DISABILITY AND REHABILITATION RESEARCH COALITION
1501 M STREET, N.W. SUITE 700 WASHINGTON, D.C. 20005

March 15, 2012

Joe V. Selby, M.D., M.P.H.
Patient-Centered Outcomes Research Institute (PCORI)
1701 Pennsylvania Ave. NW
Suite 300
Washington, DC 20006

RE: Comparative Effectiveness Research and PCORI's National Research Priorities

Dear Dr. Selby:

The Disability and Rehabilitation Research Coalition (DRRC) is a coalition of national non-profit organizations committed to improving the science of rehabilitation and disability. The DRRC seeks to maximize the return on the federal investment in such research with the goal of improving the ability of Americans with disabilities to contribute to the health and economic well-being of our nation.

Three years ago, we worked extensively with Michael Marge at the Department of Health and Human Services Office on Disability on a submission to the HHS Advisory committee regarding Comparative Effectiveness Research (CER) for people with disabilities. We engaged rehabilitation stakeholders in order to develop a consensus statement. Regrettably, the vast majority of these recommendations from three years ago are just as salient today. We, therefore, are forwarding the final document and asking you to review it at your convenience.

We appreciate your consideration of this document and hope it informs your revisions of the National Priorities for Research and Research Agenda to include comparative effectiveness research for people with disabilities. For more information, please do not hesitate to contact Peter Thomas at Peter.Thomas@ppsv.com or call: 202-466-6550.

Sincerely,

Peter Thomas
Counsel, DRRC

Bobby Silverstein
Counsel, DRRC

PROPOSAL FOR A DISABILITY COMPARATIVE EFFECTIVENESS RESEARCH PROGRAM

Michael Marge, Ed.D.*

Member, HHS Federal Coordinating Council on Comparative Effectiveness Research

U.S. Department of Health and Human Services

June 11, 2009

Disability and Health Intervention Research Organizational Framework

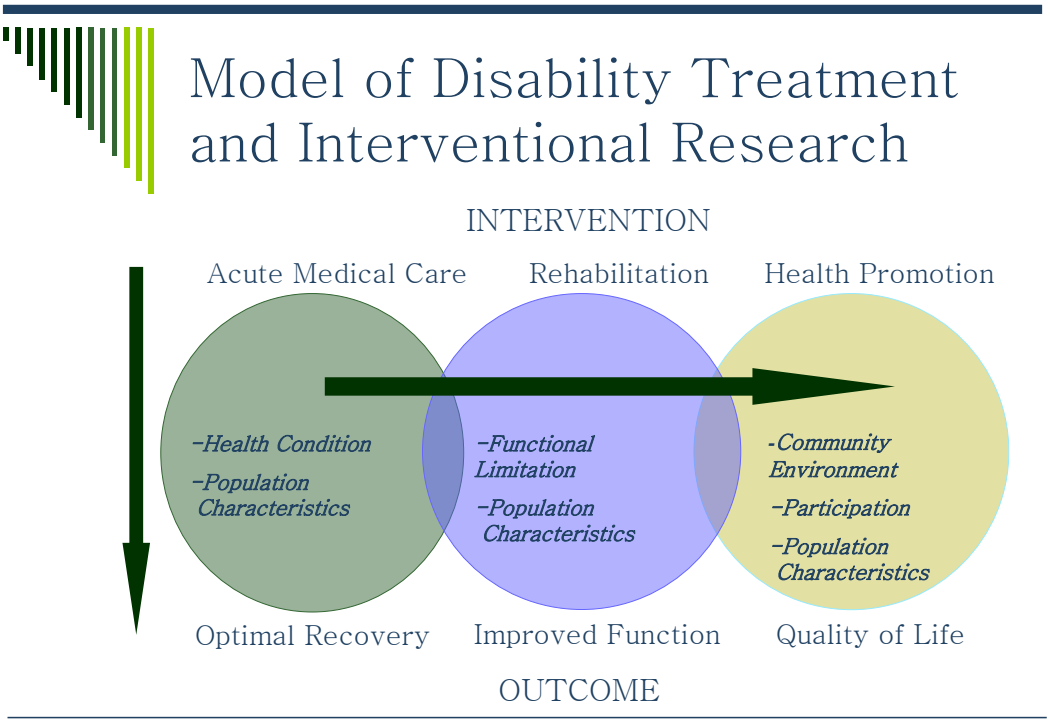
The ideal outcome for a comprehensive healthcare system is good health-related quality of life (HRQOL) for all participants. Medical and Public Health research over the past 100 years has led to enormous gains in health-related quality of life around the world.

