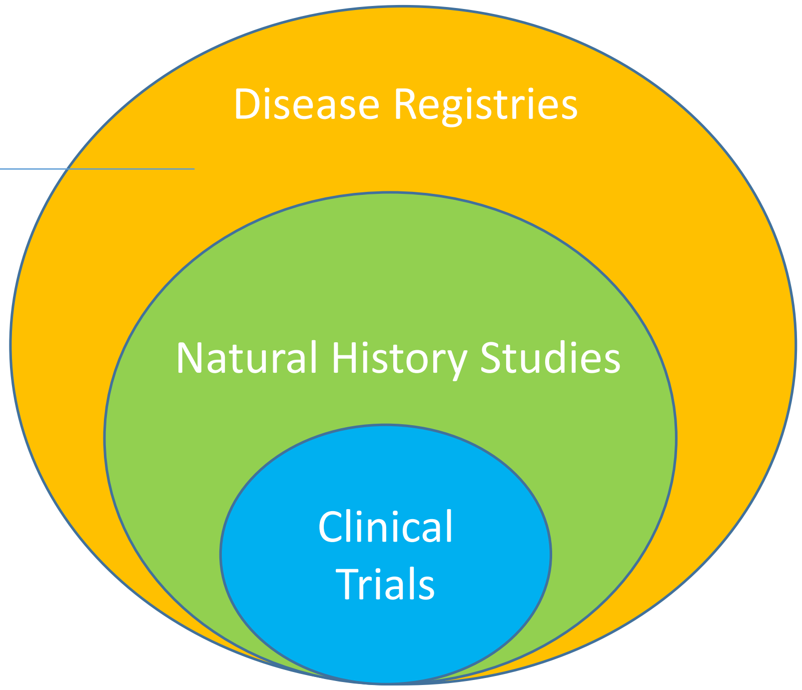
Studies that look at the natural course (e.g. no drug or procedure) of a disease

Studies that assess at the safety and/or effectiveness of a drug (or procedure)

There are many ways of studying diseases and treatments

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

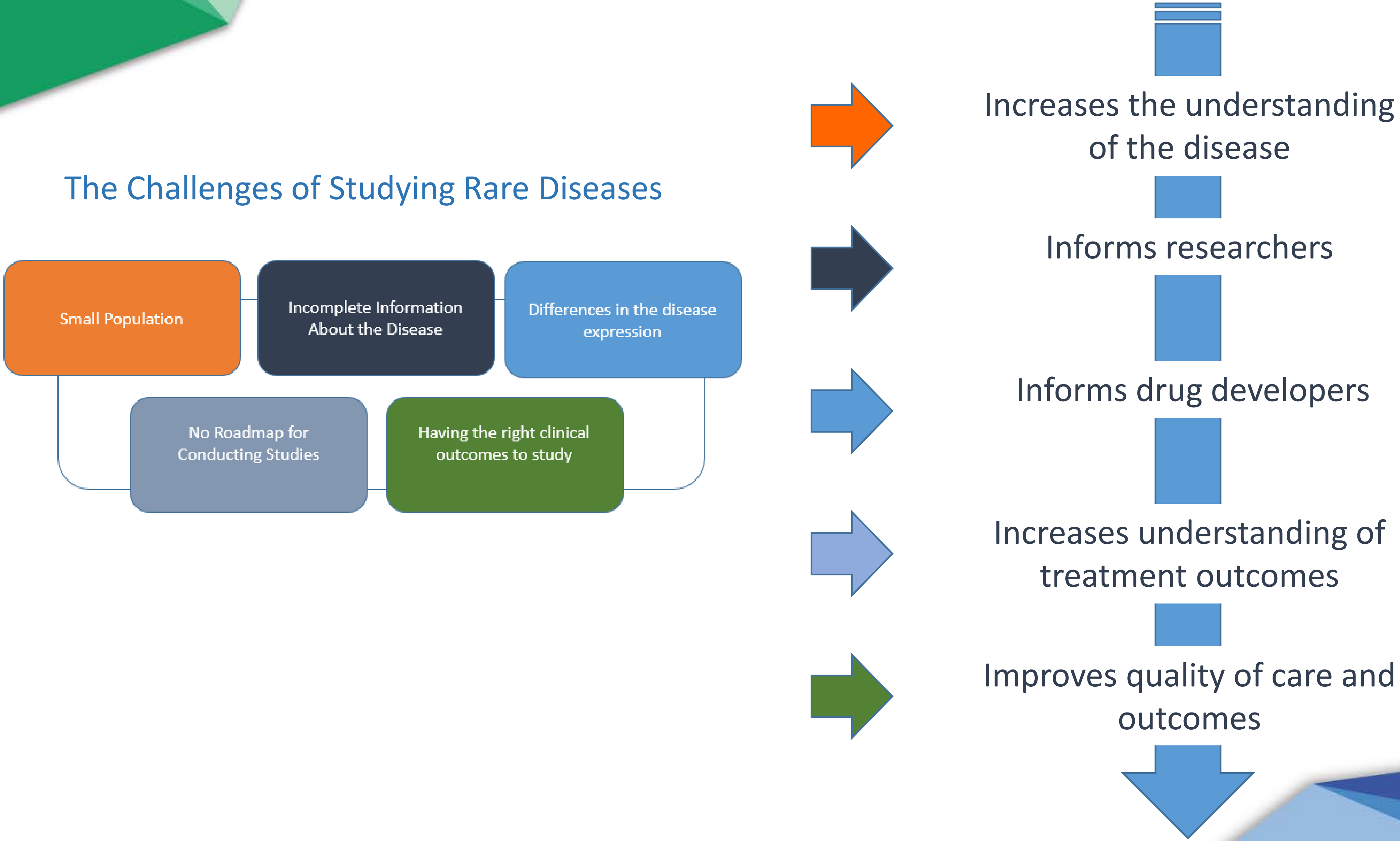
- ✓ "Real World" data (vs controlled)
- ✓ Everyone with the disease can participate
- ✓ Global representation
- ✓ Data collection at home
- ✓ Indefinite timeline (long-term)



