EARLY ACCESS POLICY

At Clementia, our mission is to bring innovative and effective treatments to individuals who currently have none. With this goal in mind, we are developing palovarotene, an investigational drug for fibrodysplasia ossificans progressiva (FOP) and multiple osteochondromas (MO).

Successful development of any new medicine, including palovarotene, requires three main steps:

1. **Discovery.** Identifying a potential medicine (drug candidate) for a specific disease.
2. **Development.** Conducting rigorous scientific research, following specific regulations, to evaluate the drug candidate through clinical trials. Once the drug candidate has entered clinical trials, it is referred to as an investigational drug.
3. **Delivery.** Securing approval from regulatory authorities to make the drug available as a treatment for individuals with the disease, based on a review of its potential benefits and potential risks. Once the drug is approved, it becomes available to people with a specific disease by physician prescription.

Regulatory authorities in many countries have programs to allow for expanded access to investigational drugs prior to approval, for people with urgent medical needs who are unable to participate in a clinical trial, and for whom there is no comparable or satisfactory alternative therapy. These programs are called many different names, including compassionate use, early access, pre-approval access, expanded access, and named patient use.

Clementia's Policy for Early Access to Investigational Drugs

Clementia believes that participation in a clinical trial is the most appropriate way to access investigational drugs. The completion of these rigorous scientific studies is a critical step to securing the regulatory approval needed to provide access to treatment for the greatest number of patients with a specific disease.

We also recognize that not all patients may be eligible or able to enroll in a clinical trial. We support expanded access to eligible investigational drugs for people with a serious or life-threatening disease or condition who meet specific requirements. Any requests for access will be evaluated on a case by case basis. When evaluating requests for pre-approval access, we consider the following (other considerations may also apply):

- Is the patient unable or ineligible to access the investigational drug through a clinical trial, with no satisfactory approved therapies available?
- Does early access interfere with our efforts to provide future access to the broader patient community or otherwise compromise the potential development of the investigational drug?
- Do local laws and regulations allow for early access, and do all parties involved agree to follow applicable legal/regulatory requirements?
- Are both the patient and a licensed physician interested in securing early access, and has the physician determined medical necessity and provided appropriate documentation to support the request? Does the patient meet any pertinent medical criteria for participation in early access program?

- Do available efficacy and safety data demonstrate that the potential benefits of treatment for the specific patient outweigh potential or known risks of the investigational product, and those risks are not unreasonable in the context of the disease or condition?
- Are both the physician and patient willing to complete the following?
    - Undergo any necessary training and certification to support the administration and use of the investigational product within Clementia's early access program.
    - Follow specific requirements necessary for Clementia to administer the program and comply with local regulations, including safety monitoring.
- Does Clementia have the resources and manufactured product necessary to support the request?

As with any investigational medicine that has not been approved by regulatory authorities, palovarotene may or may not be effective in treating the disease or condition of interest, and there may be risks associated with its use. If you are a patient interested in participating in Clementia's early access program for palovarotene, you should talk to your physician about the potential benefits and risks of taking part in the program.

Physicians who are interested in requesting early access to palovarotene for a patient may contact Clementia, an Ipsen company, at GlobalMedInfo@ipsen.com. We will acknowledge receipt and respond to inquiries within three (3) business days. As more data becomes available, Clementia may update its early access policy.