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Outcomes Research: Past, Present, and Future

Jay F. Piccirillo, MD, FACS

June 21, 2013

WASHINGTON UNIVERSITY IN ST. LOUIS
Outcomes research — the study of the end results of health services that takes patients’ experiences, preferences, and values into account — is intended to provide scientific evidence relating to decisions made by all who participate in health care.

Outcomes Research History

- Geographic Variation Studies
- Appropriateness Research
Findings: Wide geographic variation in surgical procedures without identifiable differences in pre-treatment medical condition

Example: Five-fold difference in tonsillectomy rates in counties of Vermont

“There are no data available that would allow us to relate these variations to the prevalence of tonsillitis, but it appears that the variations are more likely to be associated with differences in beliefs among physicians concerning the indications for, and efficacy of, the procedure.”

John E. Wennberg, MD, MPH 1973
% Decedents Seeing 10 or more Physicians in the Last 6 Months of Life

BJH: 39.8%

Variation in End-of-Life Care Dartmouth Atlas of Health Care
% Decedents Seeing 10 or more Physicians in the Last 6 Months of Life

BJH: 39.8%

60.8%
Hospital Days per Decedent in Last 6 Months of Life

COTH Institution

BJH: 17.3
Appropriateness Research

- Attempt to explain geographic variation
- Panel of “experts” assembled to establish guidelines for evaluation of appropriateness
- Findings: Large percentage of coronary angiography, carotid endarterectomy, and other procedures performed with “inappropriate” or “equivocal” indications
- Degree of inappropriateness similar in both high and low-use areas!
## Ratings of Appropriateness

<table>
<thead>
<tr>
<th>Procedure</th>
<th>Inappropriate (%)</th>
<th>Equivocal (%)</th>
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<td>Angiography (N=1677)</td>
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<td>Endarterectomy (N=1302)</td>
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Chassen MR et al *JAMA* 1987;258:2543-2547  
Winslow CM et al *NEJM* 1988;318: 721-727
### Appropriateness of Use of Six Different Procedures with 95% Confidence Intervals

<table>
<thead>
<tr>
<th>Appropriateness Category %</th>
<th>Tympanostomy Tubes(^a) (N=6429)</th>
<th>Coronary Angiography(^b) (N=1335)</th>
<th>Carotid Endarterectomy(^c) (N=1302)</th>
<th>Upper GI Endoscopy(^d) (N=1585)</th>
<th>Hysterectomy(^e) (N=642)</th>
<th>Sinus Surgery(^f) (N=55)</th>
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<td>17 (15-19)</td>
<td>16 (9-23)</td>
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\(^a\) Kleinman LC et al *JAMA* 1994;271(16):1250-1255  
\(^b\) Bernstein SJ et al *JAMA* 1993;269(6):766-769  
\(^c\) Winslow CM *NEJM* 1988;318:721-727  
\(^e\) Bernstein SJ *JAMA* 1993;269:2398-2402  
\(^f\) Piccirillo JF et al *Laryngoscope* 1998; 108(3):332-338
Differences Between Outcomes Research and Traditional Clinical Research

- New Research Methodologies
- Inclusion of Patient-Reported Description of Illness and Outcome
- Attention to Comorbidities
New Research Methodologies

- Prospective observational studies of single or multiple therapies for a specific disease

- Para-analysis of results of therapy from large computerized, administrative, and financial databases

- Meta-analysis, Literature Review, and Consensus Techniques
Prospective, Observational Studies of Single or Multiple Therapies

- Patients studied in “natural” clinical setting
- No attempts to select or control treatments
- Primary data
Adult Cardiac Surgery in New York State

- For many years, the NYS Department of Health studied effects of patient and treatment characteristics on outcomes for patients with heart disease.

- Hospitals and doctors involved in cardiac care “cooperated” with DOH and the NYS Cardiac Advisory Committee to compile accurate and meaningful data to enhance quality of care

Hannan EL et al *JAMA.* 1994;271:761-766
The results used to create a cardiac profile system, which assesses the performance of hospitals and surgeons over time, independent of the severity of each individual patient’s pre-operative conditions.

Isolated CABG is the most common of the many types of cardiac surgery performed on adults.