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



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

Increases the understanding of the disease

Informs researchers

Informs drug developers

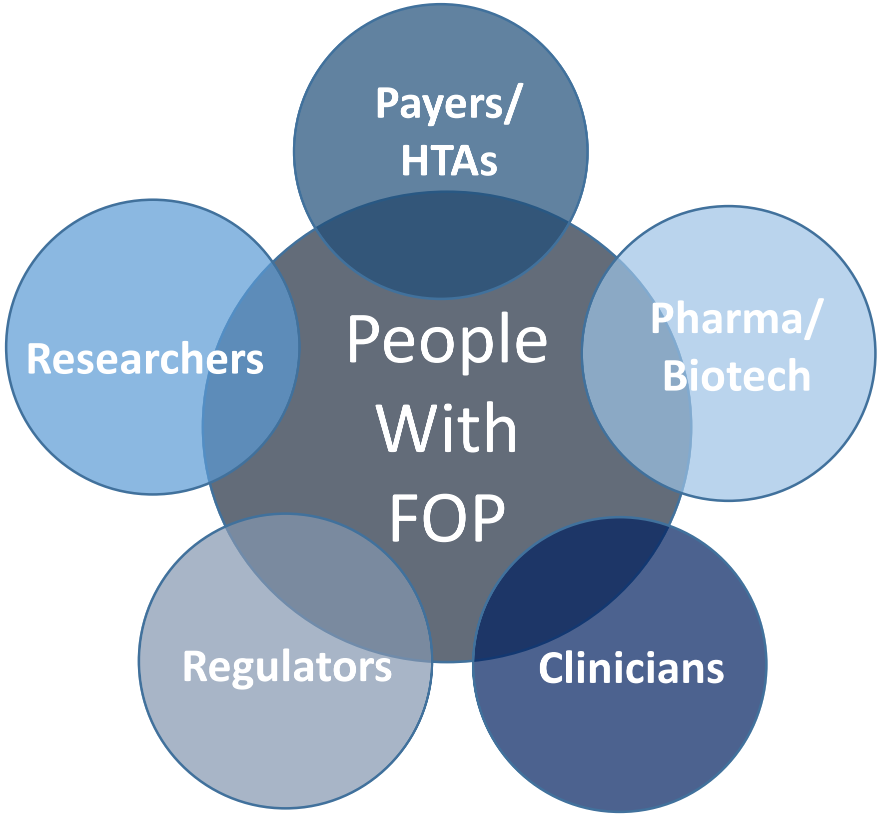
Increases understanding of treatment outcomes