For the 54 million Americans with disability, the gold standard of HRQOL is participation in community life. The World Health Organization's International Classification of Function, Disability, and Health (ICF) specifies this outcome as a result of an interaction between the individual with a disability and his or her environment. Three practice disciplines with scientific foundations address the dimensions of the ICF, including medicine, rehabilitation, and health promotion. Each addresses the individual and the environment at different levels.

The figure below depicts a conceptual continuum of intervention research in disability and rehabilitation that encompasses interventions at both the individual and environmental levels. It begins with acute medical services and shows linkages through rehabilitation to health promotion and quality of life. Notice the areas of overlap where research may address questions that encompass both Acute Medical Care and Rehabilitation Treatments and Therapies. The model may be used to organize the development of comparative effectiveness research in disability and rehabilitation that leads to improved health-related quality of life.

*with the assistance of Ruth Brannon, NIDRR/Dept. of Education; Arthur Sherwood, Ph.D., Dept. of Education; Michael Weinrich, MD, NCMRR/NIH; Louis Quatrano, Ph.D., NCMRR/NIH; Ramona Hicks, Ph.D., NINDS/NIH; Robert Jaeger, Ph.D., VA; Dorothy Shannon, Ph.D., CMS/HHS; Gloria Krahn, Ph.D., CDC/HHS; Scott Grosse,

Ph.D., CDC/HHS; Glen White, Ph.D., University of Kansas; Michael Fox, Ph.D., University of Kansas; David Stapleton, Ph.D., Mathematica; Thomas Seekins, Ph.D., University of Montana; Craig Ravesloot, Ph.D., University of Montana; Patricia Sullivan, Ph.D., Creighton University Medical School; Nancy Roizen, MD, Cleveland Clinic; Peter Thomas, J.D., Power, Pyles, Sutter and Verveille; Lisa Iezzoni, M.D.,M.Sc., Harvard University Medical School; Margaret Turk, MD, SUNY Upstate Medical University; Roberta Carlin, J.D., American Association on Health and Disability; Anthony Cahill, Ph.D., University of New Mexico; David Gray, Ph.D., Washington University; Charles Drum, Ph.D., Oregon Health Sciences University; ; Elena Andresen, Ph.D., University of Florida.



Reading the diagram from left to right, medical research examines treatment of acute conditions. The outcome of these experimental medical procedures is a function of patient characteristics such as existing health condition and population characteristics (e.g., age, gender, race). When medical interventions are 100% effective, individuals are returned to full health and pre-intervention levels of participation and quality of life. Of course, not all medical procedures lead to 100% recovery, or cure.

The value of medical interventions is determined by the interaction of effect by cost. For example, the cost-effectiveness of treating a broken leg is not debated as these treatments are

nearly 100% effective and relatively inexpensive. On the other hand, the cost of some late stage cancer treatment is debated. These debates are often framed in terms of quality adjusted life years, a metric of life expectancy by expected quality. However, use of this metric for medical procedures is flawed. Future quality of life following an acute medical procedure is related to a variety of factors beyond the intervention itself. These factors are addressed in the next two circles of the framework.

When medical outcomes do not lead to full recovery, rehabilitation interventions may be used to improve functional outcome for accessing the community. These interventions range from increasing the function of particular body structures (e.g., vocal cords via speech therapy) through the use of assistive technology like wheelchairs. Again, the outcome from rehabilitation procedures is considered a function of patient characteristics (i.e. impairment type and age). Disciplines involved in rehabilitation include medicine, psychology, physical and occupational therapy, social work, engineering, and speech.

