FOP Connection Registry: A Patient Registry Directed by the FOP Community

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Objective

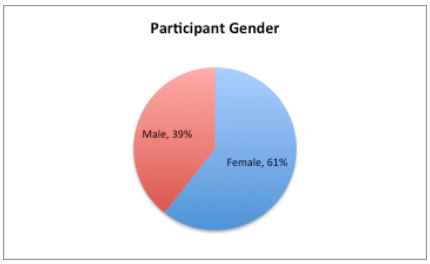
The objectives are to organize the international Fibrodysplasia Ossificans Progressiva (FOP) community for participation in clinical trials; to enable FOP patients worldwide to report data in a shared forum; to improve the collective understanding of FOP natural history; and to advance the understanding of FOP treatment outcomes.

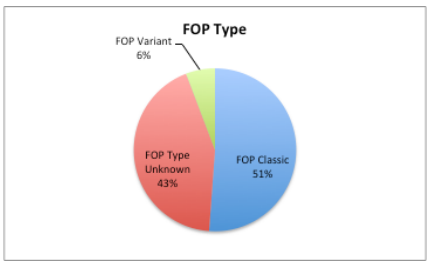
Methods

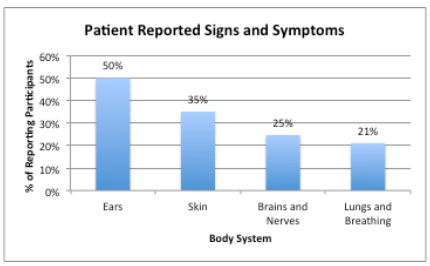
The Registry is a global, non-interventional, voluntary database that captures demographic and disease data directly from FOP patients and their caregivers via a secure, web-based patient portal. A physician portal (in development) will allow physicians to enter clinical data about their patients.

Results

Data from the first 150 FOP patients from 26 countries were analyzed. 39% (59/150) are male. The average age is 25 years (n=145) with a range of 1 to 75 years. 38% (55/145) are minor children age 17 years and younger. 59% (89/150) provided baseline data. The average age of the patients with baseline data is 24 years (n=84) with a range of 1 to 57 years. 38% (32/84) are minor children age 17 years and younger. 86 patients provided information about their FOP type. 51% (44/86) reported FOP Classic, 43% (37/86) reported FOP Type Unknown, and 6% (5/86) reported FOP Variant. 81% (77/89) of patients provided information about doctor visits, hospital admissions, and dental care not requiring hospitalization in the past 12 months prior to enrolling in the Registry. The average number of doctor visits was 5 times, with a range of 0 to greater than 25 visits. The average number of hospital admissions was 0.6, with a range of 0 to 14 admissions. Common reasons for hospital admissions included respiratory infections, falls or other injuries, dental procedures, and surgical procedures. The average number of times patients sought dental care that did not require a hospitalization was 2 times, with a range of 0 to 10 times. The most common reason for seeking dental care was for a routine dental check-up and cleaning. 87% (77/89) of the patients who provided baseline information reported possible FOP signs in other body systems in addition to heterotopic ossification. The most commonly reported signs were symptoms related to the ears (50% of responding patients; 38/76), followed by those related to the skin (35%; 27/77), the nerves and brain (25%; 19/77), and the lungs and breathing (21%; 15/76). 90% (80/89) of the patients who provided baseline data completed all three of the quality of life questionnaires – the Assistive Devices and Adaptations Questionnaire, the PROMIS Global Health Scale, and the FOP Physical Functioning Questionnaire. These data have not yet been analyzed.







Conclusion

The first 150 FOP patients in the Registry represent almost 20% of the approximately 800 known cases of FOP in the world. The IFOPA hopes to expand global participation when the patient portal is translated into other languages in 2016. Data from the patient portal add an important view of the clinical understanding and impact of FOP from patients', parents', and caregivers' perspectives. For example, the data on the frequency of medical and dental care, the effects of FOP on different body systems, and on physical functioning provide a well-rounded picture of the burden of FOP disease, not only on the patient, but also on their families and caregivers. The data support prior published reports that FOP affects many different body systems beyond the bones and joints, and may provide FOP patients and their physicians with valuable insights on holistic, patient-specific approaches to treating FOP. The Registry has already been helpful in supporting the IFOPA's ongoing efforts to collaborate with physician researchers and biopharmaceutical companies and to support drug development activities. As part of an innovative data-sharing agreement between IFOPA and Clementia Pharmaceuticals, the Registry is using a novel disease-specific physical functioning questionnaire, enabling both Clementia and the FOP community to learn more than any one group could do alone. Assembling the unique stories from individuals with FOP over time into one registry database will give individual patients more context on how similar or different their experience is compared to others. It will also allow physicians and researchers to better understand FOP clinical characteristics and disease progression among the larger FOP community, which will contribute to better patient care and the growing research efforts that are focused on bringing new FOP treatments to patients. The FOP Connection Registry is a strong confirmation of the patient community's commitment to advance research in FOP disease.