Improves quality of care and outcomes

FOP Connection Registry

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



Payers/ HTAs

Researchers

People With FOP

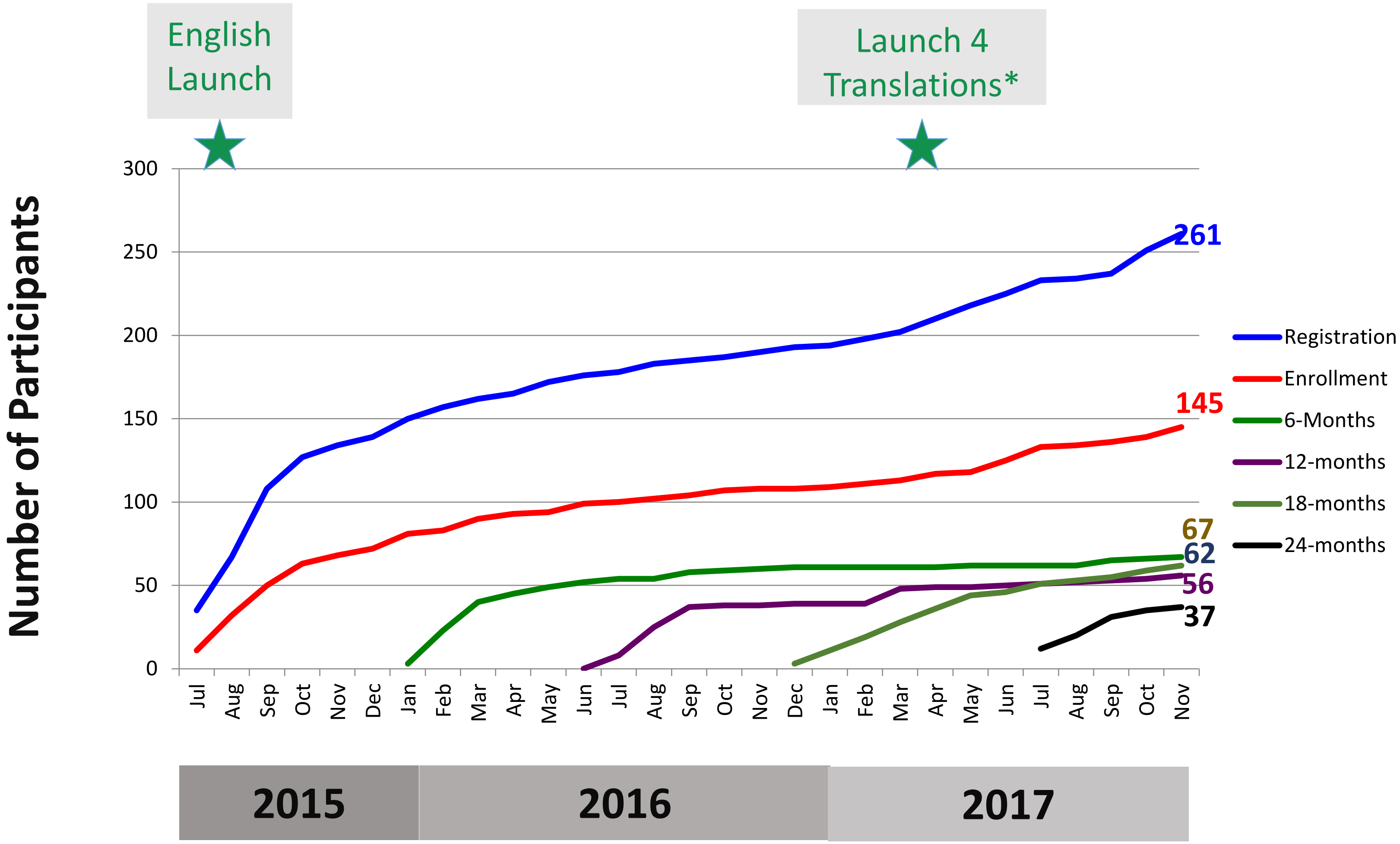
Pharma/ Biotech

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

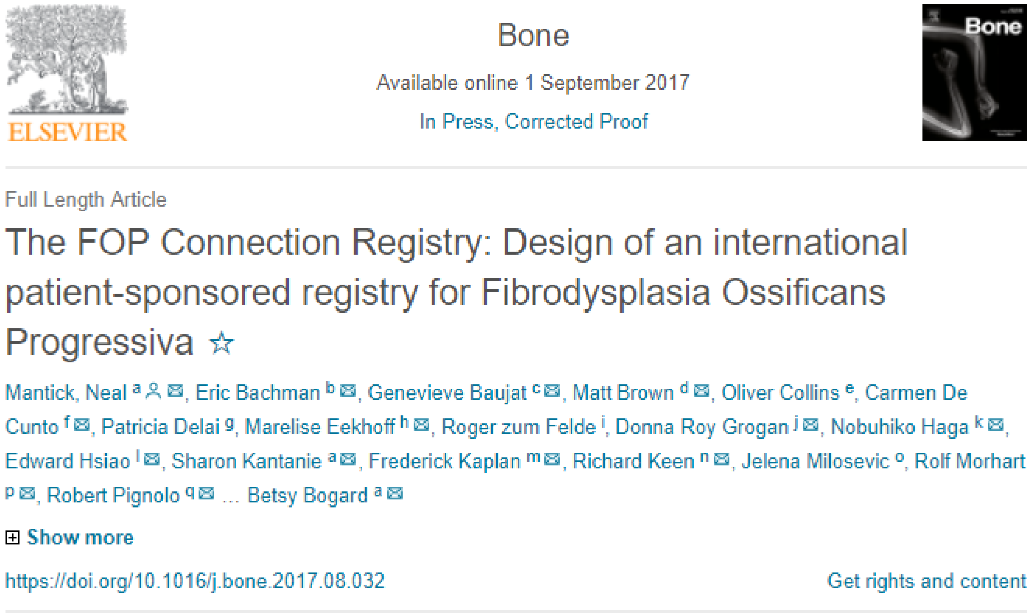
iPhone app in development

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



Bone

Available online 1 September 2017

In Press, Corrected Proof

Full Length Article

The FOP Connection Registry: Design of an international patient-sponsored registry for Fibrodysplasia Ossificans Progressiva ☆

