



Clinician Update

Three FOP clinical trials enrolling now!

There are clinical trials for three potential treatments for Fibrodysplasia Ossificans Progressiva currently enrolling, including one planned to open in Australia.

- The Clementia phase III **palovarotene** trial (clinicaltrials.gov code NCT03312634) is now enrolling and is expected to open in Australia sometime in 2018. FOP Australia remain in contact with Clementia and the planned trial team at QUT and will keep you updated.
- Regeneron's novel activin-A antibody (**REGN2477**) is now undergoing a phase II trial (NCT03188666). There will not be an Australian site for this trial but we will remain in contact with Regeneron regarding their progress and future opportunities.
- **Rapamycin** is being investigated to prevent heterotopic ossification in FOP in a phase trial through Kyoto University, enrolling in Japan only. The trial design was presented at the International FOP Association (IFOPA) /FOP Italia Drug Development Forum in October 2017, and we look forward to hearing of further progress.

With three agents being investigated for treatment of FOP, this is an exciting and challenging time for the small global FOP population. Judicious trial design will be essential to ensure each trial maximises the potential benefit from each participant's involvement for the overall research effort.

Your patients may have questions about upcoming trials. There is general patient information about clinical trials available at www.ifopa.org/clinical_trials that may assist.

FOP Australia is a small registered charity run by volunteers. We send concise updates twice yearly to any clinicians involved or interested in **Fibrodysplasia Ossificans Progressiva**

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2017 Drug Development Forum Report

A summary of the IFOPA/FOP Italia 2017 Drug Development Forum report is available through links in the news and research sections of fopaustralia.org.

This website is designed for general community access but also includes links to the current guidelines and other useful information for clinicians.

Registry update

The FOP Connection registry (NCT02745158) is now entering its third year of data collection and has over 260 patients enrolled from 46 countries. The registry is already enabling new insights in to the disease process and will provide vital information to guide assessments of potential treatments. Please check that your FOP patients (or their parents) are enrolled and entering data every six months. We will inform you when the clinicians' Medical Portal opens.

Focus on: Respiratory health

As with other diseases that cause progressive restriction/dysfunction of the chest wall, respiratory health is important in FOP. Preserving lung function by recognising and managing respiratory comorbidities and avoiding infections is recommended (Kusssmaul et al., 1998; Kaplan & Glaser, 2005; Kaplan et al., 2010).

Many patients, parents and clinicians encourage the use of incentive spirometers as a potential method for respiratory exercise. (IFOPA/FOP Italia 2017 DDF Panel Session).

Current guidelines are available via our website and include more information on cardiopulmonary function in FOP.

Spread the word

Have you got colleagues, trainees, or students that should know about FOP? Delayed or incorrect diagnosis of FOP results in unnecessary investigations, trauma and progression of immobility. Please help FOP Australia promote better recognition of FOP by linking others to us at info@fopaustralia.org.

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