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Ethical Issues in Clinical Trials

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New York Region, Protocol 201

The Communication Sciences and Disorders Clinical Trials Research Group (CSDRG) Protocol 201, Randomized Study of Two Interventions For Aspiration, has been active since 1998. This randomized, multi-center study of treatment efficacy in dysphagia is the first of its kind in the field of speech-language pathology. The protocol is described in this issue of the Division 13 Newsletter (See 201: A NIDCD Funded Multi-Site Clinical Trial in Swallowing–Progress Report).

After several years of participation in the study, the clinicians involved have encountered numerous challenges. A major issue has been enrolling sites into the project, and once enrolled, motivating those clinicians to maintain the study as a priority in their daily routines. Because Protocol 201 is a clinical trial, subject accrual depends largely on the efforts of the “clinician on the street”, those involved in the care of individuals with dysphagia in acute, subacute, rehabilitation, extended care, and home care settings. Another concern has been how to encourage clinicians to continue the study when the needs of the Protocol may appear at odds with routine clinical practice. Because Protocol 201 is a clinical trial, subject accrual depends largely on the efforts of the “clinician on the street”, those involved in the care of individuals with dysphagia in acute, subacute, rehabilitation, extended care, and home care settings. Another concern has been how to encourage clinicians to continue the study when the needs of the Protocol may appear at odds with routine clinical practice. The CSDRG and the participants in Protocol 201 are constantly weighing all issues, and searching for ways to best encourage dysphagia clinicians to “buy into” randomized clinical trials in general, and Protocol 201 specifically.

The first and perhaps most timely issue for all clinicians interested in clinical trials should be the need for evidence based practice in our field. During a time when third party payers are challenging and limiting many areas of payment for medical and ancillary treatments, evidence about the effectiveness of our treatments is crucial. Despite an increasing number of single subject and anecdotal reports, evidence based practice in dysphagia is limited. For example, how consistently do clinicians use treatment strategies, which have been assessed during a competent instrumental evaluation and found to be effective for that individual patient? This process represents evidence-based practice on the individual patient level. Logemann (2001) notes that evidence based practice involves integrating current best practice, clinical expertise, pathophysiological knowledge, and patient preferences into patient care. Clinicians must regularly examine recent literature in order to select information that appears relevant to their patients, and then use clinical expertise to select techniques that will assist their individual patients. Randomized clinical trials are one area from which clinicians can obtain relevant information. Participation in a clinical trial is perhaps one of the best ways to learn first hand the best way to apply evidence-based practice in daily care.

Clinical trials also provide a venue for ongoing education. The study chair and principal investigators of Protocol 201 host continuing education conference calls. Protocol participants from around the country participate in these calls. Topics are usually selected by participants and involve issues in the care of dysphagic patients. Conference calls have ranged from the use of unsubstantiated evaluation and therapy techniques, to how to accurately assess aspiration in dysphagic patients.

Clinical trials also allow participants to examine ethical issues in practice. For example, the ASHA Code of Ethics states, under Principles of Ethics I, that “individuals should fully inform the persons they serve of the nature and possible effects of services rendered
and products dispensed” (ASHA, 1994). This principle is addressed in the practice of informed consent for any patient identified as a possible subject in Protocol 201. Another rule of ethics states that, “individuals shall use every resource...to insure that high-quality service is provided” (ASHA).

The following case study presents an excellent example of what may occur when the needs of an individual patient outweigh the guidelines of a clinical trial. In this example, while the clinicians used evidence-based practice to provide services to the individual patient, they believed that ethically his continuation in the clinical trial could not be recommended.

**Case Study**

L.M. is an 83-year old Hispanic male who was admitted to Franklin Center for Rehabilitation and Nursing (FCRN) on November 22, 2000. He was diagnosed with Parkinson’s disease, without dementia, 6 years ago. His primary language is Spanish and he was a tailor by trade for many years. L.M. presented with multiple medical problems including major depression, recurrent urinary tract infections, congestive heart failure, and esophagitis. Prior to entering the long-term facility, he was hospitalized from October 9 through November 22, 2000. His hospital course was extremely complicated and prognosis guarded. L.M. was ventilator dependent for a prolonged period of time following an episode of left lower lobe pneumonia. Aspiration secondary to dysphagia was the suspected etiology for the pneumonia. Subsequently, a percutaneous endoscopic gastrostomy tube was placed on November 17, 2000 for nutrition and hydration.

On admission to FCRN, L.M. presented with aphasis. He attempted to communicate via whispering with obvious difficulties with intelligibility. He was not-ambulatory and received only non-oral feedings. An initial speech-language assessment revealed intact cognition and decision-making ability. L.M. expressed appropriate concerns and questions regarding his care in the long-term facility. He was concerned about his NPO status and often requested he at least be able to drink some water.

The speech-language pathologist at FCRN, also a Nursing Home clinician participating in Protocol 201, completed a clinical dysphagia evaluation on November 30, 2000. Results revealed oropharyngeal dysphagia with marked deficits in oral functioning and laryngeal elevation. He was unable to produce a volitional cough. A severely diminished reflexive cough in response to thin liquid trials was noted. The recommendations were for continued non-oral feedings and L.M. subsequently began indirect dysphagia and voice treatment programs. A physical therapy program was initiated as well. He remained motivated and cooperative throughout the course of treatment, but fatigued easily. Participation was inconsistent at times due to his overall medical status. Progress was slow but his motivation persisted.