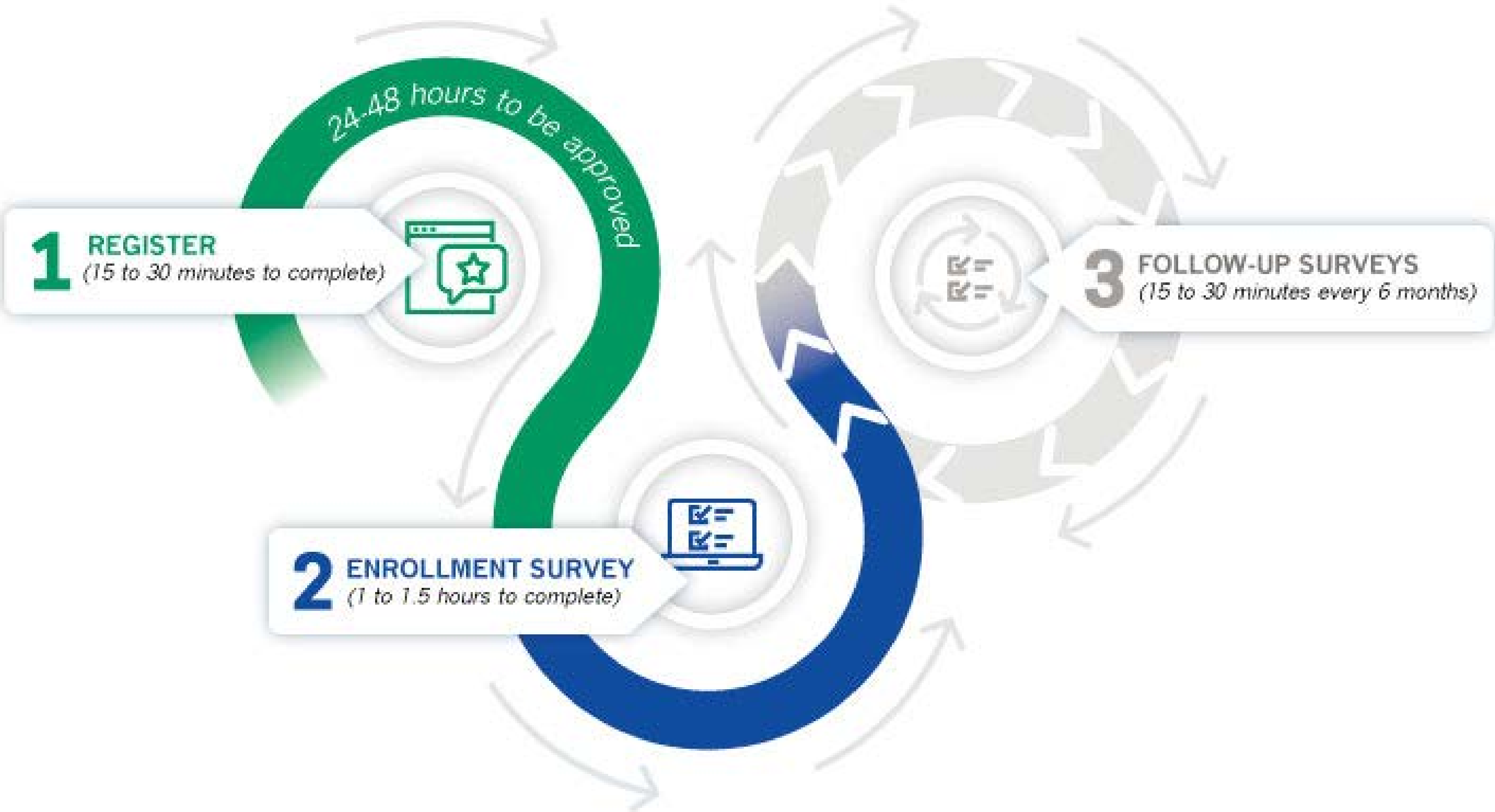
Mantick, Neal [a] 👤 ✉, Eric Bachman [b] ✉, Genevieve Baujat [c] ✉, Matt Brown [d] ✉, Oliver Collins [e], Carmen De Cunto [f] ✉, Patricia Delai [g], Marelise Eekhoff [h] ✉, Roger zum Felde [i], Donna Roy Grogan [j] ✉, Nobuhiko Haga [k] ✉, Edward Hsiao [l] ✉, Sharon Kantanie [a] ✉, Frederick Kaplan [m] ✉, Richard Keen [n] ✉, Jelena Milosevic [o], Rolf Morhart [p] ✉, Robert Pignolo [q] ✉ ... Betsy Bogard [a] ✉

⊞ Show more

https://doi.org/10.1016/j.bone.2017.08.032

Get rights and content

How does the Registry’s patient portal work?



