Non-Small Cell Lung Cancer Working Group
Presented at the Eighth Annual PRO Consortium Workshop – Bethesda, MD – April 26-27, 2017

Milestones

<table>
<thead>
<tr>
<th>Milestone Description</th>
<th>Completed Date</th>
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<tbody>
<tr>
<td>Vendor selection and contracting</td>
<td>SEP 2012</td>
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<tr>
<td>Completion of background research (literature review and 1st expert panel)</td>
<td>FEB 2013</td>
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<tr>
<td>Submit Concept Elicitation Protocol to FDA for consultation and advice</td>
<td>OCT 2013</td>
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<tr>
<td>Draft Instrument: - Complete initial qualitative research and generate items</td>
<td>DEC 2013</td>
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<tr>
<td>Submit Qualitative Research Summary Briefing Document for quantitative study to FDA</td>
<td>JUN 2014</td>
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<tr>
<td>Submit updates to FDA for review and feedback (rounds 2-3 cognitive interviews)</td>
<td>APR 2015</td>
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<tr>
<td>Discussion with FDA for review and feedback (updated instrument) prior to launch</td>
<td>JUN 2015</td>
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<tr>
<td>Complete documentation of content validity and cross-sectional evaluation of</td>
<td>OCT 2016</td>
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<tr>
<td>Submit Qualification Briefing Package to FDA for exploratory use of NSCLC-SAQ</td>
<td>1Q 2017</td>
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Goal of the NSCLC WG

- To develop a concise PRO measure for patient-experienced symptoms in advanced NSCLC

Rationale for Non-Small Cell Lung Cancer (NSCLC) Working Group (WG)

- PRO Consortium member firms and FDA advisors identified NSCLC as a priority area
- Current therapies for advanced NSCLC are not curative, any new therapy should demonstrate control of distressing disease symptoms, including this in the product label would enable a standard method for patients and providers to compare benefit among treatments

Core Items: Seven items addressing five symptom domains

Recall Period: 7-day

Response Options: 5-level verbal rating scale

Symptom Attributes: Intensity or frequency as a measure of severity

Data Collection Mode: Tablet computer used for the quantitative pilot study

Completed Activities

- Quantitative Pilot Study Results:
  - All seven items were appropriately ordered
  - Person-item distribution for the NSCLC-SAQ items showed that the items cover the range of severities experienced within the study sample, with no large gaps in concept coverage
  - NSCLC-SAQ is able to differentiate between known-groups
  - Internal consistency reliability indicated a reliable scale
  - Test-retest reproducibility (using intraclass correlation coefficient and Pearson’s product-moment correlation) showed that the NSCLC-SAQ demonstrated good test-retest reliability
  - At FDA request, Quantitative Pilot Study Report and quantitative data submitted to FDA as part of Qualification Briefing Package instead of as separate submission

Information Dissemination

- Poster titled ‘Evaluating the Non-Small Cell Lung Cancer Symptom Assessment Questionnaire (NSCLC-SAQ): Results from the Quantitative Pilot Study presented at the 17th World Congress on Lung Cancer, December 4-7, 2016 in Vienna, Austria
- A manuscript on quantitative testing of the NSCLC-SAQ will be written and submitted for publication following its qualification for exploratory use

Working Group Updates

- Endpoints
  - Progression-Free Survival (PFS) - Response Evaluation Criteria in Solid Tumors (RECIST)
  - Overall Survival
  - Improvement in NSCLC symptoms – NSCLC-SAQ
  - Delay in time to deterioration of NSCLC symptoms – NSCLC-SAQ

Goal of the NSCLC WG

- To develop a concise PRO measure for patient-experienced symptoms in advanced NSCLC

Target Population

- Patients 18 years and older
- Advanced NSCLC (Stage IIIIB/IV) with ECOG performance status of 0-2, regardless of line of therapy

Conceptual Framework

**Measure – Non-Small Cell Lung Cancer Symptom Assessment Questionnaire (NSCLC-SAQ)**

- **Core Items**: Seven items addressing five symptom domains
- **Recall Period**: 7-day
- **Response Options**: 5-level verbal rating scale
- **Symptom Attributes**: Intensity or frequency as a measure of severity
- **Data Collection Mode**: Tablet computer used for the quantitative pilot study

**Completed Activities**

- Submit Concept Elicitation Protocol to FDA for consultation and advice
- Draft Instrument: - Complete initial qualitative research and generate items
- Submit Qualitative Research Summary Briefing Document for quantitative study to FDA for review and feedback
- Submit updates to FDA for review and feedback (rounds 2-3 cognitive interviews, final cognitive interview report, expert panel meeting, and updated instrument)
- Discussion with FDA for review and feedback (updated instrument) prior to launch of quantitative pilot study
- Conduct quantitative pilot study
- Complete documentation of content validity and cross-sectional evaluation of other measurement properties
- Submit Qualification Briefing Package to FDA for exploratory use of NSCLC-SAQ

**Working Group Participants**

- **Company/Organization**: Health Research Associates (HRA)
- **Representative**: Don Bushnell, MA; Mona Martin, RN, MPA; Kelly McCarry, PhD, MPH; Larissa Stassak; Thomas Atkinson, PhD (MSKCC)

- **Company/Organization**: AstraZeneca
- **Representative**: Anna Ryden, PhD

- **Company/Organization**: Eli Lilly and Company
- **Representative**: Astra Life, PharmD (Co-Chair); David Ayer, PhD

- **Company/Organization**: Novartis Pharmaceuticals
- **Representative**: Denise D’Alessio, MBA

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