NYS reported risk-adjusted outcomes for isolated CABG surgery for over twenty years.
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*The 1992 totals differ slightly from other tables since they do not include the cases in one hospital that began performing cardiac surgery in 1992.
†Risk-adjusted rate was significantly lower than the statewide rate based on a statistical model for that year.
‡Risk-adjusted rate was significantly higher than the statewide rate based on a statistical model for that year.
Otolaryngology Examples


- Weaver et al. Survival of veterans with sleep apnea: continuous positive airway pressure versus surgery. *Otolaryngol Head Neck* 2004;130:659-665
OBJECTIVES: Continuous positive airway pressure (CPAP) improves sleep apnea survival. We tested whether CPAP is associated with better survival than uvulopalatopharyngoplasty (UPPP).

STUDY DESIGN AND METHODS: This retrospective cohort database study included all sleep apnea patients treated with CPAP or UPPP in Veteran Affairs facilities from October 1997 through September 2001. Treatment groups were compared with Cox regression, adjusting for age, gender, race, year treatment was initiated, and comorbidity. Sleep apnea severity and CPAP use data were not available.

RESULTS: By September 2002, 1339 (7.1%) of 18,754 CPAP patients and 71 (3.4%) of 2,072 UPPP patients were dead ($P < 0.001$). After adjustment, CPAP patients had 31% (95% confidence interval, 3% to 67%, $P = 0.03$) higher probability of being dead at any time, relative to UPPP patients.

CONCLUSIONS: UPPP confers a survival advantage over CPAP, after adjustment for age, gender, race, year of treatment, and comorbidity. However, we were unable to adjust for sleep apnea severity or CPAP use. Surgical treatment should be considered in sleep apnea patients who use CPAP inadequately. (Otolaryngol Head Neck Surg 2004;130:659-65.)
UPPP Survival

P<0.0001 by Log Rank

Weaver et al Otolaryngol HNS 2004;130:659-665
UPPP Mortality

Adjusted* Hazard Ratio of Death

P = 0.03

Adjusted for age, sex, race, year, comorbidity.
CPAP vs UPPP

Outcome

Bad

Good

UPPP  CPAP
CPAP v UPPPP

Outcome

Bad

Good

UPPP

CPAP

Non-Users

Users
Analysis from Large, Computerized, Administrative, and Financial Data Bases (e.g., Medicare)

- Study results of treatment over wide geographic areas and large numbers of patients

- Secondary data
Examples


Iatrogenic Esophageal Perforation in Patients with Head & Neck Cancer

- Goals: 1) Determine rate of iatrogenic esophageal perforation in head and neck cancer patients, 2) Identify risk factors for perforation, and 3) Determine effect of perforation on mortality.

- Secondary data analysis – SEER-Medicare

1° outcome - rate of iatrogenic esophageal perforation

- 152 perforations in 126 patients
  - 2.7% (95% CI 2.3 to 3.2) per patient (n = 4659)
  - 1.4% (95% CI 1.2 to 1.7) per esophagoscopy (n = 10,529)
Meta-Analysis, Literature Review, and Consensus Techniques

- Analysis of the results of therapies from the published literature
- Expert opinion for the determination of preferred therapies
Otolaryngology Examples


- Sher AE, Schectman KB, Piccirillo JF. The efficacy of surgical modifications of the upper airway in adults with obstructive sleep apnea syndrome. *Sleep* 1996;19:156-177
**Expanded Use of Patient-Reported Description of Illness and Outcome**

- For conditions defined by symptoms, diagnosis and management is almost entirely defined by symptoms and their impact on daily function and well-being.

- Evaluation of treatment effectiveness should be based on subjective assessment of symptoms and function.

- Subjective measures provide valuable information on aspects of disease impact that are most bothersome to the patient and evidence for assessment of treatment effectiveness.
Patient-Reported Outcome Measures

- Previously, measures thought to be more “objective” were included to the exclusion of subjective patient-reported outcome measures.

- Growing recognition that
  - “Objective” measures not so objective
  - “Objective” measures may not capture essence of illness
  - Lack of correlation between “objective” and subjective measures
Clinimetrics

Alvan R. Feinstein
While we can measure a biological response, we may not be able to determine whether that response makes a noticeable difference to the patient.