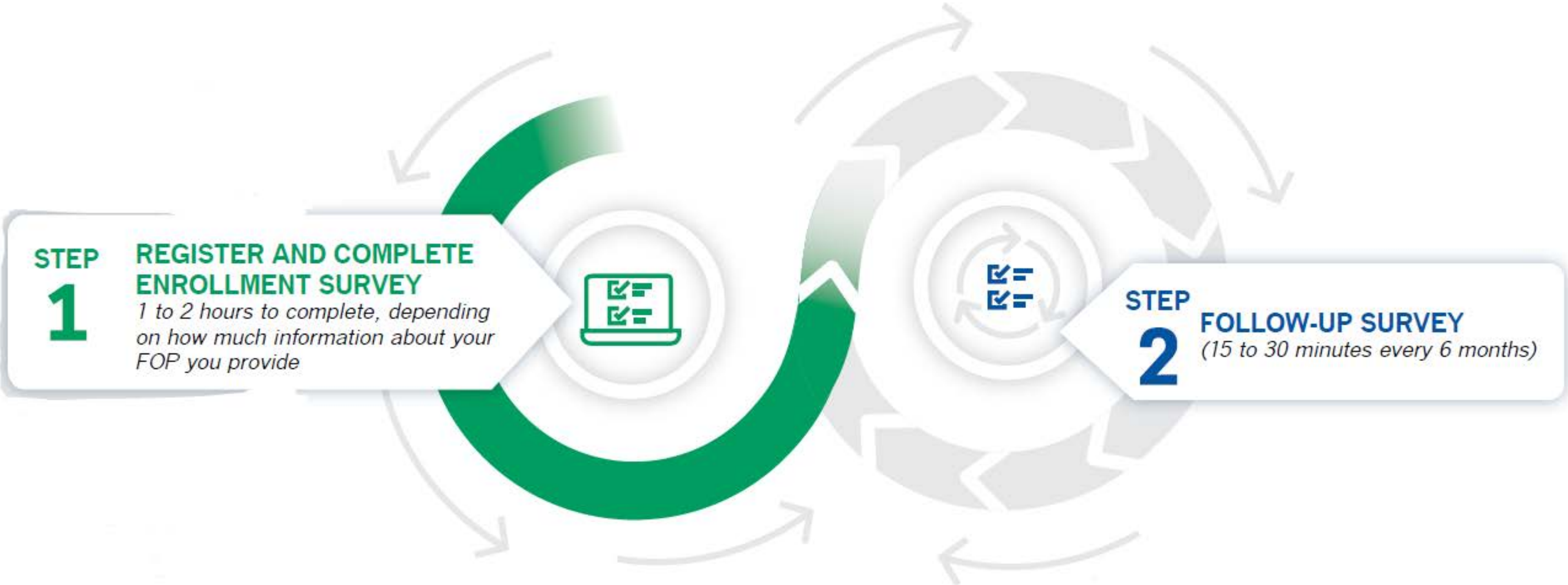
24-48 hours to be approved

1 REGISTER
(15 to 30 minutes to complete)

2 ENROLLMENT SURVEY
(1 to 1.5 hours to complete)

3 FOLLOW-UP SURVEYS
(15 to 30 minutes every 6 months)

Simplifying participation in the Registry’s patient portal



STEP 1

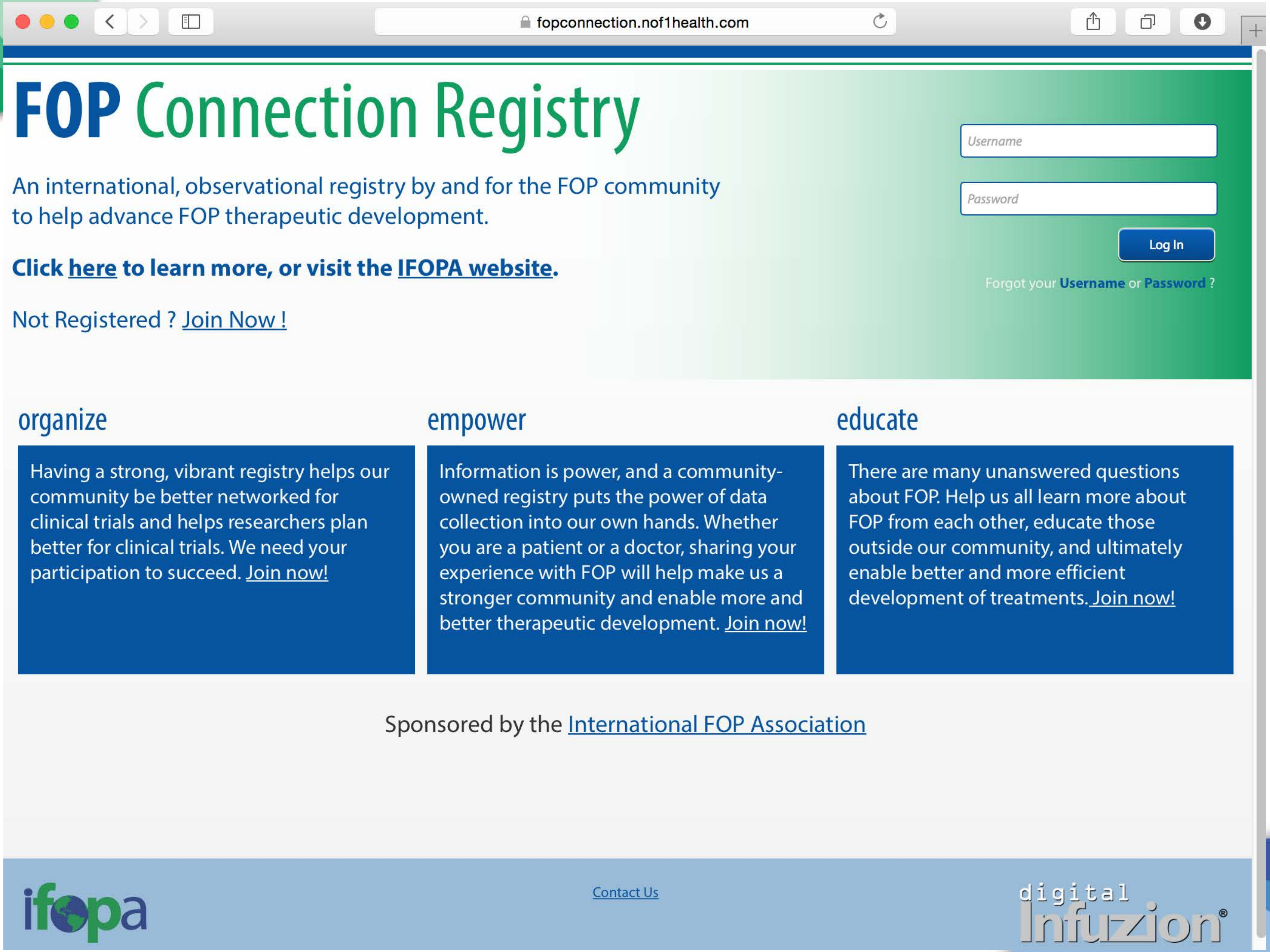
REGISTER AND COMPLETE ENROLLMENT SURVEY

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.

Forgot your **Username** or **Password** ?

