

# PATIENT-FOCUSED DRUG DEVELOPMENT MEETING FOR EPIDERMOLYSIS BULLOSA (EB) AND PACHYONYCHIA CONGENITA (PC)



Pachyonychia Congenita Project

*Fighting for a cure. Connecting & helping patients. Empowering research.*

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because the cost of doing nothing is too great

# DOWNLOAD MEETING APP

Each session in today's meeting will include a series of polling questions on PC and EB. Patients and caregivers are encouraged to use their cell phones or computers to participate in the polling questions.

Please download the meeting app, and when instructed, answer the corresponding polling questions.

## ***FROM A CELL PHONE***

- Open the following link from your cell phone:  
**<https://crowd.cc/s/1uLms>**
- The link will bring you to a webpage that lists an app called Crowd Compass Attendee Hub. Click the Play Store or iStore logo.
- Download the Crowd Compass Attendee Hub app; then click open.
- Enter the meeting name **EB-PC FDA Meeting** and click the search button.
- The meeting will come up; then click download.
- There will then be a log in screen.
- Enter your first and last name; then click next.
- Enter your email; then click next.
- A 4 digit verification code will be emailed to you.
- Enter the 4 digit code into the meeting app and hit verify.
- Answer the optional questions (do you want to link to these social media, etc).
- Select to make your name public (within the app).
- You'll then be taken to the home screen.

## ***ON A COMPUTER***

- Go to **<https://crowd.cc/eb-pc-fda>**
- Click Log In
- Enter your first and last name; then click next.
- Enter your email; then click next.
- A 4 digit verification code will be emailed to you.
- Enter 4 digit code and click verify.
- You'll be logged in to the site.

# WELCOME TO THE PC & EB EL-PFDD

Welcome to the externally led Patient-Focused Drug Development Meeting for Pachyonychia Congenita (PC) and Epidermolysis Bullosa (EB).

PC Project and debra of America are pleased to have each of you in attendance. We are honored to have representation from key stakeholders at this meeting – senior leaders from the Food and Drug Administration (FDA), industry professionals, members of academia, clinicians, and individuals affected with PC and EB and their families. We thank all of you for coming together today to show your support and let your voices be heard.

Bringing the patient’s voice to guide the evaluation of future therapeutics for PC and EB and enhancing the FDA’s ability to assess the benefits and risks of a particular therapy is directly connected to the mission of PC Project and debra of America.

Finally, we thank each of you for your ongoing commitment to finding a treatment for these devastating diseases. We know that each and every participant in the EB and PC communities plays an essential role in our mission to find a cure.

## MEETING AGENDA: FRIDAY, APRIL 6, 2018

7:00 am	<b>BREAKFAST</b>
8:30 am	<b>OPENING REMARKS</b> Janice Schwartz, Chair, Pachyonychia
8:35 am	<b>WELCOME REMARKS</b> Julie Beitz, M.D., Director, Office of Drug Evaluation III, Division of Dermatology and
8:50 am	<b>INTRODUCTION AND MEETING OVERVIEW</b> James Valentine, J.D., M.H.S., Hyman, Phelps

# PACHYONYCHIA CONGENITA (PC) SESSION

8:55 am	<b>PC CLINICAL OVERVIEW</b> Anna Bruckner, M.D., University of Colorado—Denver
9:05 am	<b>PC DEMOGRAPHIC LIVE POLLING</b>
9:15 am	<b>TOPIC 1: LIVING WITH PC</b> <ul style="list-style-type: none"><li>• PC Panel discussion: Jack Padovano, Christine Block, Nykole Lee, James Rittle, Tara Atae, PC Video</li><li>• Living with PC live polling</li><li>• Moderated audience discussion</li></ul>
10:35 am	<b>BREAK</b>
10:45 am	<b>TOPIC 2: CURRENT &amp; FUTURE TREATMENTS</b> <ul style="list-style-type: none"><li>• PC Panel discussion: Andrew Butler, Julianne Bennett, Roseann McGrath, Austin Pinardo, Rylee Defenbaugh, Janice Schwartz</li><li>• Managing PC live polling</li><li>• Moderated audience discussion</li></ul>
11:55 am	<b>PC SESSION SUMMARY</b> Kendall Marcus, M.D., Director, Division of Dermatology and Dental Products
12:10 pm	<b>LUNCH</b>
1:10 pm	<b>AFTERNOON WELCOME &amp; OVERVIEW</b> James Valentine, J.D., M.H.S., Meeting