Prospective comparison of patient-based symptoms of sinusitis with imaging findings

221 subjects participated by completing SNOT-20 immediately before undergoing CT

Radiologists scored the degree of mucosal thickening at each of 12 sites on CT scans using a staged scale of severity

Correlation of total Sino-Nasal Outcome Test-20 (SNOT-20) scores and total computed tomographic (CT) scores.
Figure 2. Scatterplot of relationship between computed tomography (CT) scan findings using Lund staging system and patient-based sinusitis severity using Chronic Sinusitis Survey (CSS), of group of patients (n = 43) from BCM. Correlation coefficient = 0.05 (p = 0.79).
Methodological Requirements for Outcomes Research

- Establish diagnostic criteria for disease and population under study; use methods to avoid bias in collection
- Create clinical-severity index for prognostic stratification
- Identify and measure comorbid conditions
- Establish outcomes measures which incorporate traditional end-points with assessments of symptoms, functional capacity, quality of life, and satisfaction with care
Diagnostic Criteria for Disease

- Consensus Conference
- Literature Review
- Clinical Research
Create Clinical-Severity Index

- Clinical-severity implies the seriousness or prognosis of disease

- The need to define how sick a patient is in order to
  - Assess diagnostic efficiency
  - Refine prognosis
  - Evaluate therapeutic effectiveness
Identify and Measure Comorbid Conditions

- Comorbidity--the presence of concomitant disease, not related to the index disease which may affect the diagnosis, treatment, and prognosis for the patient.
- Prognostic comorbidity--concomitant disease severe enough to impact on outcome of interest.
- Therapeutic comorbidity--concomitant disease which prevents use of ideal or preferred therapy.
Comorbidity Data Collection Form

Identify the important medical comorbidities and grade severity using the index. Overall Comorbidity Score is defined according to the highest ranked single ailment, except in the case where two or more Grade 2 ailments occur in different organ systems. In this situation, the overall comorbidity score should be designated Grade 3.