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

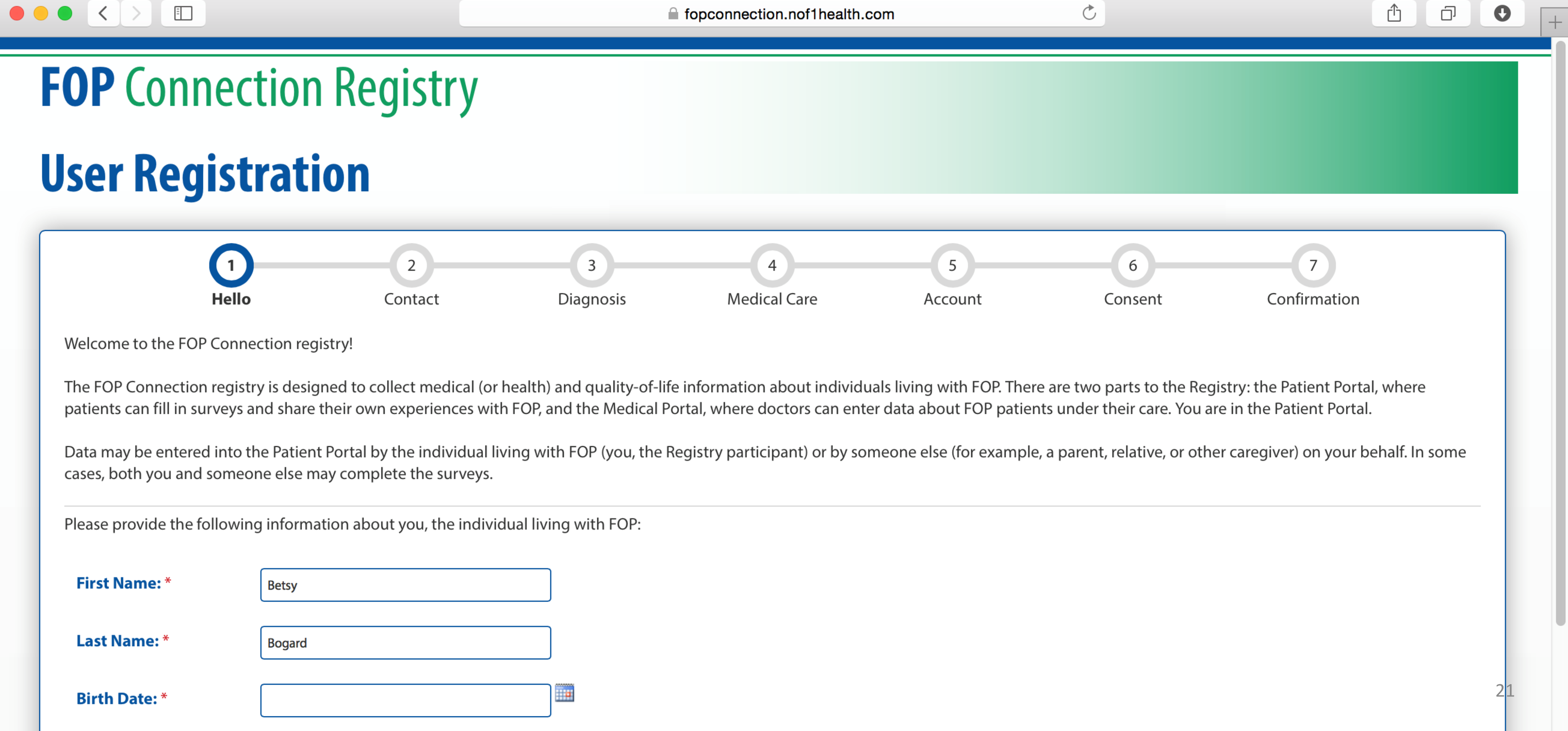
There are many unanswered questions about FOP. Help us all learn more about FOP from each other, educate those outside our community, and ultimately enable better and more efficient development of treatments. Join now!

Sponsored by the International FOP Association

Contact Us

How do you participate in the Registry?

1. Register and provide informed consent.



FOP Connection Registry

User Registration

Welcome to the FOP Connection registry!

The FOP Connection registry is designed to collect medical (or health) and quality-of-life information about individuals living with FOP. There are two parts to the Registry: the Patient Portal, where patients can fill in surveys and share their own experiences with FOP, and the Medical Portal, where doctors can enter data about FOP patients under their care. You are in the Patient Portal.

Data may be entered into the Patient Portal by the individual living with FOP (you, the Registry participant) or by someone else (for example, a parent, relative, or other caregiver) on your behalf. In some cases, both you and someone else may complete the surveys.

Please provide the following information about you, the individual living with FOP:

