Thank you for your interest in the M-CERSI Conference on Patient-Focused Drug Development, a conference by the University of Maryland, with planning members from the University of Maryland Center of Excellence in Regulatory Science and Innovation (M-CERSI), Food and Drug Administration, academia, and industry.

The M-CERSI Conference on Patient-Focused Drug Development will be held on Monday, March 9, 2015 at the Hyatt Regency Hotel in Bethesda, MD. The conference will provide a forum for all patient-focused drug development (PFDD) stakeholders to gather for an open dialogue. Patients, caregivers, and patient advocacy groups, as well as regulators from the Food and Drug Administration (FDA); researchers from academia, industry, and other agencies; payers; and other stakeholders will come together to discuss the following topics:

- Definition of PFDD
- Essential components that need to be included in successful PFDD
- Current initiatives, such as those of the FDA, patient advocacy groups, and pharmaceutical companies
- Challenges to achieving PFDD and methods to overcome those challenges
- Recommendations on a plan of action that includes a description of stakeholders' roles to move PFDD forward

Conference deliverables will include a "Patient Engagement Rubric" for PFDD.

For more information, please visit www.pharmacy.umaryland.edu/patient_focused_drug_development.

Please provide the following information:

Name

Address

Phone

Email

Title and Company/School/Agency

Please indicate highest degree obtained:

☐ High School
☐ Bachelor’s Degree
☐ Master’s Degree
☐ Doctorate

Please indicate which category best describes you:

☐ Faculty, Staff, Student from the University of Maryland, Baltimore or College Park Campus
☐ M-CERSI Industrial Consortia Member
☐ Federal Government Employee
☐ Other Participant

Registering by mail? Please detach this form and submit it to the address below. All participants can also register online at www.pharmacy.umaryland.edu/patient_focused_drug_development.
## CONFERENCE AGENDA

### March 9, 2015

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<th>Time</th>
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<tbody>
<tr>
<td>8:30-9:00 a.m.</td>
<td><strong>Registration and Continental Breakfast</strong></td>
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<tr>
<td>9:00-9:15 a.m.</td>
<td><strong>Welcome</strong>&lt;br&gt;James Polli, PhD&lt;br&gt;Shangraw/Noxell Endowed Chair in Industrial Pharmaceutics&lt;br&gt;Co-Principal Investigator, Center for Excellence in Regulatory Science and Innovation&lt;br&gt;University of Maryland School of Pharmacy</td>
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<td></td>
<td><strong>Introduction and Objectives</strong>&lt;br&gt;Eleanor Perfetto, PhD, MS&lt;br&gt;Professor, Department of Pharmaceutical Health Services Research&lt;br&gt;University of Maryland School of Pharmacy</td>
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<td>Pat Furlong&lt;br&gt;Founding President and CEO&lt;br&gt;Parent Project Muscular Dystrophy (PPMD)</td>
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<tr>
<td>9:15-11:00 a.m.</td>
<td><strong>FDA Activities in Patient-Focused Drug Development</strong>&lt;br&gt;&lt;br&gt;<strong>Moderator:</strong> Sara Eggers, PhD (FDA)&lt;br&gt;&lt;ul&gt;&lt;li&gt;How FDA defines PFDD&lt;/li&gt;&lt;li&gt;Current FDA activities in PFDD&lt;/li&gt;&lt;li&gt;PRO's in PFDD&lt;/li&gt;&lt;li&gt;Future Directions&lt;/li&gt;&lt;/ul&gt;</td>
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<td><strong>An Overview of FDA's Patient-Focused Drug Development Initiative</strong>&lt;br&gt;Theresa Mullin, PhD&lt;br&gt;Director, Office of Strategic Programs&lt;br&gt;Food and Drug Administration</td>
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<td><strong>The Role of Patients in Health Outcomes Assessment</strong>&lt;br&gt;Ashley Slagle, PhD&lt;br&gt;Study Endpoint and Labeling Division&lt;br&gt;Food and Drug Administration</td>
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<td><strong>TBD</strong>&lt;br&gt;Richard Klein&lt;br&gt;Director, Patient Liaison Program&lt;br&gt;Food and Drug Administration</td>
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<td>11:00-11:15 a.m.</td>
<td><strong>Break</strong></td>
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**Patient-Focused Drug Development**
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<th>Time</th>
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| 11:15-1:00 p.m. | **Patient Engagement in Patient-Focused Drug Development**  
**Moderator:** Pat Furlong (PPMD)  
- Patient and Patient Advocate definition of PFDD  
- View of Patient and Advocacy Roles within PFDD  
- Current Activities in PFDD  
- Future Directions  
**Dialogue on Patient Engagement in Drug Development**  
Marc Boutin, JD  
Chief Executive Officer  
National Health Council  
**Patient-Focused Drug Development: Exploring from a Patient-First Perspective**  
Sally Okun, RN  
Vice President, Advocacy, Policy & Patient Safety  
PatientsLikeMe  
Tom Murphy  
PatientsLikeMe  
Patients Advocate for ALS  
**Patient Organization Action Steps to Engage in New Therapy Development**  
Christine Brown, MS  
Executive Director  
National PKU Alliance |
| 1:00-2:00 p.m. | **Lunch with Keynote Speaker**  
George Vradenburg  
Convener  
The Global CEO Initiative on Alzheimer’s Disease |
# CONFERENCE AGENDA

**March 9, 2015**

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<td><strong>2:00-3:00 p.m.</strong></td>
<td><strong>How Companies Are Preparing for Patient-Focused Drug Development</strong></td>
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<td><strong>Moderator:</strong></td>
<td>Lisa Egbuina-Davis, MD, MPH, MBA, Co-Founder and Director, ROI Squared, and Senior Advisor, Avalere Health</td>
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<tr>
<td><strong>Industry Definition of PFDD</strong></td>
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<td><strong>Industry Current Activities</strong></td>
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<td><strong>Industry View of Its Role</strong></td>
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<td><strong>Future Directions</strong></td>
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**Pharma’s Role in Getting Patients Ready to Partner in Development**

Anne Beal MD, MPH  
Chief Patient Officer  
Sanofi

**Patient-centricity: Making Stone Soup**

Roslyn Schneider, MD, MSc  
Global Patient Affairs  
Pfizer

**Patient Focused Drug Development: The Time is Now**

Jorie Gatlin, MD  
VP & Head of Regulatory A&P and Risk Management  
Novartis

| 3:00-3:15 p.m. | **Break** |

| 3:15-4:45 p.m. | **Panel Discussion: Future Directions and Opportunities for Collaboration**  |
| **How do we create a model for Patient-Focused Drug Development that will meet the needs of Patients, Drug Regulators, Drug Developers, and Payers?** |  |
| **Moderator:** | Eleanor Perfetto, PhD, MS (UMSOP)  |
| **Participants:** | Murray Ross, PhD (Kaiser-Permanente), Robert Epstein, MD, MS (Epstein Health, LLC), Marc Boutin, JD (National Health Council), Kristin Van Goor, PhD (PhRMA), Theresa Mullin, PhD (FDA), Suzanne Schrandt, JD (PCORI). |

| 4:45-5:00 p.m. | **Closing Remarks and Adjourn**  |
| **Eleanor Perfetto, PhD, MS and Pat Furlong** |  |