Welcome and PRO Consortium Update

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SEVENTH ANNUAL
PATIENT-REPORTED OUTCOME CONSORTIUM WORKSHOP

April 27-28, 2016 ■ Silver Spring, MD

CRITICAL PATH INSTITUTE
a decade of excellence

10 YEARS

Acknowledgments

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Workshop Packet Contents

- Welcome Letter
- Workshop Agenda
- PRO Consortium Fact Sheet
- Presenters and Panelists Biographical Sketches
- Pre-Registrant List
- Workshop Feedback Form

Active Participation is Encouraged

Before you speak, please step to a micro-phone or let us bring a microphone to you

The workshop is being audio recorded

Please turn off cell phones or set to vibrate

PRO Consortium Member Firms

Working Groups

PRO CONSORTIUM
CRITICAL PATH INSTITUTE

Asthma – 10 firms
Cognition – 10 firms
Depression – 9 firms
Functional Dyspepsia – 2 firms
Irritable Bowel Syndrome (IBS) – 3 firms
Non-Small Cell Lung Cancer (NSCLC) – 8 firms
Multiple Sclerosis – 5 firms
Myelofibrosis – 2 firms
Rheumatoid Arthritis – 5 firms

Goal of Working Groups*

To produce and/or compile the necessary evidence to enable new or existing clinical outcome assessment (COA) tools to be qualified by the FDA for use in clinical trials where COA endpoints can be used to support product labeling claims.

* Myelofibrosis WG is the one exception

Working Group Updates

During breaks in today's Workshop, please view the seven working group posters at the back of the room. They will also be on display during the reception in the Magnolia Ballroom from 5:30 pm – 7:00 pm this evening.

Asthma Working Group

Co-Chairs: Linda Nelsen (GlaxoSmithKline LLC) and TBD

Target population: Adults and adolescents with a clinical diagnosis of mild to severe persistent asthma

Measurement concept: Daytime and nighttime asthma symptoms

Role in endpoint hierarchy: Co-primary or secondary endpoint to establish or support treatment benefit

Name of PRO instrument: *Asthma Daily Symptom Diary* (*ADSD*)

VALUE IN HEALTH ▮ (2016) ▮▮▮–▮▮▮

ELSEVIER

Available online at www.sciencedirect.com

ScienceDirect

journal homepage: www.elsevier.com/locate/jval

Assessing Asthma Symptoms in Adolescents and Adults: Qualitative Research Supporting Development of the Asthma Daily Symptom Diary

Adam Gater, MSc[1,], Linda Nelsen, MHS[2], Sarah Fleming, MPH[3], J. Jason Lundy, PhD[4], Nicola Bonner, MSc[1], Rebecca Hall, MMedSci[1], Chris Marshall, MSc[1], Hannah Staunton, MSc[1], Jerry A. Krishnan, MD, PhD[5], Stuart Stoloff, MD[6], Michael Schatz, MD[7], John Haughney, MD[8], on behalf of the Patient-Reported Outcome Consortium's Asthma Working Group*

Cognition Working Group

Co-Chairs: Julie Chandler (Merck Sharp & Dohme Corp) and Scott Andrews (Eli Lilly and Company)

Target population: Adults with a clinical diagnosis of mild cognitive impairment (MCI) due to Alzheimer's disease

Measurement concept: day-to-day functioning

Role in endpoint hierarchy: Co-primary endpoint to establish treatment benefit

Name of PerfO measure: TBD

Name of previously developed PRO instrument: *Interpersonal Function and Daily Activities Questionnaire* (*IFDAQ*)

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CrossMark

Alzheimer's & Dementia 12 (2016) 75-84

Alzheimer's & Dementia

Review Article

Development of a patient-reported outcome instrument to assess complex activities of daily living and interpersonal functioning in persons with mild cognitive impairment: The qualitative research phase

Mark Forrest Gordon[a,*], William R. Lenderking[b], Amy Duhig[c], Julie Chandler[d], J. Jason Lundy[e], David S. Miller[f], Elisabeth Piault-Louis[g], Rachelle S. Doody[h], Douglas Galasko[i], Serge Gauthier[j], Lori Frank[k], on behalf of the Patient-Reported Outcome Consortium's Cognition Working Group

Depression Working Group

Co-Chairs: Nicki Bush (Eli Lilly and Company) and Lucy Abraham (Pfizer, Inc.)

Target population: Adults with a clinical diagnosis of major depressive disorder

Measurement concepts: Symptoms of major depressive disorder

Role in endpoint hierarchy: Primary or secondary endpoint to establish or support treatment benefit

Name of PRO instrument: *Symptoms of Major Depressive Disorder Scale* (*SMDDS*)

Patient (2016) 9:117–134
DOI 10.1007/s40271-015-0132-1

ORIGINAL RESEARCH ARTICLE

Patient-Centered Research to Support the Development of the Symptoms of Major Depressive Disorder Scale (SMDDS): Initial Qualitative Research

Kelly P. McCarrier[1] · Linda S. Deal[2] · Lucy Abraham[3] · Steven I. Blum[4] · Elizabeth Nicole Bush[5] · Mona L. Martin[1] · Michael E. Thase[6,7] · Stephen Joel Coons[8] · On behalf of the PRO Consortium's Depression Working Group

Functional Dyspepsia Working Group

Co-Chairs: Robyn Carson (Allergan) and David Reasner (Ironwood)

Target population: Adults with a clinical diagnosis of functional dyspepsia

Measurement concepts: Symptoms of functional dyspepsia

Role in endpoint hierarchy: Primary endpoint to establish treatment benefit

Name of PRO instrument: *Functional Dyspepsia Symptom Diary* (*FDSD*)