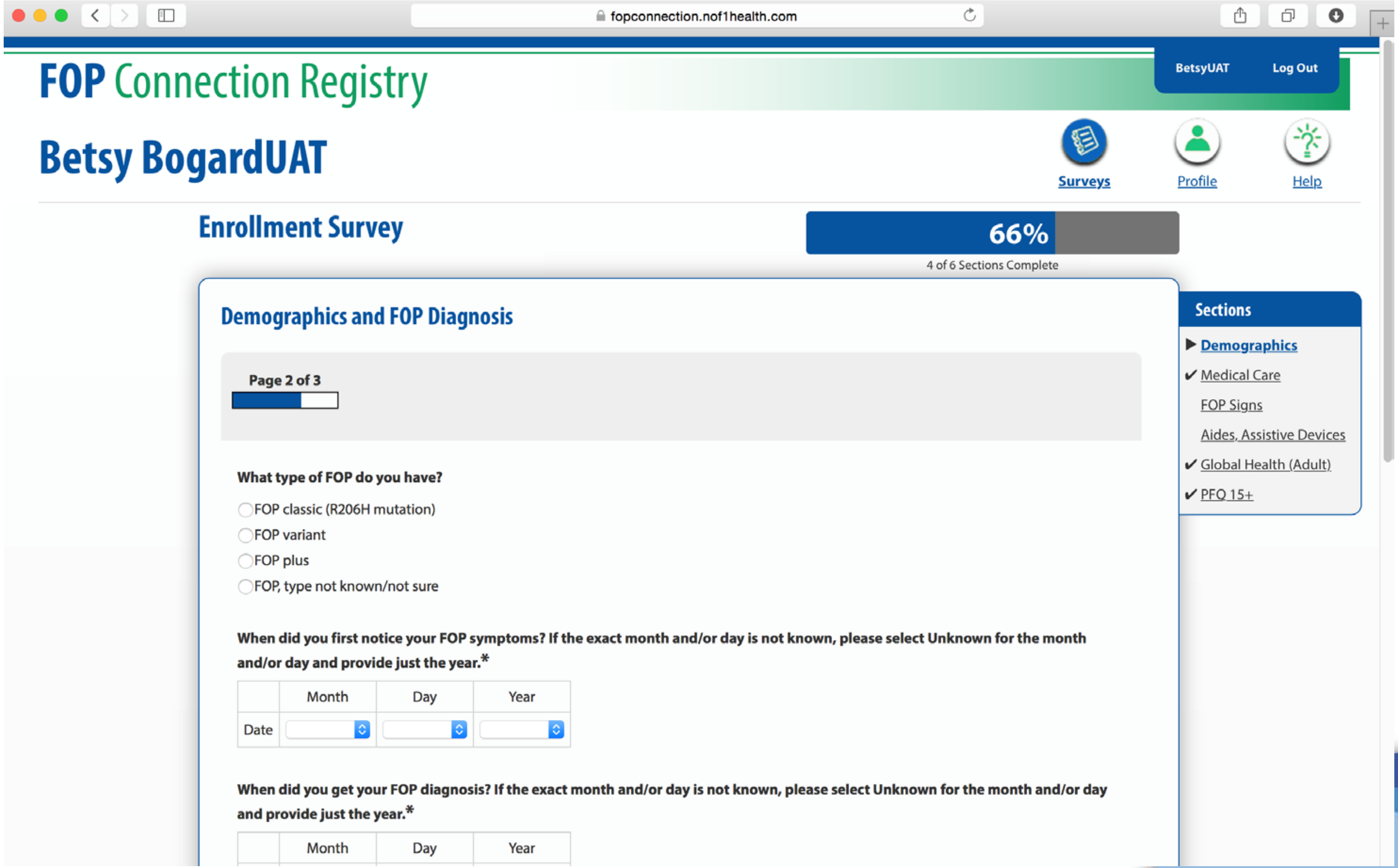
First Name: * Betsy

Last Name: * Bogard

Birth Date: *

How do you participate in the Registry?

2. Enter your past information one time.



Betsy BogardUAT

Enrollment Survey

66%
4 of 6 Sections Complete

Demographics and FOP Diagnosis

Page 2 of 3

What type of FOP do you have?

○ FOP classic (R206H mutation)
○ FOP variant
○ FOP plus
○ FOP, type not known/not sure

When did you first notice your FOP symptoms? If the exact month and/or day is not known, please select Unknown for the month and/or day and provide just the year.*

	Month	Day	Year
Date			

When did you get your FOP diagnosis? If the exact month and/or day is not known, please select Unknown for the month and/or day and provide just the year.*

