# Orphan Drug Assessment and Pricing Summit

**Wednesday, May 31, 2017**  
Kaiser Center for Total Health  
Washington, DC

## Agenda as of 5/10/17

<table>
<thead>
<tr>
<th>Time</th>
<th>Session Title</th>
<th>Speaker(s)</th>
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<tr>
<td>9:00 AM – 9:30 AM</td>
<td>Welcome and Goal Setting</td>
<td>Steven Pearson, MD, MSc</td>
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<td>9:30 AM – 10:00 AM</td>
<td>Overview of the Briefing Paper</td>
<td>Dan Ollendorf, PhD Rick Chapman, PhD</td>
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| 10:00 AM – 10:45 AM| Issue 1 – Contextual Considerations and Ethical Issues                      | *Moderated panel discussion:*
|                   | What’s an ultra-orphan drug? What are the ethical considerations for and against giving special treatment to clinical and value assessments of ultra-orphan drugs? Moderator will pose the above questions to a panel of different perspectives and will moderate a conversation that touches on certain additional considerations related to treatments for rare conditions from a societal and ethical perspective, and how they should influence the judgments of strength of evidence and analysis of reasonable long-term value for money. Considerations may include:
|                   | • First-ever treatments for serious illnesses  
|                   | • Preferences for illnesses of children  
|                   | • Environment/incentives to support investment in future treatments         | • Diane Berry, PhD Sarepta (Confirmed)  
|                   | • Paul Melmeyer, MPP, NORD (Confirmed)                                       | • Jim Sabin, MD, Harvard Medical School (Confirmed)  
|                   | • John Watkins, PharmD, MPh, Premera (Confirmed)                             | • Clark Paramore, MSPH, bluebird bio (Confirmed)  
| 10:45 AM – 11:00 AM| Break                                                                        |                                                                           |
| 11:00 – 11:45 AM | Issue 2 – Comparative Clinical Effectiveness                                |                                                                           |
|                   | ICER has a standard approach for judging net health benefit of new treatments. Should that approach change for ultra-orphan drugs? If yes, how? If no, why not? |                                                                           |
**Moderator** will pose the above questions to a panel of different perspectives, and touch on key considerations such as:

- Small clinical trial populations
- Different types and quantities of evidence / Accelerated approval pathway
- Use of surrogate outcomes
- Durability of effect
- How do the above impact assessment of clinical effectiveness?

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| 11:45 AM – 12:30 PM | **Issue 3 – Other Benefits or Disadvantages**  
ICER has a standard approach to discussing and identifying other benefits or disadvantages of new treatments. Do ultra-orphan drugs require a change to this approach, or another approach all together?  
**Moderator** will pose the above questions to a panel of different perspectives, and touch on key considerations such as:  
- Circumstances and conditions that surround many rare conditions, such as unmeasured patient health benefits, benefits that take years to measure, or caregiver burden  
- How to weigh the above in the assessment of value of ultra-orphan drugs  
  
Other Benefits or Disadvantages may include:  
- Direct patient health benefits that are not adequately captured by the QALY.  
- Reduced complexity that will significantly improve patient outcomes.  
- Reduction of important health disparities across racial, ethnic, gender, socio-economic, or regional categories.  
- Significant reduction of caregiver burden.  
- Novel mechanism of action or approach that will allow successful treatment of many patients who have failed other available treatments.  
- Significant impact on improving return to work and/or overall productivity  |
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|  | **Issue 4 – Price, Cost-effectiveness, and Affordability**  
ICER has a standard approach to cost-effectiveness analysis (long-term value for money) and potential  |
| Lunch |  | **Moderator** will pose the above questions to a panel of different perspectives, and touch on key considerations such as:  
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| **Panel Members:**  
- David Rind, MD, ICER (Confirmed)  
- Michael Sherman, MD, Harvard Pilgrim Health Care (Confirmed)  
- Clinical Experts Invited  
- Patient Advocate Invited  |
| **Moderated audience reaction:**  
**Panel Members:**  
- Elizabeth Cobbs, PhD, Merck (Confirmed)  
- Kim Lenz, PharmD, MassHealth (Confirmed)  
- A. Gordon Smith, MD, University of Utah School of Medicine (Confirmed)  
- Parents of children with rare diseases (Invited)  |
| **Moderated audience reaction:**  
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budget impact (affordability). Do these approaches need to change for ultra-orphan drugs? If so, how should they change?

Moderator will pose the above questions to a panel of different perspectives, and touch on key considerations such as:

- The role of cost of development, cost-effectiveness, and potential budget impact in assessing value for ultra-orphan treatments
- Are development and manufacturing costs relevant to pricing and assessment of value?
- What is the role of cost-effectiveness analysis using cost/QALY thresholds? Do we need different thresholds for (ultra)orphan treatments?
- Do small patient populations justify high prices?
- Good value but not affordable? What should the role of potential budget impact be in considerations of pricing, coverage, and payment?

- Rick Chapman, PhD, ICER (Confirmed)
- Pat Gleason, PharmD, Prime Therapeutics (Confirmed)
- Kenneth Hobby, MBA, CureSMA (Confirmed)
- David Mitchell, Patients for Affordable Drugs (Confirmed)
- Lisa Prosser, PhD, University of Michigan (Confirmed)
- Matt Rousculp, PhD, GlaxoSmithKline (Confirmed)

Moderated audience reaction

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<tr>
<td>3:00 PM – 4:00 PM</td>
<td>Guiding Principles for Orphan Drug Value Assessment</td>
<td>Moderated discussion with audience</td>
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<tr>
<td>4:00 PM</td>
<td>Adjournment</td>
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