Abstract—Swallowing problems (dysphagia) can occur at any age but are most prevalent in elderly individuals and are a growing healthcare concern as the geriatric population expands. Without effective diagnosis and treatment, dysphagia may lead to serious medical conditions such as pneumonia, dehydration, and malnutrition. Experts in the field of dysphagia met on August 21, 2001, in Rockville, Maryland, to respond to this heightened healthcare need and to determine the course of dysphagia research. Presentations at the meeting included epidemiological data, geriatric-specific issues, diagnostic techniques, risk factors for pneumonia, and recent relevant trials. The experts identified outstanding issues in dysphagia research, such as study design, population selection, and the standardization of diagnostic and treatment protocols. They designed a clinical trial that represents what they deem is one of the greatest needs in dysphagia research, providing a critical springboard for research endeavors with far-reaching implications.

Key words: clinical trials, deglutition disorder, group meetings, therapeutics.

INTRODUCTION

Swallowing problems (dysphagia) can occur at any age but are most prevalent in elderly individuals and are a growing healthcare concern as the geriatric population expands[1,2]. Without effective diagnosis and treatment, dysphagia can lead to dehydration, malnutrition, reduced rehabilitative potential after injury or illness, pulmonary complications related to chronic aspiration, and associated reductions in quality of life (QOL). As the population ages, an estimated 16,500,000 individuals will require care for dysphagia by the year 2010 [3].

A meeting of experts in the field of dysphagia was convened on August 21, 2001, in Rockville, Maryland, in response to this heightened healthcare need and to determine the course of dysphagia research in the wake of a 1999 evidence report, entitled “Diagnosis and Treatment of Swallowing Disorders (Dysphagia) in Acute-Care Stroke Patients.” The report was provided by the Emergency Care Research Institute (ECRI), an Evidence-Based Practice Center (EPC), engaged by the Agency for Healthcare Research and Quality (AHRQ). Martin Erlichman, MS, Senior Health Science Analyst in the Center for Practice and Technology Assessment at AHRQ in Rockville, Maryland; JoAnne Robbins, PhD, Associate Director of Research Geriatric Research, Education and Clinical Center (GRECC) at the William S. Middleton Memorial Veterans Hospital.
Veterans Memorial Hospital and Associate Professor, Department of Medicine, at the University of Wisconsin; and Susan Langmore, PhD, Associate Clinical Professor Department of Neurology at the University of California, invited various experts in the field of dysphagia, including speech pathologists, geriatricians, pulmonologists, otolaryngologists, statisticians, epidemiologists, healthcare managers, and representatives from funding agencies for this one-day meeting to shape the future of dysphagia research.

Background

ECRI Report

In 1999, AHRQ published a systematic review of the clinical evidence regarding the “Diagnosis and Treatment of Swallowing Disorders (Dysphagia) in Acute-Care Stroke Patients” completed by ECRI. David Doggett, PhD, Senior Research Analyst at ECRI and contributing researcher on the EPC report, provided an overview and summary of the 1999 dysphagia study. He summarized the report, including research specific to the prevention of aspiration pneumonia and to the cost effectiveness of dysphagia evaluation techniques for stroke patients. Recommendations from the ECRI report pointed toward the need for more quality information in all areas of dysphagia research. Dr. Doggett noted the need for further research on (1) diagnosis and treatment; (2) instrumental and non-instrumental dysphagia exams; (3) controlled-therapy options for swallowing disorders; (4) methodological issues, including the necessity of having a homogenous population and standardization; and (5) benefit-harm studies of tube feeding for dysphagic individuals.

Epidemiology

William Baine, MD, Senior Medical Advisor in AHRQ’s Center for Outcomes and Effectiveness Research, presented the results of a review of data from the Centers for Medicare and Medicaid Services (CMS) Medicare Provider Analysis and Review (MEDPAR) files on hospitalization for elderly aspiration pneumonia and general pneumonia from 1991 to 1998, concentrating on principal diagnosis. Dr. Baine reported that there was a 93.5 percent increase in hospitalization because of aspiration pneumonia, while other types of pneumonia experienced decreases. Aspiration pneumonia has the highest case fatality rate of all pneumonia classifications and is one of the most costly. Undiagnosed and untreated dysphagia has serious morbidity and mortality consequences.