Patient
DOI 10.1007/s40271-016-0164-1

ORIGINAL RESEARCH ARTICLE

Development of a Symptom-Based Patient-Reported Outcome Instrument for Functional Dyspepsia: A Preliminary Conceptual Model and an Evaluation of the Adequacy of Existing Instruments

Fiona Taylor[1] · David S. Reasner[2] · Robyn T. Carson[3] · Linda S. Deal[4] · Catherine Foley[1] · Ramon Iovin[1] · J. Jason Lundy[5] · Farrah Pompilus[1] · Alan L. Shields[1] · Debra G. Silberg[6]

IBS Working Group

Co-Chairs: Robyn Carson (Allergan) and Jennifer Hanlon (Ironwood)

Target population: Adults with a clinical diagnosis of IBS, including the three main subtypes: IBS-C (constipation predominant, IBS-D (diarrhea predominant) and IBS-M (mixed)

Measurement concepts: Abdominal symptoms and bowel movement-related symptoms

Role in endpoint hierarchy: Primary endpoint to establish treatment benefit

Name of instruments: *Diary for Irritable Bowel Syndrome Symptoms – Constipation (DIBSS-C), DIBSS-D*, and *DIBSS-M*

Multiple Sclerosis Working Group

Co-Chairs: TBD

Target population: Adults with a clinical diagnosis of relapsing-remitting MS

Measurement concepts: Symptom severity and functional impact

Role in endpoint hierarchy: Secondary endpoint supporting treatment benefit

NSCLC Working Group

Co-Chairs: Astra Liepa (Eli Lilly and Company) and Kendra DeBusk (Genentech, Inc.)

Target population: Adult patients with advanced NSCLC (stages IIIb/IV and ECOG performance status of 0-2)

Measurement concepts: Pulmonary and non-pulmonary symptoms of NSCLC

Role in endpoint hierarchy: Secondary endpoint to support treatment benefit

Name of PRO instrument: *Non-Small Cell Lung Cancer Symptom Assessment Questionnaire* (*NSCLC-SAQ*)

Clinical Therapeutics/Volume ∎, Number ∎, 2016

Qualitative Development and Content Validity of the Non–Small Cell Lung Cancer Symptom Assessment Questionnaire (NSCLC-SAQ), A Patient-Reported Outcome Instrument

Kelly P. McCarrier, PhD, MPH[1]; Thomas M. Atkinson, PhD[2]; Kendra P.A. DeBusk, PhD[3]; Astra M. Liepa, PharmD[4]; Michael Scanlon, MA[1]; Stephen Joel Coons, PhD[5]; and on behalf of the Patient-Reported Outcome Consortium, Non–Small Cell Lung Cancer Working Group[†]

Rheumatoid Arthritis Working Group

Co-Chairs: April Naegeli (Eli Lilly and Company) and Enkeleida Nikai (Eli Lilly and Company)

Target population: Adults with a clinical diagnosis of mild to severe rheumatoid arthritis

Measurement concept: Rheumatoid arthritis-related fatigue

Role in endpoint hierarchy: Secondary endpoint supporting treatment benefit

Name of PRO instrument: PROMIS – Fatigue (possibly 7- or 8-item static form)

Myelofibrosis Working Group

Goal: Develop a harmonized myelofibrosis symptom questionnaire for use as a secondary endpoint measure. Questionnaire intended to include fatigue in addition to the following symptoms: night sweats, itching, abdominal discomfort, early satiety, pain under ribs on left side, and bone pain.

Milestone: Harmonization Panel Meeting held on March 2, 2016, which produced the *Myelofibrosis Symptom Assessment Form* (*MFSAF*) v3 (24-hour and 7-day recall)

Next steps: Submission of manuscript, development of user manual, collection of quantitative data, and potential submission of LOI for qualification program

Since Last Year's Workshop…

Two articles published based on collaborations between the PRO Consortium and ePRO Consortium:

Fleming S, Barsdorf A, Howry C, O'Gorman H, Coons SJ. Optimizing electronic capture of clinical outcome assessment data in clinical trials: The Case of Patient-Reported Endpoints. *Therapeutic Innovation & Regulatory Science* 2015;49:797–804.

O'Donohoe P, Lundy JJ, Gnanasakthy A, Greene A. Considerations for requiring subjects to provide a response to electronic patient-reported outcome instruments. *Therapeutic Innovation & Regulatory Science* 2015;49:792–6.

Since Last Year's Workshop…

Two articles published based on sessions at the Sixth Annual PRO Consortium Workshop:

Coon C, Cappelleri JC. Interpreting change in scores on clinical outcome assessments. *Therapeutic Innovation & Regulatory Science* 2016;50:22-29.

Kovacs S, Turner-Bowker D, Calarco (Smith) G, Mulberg A, Paty J. Practical considerations for the use of clinical outcome assessments (COAs) in pediatric clinical research with examples from pediatric gastroenterology. *Therapeutic Innovation & Regulatory Science* 2016;50:37-43.

Since Last Year's Workshop…

Co-Sponsored with FDA

Workshop on Clinical Outcome Assessments (COAs) in Cancer clinical trials on April 26, 2016, in Silver Spring, MD

Near Future

Use of PRO Consortium instruments in member firms' clinical trials (pre-qualification)

- *ADSD*
  - Genentech
  - GlaxoSmithKline
- *DIBSS-C*
  - Allergan/Ironwood
- *NSCLC-SAQ*
  - Boehringer Ingelheim