When rehabilitation outcomes are100% effective the patient has regained full function to participate in the community. Using the best available rehabilitation technology, full function could be a common rehabilitation outcome. However, like medical interventions, rehabilitation outcomes have a range of cost-effectiveness. Often, the cost of providing assistive technology for accessing the environment is prohibitive. This can be viewed as either a technological or an environmental problem. Accessible environments require less expensive rehabilitation equipment for participation. For example, the Ibot, an expensive wheelchair that can climb stairs, is unnecessary in ramp and lift equipped environments. Likewise, adequately structured work environments require less job coaching for people with intellectual disabilities.

The last block of the framework picks up where the previous two blocks end. Ultimately, the translation of medical and rehabilitative procedures into health-related quality of life depends on the behavioral choices available to the individual. These choices occur at the intersection of the individual in interaction with his or her environment; the richer and more accessible the environment, the greater the, opportunity to participate in community life. Traditionally, health promotion interventions aim to reduce health risk factors and increase health protective factors to reduce morbidity and mortality. When considering health-related quality of life outcomes for people with functional loss, health-related quality of life also must include features of the

environment that facilitate or impede participation. From this perspective, the absence of participation opportunities is a health risk factor.

The outcomes of experimental health promotion interventions to increase health-related quality of life for people with functional loss result from the interaction of personal and environmental characteristics, an interaction effect exemplified by the International Classification of Functioning, Disability, and Health (WHO, 2001). More importantly, in a comprehensive healthcare system, health promotion engages medical and rehabilitation patients to maximize functional outcome via personal and environmental interventions. For example, the symptom presentation from many chronic illnesses interrupts participation through both functional limitations due to untreatable symptoms and from the demands of rigorous medical and self-management procedures. For these individuals, health promotion supports self-management of the disease process as well as participation in community life. Health-related quality of life and other factors associated with HRQOL also may be addressed by interventions that focus at the systems level, as well as the individual level. For example, a health promotion intervention might target the establishment of programs or polices that increase the accessibility of the built environment (such as trails or public places) or address social environments (such as modifying negative attitudes).

The Need for Comparative Effectiveness Research that is Patient-Centered

Three Categories of Recommended Disability Comparative Effectiveness Research:

Category I: Rehabilitation Therapies and Treatments

Rehabilitation is a concept that has at its core, the promotion of the highest health, physical, psychologic, cognitive, vocational, educational, avocational, and social function possible consistent with the physiologic or anatomic impairment or environmental barriers for those with disabilities. There is a dynamic interplay among the many components of rehabilitation (e.g.

medical and pharmacologic interventions, nursing, speech-language/occupational and physical therapies, education, orthotics and prosthetics, counseling, social service support, durable medical equipment, spiritual support, and vocational counseling), as the individual's condition stabilizes and skills develop. Realistic and practical goals are established, and usually change over the course of the disability evolution and the individual's lifetime. There may be a variety of sites of service and components of rehabilitation, based on medical and functional needs, which also change with time.

Rehabilitation and disability research has expanded the measurement instruments used to identify impairment and function levels (e.g. classification of spinal cord injury [ASIA], NIH Stroke Scale); burden of care (e.g. functional independence measure [FIM™]); barriers to function, social interaction, or work involvement; and outcome measurements in the context of health, function, and societal participation. It has also become clear that people with disabilities assess their health, quality of life, and satisfaction within a different context than do those without disabilities (Drum et al, 2008; Palsbo, 2007).

Rehabilitation intervention research typically examines either very broad (e.g. timing of rehabilitation initiation, care settings, organizational milieus, the full array of rehabilitation care) or very specific intervention strategies (e.g. constraint induced therapy, intrathecal baclofen pump medication delivery, body weight support therapy, use of floor reaction ankle foot orthoses, neuromuscular electrical stimulation). It is difficult to assess discreet interventions, their timing or dosing, when they are delivered within a multidisciplinary, comprehensive program. However, efforts are now being made to examine individual services within the context of the full range of rehabilitation programs (LivnehH, 1989; DejongG et al, 2004). Also medical informatics offers a means to assess the benefit of individual services within the context of a multiple service program through the analysis of very large databases.