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</tr>
</thead>
<tbody>
<tr>
<td><strong>Cardiovascular System</strong></td>
<td><strong>Severe Decompensation</strong></td>
<td><strong>Moderate Decompensation</strong></td>
<td><strong>Mild Decompensation</strong></td>
</tr>
<tr>
<td>Myocardial Infarct</td>
<td>▪ MI ≤ 6 months</td>
<td>▪ MI &gt; 6 months ago</td>
<td>▪ Old MI by ECG only, age undetermined</td>
</tr>
<tr>
<td>Angina / Coronary Artery Disease</td>
<td>▪ Unstable angina</td>
<td>▪ Chronic exertional angina</td>
<td>▪ ECG or stress test evidence or catheterization evidence of coronary disease without symptoms</td>
</tr>
<tr>
<td></td>
<td></td>
<td>▪ Recent (≤ 6 months) Coronary Artery Bypass Graft (CABG) or Percutaneous Transluminal Coronary Angioplasty (PTCA)</td>
<td>▪ Angina pectoris not requiring hospitalization</td>
</tr>
<tr>
<td></td>
<td></td>
<td>▪ Recent (≤ 6 months) coronary stent</td>
<td>▪ CABG or PTCA (&gt;6 mos.)</td>
</tr>
<tr>
<td></td>
<td></td>
<td>▪ Ejection fraction &lt; 20%</td>
<td>▪ Coronary stent (&gt;6 mos.)</td>
</tr>
<tr>
<td>Congestive Heart Failure (CHF)</td>
<td>▪ Hospitalized for CHF within past 6 months</td>
<td>▪ Hospitalized for CHF &gt;6 months prior</td>
<td>▪ CHF with dyspnea which has responded to treatment</td>
</tr>
<tr>
<td></td>
<td>▪ Ejection fraction &lt; 20%</td>
<td>▪ CHF with dyspnea which limits activities</td>
<td>▪ Exertional dyspnea</td>
</tr>
<tr>
<td></td>
<td></td>
<td>▪ CHF with dyspnea which limits activities</td>
<td>▪ Paroxysmal Nocturnal Dyspnea (PND)</td>
</tr>
<tr>
<td>Arrhythmias</td>
<td>▪ Ventricular arrhythmia ≤ 6 months</td>
<td>▪ Ventricular arrhythmia &gt; 6 months ago</td>
<td>▪ Sick Sinus Syndrome</td>
</tr>
<tr>
<td></td>
<td></td>
<td>▪ Chronic atrial fibrillation or flutter</td>
<td></td>
</tr>
<tr>
<td></td>
<td></td>
<td>▪ Pacemaker</td>
<td></td>
</tr>
<tr>
<td>Hypertension</td>
<td>▪ DBP≥130 mm Hg</td>
<td>▪ DBP 115-129 mm Hg</td>
<td>▪ DBP 90-114 mm Hg</td>
</tr>
<tr>
<td></td>
<td>▪ Severe malignant papilledema or other eye changes</td>
<td>▪ Secondary cardiovascular symptoms: vertigo, epistaxis, headaches</td>
<td>▪ DBP &lt;90 mm Hg while taking antihypertensive medications</td>
</tr>
<tr>
<td></td>
<td>▪ Encephalopathy</td>
<td>▪</td>
<td></td>
</tr>
<tr>
<td>Venous Disease</td>
<td>▪ Recent PE (≤ 6 mos.)</td>
<td>▪ DVT controlled with Coumadin or heparin</td>
<td>▪ Old DVT no longer treated with Coumadin or Heparin</td>
</tr>
<tr>
<td></td>
<td>▪ Use of venous filter for PE’s</td>
<td>▪ Old PE &gt; 6 months</td>
<td></td>
</tr>
<tr>
<td>Peripheral Arterial Disease</td>
<td>▪ Bypass or amputation for gangrene or arterial insufficiency &lt; 6 months ago</td>
<td>▪ Bypass or amputation for gangrene or arterial insufficiency &gt; 6 months</td>
<td>▪ Intermittent claudication</td>
</tr>
<tr>
<td></td>
<td>▪ Untreated thoracic or abdominal aneurysm (≥6 cm)</td>
<td>▪ Chronic insufficiency</td>
<td>▪ Untreated thoracic or abdominal aneurysm (&lt; 6 cm)</td>
</tr>
<tr>
<td></td>
<td></td>
<td>▪</td>
<td>▪ s/p abdominal or thoracic aortic aneurysm repair</td>
</tr>
</tbody>
</table>
Adult Comorbidity Evaluation-27
Comorbidity Calculator

Available on the Internet!

http://otooutcomes.wustl.edu/prognostigram/Documents/calc.htm
### IMPACT OF PROGNOSTIC COMORBIDITY ON FIVE-YEAR SURVIVAL RATES

<table>
<thead>
<tr>
<th>Prognostic Comorbidity</th>
<th>Rectum Cancer</th>
<th>Larynx Cancer</th>
<th>Endometrial Cancer</th>
<th>Larynx Cancer</th>
<th>Prostate Cancer</th>
</tr>
</thead>
<tbody>
<tr>
<td>Absent</td>
<td>85/264 (32%)</td>
<td>93/172 (54%)</td>
<td>102/131 (78%)</td>
<td>123/166 (74%)</td>
<td>137/229 (60%)</td>
</tr>
<tr>
<td>Present</td>
<td>6/54 (11%)</td>
<td>3/20 (15%)</td>
<td>3/11 (27%)</td>
<td>4/27 (15%)</td>
<td>6/38 (16%)</td>
</tr>
<tr>
<td>Total</td>
<td>91/318 (29%)</td>
<td>96/192 (50%)</td>
<td>105/142 (74%)</td>
<td>127/193 (66%)</td>
<td>143/267 (54%)</td>
</tr>
</tbody>
</table>

| $\chi^2$ | 9.76 | 10.94 | 3.54 | 36.27 | 25.41 |
| p value   | 0.0018 | 0.0009 | 0.0599 | <0.0001 | <0.0001 |

Denominators- number of patients in each category  
Numerators- corresponding number of five-year survivors
Establish Outcome Measures

