

PRINTED HANOUT: Outcomes and Quality Measures in Allergy Practice and Research

Seminar 4010

Monday, March 3, 2014

Learning Objectives:

1. Identify the most appropriate outcomes measures for assessment of quality ambulatory and inpatient practice in asthma
 1. Federal Measures
 2. Patient centric measures
 3. Cost effectiveness measures
2. Discuss quality measures in the ambulatory setting for allergic diseases
 1. Checklist for safety and accuracy in Immunotherapy
 2. Quality control in ambulatory spirometry
 - 3.
3. Learn how to evaluate outcome studies of asthma.
 1. Concepts of Minimally Important Clinical Difference in Quality of Life
 2. Pitfalls in comparing therapeutic efficacy using exacerbation as an endpoint
 1. Issues in the calculation of Numbers Needed to Treat

Pertinent Bibliography:

Designing clinical trials to address the needs of childhood and adult asthma: The National Heart, Lung, and Blood Institute's AsthmaNet . J Allergy Clin Immunol 2014;133:34-8
E. Rand Sutherland, MD, MPH, and William W. Busse, MD,

TABLE I. Critical pediatric asthma questions identified by AsthmaNet members during protocol planning

- Does immunomodulation prevent asthma or improve control?
- Does treatment in childhood prevent morbidity in later life?
- What is the best approach to step 2-4 therapy in children (both step-up and step-down therapy)?
- Can anticholinergic agents replace β -agonists in patients with suboptimally controlled asthma?
- Does early treatment of exacerbations reduce their severity or duration?
- Which is better: intermittent or continuous therapy for exacerbations?
- Is there a role for macrolide antibiotics in acute exacerbations?
- What are the optimal objective measurements of disease in children unable to perform spirometry?
- Can we use biomarkers to predict treatment response in children?
- What are optimal drug delivery strategies for children with asthma?
- Can we enhance adherence in adolescents?
- Does parental education enhance treatment efficacy?

TABLE II. Critical adult asthma questions identified by AsthmaNet members during protocol planning

- What are the optimal approaches for ICS/long-acting β -agonist step-down therapy?
- What are the optimal approaches for treatment of intermittent disease?
- Are symptom-based treatments appropriate in patients with moderate asthma?
- What is the optimal clinical evaluation for patients with refractory asthma?
- What approaches are best when step 6 therapy fails?
- Can we prevent exacerbations or manage them more effectively?
- What is the optimal approach to treating asthmatic patients who smoke?
- How does obesity affect the diagnosis and management of asthma?
- How do we treat comorbid gastroesophageal reflux disease, and is there benefit to doing so?
- Are tailored approaches based on race/ethnicity needed?
- Can we use phenotypes to optimize therapy and define prognosis?
- What is the pathobiology of fixed airflow limitation?

- **Assessing Participant-Centered Outcomes to Improve Clinical Research.**
- Rhonda G. Kost, M.D., Laura M. Lee, M.S., R.N., Jennifer Yessis, Ph.D., Robert A. Wesley, Ph.D., David K. Henderson, M.D., and Barry S. Collier, M.D. NEJM 369 23 2179-2181
- J Allergy Clin Immunol. 2014 Jan;133(1):27-33. doi: 10.1016/j.jaci.2013.10.026.

- Researching asthma across the ages: Insights from the National Heart, Lung, and Blood Institute's Asthma Network. Cabana MD¹, Kunselman SJ², Nyenhuis SM³, Wechsler ME⁴.
- J Allergy Clin Immunol. 2013 Dec 20. pii: S0091-6749(13)01708-9. doi: 10.1016/j.jaci.2013.11.001. [Epub ahead of print]. Validation studies of asthma patient-reported outcomes: "We want more!" Sorkness CA.
- Busse WW, Morgan WJ, Taggart V, Togias A. Asthma outcomes workshop: overview. J Allergy Clin Immunol 2012;129(Suppl):S1-8.
- Interventions to modify health care provider adherence to asthma guidelines: a systematic review. Okelo SO, Butz AM, Sharma R, Diette GB, Pitts SI, King TM, Linn ST, Reuben M, Chelladurai Y, Robinson KA. Pediatrics. 2013 Sep;132(3):517-34. doi: 10.1542/peds.2013-0779. Epub 2013 Aug 26. Review.
- Have expert guidelines made a difference in asthma outcomes? Podjasek JC, Rank MA. Curr Opin Allergy Clin Immunol. 2013 Jun;13(3):237-43. doi: 10.1097/ACI.0b013e32836093c3.
- Johnson R, **Dinakar C**. Pediatric pay-for-performance: Who Pays? Curr Allergy Asthma Rep. 2010 Aug 20

TABLE I. Challenges with studies evaluating cross-age response to asthma treatment

Issue	Examples
Recruitment	<ul style="list-style-type: none"> • Need for culturally diverse recruitment materials • Need for age-appropriate recruitment strategies
Consent/assent	<ul style="list-style-type: none"> • Requirement of parental/guardian consent for minors • Incorporation of assent procedures for children
Intervention selection	<ul style="list-style-type: none"> • Difficulty in maintaining consistency of intervention therapy because of differences in dosing and drug availability
Control selection	<ul style="list-style-type: none"> • Limitations in the use of placebo controls for children
Outcome measurement	<ul style="list-style-type: none"> • Dependence on proxy reporting of outcomes for children • Dearth of cross-age instruments to measure QOL, asthma control, symptoms, and exacerbations
Participant growth and development	<ul style="list-style-type: none"> • Need to account for physical growth (eg, weight and height) of pediatric participants
Physical limitations in data collection	<ul style="list-style-type: none"> • Physical and developmental limits for testing and data collection (eg, phlebotomy, spirometry, and sputum induction) • Increased safety restrictions in testing of children (eg, methacholine bronchoprovocation)
Consideration of long-term effects	<ul style="list-style-type: none"> • Careful monitoring and follow-up for long-term developmental effects in children
Reporting of results	<ul style="list-style-type: none"> • Determination of how data across ages will be subdivided (eg, age cutoffs, including adolescents as adults or children)

TABLE III. Conditions under which placebos can be ethically used in pediatric drug research*

1. When there is no commonly accepted therapy for the condition and the agent under study is the first that can modify the course of the disease process
2. When the commonly used therapy for the condition is of questionable efficacy
3. When the commonly used therapy for the condition carries with it a high frequency of undesirable adverse effects and the risks might be significantly greater than the benefits
4. When the placebo is used to identify the incidence and severity of adverse effects produced by adding a new treatment to an established regimen
5. When the disease process is characterized by frequent and spontaneous exacerbations and remissions and the efficacy of the therapy has not been demonstrated

*Modified from the American Academy of Pediatrics.³¹