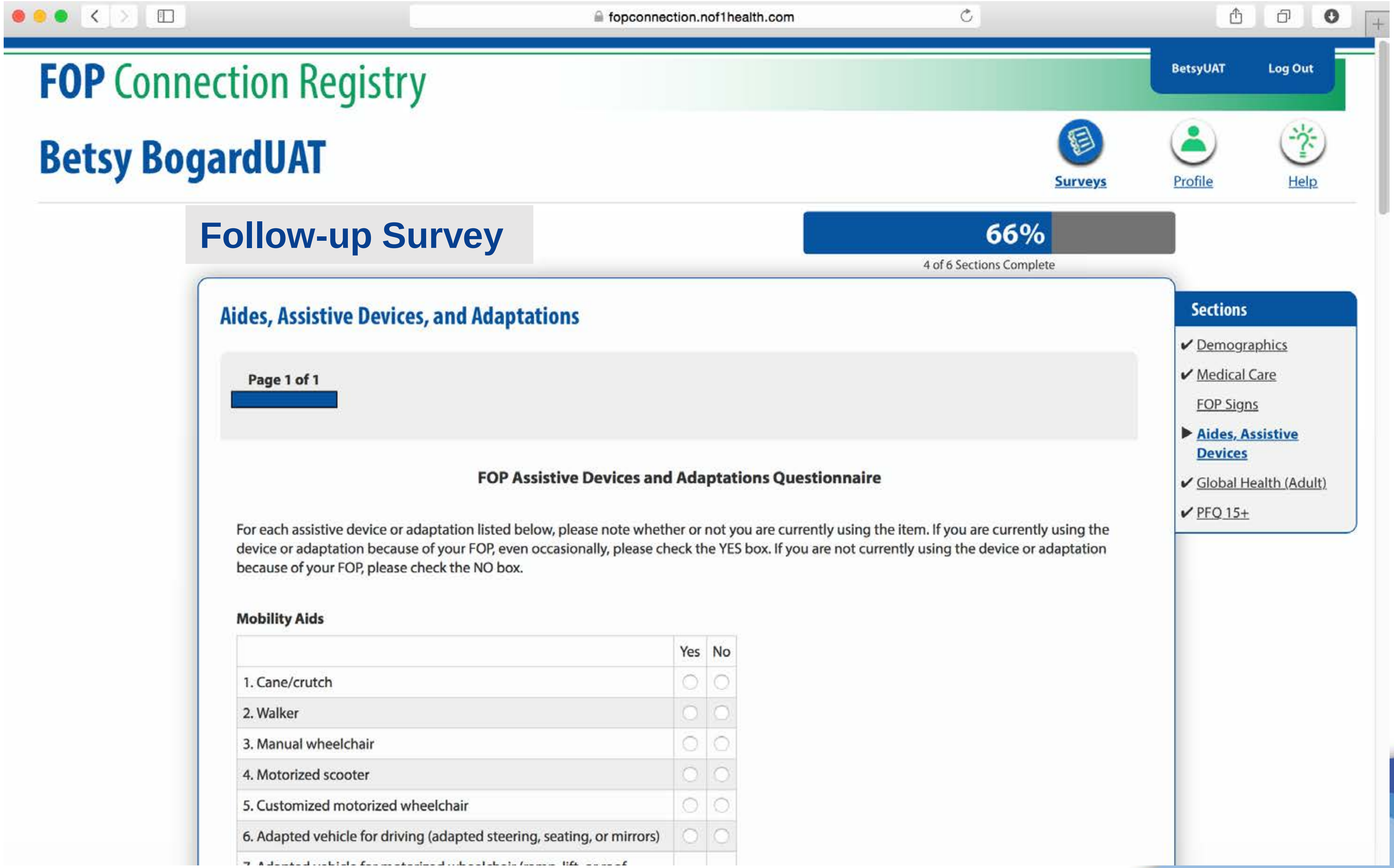
	Month	Day	Year

Sections

- ▶ Demographics
- ✔ Medical Care
- FOP Signs
- Aides, Assistive Devices
- ✔ Global Health (Adult)
- ✔ PFQ 15+

How do you participate in the Registry?

3. Update your information every six months.



FOP Connection Registry

Betsy BogardUAT

Follow-up Survey

66%

4 of 6 Sections Complete

Aides, Assistive Devices, and Adaptations

Page 1 of 1

Sections

- ✔ Demographics
- ✔ Medical Care
- FOP Signs
- ▶ **Aides, Assistive Devices**
- ✔ Global Health (Adult)
- ✔ PFQ 15+

FOP Assistive Devices and Adaptations Questionnaire

For each assistive device or adaptation listed below, please note whether or not you are currently using the item. If you are currently using the device or adaptation because of your FOP, even occasionally, please check the YES box. If you are not currently using the device or adaptation because of your FOP, please check the NO box.

Mobility Aids

	Yes	No
1. Cane/crutch	○	○
2. Walker	○	○
3. Manual wheelchair	○	○
4. Motorized scooter	○	○
5. Customized motorized wheelchair	○	○
6. Adapted vehicle for driving (adapted steering, seating, or mirrors)	○	○
7. Adapted vehicle for motorized wheelchair (ramp, lift, or roof		

Work has begun to develop the FOP Connection Registry’s medical portal.

The FOP Connection Registry

✓**Launched**

Patient Portal

=

Target: 1H2018

Medical Portal

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

2018 FOP Registry Raffle – Your chance to win a iPhone

January – July 2018

FOP Connection Registry

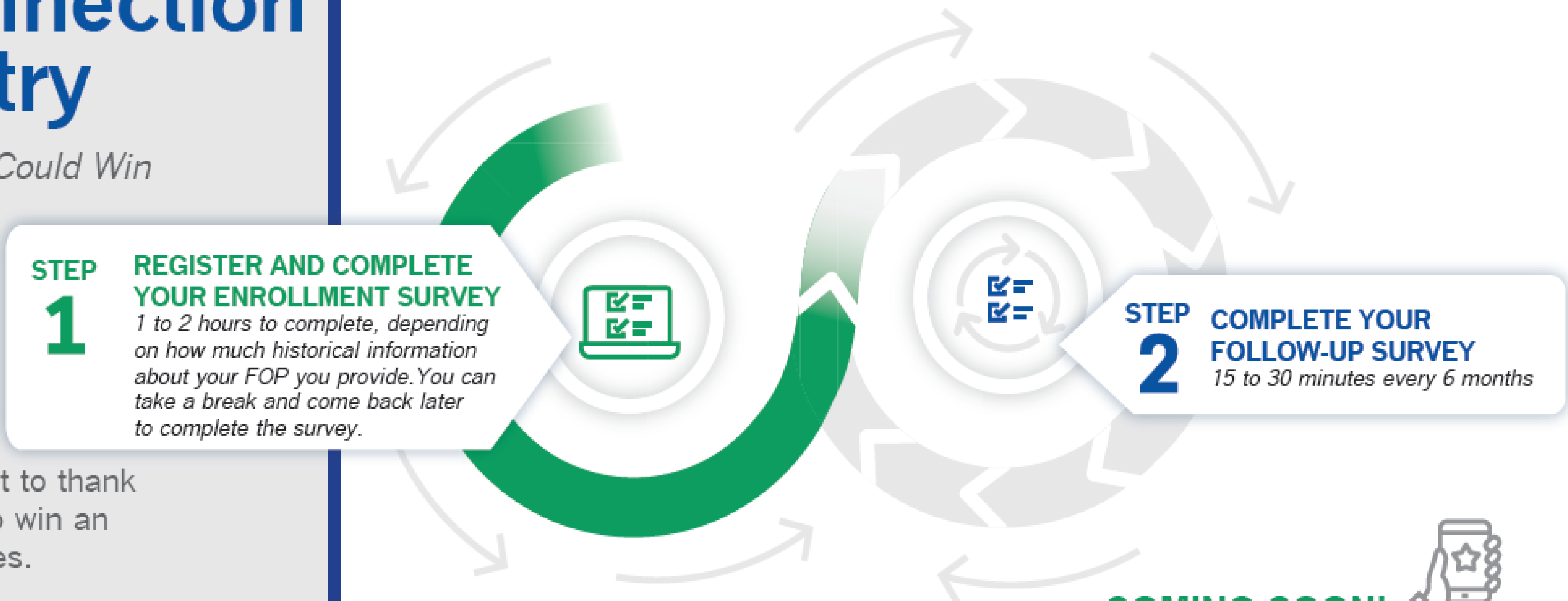
Participate and You Could Win

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)

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

Please register!

Adam Sherman

www.ifopa.org
www.fopconnection.org
registry@fopconnection.org
adam.sherman@ifopa.org