- Mortality
- Morbidity
- Health Status (General/Disease-Specific)
  - Physical
  - Functional
  - Emotional
- Health-Related Quality of Life
- Satisfaction with Care
Medical Outcomes Study SF-36
- Originally developed for study of utilization of health insurance
- 36 items
- Measures health status in 8 domains
  - PF, RP, BP, GH, VT, SF, RE, and MH
- Scores range from 0-100 on each domain

Ware JE, Sherbourne CD. *Medical Care* 1992;30(6):473-483
McHorney CA, Ware JE, Raczek AE. *Medical Care* 1993;31:247-263
### Eight Subscales of General Health

<table>
<thead>
<tr>
<th>Subscale</th>
<th>Definition</th>
</tr>
</thead>
<tbody>
<tr>
<td>PF</td>
<td>Limitations on physical activities such as walking, bathing, and strenuous sports</td>
</tr>
<tr>
<td>RP</td>
<td>Problems with work or other daily activities as a result of physical health</td>
</tr>
<tr>
<td>BP</td>
<td>Intensity of bodily pain or limitations due to pain</td>
</tr>
<tr>
<td>GH</td>
<td>Perception of current health and health outlook</td>
</tr>
<tr>
<td>VT</td>
<td>Level of energy</td>
</tr>
<tr>
<td>SF</td>
<td>Extent health interferes with normal social activities</td>
</tr>
<tr>
<td>RE</td>
<td>Problems with daily activities as a result of emotional issues</td>
</tr>
<tr>
<td>MH</td>
<td>Mental health screening</td>
</tr>
</tbody>
</table>
SF-36 General Health Survey
National Norms and Rhinosinusitis

Domain Score

PF RP BP GH VT SF RE MH

Khalid AN, Quraishi SA, Kennedy DW. *Am J Rhinology* 2004;18(3):131-136
**Otolaryngology Examples**


**Disease-Specific Health Status**

Sino-Nasal Outcome Test-20 (SNOT-20)

- 20 Sino-nasal specific items
- Identified from focus group discussions
- Response category for each item none, mild, moderate, and severe
- Patients identify important items

Piccirillo JF, Merritt MG, Richards ML. *Otolaryngol Head Neck Surg* 2002;126:41-47
**SINO-NASAL OUTCOME TEST (SNOT-20)**

Below you will find a list of symptoms and social/emotional consequences of your rhinosinusitis. We would like to know more about these problems and would appreciate your answering the following questions to the best of your ability. There are no right or wrong answers, and only you can provide us with this information. Please rate your problems as they have been over the past two weeks. Thank you for your participation. Do not hesitate to ask for assistance if necessary.

1. Considering how severe the problem is when you experience it and how frequently it happens, please rate each item below on how “bad” it is by circling the number that corresponds with how you feel using this scale: →

<table>
<thead>
<tr>
<th>Item</th>
<th>No problem</th>
<th>Very mild problem</th>
<th>Mild or slight problem</th>
<th>Moderate Problem</th>
<th>Severe Problem</th>
<th>Problem as bad as it can be</th>
</tr>
</thead>
<tbody>
<tr>
<td>1. Need to blow nose</td>
<td>0</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
<tr>
<td>2. Sneezing</td>
<td>0</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
<tr>
<td>3. Runny nose</td>
<td>0</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
<tr>
<td>4. Cough</td>
<td>0</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
<tr>
<td>5. Post-nasal discharge</td>
<td>0</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
<tr>
<td>6. Thick nasal discharge</td>
<td>0</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
<tr>
<td>7. Ear fullness</td>
<td>0</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
<tr>
<td>8. Dizziness</td>
<td>0</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
<tr>
<td>9. Ear pain</td>
<td>0</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
<tr>
<td>10. Facial pain/pressure</td>
<td>0</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
<tr>
<td>11. Difficulty falling asleep</td>
<td>0</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
<tr>
<td>12. Wake up at night</td>
<td>0</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
<tr>
<td>13. Lack of a good night’s sleep</td>
<td>0</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
<tr>
<td>14. Wake up tired</td>
<td>0</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
<tr>
<td>15. Fatigue</td>
<td>0</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
<tr>
<td>16. Reduced productivity</td>
<td>0</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
<tr>
<td>17. Reduced concentration</td>
<td>0</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
<tr>
<td>18. Frustrated/restless/irritable</td>
<td>0</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
<tr>
<td>19. Sad</td>
<td>0</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
<tr>
<td>20. Embarrassed</td>
<td>0</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
</tbody>
</table>

