



# ASTHMA OUTCOMES WORKSHOP

# SYMPTOMS COMMITTEE

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Public Health



# Committee Members

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Robert F. Lemanske, Jr., M.D. (Co-chair)

Jerry Krishnan, M.D., Ph.D. (Co-chair)

Glorisa Canino, Ph.D.

Kurt Elward, M.D., MPH

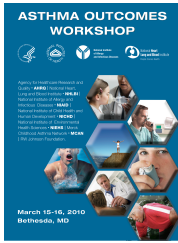
Meyer Kattan, M.D.

Elizabeth Matsui, M.D.

Herman Mitchell, Ph.D.

Rand Sutherland, M.D., MPH

Michael Minnicozi, Ph.D. (NIH liaison)



# Background

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- Sx (dyspnea, cough, wheeze, chest tightness) are used to dx asthma, to monitor response to tx, and assess control
- Sx can be measured alone or as part of a composite measure (e.g., with lung function, activity)
- Measuring Sx
  - Self-administered paper or electronic diaries prospectively (**daily diaries**)
  - Interviewer-administered questionnaires at study visits (**retrospective questionnaire**)



# Task Assigned to Symptom Committee

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- To identify and review the validity of tools that attempt to assess asthma symptoms alone
- RAND completed a comprehensive literature search to identify validated daily diaries and retrospective questionnaires about symptoms in children and adults with asthma



Author, year	Santanello et al 2000	Santanello et al 1999	Santanello et al 1997	Revicki et al, 1998
Type of instrument	Daily diary	Daily diary	Daily diary	Retrospective questionnaire
Name of instrument	Pediatric Asthma Caregiver Diary (PACD)	Pediatric Asthma Symptom Diary Scale	Daytime Symptom Diary Scale; Nocturnal Diary Scale	Asthma Symptom Utility Index (ASUI)
For children or adults	Children; 2-5 yrs	Children, 6-14 yrs	Adults	Adults
Completed by self or by care-giver	Caregiver	Child; some assistance from parents allowed; 2 <sup>nd</sup> grade reading level	Self	Self
Requires permission for use (e.g., copyright)	Yes, Merck and Co	Yes, Merck and Co	Yes, Merck and Co	No
Cost (unit price)	Not stated	Not stated	Not stated	None
Electronic or paper	Paper	Electronic	Paper	Paper

# Research of Literature Search

Key Article With Validated Instrument	Number of Citations	Number of Full-Text Reviews
Symptom Diary		
Santanello, N. C., B. L. Barber, et al. "Measurement characteristics of two asthma symptom diary scales for use in clinical trials." 1997; Eur Respir J 10(3): 646-51.	79	45
Santanello, N. C., G. Davies, et al. "Validation of an asthma symptom diary for interventional studies." Arch Dis Child 1999; 80(5): 414-20.	58	18
Santanello NC, DeMuro-Mercon C, Davies G, Ostrom N, Noonan M, Rooklin A, Knorr B. Validation of a pediatric asthma caregiver diary. J Allergy Clin Immunol 2000; 106:861-6.	20	10
Symptom Questionnaire		
Revicki DA, Leidy NK, Brennan-Diemer F, Sorensen S, Togias A. Integrating patient preferences into health outcomes assessment. Chest 1998;114:998-1007.	80	21
<b>TOTAL</b>	<b>237</b>	<b>94</b>



# DRAFT Recommendations: Adults

	<b>Characterization of Study Population for Prospective Clinical Trials</b>	<b>Prospective Clinical Trial Efficacy / Effectiveness Outcomes</b>	<b>Observational Study Outcomes</b>
<b>CORE</b>	NONE	NONE	NONE
<b>SUPPLEMENTAL</b>	1. Asthma Symptom Utility Index 2. Santanello Daytime Symptom Diary Scale and Nocturnal Diary Scale	1. Asthma Symptom Utility Index 2. Santanello Daytime Symptom Diary Scale and Nocturnal Diary Scale	1. Asthma Symptom Utility Index 2. Santanello Daytime Symptom Diary Scale and Nocturnal Diary Scale
<b>EMERGING</b>	1. Asthma Burden Index 2. Various symptom composite indices: Symptom Free Days, Episode Free Days, Maximum Symptom Days, Asthma Control Days	1. Asthma Burden Index 2. Various symptom composite indices: Symptom Free Days, Episode Free Days, Maximum Symptom Days, Asthma Control Days	1. Asthma Burden Index 2. Various symptom composite indices: Symptom Free Days, Episode Free Days, Maximum Symptom Days, Asthma Control Days



# DRAFT Recommendations: Children

	<b>Characterization of Study Population for Prospective Clinical Trials</b>	<b>Prospective Clinical Trial Efficacy / Effectiveness Outcomes</b>	<b>Observational Study Outcomes</b>
<b>CORE</b>	NONE	NONE	NONE
<b>SUPPLEMENTAL</b>	Pediatric Asthma Caregiver Diary (daily diary; Santanello, 2000)	Pediatric Asthma Caregiver Diary (daily diary; Santanello, 2000)	Pediatric Asthma Caregiver Diary (daily diary; Santanello, 2000)
<b>EMERGING</b>	1. Asthma Symptom Diary Scale 2. Asthma Burden Index 3. Various symptom composite indices: Symptom Free Days, Episode Free Days, Maximum Symptom Days, Asthma Control Days	1. Asthma Symptom Diary Scale 2. Asthma Burden Index 3. Various symptom composite indices: Symptom Free Days, Episode Free Days, Maximum Symptom Days, Asthma Control Days	1. Asthma Symptom Diary Scale 2. Asthma Burden Index 3. Various symptom composite indices: Symptom Free Days, Episode Free Days, Maximum Symptom Days, Asthma Control Days





# Symptoms Committee: Final Recommendations

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- Recommend measuring symptoms as an outcome measure.
- Of the instruments to measure symptoms currently available, NONE are considered to be core.



# Symptoms Committee: Final Recommendations

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- Until instruments that qualify as core are further refined and/or newly developed, researchers may choose from a set of tools to measure symptoms.



# Symptoms Committee: Future Research Recommendations

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- Development of retrospective symptom questionnaire
  - Recording of symptoms
    - Frequency and/or severity
    - Individual symptoms—wheeze, cough, SOB, chest tightness  
nocturnal vs daytime symptoms—versus overall
  - Recall period
    - Preferably two wks; no more than four wks
  - In what populations do they apply?
    - Age, gender, culture, race, ethnicity, socioeconomic status
    - For characterization and to serve as endpoints in clinical trials and observational studies
- Comparisons of data collected using a diary versus retrospective questionnaires need to be performed



# Symptoms Committee: Future Recommendations

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- The instruments used to measure symptoms in a study should be specifically and completely described in the publication (e.g., methods section) to facilitate evaluation of results across studies as much as possible.



# Symptoms Committee: Future Recommendations

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- Research to develop validated instruments (more than face validity) to evaluate symptoms that could be considered “core” is needed.
- Symptom assessments are embedded in asthma control and quality of life instruments.
  - How much overlap is there between these and “symptom only” instruments?
  - If overlap exists, can this be disentangled to minimize study burden to the subject and coordinator?