

# Hardened Hope: Care Advances for Patients with Fibrodysplasia Ossificans Progressiva

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## Program Overview/Statement of Need

As a rare disease, fibrodysplasia ossificans progressiva (FOP) poses substantial diagnostic and treatment challenges for pediatricians and pediatric orthopedists. It is frequently confused with other development bone diseases due to similar presentation, and treatment options are lacking. Currently, treatment of FOP is limited to symptomatic management of disease flare-ups. In recent years, however, improved understanding of the genetics and pathophysiology of FOP have led to improvements in diagnosis and treatment, as well as the identification of multiple new therapeutic targets. Novel agents that have demonstrated efficacy in minimizing disease symptoms and slowing progression of disease are currently in late-stage clinical trials and may soon be available for FOP treatment.

This activity, *Hardened Hope: Care Advances for Patients with Fibrodysplasia Ossificans Progressiva*, will provide clinicians with expert advice and guidance on how to identify and diagnose FOP in their patients, current best practices for FOP management, and the most up-to-date information on novel therapies currently in clinical trial for treatment of FOP.

## Target Audience

The intended audiences for this initiative are pediatricians and pediatric orthopedists, the clinicians who diagnose and manage patients with FOP.

## Educational Objectives

This program is designed to address ACGME and NAM competencies, including delivering patient-centered care, practicing evidence-based medicine, and focusing on quality improvement.

*At the conclusion of this education, participants should be able to:*

- Distinguish patients with FOP using the latest recommendations and criteria
- Develop effective FOP treatment plans which incorporate the most recent clinical evidence
- Evaluate the latest safety and efficacy data with FOP agents in late-stage development

## Faculty

**Peter Kannu, MB ChB, PhD, DCH, FRACP, FRCPC**

Associate Staff

The Hospital for Sick Children and Peter Gilgan Centre for Research and Learning

Chair of Medical Genetics

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**Charles E. Levy, MD**

Courtesy Research Scholar

Center for Arts in Medicine

College of the Arts

University of Florida

Gainesville, FL

Editorial assistance was provided by Barbara J. Martin, MD.

## Physician Accreditation Statement

This activity has been planned and implemented in accordance with the accreditation requirements and policies of the Accreditation Council for Continuing Medical Education (ACCME) through the joint providership of

the Potomac Center for Medical Education and Rockpointe. The Potomac Center for Medical Education is accredited by the ACCME to provide continuing medical education for physicians.

## Physician Credit Designation Statement

The Potomac Center for Medical Education designates this journal-based CME activity for a maximum of 1.0 *AMA PRA Category 1 Credit*<sup>™</sup>. Physicians should claim only the credit commensurate with the extent of their participation in the activity.

For information about the accreditation of this activity, please email: [contact@potomacme.org](mailto:contact@potomacme.org).



## ABP MOC Recognition Statement

Successful completion of this CME activity, which includes participation in the evaluation component, enables the learner to earn up to 1 MOC point in the American Board of Pediatrics (ABP) Maintenance of Certification (MOC) program. It is the CME activity provider's responsibility to submit

learner completion information to ACCME for the purpose of granting ABP MOC credit.

## Instructions for Obtaining Credit

To receive credit, learners must complete the online post-test, with a score of 75% or better, and evaluation located at [www.rockpointe.com/FOPsupplement](http://www.rockpointe.com/FOPsupplement).

## Fee Information

There is no fee for this educational activity.

## Disclosure Statement

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## Disclosures

### Program Faculty

*The faculty, authors and content developers reported the following relevant financial relationships that they or their spouse/partner have with commercial interests:*

**Peter Kannu, MB ChB, PhD, DCH, FRACP, FRCPC:** *Honoraria:* Alexion, Ipsen

**Charles E. Levy, MD:** Nothing to disclose

**Barbara Martin, MD:** Nothing to disclose

## Planners and Managers

*The planners and managers reported the following relevant financial relationships that they or their spouse/partner have with commercial interests:*

**Chelsey Goins, PhD:** Nothing to disclose

#### **Content Reviewers**

*The content reviewers reported the following relevant financial relationships that they or their spouse/partner have with commercial interests:*

**Katie Propst, PhD:** Nothing to disclose

#### **FDA Disclosure**

The contents of some CME/CE activities may contain discussions of non-approved or off-label uses of some agents mentioned. Please consult the prescribing information for full disclosure of approved uses.

Jointly provided by Potomac Center for Medical Education and Rockpointe



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#### **Post-Test**

In order to receive credit, please complete the online CME post-test, with a score of 75% or better, and evaluation form at [www.rockpointe.com/FOPsupplement](http://www.rockpointe.com/FOPsupplement). The post-test questions are listed below for your

reference and convenience. They are identical to the post-test you will find online.

However, to receive credit you must complete the online form at: [www.rockpointe.com/FOPsupplement](http://www.rockpointe.com/FOPsupplement). If you are experiencing problems or have any questions, please email [cme@rockpointe.com](mailto:cme@rockpointe.com).

1. Which of the below is considered the classic sign defining FOP?
  - a. Tumor-like swellings on the neck, back, and shoulders
  - b. Malformation of the great toes
  - c. Heterotopic ossification of the subcutaneous fat and deep connective tissue
  - d. Increased expression of bone morphogenic protein 4
2. For management of a 15-year-old patient with FOP who is experiencing local inflammation following major blunt trauma to the shoulder, which of the following approaches would you consider most appropriate?
  - a. NSAIDs to manage pain
  - b. Surgical removal of resulting bony growths
  - c. Corticosteroid prophylaxis
  - d. Intravenous bisphosphonates
3. Which of the following results was seen in a phase 2b trial of palovarotene in FOP?
  - a. Decrease in pain associated with HO formation
  - b. 72% reduction in new HO at 12 weeks
  - c. No new HO during study period
  - d. 46% reduction in HO formation at 8 weeks
4. A 22-year-old patient with FOP requires dental surgery but is concerned about long-term impact on jaw mobility. When discussing potential anesthesia options with him, which of the following would you recommend?
  - a. General anesthesia with no airway support
  - b. General anesthesia followed by endotracheal intubation
  - c. Use of a cuffed tracheostomy device followed by general anesthesia
  - d. Intranasal fiberoptic intubation followed by general anesthesia