2. Please mark the most important items affecting your health (maximum of 5 items) ________________________________

---

Copyright © 1996 by Jay F. Piccirillo, M.D., Washington University School of Medicine, St. Louis, Missouri
Rhinosinusitis Scores
Baseline and 6 Weeks

Rhinosinusitis Scores
Baseline
6 weeks

Nasal: 2, 1.5
Eye: 1.5, 1.3
Sleep: 2.1, 2.0
Ear: 1.5, 1.7
General: 2.0, 1.9
Prac: 1.8, 1.7
EMO: 1.9, 1.6
Total: 2.0, 1.8

Error bars represent 95% Confidence Limits.
### Correlation Between SF-36 and SNOT-20 Domain Scores

<table>
<thead>
<tr>
<th></th>
<th>PF</th>
<th>RP</th>
<th>BP</th>
<th>GH</th>
<th>VT</th>
<th>SF</th>
<th>RE</th>
<th>MH</th>
</tr>
</thead>
<tbody>
<tr>
<td>Nasal</td>
<td>0.13</td>
<td>0.22</td>
<td>0.10</td>
<td>0.36</td>
<td>0.24</td>
<td>0.16</td>
<td>0.15</td>
<td>0.21</td>
</tr>
<tr>
<td>Eye</td>
<td>0.29</td>
<td>0.25</td>
<td>0.37</td>
<td>0.26</td>
<td>0.37</td>
<td>0.38</td>
<td>0.30</td>
<td>0.31</td>
</tr>
<tr>
<td>Ear</td>
<td>0.10</td>
<td>0.19</td>
<td>0.20</td>
<td>0.16</td>
<td>0.24</td>
<td>0.28</td>
<td>0.04</td>
<td>0.16</td>
</tr>
<tr>
<td>Sleep</td>
<td>0.31</td>
<td>0.28</td>
<td>0.38</td>
<td>0.41</td>
<td>0.51</td>
<td>0.42</td>
<td>0.36</td>
<td>0.43</td>
</tr>
<tr>
<td>General</td>
<td>0.24</td>
<td>0.49</td>
<td>0.52</td>
<td>0.43</td>
<td>0.59</td>
<td>0.59</td>
<td>0.31</td>
<td>0.37</td>
</tr>
<tr>
<td>Practical</td>
<td>0.19</td>
<td>0.18</td>
<td>0.01</td>
<td>0.33</td>
<td>0.25</td>
<td>0.18</td>
<td>0.15</td>
<td>0.22</td>
</tr>
<tr>
<td>Emotional</td>
<td>0.27</td>
<td>0.36</td>
<td>0.28</td>
<td>0.48</td>
<td>0.47</td>
<td>0.44</td>
<td>0.36</td>
<td>0.46</td>
</tr>
<tr>
<td>Total</td>
<td>0.29</td>
<td>0.40</td>
<td>0.38</td>
<td>0.48</td>
<td>0.53</td>
<td>0.49</td>
<td>0.32</td>
<td>0.42</td>
</tr>
</tbody>
</table>

Correlations ≥ 0.40 are shown in green.
Otolaryngology Examples


**Patient Satisfaction with Medical Care**

- Direct measures involve asking patients to evaluate their satisfaction.
- Patients’ judgments of their medical care can be measured reliably and accurately.
- These measurements can be used to compare how patients evaluate different practice styles, administrative arrangements, and treatment modalities.
Patient Visit Rating Questionnaire*

Instructions: Here are some questions about the visit you just made. In terms of your satisfaction, how would you rate each of the following:

The visit overall
The technical skills (thoroughness, carefulness, competence) of the person you saw
The personal manner (courtesy, respect, sensitivity, friendliness) of the person you saw
How long you waited to get an appointment
Convenience of the location of the office
Getting through to the office by phone
Length of time spent waiting at the office
Time spent with the person you saw
Explanation of what was done for you

*Response categories: poor, fair, good, very good, and excellent

Rubin RR et al JAMA 1993;270(7):835-840
Otolaryngology Examples


Translation of Outcome Research

- Quality Improvement
- Safety Initiatives
- Effectiveness Research
Washington State’s Surgical Care and Outcomes Assessment Program (SCOAP)

- Regional approach to surgical quality improvement
- Peer-to-peer collaborative to determine process of care metrics of “perfect” operation
- Track risk-adjusted outcomes specific to a given operation
- Create interventions to correct under performance in both the use of these process measures and outcomes.