An area of rehabilitation intervention that is often overlooked is the dynamic rehabilitation medical demands in early or acute phases of disability onset or diagnosis, or in progressive or chronic conditions. These interventions focus on optimizing physiologic function (e.g. treatment of evolving agitation after brain injury, management of changing spasticity and tone with cerebral palsy) and addressing ongoing co-morbidities (e.g. hypertension and diabetes management following stroke) and medical issues (e.g. infection, neurogenic bladder, nutritional

management with dysphagia) while supporting participation in the function-restorative rehabilitation process. People with lifelong disabilities should anticipate aging changes and susceptibility to secondary conditions, that may require acute and ongoing rehabilitation interventions. There are also transitions of care that require facilitation through the rehabilitation process. Those with acute onset disabilities or adults with childhood onset disabilities require support, education, and empowerment to be able to effectively manage their care or to determine someone with capacity to partner in achieving ongoing care.

Rehabilitation and disability science has established a sufficient core of knowledge during the past two decades, such that comparative effective research is now warranted.

Examples of suggested areas of CER are found in Appendix A.

Category II: Environmental Interventions: Assistive Devices and Technologies

Examination of the effectiveness of environmental modifications or interventions is needed to improve the health, physical function and participation of people with disabilities. Categories of environmental interventions include but are not limited to provision of assistive technologies, personal assistants, home modifications and community access.

The narrow focus on restorative rather than compensatory care is a well documented problem for people with chronic diseases and disabilities despite a growing consensus that the primary outcome measure of rehabilitation effectiveness is community participation. For millions of Americans with disabilities, assistive technologies (AT) are key environmental factors in helping them to return to or remain in their homes and communities. AT has been defined as "any item, piece of equipment, or product system whether acquired commercially or off the shelf, modified, or customized that is used to increase, maintain, or improve functional capabilities of people with disabilities" (Technology-Related Assistance for Individuals with Disabilities Act, 1988). Studies have found that provision of AT can enhance performance of self care activities, independent communication, work skills, mobility and community participation. However, comparative analyses of the differential effectiveness are limited since few currently used measurements assess AT use even for prevention of health conditions (e.g. skin ulcers, shoulder injuries, obesity, urinary tract infections, scoliosis).

Studies of different approaches to the provision of AT that enhances performance (e.g. advanced communication devices, lightweight manual wheelchairs, multifunction power wheelchairs, pressure sensitive seating cushions and advanced prosthetics) could provide guidance on the most effective approach to enabling people with significant disabilities to live in their homes and communities instead of in costly nursing homes. Advanced AT may help to reduce secondary conditions, improve the rate of people who return to work and allow people to remain in their homes. Assistive technology holds promise as a means of improving self-care and may reduce the need for both paid and unpaid help.

Examples of suggested areas of research for Category II are found in Appendix B.

Category III: Health Promotion and Wellness Interventions for People with Disabilities

It is only within the last decade or so that contemporary public health efforts such as the International Classification of Functioning, Disability and Health (WHO 2001) and the Surgeon General's Call to Action to Improve the Health and Wellness of Persons with Disabilities (USDHHS 2005) have resulted in broader recognition that disability is **not** equivalent to ill health and that people can experience disability and good quality health. Traditionally, public health approaches measure health outcomes in terms of reducing cases and symptoms of disease (reducing morbidity rates) and avoiding early deaths (reducing mortality rates). For example, children born with genetic or congenital anomalies and adults acquiring disabilities through injury or chronic disease are tallied within a morbidity count. Health promotion in this context focuses on primary prevention and views disability as incompatible with health and the ability to achieve health.

What is health promotion and does it differ in the context of disability? Last (2007, p. 159) defines health promotion as "The policies and processes that enable people to increase control over and improve their health. These address the needs of the population as a whole in the context of their daily lives, rather than focusing on people at risk for specific diseases, and are directed toward action on the determinants or causes of health." According to the Ottawa Charter for Health Promotion (World Health Organization, 1986), health promotion is the process of enabling people to increase control over, and to improve, their health. As adopted by the *(American Journal of Health Promotion (1989),*"Health promotion is the science and art of

helping people change their lifestyle to move toward a state of optimal health. Optimal health is defined as a balance of physical, emotional, social, spiritual, and intellectual health. Lifestyle change can be facilitated through a combination of efforts to enhance awareness, change behavior and create environments that support good health practices. Of the three, supportive environments will probably have the greatest impact in producing lasting change."

