Toward Consensus Development: Qualifying Endpoint Measures for Rheumatoid Arthritis Clinical Trials

Silver Spring, MD

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Sponsored by the

Critical Path Institute's

Patient-Reported Outcome (PRO) Consortium

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Critical Path Institute

Welcome and Introduction

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Patient-Reported Outcome (PRO) Consortium

Critical Path Institute

Welcome

- Patient representatives
- Representatives from the FDA, including the Division of Pulmonary, Allergy, and Rheumatology Products (DPARP) and the Study Endpoints and Labeling Development (SEALD) team
- Clinical experts, including representatives from the American College of Rheumatology (ACR), Outcome Measures in Rheumatology (OMERACT), and European League Against Rheumatism (EULAR)
- Representatives from the National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS)
- Members of the PRO Consortium's RA Working Group

House-keeping Considerations

- Content of workshop packet
  - Agenda and objectives
  - Background material
  - Participant bio sketches
  - PRO Consortium overview article
  - RA Workshop etiquette
- Workshop is audio-recorded
- Lunch in Elm Rooms 1 & 2

Workshop: Principal Objective

To identify rheumatoid arthritis-related concepts best assessed through patient self-report that could be further investigated to determine their potential role in the documentation of treatment benefit in rheumatoid arthritis clinical trials.

Critical Path Institute and the Patient-Reported Outcome (PRO) Consortium

Critical Path Institute (C-Path)

Established in 2005 by the University of Arizona and the US Food and Drug Administration (FDA)

An independent, non-profit organization

Dedicated to implementing FDA's *Critical Path Initiative* - A strategy for transforming the way FDA-regulated products are developed, evaluated, manufactured, and used

C-Path
(www.c-path.org)

Provides a neutral, pre-competitive venue for collaboration aimed at accelerated development of safe and effective medical products

Primary sources of funding for C-Path's operations:

- government agency grants (e.g., FDA, SFAz)
- foundation grants/contracts (e.g., Gates, PKD)
- private philanthropy

Less than 20% of C-Path's operational expenses are paid by funds from commercial firms.

PRO Consortium

Formed in late 2008 by C-Path, in cooperation with the FDA and the pharmaceutical industry

- **Membership**
  - Only available to medical product companies
  - 25 members in 2012

- **Non-Voting Participants**
  - Representatives of governmental agencies
  - Clinical consultants, patients, academic researchers, and CROs partnering in the development of the PRO instruments

PRO Consortium: Members

Abbott
A Promise for Life

ACTELION

AMGEN

astellas
Leading Light for Life

AstraZeneca

Boehringer Ingelheim

Bristol-Myers Squibb

Daiichi-Sankyo

Eisai

Forest Laboratories, Inc.

gsk GlaxoSmithKline

HORIZON PHARMA

Ironwood

Johnson & Johnson

Lilly

MERCK

NOVARTIS

novo nordisk®

Pfizer

Roche

SANOFI

Shire

SUNOVION

Takeda

ucb

PRO Consortium: Goals

- Enable pre-competitive collaboration that includes FDA input/expertise
- Develop qualified, publicly available PRO instruments
- Avoid development of multiple PRO instruments for the same purpose
- Share costs of developing new PRO instruments
- Facilitate FDA's review of medical products by standardizing PRO endpoints

PRO Consortium Working Groups

- Asthma
- Cognition (mild cognitive impairment due to AD)
- Depression
- Functional Dyspepsia
- Irritable Bowel Syndrome
- Lung Cancer (NSCLC)
- **Rheumatoid Arthritis**

RA Working Group: Member Firms

PRO CONSORTIUM
CRITICAL PATH INSTITUTE

Boehringer Ingelheim

gsk GlaxoSmithKline

HORIZON PHARMA

Johnson & Johnson

Lilly

MERCK

novo nordisk®

Roche

ucb

Goal of each Working Group

To produce and/or compile the necessary evidence to enable new or existing PRO instruments to be “qualified” by the FDA for use in clinical trials where PRO endpoints can be used to support product labeling claims.

Qualification

- Qualification is based on an FDA review of evidence that supports the conclusion that a PRO instrument provides a **well-defined and reliable assessment of a targeted concept in a specified context of use**.