Swallowing and Older Adults

JoAnne Robbins, PhD, Associate Professor of Medicine, Sections of Gastroenterology and Geriatrics at the University of Wisconsin, and Associate Director for Research of the GRECC at the William S. Middleton Veterans Memorial Hospital, provided participants with an overview of dysphagia, specifically as it pertains to the elderly population. Dr. Robbins highlighted the growth in numbers of the geriatric population and the clinical complexity of the oropharyngeal swallow. The condition of sarcopenia, an age-related diminishment of muscle mass and strength, was presented as a major source of reduced reserve capacity characteristic of presbyphagia [4–7]. Dr. Robbins expressed that the key to the treatment and management of dysphagia and/or aspiration is identifying the etiology, that is, the underlying physiological or anatomical reason.

Diagnostic Techniques

Susan Langmore, PhD, Associate Clinical Professor of Neurology at the University of California, San Francisco, provided a comparison of assessment techniques for dysphagia and an overview of risk factors for pneumonia. Dr. Langmore compared the assessment techniques of fiber-optic endoscopic evaluation of swallowing (FEES) and videofluoroscopic swallowing study (VSS). While both the FEES and VSS assessment tools have the same goal (determination of the underlying pathophysiology of dysphagia and “on-line” imaging results of treatment), each tool has advantages and disadvantages. FEES is more portable than VSS and requires no radiation exposure, and the patient can eat real food during the examination. VSS provides an image of the entire duration of the swallow and allows for viewing of the complete oropharyngeal aerodigestive complex.

Risk Factors for Aspiration Pneumonia

Dr. Langmore reviewed research on the risk factors for pneumonia to elucidate the relationship between pneumonia and dysphagia. Aspiration pneumonia was presented as a three-phase process in which the patient (1) colonizes pathogenic bacteria in the oropharynx, (2) aspirates the bacteria into the airway, and (3) is unable to clear the material and subsequently develops a bacterial infection in the respiratory system. In addition to dysphagia,
other risk factors associated with aspiration pneumonia include dependence on others for feeding, multiple medical conditions, smoking, tube feeding, and dependence for oral care [8]. Dr. Langmore expressed a great need for prospective studies in this area.

Recent Relevant Trials

Dr. Langmore reviewed three trials comparing diagnostic techniques, including studies by Logemann et al., Smithard et al., and Aviv et al [9–11]. All studies, while praised for their attempts to answer difficult questions, were cited as being underpowered. Other methodologic issues included concern about outcome measures, randomization methods, and variable controls.

Dr. Robbins presented information about Protocol 201, an ongoing National Institutes of Health (NIH)-funded, multisite clinical trial for dysphagia treatment [12]. The trial is a randomized study of two commonly used interventions for liquid aspiration in patients with dementia with or without Parkinson’s disease to determine the effects of either chin-down posture or thickened liquids on frequency of aspiration (short-term) and pneumonia (long-term). Specific challenges that Dr. Robbins noted included clinician dedication, standardization of materials and protocols, continuous recruitment of new sites, and patient accrual.

RESULTS

Outstanding Issues in Dysphagia Research

After the introductory presentations provided some perspective on the issues of dysphagia research, participants spent the remainder of the meeting discussing outstanding issues in swallowing research and proposing future directions for clinical trials. Specific issues follow.

Building an Evidence Database

Participants generally agreed on the need for more research in all areas of dysphagia to provide quality evidence from published clinical trials. Participants cited a need for more definitive information on the epidemiology of dysphagia, the physiology of swallowing, and the efficacy of assessment and treatment techniques. Some participants expressed concern that no concrete evidence has been found to show that interventions are more effective than no interventions, and stressed that anecdotal experience within clinical settings cannot substitute for evidence obtained from randomized clinical trials.

Securing Funding

Given the need for more definitive research, participants expressed frustration with the lack of funding available to obtain the evidence on which to base practice. Funding agencies represented at the meeting included the National Institute on Deafness and Other Communication Disorders (NIDCD), the National Institute on Aging (NIA), the National Institute of Child Health and Human Development (NICHD), and the Department of Veterans Affairs (VA). Representatives from these agencies recommended that the experts prioritize the outstanding issues to provide funding agencies with the most important concerns to be addressed by dysphagia research today.

