On Friday October 24, the European Medicines Agency's (EMA's) Committee for Medicinal Products for Human Use (CHMP) concluded its scientific evaluation of Clinuvel's drug SCENESSE® (afamelanotide 16mg) for adult patients diagnosed with erythropoietic protoporphyria (EPP). Based on its evaluation, the CHMP has issued a positive opinion under exceptional circumstances in compliance with EC Regulation 726/2004 14(8). Clinuvel has compiled this brief Q&A document to address some of the questions anticipated from the global EPP community as a result of the EMA decision.

What was the decision of the CHMP?
Based on its evaluation, the CHMP has issued a positive opinion, recommending that the EMA allow marketing authorisation for SCENESSE® under exceptional circumstances in compliance with EC Regulation 726/2004 14(8).

What are 'exceptional circumstances'?
This is a specific regulatory term applied to certain drug approvals. It means that the drug is recommended for approval with strict conditions which Clinuvel must fulfil once the drug is being commercially distributed to patients. The main activity will be to follow up patients for long time to ensure that safety and efficacy of the drug is maintained.

Does this mean I can access the drug in Europe?
Not immediately. The CHMP's decision must still be ratified by the European Commission in Brussels, a process which will take time and may not be completed until the end of the year or early 2015. In parallel, Clinuvel must complete the distribution requirements and logistics in various European countries. Following this process, it is envisaged that the drug will be made available to EPP patients through the specialist treatment centres in each country.

How much will the drug cost?
It is impossible to answer this question at present. It is Clinuvel's intention to facilitate drug access for as many EPP patients as is possible in a sustainable manner.

When will more information be made available about availability in Europe?
We expect to provide the community with an update before the end of 2014.

What impact does this decision have on the US FDA/SwissMedic/Australian TGA and other jurisdictions?
The main impact is that, as planned, Clinuvel will now approach and conclude discussions with other main and leading regulatory authorities in each country to discuss what would be required to file for marketing authorisation in their jurisdictions. No timeline can be provided for these discussions, however we will keep the community updated on progress.

Where can I ask specific questions?
Please contact Lachlan Hay at Clinuvel Pharmaceuticals via email: Lachlan.Hay@clinuvel.com

October 24, 2014