Flum Surgery 2012;151:146-52
Development of Risk Calculators

- Data from ACS NSQIP colorectal 2006 to 2007
- 28,863 patients at 182 hospitals
- Logistical prediction models for 30-day morbidity, serious morbidity, and mortality
- 15-variable predictor model
- Risk calculator allows surgeons to preoperatively provide patients personal risk

Creating Healthcare Excellence through Education and Research

CHEER

- Practice-based clinical research in hearing and communication sciences
- Provide necessary infrastructure to accelerate clinical research in order to improve patient outcomes.
- 26 academic and community research sites across 16 states
- David Witsell, PI, Duke Medical Center
**Otology Data Collection**

- Prospective observational study of CHEER infrastructure
- Convenience sample of patients presenting with tinnitus, dizziness, or a combination of these symptoms
- Data collection exercise
  - Demographic
  - Clinical
  - Treatment
  - Health-related quality-of-life surveys

Safety Initiatives

- IOM estimates that >300,000 deaths occur each year as a result of lapses in patient safety
- Focus of research safety
  - Reduction of errors
  - Misuse of medical therapies
  - Oversight in clinical care
  - Illuminate gaps in knowledge and areas for more research
Complications of Primary and Revision FESS for Chronic Rhinosinusitis

- Retrospective cohort analysis
- California and Florida Healthcare Cost and Utilization Project (HCUP) databases
- Between January 2005 to December 2008
- Rates of complications after primary and revision FESS were calculated
- Multivariate model used to determine risk factors for the occurrence of major complications

Getz A. *Laryngoscope*, 2013 accepted for publication
Major complications

- Primary FESS cases - 288/78,944 (0.36%)
- Revision FESS cases – 19/4,151 (0.46%)

Multivariate analysis showed that patients:

- Over 40 years old
- Medicare
- Surgery of the frontal sinus
- Image guidance during surgery

were at higher risk for reported complications
To compare the effectiveness and cost of first-line with second-line antibiotics for acute sinusitis

Retrospective cohort pharmaceutical database

29,102 patients receiving antibiotics

Between July 1996 and June 1997

Main outcome – absence of additional claim for antibiotics in 28 days after initial prescription

Additional outcomes – serious complications, direct charges

Results

- 17 different antibiotics prescribed
- 17,328 first-line antibiotics
- 11,773 second-line antibiotics
Overall success rate 90.4% (95% CI 90.0% to 90.8%)
- First-line antibiotic 90.1%
- Second-line antibiotic 90.8%
- Difference of 0.7% (95% CI 0.01% to 1.40%)
- Not clinically significant

Cost of care
- First-line antibiotic $68.98
- Second-line antibiotic $135.17
- Difference of $66.19 (95% CI $64.95 to $67.43)
- Difference was entirely due to antibiotic cost
TALC - Treatment of Advanced Laryngeal Cancer

- Observational multi-site study assessing chemoradiation vs laryngectomy impacts quality of life, swallowing, and speech on patients with new tumors of the hypopharynx (T2, T3) and cartilage-invading larynx (T3, T4)
- PI – Bevan Yueh, MD, MPH

https://talc.ahc.umn.edu/
• Goals
  • Identify pre-treatment predictors of swallowing function after treatment
  • Explore the relative impact of chemoradiation vs. laryngectomy on swallowing, with secondary analyses of outcomes such as self-reported health status, H&N-specific function, and speech.

