


# Homocystinuria (HCU) EL-PFDD Proposed Agenda

October 27, 2023 | 10:00am-3pm ET

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| <b>10:00 am- 10:05 am</b> | <b>Welcome (5 mins)</b> <ul style="list-style-type: none"><li>• Danae Bartke, Executive Director, HCU Network America</li></ul>                 |
| <b>10:05 am-10:15 am</b>  | <b>FDA Opening Remarks (10 mins)</b> <ul style="list-style-type: none"><li>• Dr. Mehul Desai, DRDMG, CDER, FDA</li></ul>                        |
| <b>10:15 am-10:30 am</b>  | <b>HCU Clinical Overview (15 mins)</b> <ul style="list-style-type: none"><li>• Dr. Kimberly Chapman, Children’s National DC</li></ul>           |
| <b>10:30 am-10:35 am</b>  | <b>Introduction and Meeting Overview (5 mins)</b> <ul style="list-style-type: none"><li>• James Valentine, JD, MHS, Meeting Moderator</li></ul> |
| <b>10:35 am-10:45 am</b>  | <b>Demographic Polling (10 mins)</b>  |

## **Session 1 – Living with HCU Symptoms and Daily Impact**

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| <b>10:45 am-11:10 am</b> | <b>Patient/Caregiver Panel 1 (25 mins)</b> <ul style="list-style-type: none"><li>• 5 patient/caregiver panelists</li></ul>   |
| <b>11:10-12:30</b>       | <b>Audience polling &amp; moderated discussion (80 mins)</b> <ul style="list-style-type: none"><li>• Including 5 patient/caregiver discussion starters</li><li>• Telephone call ins</li><li>• Written comments</li></ul> |
| <b>12:30pm- 1:00 pm</b>  | <b>Lunch (30 mins)</b>   |

## **Session 2 – Current and Future Treatments for HCU**

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| <b>1:00 pm-1:10 PM</b>  | Margie McGlynn, R.Ph., Hon DSci, President, HCU Network America  |
| <b>1:10 pm-1:35 PM</b>  | <b>Patient/Caregiver Panel 2 (25 mins)</b> <ul style="list-style-type: none"><li>• 5 patient/caregiver panelist</li></ul>  |
| <b>1:35 pm-2:40 PM</b>  | <b>Audience polling &amp; moderated discussion (75 mins)</b> <ul style="list-style-type: none"><li>• Including 5 patient/caregiver discussion starters</li><li>• Telephone call ins</li><li>• Written comments</li></ul> |
| <b>2:40 pm-2:50 PM</b>  | <b>Summary Remarks (10 mins)</b> <ul style="list-style-type: none"><li>• Larry Bauer, RN, MA</li></ul>   |
| <b>2:50 pm- 3:00 pm</b> | <b>Closing Remarks (10 mins)</b> <ul style="list-style-type: none"><li>• Danae Bartke, Executive Director, HCU Network America</li></ul>   |
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