These contemporary definitions of health promotion seem to have been developed precisely for people with disabilities rather than as a means of excluding them. Compared to the general population, people with disabilities experience important health differences such as lower levels of general health, higher levels of unmet health care needs, lower levels of preventive services, and higher levels of preventable secondary conditions, chronic conditions, and early mortality. There are also recognized differences in health behaviors, including higher rates of cigarette smoking and lower participation in physical activity and exercise than the general adult population. It is also evident that the social determinants of health differentially impact people with disabilities, including socioeconomic determinants (e.g., education, income, and employment); psychosocial determinants (e.g., stress, social isolation, and level of control); and community and societal determinants (e.g., social support, community participation, and income inequality).

The challenge for health promotion in the context of people borne with or who acquire disabilities is to develop a better understanding of the reasons why people with disabilities experience health differences and to develop individual, systems, and policy level interventions that are effective in addressing the determinants of health.
Examples of suggested studies under Category III are found in Appendix C.

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APPENDIX A

Examples of CER in the area of Rehabilitation Therapies and Treatments

Attention Deficits in TBI: Methylphenidate vs. Attention Process Training

Nature of Problem or Research Question: Attention deficits are common after TBI across the spectrum of severity. There have been several behavioral/experiential approaches taken to attention retraining, and there has been considerable pharmacologic research. However, the multifaceted nature of attention and attention deficits and the small sizes of the studies conducted to date make it difficult to assess the differential effects of these approaches or the wisdom of combining them.

Impact/Utilization: Subtle attention deficits are among the most frequent complaints after mild TBI, and clinically obvious attention deficits are characteristic in moderate to severe injury. The most clearly described problems are slowness of information processing, difficulty with divided attention, and difficulty in maintaining attention to task in ongoing performance environments such as work.

Nominated Intervention (1): Methylphenidate

Summary of Research Findings to date: Methylphenidate is, of course, the leading agent for treatment of Attention Deficit (Hyperactivity) Disorder, and its benefit in that setting has been repeatedly replicated. The literature in TBI is smaller, with no large multicenter parallel group trials conducted to date. However, there have been several small but well controlled studies by Whyte, et al, and Willmott et al, with very consistent findings of efficacy in particular subdomains. In particular, speed of processing, caregiver ratings of attentiveness, and individual work productivity, have been seen to respond to drug in these studies.

Nominated Intervention (2): Attention Process Training

Summary of Research Findings to date: Attention Process Training is the most well described and extensively studied behavioral/experiential treatment of attention deficits after TBI. Developed by Sohlberg and Mateer and distilled into a treatment manual, the treatment focuses on exercises that "stress" specific attentional domains, but also includes a considerable amount of "metacognitive coaching" from the therapist to help the patient identify situations that are susceptible to attentional lapses and strategic compensations to be employed. APT has been evaluated in several pre-post designs, and impact appears to be less when compared to an untreated control group. However, there do appear to be process-specific benefits. That is, APT appears to have greater impact on strategic aspects of "Executive Attention" than simple vigilance or processing speed domains.

In summary, there is moderately strong support for both forms of attention treatment, but a suggestion that their primary impacts may appear in different facets of the complex array of attentional functions. Ultimate clinical recommendations, therefore, may be in the form of

defining which outcomes are most powerfully affected by which treatment in which patient subgroups.

Proposed Study Design:

Design: Parallel 3-group design with an APT group, a medication group, and a combined medication and APT group. Further discussion is needed regarding the control condition(s). It would be very difficult and expensive to create a "sham APT" treatment, since the treatment would need to be plausible to patients and therapists, distilled into a manual, and yet unlikely to have positive effects on attentional function. The best compromise may be to use placebo and active methylphenidate, but to have "open-label" APT.