Study Design

Because of the difficulty in recruiting and maintaining a large enough sample of homogenous subjects, participants supported the idea of running multi-institutional clinical studies to obtain enough data to be statistically significant with adequate power and confidence. Possible research settings included acute care, subacute care, extended care, assisted living, and nursing homes. Participants also stressed the need for trials to be truly randomized.

Population Selection

Participants discussed the need to enroll a homogenous group of subjects into future clinical trials, but noted the difficulty with finding and maintaining such a group among the older population. Many agreed that the most comprehensive at-risk group is the frail elderly, not only stroke victims or persons with degenerative neurologic disease. In fact, the latter two groups are subgroups of the larger frail elderly population [13]. Thus by definition, frail elders are a heterogeneous group in terms of disease and condition, which makes the issue of population very complex for dysphagia research. The challenge is to address the needs of the largest numbers with the best research design to ensure meaningful interpretation and generalization of results.

Standardization

A significant recurring theme at the meeting was a need for standardization in both research and clinical
settings. Participants noted from experience that many clinicians are not knowledgeable about the latest swallowing treatments and that there is need for more standardization of training and protocols.

Outcome Measures
Participants discussed a need to find patient-centered outcome measures such as QOL, in addition to more traditional clinician-driven outcomes. Onset of pneumonia is a challenging outcome for evaluating interventions, because the diagnosis is sometimes problematic, particularly in geriatric patients residing in extended-care facilities, and the time frame during which subjects develop pneumonia may vary. Suggestions for other potential outcome measures were malnutrition, dehydration, and return to oral intake from NPO (nil per oral) status.

Comparing Assessment Tools
Participants agreed that an appropriate starting point for a large clinical trial is to compare the most common assessment tools such as the bedside swallowing examination (BSE), VSS, and FEES. They stressed the importance of not only comparing instrumental exams with each other but also with noninstrumental exams as well. Discussion ensued regarding the ethics of providing BSE only. The majority of participants agreed that BSE alone as one of the conditions is justified in light of its frequency of use in various clinical settings and preliminary efficacy data.

Design of Future Research
Given the many outstanding issues in dysphagia research, the experts agreed that future research needs to address two basic components: (1) epidemiological/longitudinal studies of dysphagia and swallowing disorders and (2) clinical trials to determine the efficacy of assessment and treatment techniques.

Participants stressed the need for epidemiological studies to determine the risk factors for dysphagia and aspiration pneumonia in the aging population. Possible options for these studies included adding dysphagia questions onto one of the several on-going national longitudinal studies; conducting the study within the VA Health Care System, which offers a distinct database; and completing a pilot study that focuses on frail elderly individuals to understand the overall aging process. While these ideas and options offer great hope for data collection, they pose many challenges, including feasibility and population restrictions.

The group overwhelmingly stressed the necessity of conducting a clinical trial in the next phase of dysphagia research, and many suggested the need for multiple clinical trials. Participants agreed that the design of at least one of the possible studies should involve the randomization of assessment and treatment procedures. However, they noted that designing such a trial would be a challenge in light of the routine clinical practice of providing treatment based on the assessment procedure. After much deliberation and discussion, the participants outlined the blueprint for at least one clinical trial that will focus on “clinical care packages”:

- **Study Design:** Nationwide, multisite, randomized clinical trial. Assessment (noninstrumental and instrumental) and intervention tools would be grouped together into clinical care packages. Subjects would be randomized into one of the packages and their health monitored for a period of time.
- **Population:** The frail elderly, although a heterogeneous group, was supported as the most appropriate subject population because of need. Stroke patients, a subgroup of the broader frail population, also were identified as a potential independent population.
- **Study Questions:** Does the type of dysphagia care package impact outcome? Which care package results in the fastest improvement in health?
- **Primary Outcome Measure:** Pneumonia, nutritional status hydration status, return to oral intake from NPO status, and dysphagia-related QOL were discussed as possibilities.

DISCUSSION
While simply outlined, the proposed clinical trial represents what experts in dysphagia from across the United States deem to be one of the greatest needs in dysphagia research today. The meeting sponsored by the AHRQ and hosted by Martin Erlichman, MS; JoAnne Robbins, PhD; and Susan Langmore, PhD, provided a critical springboard to important research endeavors with far-reaching implications.
REFERENCES


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