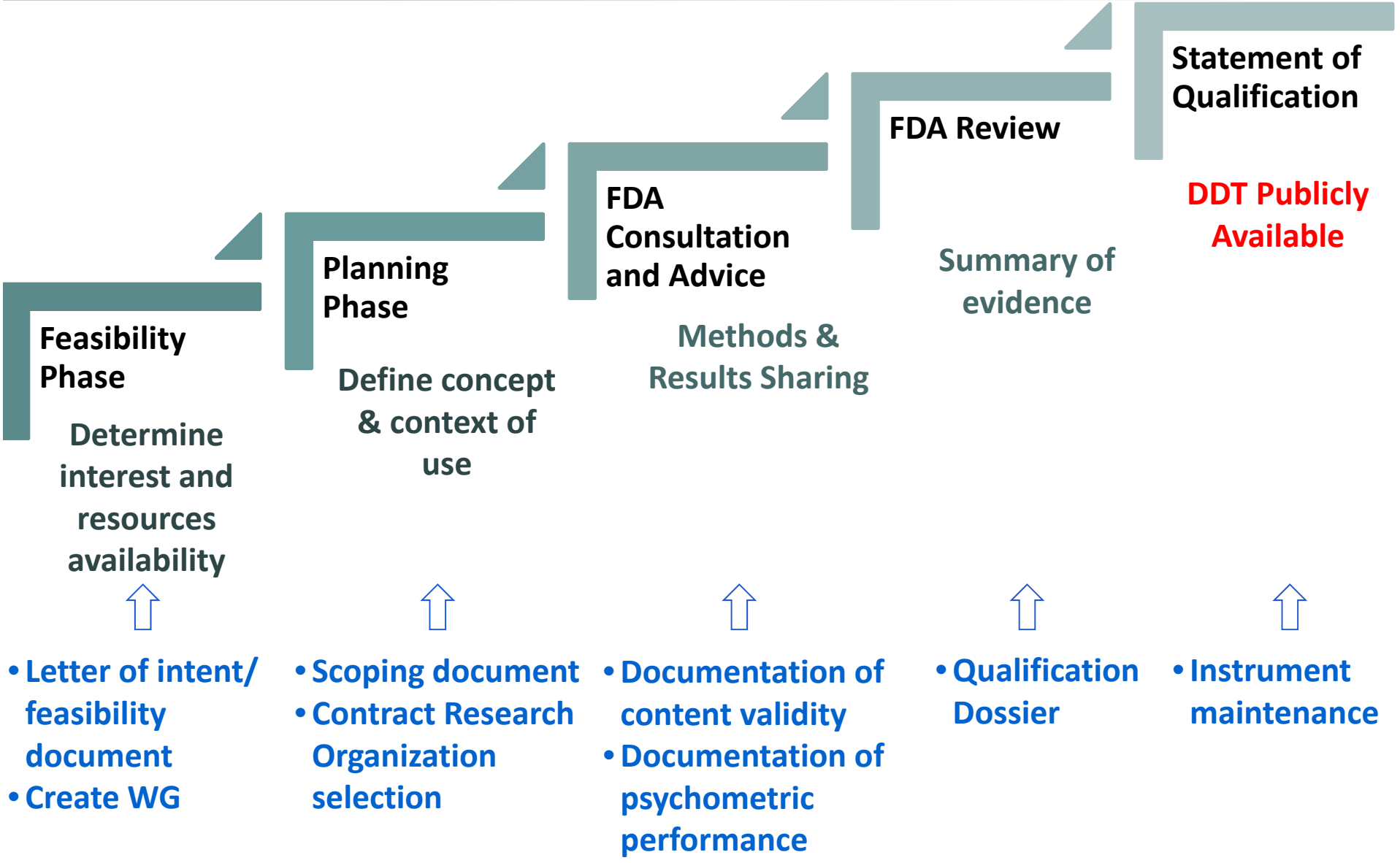
FDA's *Guidance for Industry: Qualification Process for Drug Development Tools* (draft - October 2010)

FDA's Drug Development Tool Qualification Program

- A **novel and voluntary submission** process

- "Because of the substantial work needed to achieve qualification, CDER encourages the formation of collaborative groups to undertake these….programs to increase the efficiency of joint efforts and to lessen the resource burden upon any individual person or company working to gain qualification for a tool."

Steps on the Path to PRO Instrument Qualification



PRO Instrument Qualification

…has the potential to:

- More effectively incorporate the patient's voice into the evaluation of treatment effects
- Increase number of accepted PRO measures used to support claims in product labeling
- Enhance comparability/consistency of endpoints across clinical trial
- Improve efficiency for sponsors in endpoint selection
- Improve product labeling

Words for the Day

- Collaborative
- Collegial
- Constructive
- Respectful
- Productive