# EPIDERMOLYSIS BULLOSA (EB) SESSION

1:15 pm	<b>EB CLINICAL OVERVIEW</b> Anna Bruckner, M.D., University of Colorado—Denver
1:25 pm	<b>EB DEMOGRAPHIC LIVE POLLING</b>
1:35 pm	<b>TOPIC 1: LIVING WITH EB</b> <ul style="list-style-type: none"><li>• EB Panel discussion: Daisy Sherman, Megan Gosselin, Brandon Joseph, Lizzy Hendrickson, Katie Murray, Melanie Arnold</li><li>• Living with EB live polling</li><li>• Moderated audience discussion</li></ul>
2:55 pm	<b>BREAK</b>
3:05 pm	<b>TOPIC 2: CURRENT &amp; FUTURE TREATMENTS</b> <ul style="list-style-type: none"><li>• EB Panel discussion: Brett Kopelan, Heather Federici, Annie McKenzie, Hodges Caldwell, Donna McCauley, Michelle Hall</li><li>• Treating EB live polling</li><li>• Moderated audience discussion</li></ul>
4:25 pm	<b>EB SESSION SUMMARY</b> Jill Lindstrom, M.D., Deputy Director, Division of Dermatology and Dental Products
4:40 pm	<b>CLOSING REMARKS &amp; NEXT STEPS</b> Brett Kopelan, Executive Director, debra of
4:45 pm	<b>ADJOURN</b>

# DISCUSSION QUESTIONS

## TOPIC 1: LIVING WITH - DAILY IMPACT DISCUSSION

1. Of all the symptoms that you experience because of your condition, which 1-3 symptoms have the most significant impact on your life?
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? How do your symptoms and their negative impacts affect your daily life on the best days? On the worst days?
3. How has your condition and its symptoms changed over time? Do your symptoms come and go? If so, do you know of anything that makes your symptoms better? Worse?
4. What worries you most about your condition?
5. How has your condition affected your mood (for example; depression, apathy, patience/tolerance for frustration)?

## TOPIC 2: CURRENT & FUTURE TREATMENTS DISCUSSION

1. What are you currently doing to help treat your condition or its symptoms? What specific symptoms do your treatments address? How has your treatment regimen changed over time, and why?
2. How well does your current treatment regimen treat the most significant symptoms of your disease?
  - a. How well do these treatments stop or slow the progression of your disease?
  - b. How well do these therapies improve your ability to do specific activities that are important to you in your daily life?
  - c. How well have these treatments worked for you as your condition has changed over time?
3. What are the most significant downsides to your current treatments, and how do they affect your daily life?
4. Assuming there is no complete cure for your condition, what specific things would you look for in an ideal treatment for your condition?

## SPECIAL THANKS

We give special thanks to the honorable FDA speakers: Dr. Julie Beitz, Director, Office of Drug Evaluations III, Division of Dermatology and Dental Products, Dr. Kendall Marcus, Director, Division of Dermatology and Dental Products, and Dr. Jill Lindstrom, Deputy Director, Division of Dermatology and Dental Products. Without your support, encouragement and vision for this meeting, we would not be here today.

Thank you to Dr. Anna Bruckner, University of Colorado—Denver for enthusiastically agreeing to be part of this meeting. We appreciate your commitment to EB and PC patients.

Thank you to James Valentine for guiding us every step of the way in preparing for this meeting. Your leadership and unshakable faith in our organizations have made this dream a reality.

Finally, we wish to recognize the participating panelists and audience members for selflessly giving their time and generously and vulnerably sharing their lives with each of us.

## MEETING SPONSORS



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