Sample (include target disability group, age group) Adults with self-reported or clinician-reported attention deficits and a history of moderate to severe TBI .

Inclusion/Exclusion: Individuals with moderate to severe TBI > 6 months post-injury with persistent complaints related to attention. Individuals would be excluded for significant cardiac disease or uncontrolled hypertension that would make treatment with methylphenidate unwise, for a history of stimulant abuse, for concurrent treatment with drugs that would antagonize the effects of methylphenidate or make its administration unsafe, for language comprehension deficits that would preclude active participation in APT, for severe memory impairment that precludes retention of learned strategies, or for severe behavior problems that prevent participation in treatment.

Timeline: Depends on how many centers included. Subjects would be treated in 8 week blocks, but would have an additional follow up assessment at 12 weeks (i.e., 1 month follow up).

Data Collection Plan Anticipated: Subjects screened and enrolled in 8-week program. APT program delivered in 2-hour blocks 3X/week in an outpatient setting. Methylphenidate given at dose of .3 mg/kg BID. Baseline assessment conducted with a neuropsychological test battery of attention and speed of processing measures, as well as observational rating scales (Rating Scale of Attentional Behavior, Moss Attention Rating Scale, and the Cognitive Failures Questionnaire). Follow up at 4 weeks, 8 weeks, and 12 weeks (4 weeks after completing treatment).

Data Analysis Plan Anticipated: Primary outcome would be a composite score (average of ranks across measures or average of z scores across meausures) based on the attentional rating scales, since these ratings bear the strongest relationship to real-world benefits. This would be Kruskal-Wallis comparison of the 3 treatment groups. Secondary analyses would involve assessment of treatment effects and effect sizes in each of the neuropsychological measures, as well as drop outs and adverse events, with particular attention to the possibility of differential domains of maximal treatment response for the 2 treatments. Specifically, we would predict that the drug may produce greater effects on speed of processing, whereas the APT may produce

greater benefits in executive attention measures and specific improvement on the Cognitive Failures Questionnaire.

Feasibility Assessment:

Threats to Implementation: subject recruitment, hiring and/or training therapists to deliver the APT.

Threats to study completion: subject recruitment

Potential Threats to Generalizability: exclusion of patients on many medications, patients with coexisting impairments.

Comparison of Compensatory and Restorative Remediation for Attention Deficits after Traumatic Brain Injury

Impact/Utilization: The proposed research will evaluate interventions derived from two different theoretical models proposed to underlie the rehabilitation of cognitive impairments after TBI. The research will impact the field on several levels: (1) comparison of theoretical models of improvement based on either compensation / adaptation to deficits vs. neuroplasticity / restoration of function. (2) comparison of different instructional components based on increasing metacognitive regulation and strategy use through distributed learning and error management, versus reliance on massed practice and errorless learning. (3) comparison of different service models, requiring specialty trained therapists versus automated, computerized interventions with minimal therapist involvement. The study will have implications for understanding the mechanisms of action of treatment as well as practical issues related to portability and service delivery.

Nominated Intervention (1): Compensatory attention and metacognitive strategy training.

Summary of Research Findings to date: A combination of direct attention training and metacognitive training to develop compensatory strategies for attention deficits after traumatic brain injury (TBI) is currently considered a "practice standard" within the field of cognitive rehabilitation. Sohlberg et al [1] used a crossover design to compare the effectiveness of "attention process training" (APT) brain injury education and support for 14 patients with acquired brain injury. Self-reported changes in attention and memory functioning as well as improvement on neuropsychological measures of attention-executive functioning were greater following APT than following therapeutic support. Another RCT [2] investigated the effectiveness of APT and cognitive-behavioral psychotherapy for participants with persisting complaints after mild or moderate TBI. Participants in the active treatment group demonstrated improved performance on a measure of complex attention and reduced emotional distress compared with the no-treatment control group, although there was no effect of treatment on community integration. Another RCT [3] taught 22 patients with severe TBI to compensate for slowed information processing and the experience of "information overload" in daily tasks. Participants were randomly assigned to receive either "Time Pressure Management" (TPM) or an alternative treatment of generic "concentration" training. Participants receiving TPM showed significantly greater use of self-management strategies and greater improvement of attention and memory functioning compared with participants who received the alternative treatment. Several observational studies have reported success in the use of interventions developed to address the central executive component (CE) of working memory. [4, 5] Both of these latter studies emphasize the development of compensatory strategies to manage processing demands, and training in the application of this intervention approach to participants everyday functioning. Thus, although the precise nature of

