clementia

Dear FOP community,

In my last letter to you, I quoted a passage saying that the tiniest thread can be twisted into an unbreakable cord. I feel that this is what we are achieving together!

The hope we have all shared over the last few years is now supported by our Phase 2 clinical results. Through the collective efforts of the community, including FOP families, researchers, and clinicians and their teams, we have collected data on over 100 flare-ups, including after treatment with 4 distinct regimens of palovarotene. We now have important data on 114 patients in the Natural History Study (NHS) which, through a ground-breaking agreement with IFOPA, we will share with the patient community so that the knowledge gained will be available to others coming after us.

We have observed that treating chronically with palovarotene 5 mg once daily, followed by treatment with higher doses at the earliest signs of a flare-up, appears to be the best way to reduce new bone formation after flare-ups. In fact, for those on this regimen,we detected a greater than 70% reduction in new bone volume at 12 weeks as compared to untreated flare-ups. The FDA granted us Breakthrough Therapy Designation in July in recognition of this clinical evidence indicating that palovarotene may provide substantial improvement over existing therapies for FOP. Now is the time to take everything learned and focus our energies on the efficient completion of the final step in our clinical development program for palovarotene in FOP: the Phase 3 MOVE Trial.

We are thrilled with the recruitment of the first several patients in the MOVE Trial last year and plan to continue enrolling up to 80 patients at 20 sites in 16 countries across 6 continents. Everyone who enrols in this final clinical trial will receive palovarotene, as regulatory authorities have agreed that the NHS could be used as the external control. We believe that if the trends in bone reduction observed to date continue, it is possible that palovarotene will extend the time patients retain mobility.

Less than 5 years ago, I was an individual who had never heard of FOP. I read a Nature Medicine paper and decided to change my life. I quit my job and started Clementia. Building Clementia has been a life-changing experience. I don't believe it is possible to become aware of FOP and, in particular, to meet and interact with families who live with FOP and not be transformed. I know that I speak for the entire Clementia team when I say thank you to all of you for the opportunity to serve your community. You have provided us with the most laudable goal to direct our expertise and work and inspired us beyond what we knew was possible.

Finally, while we applaud our progress, we must also acknowledge the recent loss and deep grief within the FOP community. We realize that every day poses risks for those living with FOP and are sincerely grateful for those who, having lived with FOP for decades, have courageousely kept hope alive and given of their time and energy so that others would benefit. You are the heroes of this story.

Thank you and best wishes for a transformative 2018!

Clarissa Desjardins