L.M. was identified as an eligible patient for Protocol 201 in late December. He was approached for consent by his treating speech-language pathologist and the clinical trial speech-language pathologist. Information re: the study was provided, and questions were answered, in his dominant language by the clinical trial speech-language pathologist. Informed consent was obtained. This followed an explanation of the risks and benefits of each part of the protocol, including the significance of the presence or absence of aspiration, possible treatment assignments, and the potential for oral intake. L.M.’s questions ranged from questioning the risks of developing pneumonia again, the chances of ever eating “normally” again, and the potential for removal of the PEG tube. He decided to participate in the study because it was “good to gain information both for myself and other Parkinson’s patients.” He also remained highly motivated to begin oral intake of liquids.

The patient’s daughter was also contacted by the clinical trial speech-language pathologist several times by phone as she did not live in the area. She was provided with the details of the study and was excited about the possibility of her father eating and drinking by mouth again. Extensive counseling was provided as informed consent was also obtained from the patient’s daughter. They were assured that the patient could withdraw from the study at any time if they were in disagreement with the recommendations. The patient’s daughter was in agreement with her father’s decision to participate in the study.

A videofluoroscopic examination of swallowing was done on January 2, 2001 at New York Hospital Medical Center of Queens in Flushing, New York. L.M. presented with severe oropharyngeal dysphagia. There was poor to absent laryngeal elevation and airway closure. Poor pharyngeal propulsion resulted in significant residue and aspiration after the swallow, of all consistencies. L.M. consistently aspirated 40-50% of all boluses presented. His cough response was diminished, weak, and non-productive. Implementation of both Protocol 201 interventions (nectar and honey thickened liquids, chin tuck for thin liquids) and clinical maneuvers (breath hold, effortful swallow) were ineffective in eliminating aspiration. Therefore, L.M. met the criteria for inclusion in Part II of the study, that is, aspiration on all study interventions.
Following the protocol guidelines, L.M. was randomized into Part II of the clinical trial. His assigned intervention for the anticipated 3 months was use of chin tuck posture with thin liquids. However, this was not implemented immediately. A review of the videofluorography by the clinical trial speech-language pathologist and the Protocol 201 Central Laboratory in Madison, WI, revealed that the degree of aspiration was extremely severe, as noted, 40-50% of the bolus. The degree of aspiration was one of the worst seen in Protocol participants. Therefore, although informed consent had been obtained for this patient, the clinical trial speech-language pathologist and Regional Project Investigators for New York requested that his case be discussed on the next scheduled Protocol Conference Call, before he began the assigned intervention.

The study chair, principal investigator, regional project investigators from all regions, clinical trial speech-language pathologist and nursing home clinician discussed the case. There were many contraindications to oral feedings of any sort: the severity of the dysphagia, the significant amounts of aspiration, absence of a productive cough, pneumonia history, patient’s limited mobility, current feeding method, and the lack of response to trial interventions. It was decided that although the patient had agreed to continue the Protocol interventions and begin oral intake of liquids, and was indeed anxious to begin some sort of oral intake, the speech-language pathologists involved could not recommend that he do so.

Both patient and daughter were counseled at length regarding these results, again, by the clinical trial speech-language pathologist, in Spanish. Emphasis was placed on differentiating between the recommendations made for study purposes and those made for non-study purposes. Although, for study purposes, L.M. could initiate thin liquids, under regular circumstances the treating speech-language pathologist would not recommend that he initiate any oral intake. L.M. demonstrated understanding that food and liquid entered his airway and lungs, in large amounts, and that nutrition and hydration could continue solely through tube feedings. However, he frequently asked for a prognosis as to whether, and when, he would be able to eat and drink by mouth again. The patient’s daughter was primarily concerned about a recurrent pneumonia. She described the “crisis” L.M. had been through with the previous pneumonia episode, and she asked if the risk of a repetition of that situation was high.

After much discussion with the patient and his daughter, both requested that the clinicians make the best decision. He stated several times “you are the professionals, you tell me what to do.” They both trusted and deferred to the expertise and professionalism of the speech-language pathologists. The recommendations were therefore made to continue nonoral feedings, dysphagia therapy, and re-evaluation of swallow function as specified by the treating clinician. L.M. expressed his disappointment; however, he elected to withdraw from the study and stated, “Then I’ll stay just as I am,” rather than go against recommendations. Following team discussions between all clinicians involved, it was decided that it would be in this patient’s best interests to withdraw from Protocol 201. Although L.M. was a good candidate for participation, the risks certainly outweighed the benefits of remaining in the trial, and would have been in conflict with good clinical practice.

L.M. withdrew from the clinical trial, but continued with dysphagia therapy. Indirect therapy was provided but slowly progressed to direct intervention. Therapeutic techniques identified during the previous videofluorographic study included swallow maneuvers, vocal adduction exercises, postural changes, modifications of bolus volume, and compensatory techniques. L.M. demonstrated consistent improvement in phonation and oropharyngeal swallow function. On April 25, 2001, the patient returned to NYHMCQ for an instrumental re-evaluation via videofluorography. Significant improvement in swallow physiology was evident compared to the exam done nearly 5 months earlier. The amount of aspiration decreased to 5-10% of thin liquids only, with penetration of nectar thick liquids. Cough remained ineffective. A limited oral diet of purees was therefore initiated, with honey-thickened liquids added to L.M.’s diet in therapy only. Currently, he receives a full puree diet and all hydration via mouth, with thickened liquids. He continues to use postural modifications, compensatory techniques, and swallow maneuvers. As of July 17, 2001, L.M.’s tube feedings have been discontinued. He has a significantly improved voice and now ambulates with a walker. Use of a wheelchair has been discontinued and he is being assessed for ability to use a cane. For the first time since his admission to Franklin Center for Rehabilitation and Nursing, L.M. and the treating speech-language pathologist are discussing the possibility of returning home to his wife.

Clinical trials such as Protocol 201 will serve the field of speech-language pathology well in many ways, including in the understanding of evidence-based practice and the application of ethical concerns involving our patients.
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References
