INSTITUTE FOR CLINICAL AND ECONOMIC REVIEW

Orphan Drug Assessment and Pricing Summit

Wednesday, May 31, 2017

Kaiser Center for Total Health

Washington, DC

Agenda

8:30 AM – 9:00 AM	**Registration and Light Breakfast**	
9:00 AM – 9:30 AM	**Welcome and Goal Setting**	Steven Pearson, MD, MSc
9:30 AM – 10:00 AM	**Overview of the Briefing Paper**	Dan Ollendorf, PhD Rick Chapman, PhD
10:00 AM – 10:45 AM	***Issue 1 – Contextual Considerations and Ethical Issues*** **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:*** o ***First-ever treatments for serious illnesses*** o ***Preferences for illnesses of children*** o ***Environment/incentives to support investment in future treatments***	*Moderated panel discussion:* • *Diane Berry, PhD Sarepta (Confirmed)* • *Paul Melmeyer, MPP, NORD (Confirmed)* • *Jim Sabin, MD, Harvard Medical School (Confirmed)* • *John Watkins, PharmD, MPh, Premera (Confirmed)* *Moderated audience reaction*
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?**	*Moderated panel discussion* • *Vanessa Battista, RN, MS. CPNP,*

	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?***	*Children's Hospital of Philadelphia (Confirmed)* • *Annie Kennedy, Parent Project Muscular Dystrophy (Confirmed)* • *Clark Paramore, MSPH, bluebird bio (Confirmed)* • *David Rind, MD, ICER (Confirmed)* • *Michael Sherman, MD, Harvard Pilgrim Health Care (Confirmed)* • *Perry Shieh, MD, PhD, University of California, Los Angeles (Confirmed)* *Moderated audience reaction*
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.	*Moderated panel discussion* • *Elizabeth Cobbs, PhD, Merck (Confirmed)* • *Kim Lenz, PharmD, MassHealth (Confirmed)* • *Beth Moore, Parent of Two Children with SMA (Confirmed)* • *Tom Mouser, MD, Parent of Child with DMD (Confirmed)* • *A. Gordon Smith, MD, University of Utah School of Medicine (Confirmed)*

	• 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	*Moderated audience reaction*
12:30 PM – 1:30 PM	Lunch	

1:30 PM – 3:00 PM	**Issue 4 – Price, Cost-effectiveness, and Affordability**
	ICER has a standard approach to cost-effectiveness analysis (long-term value for money) and potential 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?***
		Moderated panel discussion
		• *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

3:00 PM – 4:00 PM	**Guiding Principles for Orphan Drug Value Assessment**	*Moderated discussion with audience*
4:00 PM	**Adjournment**	