CRITICAL PATH INSTITUTE

Towards Consensus Development: Qualifying Endpoint Measures for Rheumatoid Arthritis Clinical Trails

August 28, 2012

Sheraton Silver Spring Hotel
8777 Georgia Avenue – Silver Spring, MD 20910

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Critical Path Institute

The Patient-Reported Outcome (PRO) Consortium is a public-private partnership established by the Critical Path Institute (C-Path) in cooperation with the U.S. Food and Drug Administration (FDA) and the medical products industry in 2008. The PRO Consortium brings together scientists from C-Path, industry, academia, and regulatory agencies in a pre-competitive environment for the purpose of developing, evaluating, and qualifying PRO instruments for use as primary or secondary endpoint measures in clinical trials designed to evaluate treatment benefit.

On August 28, 2012, the PRO Consortium's Rheumatoid Arthritis (RA) Working Group held a workshop in Silver Spring, Maryland titled Toward Consensus Development: Qualifying Endpoint Measures for Rheumatoid Arthritis Clinical Trials. The workshop's objective was to identify RA-related symptoms and RA-defining decrements in physical function that could be explored as potential PRO endpoints in clinical trials to support label claims for RA drugs. Along with RA Working Group members and C-Path personnel, participants included RA patients, representatives from the US Food and Drug Administration, National Institute for Arthritis and Musculoskeletal and Skin Diseases, American College of Rheumatology, Outcome Measures in Rheumatology, and European League Against Rheumatism.

The expected outcome of the workshop was a research agenda aimed at collecting evidence for FDA qualification of one or more PRO instruments that capture concepts that are relevant to patients with RA. Once qualified, the PRO instruments will contribute to the assessment of treatment benefit in RA drug registration trials. The following agenda provides an overview of the day-long meeting as well as links to the slide sets presented and the workshop minutes.

Workshop Agenda
August 28, 2012

7:30-8:30 am	**Breakfast**
8:30-8:40 am	**Welcome and Introduction** *Stephen Joel Coons, PhD* — Executive Director, Patient-Reported Outcome Consortium, Critical Path Institute

8:40-8:50 am	**Introduction to RA Working Group, Overview of Workshop** *April Naegeli, DrPH, MPH* — Co-chair of the PRO Consortium RA Working Group, and Research Scientist, Global Health Outcomes, Center of Expertise, Eli Lilly and Company *Risa Hayes, PhD* — Research Advisor, Eli Lilly and Company
8:50-9:10 am	**PRO instrument qualification, overview and need for patient- focused drug development** *Janet Woodcock, MD* — Director, Center for Drug Evaluation and Research, Food and Drug Administration (FDA)
9:10-9:30 am	**Documentation of treatment benefit in RA: Current model and opportunities** *Sarah Yim, MD* — Associate Director, Division of Pulmonary, Allergy, and Rheumatology Products (DPARP), FDA
9:30-10:00 am	**General discussion**
10:00-10:10 am	**Break**
10:10-10:30 am	**RA patient perspective** *Amye L. Leong, MBA* — Chief Executive Officer, Healthy Motivation *Brian Taylor* — FDA Patient Representative, Rheumatoid Arthritis
10:30-10:40 am	**General concepts of measurements in RA** *Enkeleida Nikai, MSc Psych, MB* — Co-chair of the PRO Consortium RA Working Group, and Associate Director – Global Market Access, UCB Pharma
10:40-11:00 am	**General discussion**
11:00-11:15 am	**Identification of relevant patient subgroups** *Lee S. Simon, MD* — Principal, SDG LLC
11:15-11:30 am	**General discussion**
11:30 am-12:30 pm	**Lunch**
12:30-1:00 pm	**Concept of measurement: RA-Defining Symptoms** *Clifton O. Bingham III, MD* — Director, Johns Hopkins Arthritis Center, and Associate Professor of Medicine, Divisions of Rheumatology and Allergy, Johns Hopkins University
1:00-1:30 pm	**General discussion**

1:30-2:00 pm	**Concept of measurement: RA-defining decrements in physical functioning – Presentation #1** *Vibeke Strand, MD, FACP, FACR* — Clinical Professor, Adjunct, Division of Immunology and Rheumatology, Stanford **Concept of measurement: RA-defining decrements in physical functioning – Presentation #2** *Jasvinder Singh, MD, MPH* — Associate Professor of Medicine, Division of Clinical Immunology and Rheumatology, University of Alabama at Birmingham
2:00-2:30 pm	**General Discussion**
2:30-2:45 pm	**Break**
2:45-3:45 pm	**Discussion of the measurement gaps and the path forward** *Risa Hayes, April Naegeli, and Enkeleida Nikai*
3:45-4:00 pm	**Workshop Wrap Up** *Janet Woodcock and Stephen Joel Coons*
	Workshop Minutes