



Scientific Workshop on Erythropoietic Protoporphyrin (EPP)

October 24, 2016

- 9:00 – 10:00 am **Registration**
- 10:00 – 10:05 am **Welcome**
Sara Eggers, PhD
Office of Strategic Programs (OSP), CDER, FDA
- 10:05 – 10:10 am **Opening Remarks**
Kendall Marcus, MD
Director, Division of Dermatology and Dental Products (DDDP), CDER, FDA
- 10:10 – 10:20 am **The Epidemiology and Natural History of Erythropoietic Protoporphyrin**
Henry Lim, MD
- 10:20 – 10:30 am **Treatment Approaches for Erythropoietic Protoporphyrin**
Joyce Teng, MD, PhD
- 10:30 – 11:00 am **Patient Perspectives on Symptoms and Current Approaches to Treatments**
A panel of patients will provide comments on disease symptoms, daily impacts and current approaches to treatment.
- 11:00 – 12:30 pm **Large-Group Facilitated Discussion**
Patients and caregivers in the audience are invited to add to the dialogue.
- 12:30 – 12:45 pm **Open Public Comment**
- 12:45 – 1:30 pm **Lunch**
- 1:30 – 2:00 pm **An Overview of the FDA Regulatory Process**
J. Paul Phillips, MS
DDDP, CDER, FDA

Kathryn O’Connell, MD, PhD
Rare Diseases Program, CDER, FDA
- 2:00 – 2:10 pm **Challenges in Clinical Trial Design for Erythropoietic Protoporphyrin**
Elisabeth Minder, MD
- 2:10 – 3:40 pm **Panel Discussion**
A facilitated discussion with experts on specific clinical trial considerations. Patients and caregivers in the audience will also be invited to add to the dialogue.
- 3:40 – 3:50 pm **Open Public Comment**
- 3:50 – 4:00 pm **Closing Remarks**
Kendall Marcus, MD
DDDP, CDER, FDA

Discussion Questions (Morning Session):

1. Of all the symptoms that you experience because of your condition, which 1-3 symptoms have the most significant impact on your daily life? (Examples may include itching, burning, pain, scarring, etc.)
2. Are there specific activities that are important to you but that you cannot do at all or as fully as you would like because of your condition? (Examples of activities may include daily hygiene, work and school performance, participation in sports or social activities, etc.)
3. How have your condition and its symptoms changed over time?
4. What are you currently doing to manage your condition or its symptoms? (Examples may include prescription medicines, phototherapy, over-the-counter products, and other therapies including non-drug therapies such as limiting exposure to sun, diet modification, etc.)
 - a. What specific symptoms do your therapies address?
 - b. How has your treatment regimen changed over time, and why?
5. How well does your current treatment regimen control your condition?
 - a. Would you define your condition today as being well managed?
6. Assuming there is no complete cure for your condition, what specific things would you look for in an ideal treatment for your condition?
 - a. What would you consider to be a meaningful improvement in your condition (for example specific symptom improvements) that a treatment could provide?

Discussion Topics (Afternoon Session):

1. Trial population. Address:
 - a. Disease heterogeneity--possibility for enrichment
 - b. Concomitant treatment
 - c. Age--enrollment of pediatric subjects
2. Endpoints that can be reliably measured and interpreted. Address:
 - a. Demonstration of clinically meaningful benefit
 - b. Type of measure
 - i. Patient-reported outcome
 - ii. Performance measure
 - iii. Biomarker/laboratory measure
 - c. Photoprovocation
3. Other clinical trial design considerations:
 - a. Choice of control, e.g. placebo, active comparator, dose response
 - b. Trial duration
 - c. Unblinding due to side effects of the investigational product, e.g., pigmentary changes, etc.
 - d. Use of photoprovocation
4. Patient and caregiver experiences participating in clinical trials