the interventions in all of these studies differ, they share a common emphasis on the combination of direct attention training and metacognitive training, and the development of strategies to compensate for residual cognitive deficits ("strategy training") rather than attempting to directly restore the underlying impaired function ("restorative training").

Nominated Intervention (2): Computerized, restorative attaining training.

Summary of Research Findings to date: There is an emerging science and body of evidence documenting neuroplasticity in the adult brain, and a corresponding interest in developing and evaluating cognitive interventions that promote neuroplasticity as a means of restoring function. One small RCT developed also interventions based on the central executive operations of working memory, and compared this with a general stimulation approach. [6] Improvements in cognitive functions dependent on the CE as well as reduced cognitive symptoms were noted after CE training but not general stimulation. These gains were attributed to the effects of "massive practice" on CE tasks and the recovery of the underlying attentional functions, which then generalized to related cognitive operations and daily functioning. Another RCT used automated, computerized training on various working memory tasks to treat the cognitive deficits of 18 adults after stroke. [7] The intervention was based on intense, systematic practice with minimal therapist involvement, [8] under the assumption that the training leads to increased cortical activation and restoration of the underlying function. [9] This study again demonstrated gains on several measures of working memory as well as a reduction in cognitive symptoms.

Proposed Study Design:

Design: RCT

Sample (include target disability group, age group) Adults with TBI, 18 to 60 years old, minimum 6 months post injury

Inclusion/Exclusion: will include formal assessment of pre-treatment cognitive functioning. Treatment compliance will be assessed as a study variable.

Timeline. 8 week intervention period with 3 month follow-up.

Data Collection Plan Anticipated. Pre-post and follow-up testing of cognitive functioning to include working memory storage and working memory manipulation tasks; other attention, memory and executive tasks; subjective complaints. Potential for subset of participants to be evaluated with fMRI.

Data Analysis Plan Anticipated. Mixed model MANOVA

Feasibility Assessment:

Threats to Implementation. Recruitment of appropriate subjects; ability to control for other simultaneous treatments received.

Threats to study completion. Recruitment and retention of subjects

Potential Threats to Generalizability: Intervention arms may be conducted within specialized rehabilitation research centers.

Comparative Effectiveness Research Proposal for Autism Interventions

Nature of Problem or Research Question

There is empirical support demonstrating the efficacy of a range of approaches for enhancing the communication skills of individuals with autism spectrum disorders (ASD) (Dawson & Osterling, 1997; NRC, 2001; Prizant & Wetherby, 1998; Rogers, 1998). However, there are no large-scale studies directly comparing the effectiveness of different approaches using randomly assigned, matched control samples with sufficient sample sizes and adequate statistical power. Therefore, evidence that any one approach is more effective than another approach is not available to date. The proposed research question offers a comparison of different intervention approaches and suggests outcome measures that are ecologically valid. Specifically, they measure meaningful changes within natural learning environments and across natural communication partners and address the core deficits of autism—communication and social interaction (National Research Council, 2001):

> **Compare the effectiveness of social interaction approaches versus highly structured behavioral approaches on the verbal, social and nonverbal functional communication skills of preschool children with autism spectrum disorders, in terms of (a) gains made in the frequency of self-initiated spontaneous communication during functional activities and (b) the generalization of gains made across activities, interactants, and environments.**

Impact/Utilization

Comparative effective research should deepen our understanding of the types of intervention approaches that provide the most meaningful communication and social interaction outcomes for with young children with autism. Given that the core features of ASD revolve around social communication and language use, the field of speech-language pathology has much to contribute to future research evaluating the comparative effectiveness of approaches to treating social, communication, and cognitive impairments in ASD.