• Patients complete a set of 4 questionnaires regarding swallowing function and overall well-being at 3 time points

• Type of treatment is determined by the treating physician and is not altered as a result of participation in this study.
Outcomes Research Future

New questions:

• Aging population
• Growing knowledge base
• Broad range of vested interests
• Increasing sophistication of patients
• Pressure to demonstrate value of health care
Outcomes Research Future

- Advances in Health Information Technology
  - Sophistication of software
  - Real-time research and surveillance systems
  - Greater amounts of data available

- Dissemination & Implementation
Leveraging the collection of patient and practitioner data could be an important way to improve quality and efficacy of health care delivery

Murdoch & Detsky *JAMA* 2013; 309(13):1351-1352
Successful Big Data Projects

- Astronomy - Sloan Digital Sky Survey
- Retail sales
  - Walmart’s expansive number of transactions
  - Amazon.com pioneered using data to make recommendations to customers based on their past buying behavior
- Search engines – Google’s customization of individual searches based on previous web searches.
Scary Big Data Projects

- NSA intercepts 1.7 billion American electronic records and communications a day

- PRISM takes large beams of data and helps the government find discrete, manageable strands of information
Big Data To Transform Medicine

- Greatly expand the capacity to generate new knowledge.
- Clinical trials and observational research prohibitively expensive to answer many important questions
- Potential to create observational evidence base for clinical questions whose answers would not be possible
- Extends generalizability of results
Help with knowledge dissemination – for example, analyze existing electronic health records with published results in the medical literature to guide clinical decisions.

Collaboration between IBM Watson supercomputer and MSKCC to help diagnosis and propose treatment options.

Clinicians may receive messages “Your colleagues treat similar patients with XYZ Formula“
Help translate personalized medicine initiatives into clinical practice by offering opportunities to use analytical capabilities that can integrate systems biology – genomics with EHR data.

Allow for a transformation of health care by delivering information directly to patients, empowering them to play a more active role.

In the future, medical records will reside with the patient.
Surveys are now inexpensive (REDCap with iPads)

- Patients' experiences with illness and the healthcare system are easily tracked
- Tailor approaches and quickly distinguish responders from nonresponders
Follow physiological, psychological, symptom parameters over time in ways not cumbersome yet exact.

Technology can facilitate integration of dynamic decision support model, placing research back into practice in the service of clinical decisions at the bedside.
MyCaJourney.com

- Web-based portal site to support the collection of data from a large number of patients with a variety of solid tumors
- To improve the cancer patient’s experience and empower them to take more responsibility and ownership of their health and survivorship decisions
- Digital cancer patient registry

http://washu.amitechsolutions.com/Pages/default.aspx
MyCaJourney.com

Three modules

• *MyCaNavigator* — Patient-specific survival curves and comparative effectiveness

• *MyCaJournal* — Cancer Patient Report Outcomes and Survivorship Experience (CaPROSE); digital cancer patient experience

• *MyCaCommunity* — social network blog for “patients like me”
MyCaJournal

http://128.252.51.211:91/mrIWeb/mrIWeb.dll?I.Project=MYCAJOURNAL&i.test=1
As quantified in one study, only 14 percent of findings from research filter into practice, and for those that do, it takes an average of 17 years.

Dissemination & Implementation

- Tremendous knowledge gap between what we know can optimize health and healthcare delivery and what actually gets implemented.

- The science of D&I seeks to address this gap by understanding how to create, evaluate, report, disseminate, and integrate evidence-based strategies to improve health and prevent disease in clinical and public health practice settings.

Is Outcomes Research a Fad?

No!

Outcomes Research is here to stay

- Concerns and focus about health care
  - costs
  - quality
  - reform
- Information about comparative effectiveness research
- Value of various clinical strategies
- Utility of innovative approaches
Outcomes Research Conclusions

- Evolved from Geographic Variation and Appropriateness Studies
- Utilizes new methodologies for the evaluation of the effects of diverse therapies on patient outcome
- Introduces new areas of study not traditionally included in the evaluation of medical care
Outcomes research can illuminate the results of care, promote improvements, facilitate feedback, and collect data

Seeks to make visible what was formerly obscure regarding patterns of care and effect on patients

Seeks gaps in quality of care and supports constructive remedies

Voice of the patients and focuses on patient experience

Krumholz Circulation 2008; 118:309-318
Outcomes Research can provide the scholarship that can support efforts to improve medical practices and healthcare policies.