Nominated Intervention

There are many different intervention approaches that have been used for individuals with ASD. Programs differ in how goals are prioritized and the techniques used to target goals. Some programs rely heavily on singular strategies, while others are more comprehensive or eclectic. Most important is how the environment and instructional strategies support individualized goals and objectives for the individual with ASD and his or her family and other communication partners (NRC, 2001).

The major approaches currently in use are highly structured behavioral approaches (e.g., Applied Behavioral Analysis approaches) and more social interactive developmental approaches, such as (e.g., Social Communication Emotional Regulation Transactional Supports (SCERTS) and Developmental, Individual Differences, Relationship Based approach (DIR).

Summary of Research Findings to Date

Massed discrete trial methods, based on the theory of applied behavioral analysis (ABA) have been used with children with autism to teach verbal behavior (Lovaas, 1987; see summary by Koegel, 1995). Applied behavior intervention is intensive, with 30 to 40 hours of one-on-one intervention recommended on a weekly basis. Recently, a systematic review of the efficacy of applied behavior intervention was conducted with preschool children (18 months to 6 years) with autism. Outcome measures were cognition, language, and adaptive behavior (Spreckey & Boyd, 2009). Four studies had adequate data and were of sufficient quality to be included in a meta-analysis. Results of the meta-analysis did not demonstrate significant improvements in any of the outcome measures compared to other interventions for preschool children with autism. A clear need for more controlled clinical trials with additional outcomes (e.g., addressing family functioning) was demonstrated.

A major limitation of a discrete trial approach for language acquisition is the lack of spontaneity and generalization. More contemporary behavioral approaches use more naturalistic teaching methods for teaching speech, language, and communication, such as natural language paradigm (R. L. Koegel, O'Dell, & Koegel, 1987), incidental teaching (Hart, 1985; McGee, Krantz, & McClannahan, 1985; McGee, Morrier, & Daly, 1999), time delay and milieu intervention (Charlop, Schreibman, & Thibodeau, 1985; Charlop & Trasowech, 1991; Hwang & Hughes, 2000b; Kaiser, 1993; Kaiser, Yoder, & Keetz, 1992), and pivotal response training (L. K. Koegel, 1995; R. L. Koegel, Camarata, Koegel, Ben-Tall, & Smith, 1998; Whalon & Schreibman, 2003).

There are only a few studies, all using single-subject design, that have compared traditional discrete trial with naturalistic behavioral approaches. These studies have reported that naturalistic approaches are more effective at leading to generalization of language gains to natural contexts (R. L. Koegel et al., 1998; R. L. Koegel, Koegel, & Surratt, 1992; McGee et al., 1985).

Other intervention approaches also incorporate naturalistic behavior approaches and are more comprehensive and are consistent with a social interactive and developmental approach to intervention: Social Communication Emotional Regulation Transactional Supports comprehensive educational model for children with ASD (Prizant, Wetherby, Rubin, Laurent, & Rydell, 2003, 2006) and Developmental, Individual Differences, Relationship Based approach (DIR). Although the empirical support for developmental approaches is more limited than for behavioral approaches, there are a growing number of research studies that provide support for using developmental strategies (Aldred, Green, & Adams, 2004; Hwang & Hughes, 2000b; Lewy & Dawson, 1992; Mahoney & Perales, 2005; Rogers & DiLalla, 1991; Rogers & Lewis, 1989), and there are many case studies, with Greenspan and Wieder (1997) being the largest case

review. Developmental approaches share many components of contemporary naturalistic behavioral approaches and are compatible along most dimensions (Prizant & Wetherby, 1998).

Proposed Study Designs

- Single-subject cross-over designs to further investigate efficacy in specific populations not previously studied*
- Group designs comparing these approaches to further investigate generalization in specific populations not previously studied
- Random assignment, matched control samples, double-blind clinical trial

*Single-subject designs may be provide evidence of efficacy or effectiveness through multiple replications (Odom, Brown, Frey, Karasu, Smith-Canter, & Strain, 2003).

Inclusion Criteria
Preschool children with ASD

Exclusion Criteria
Preschool children with ASD with significant intellectual/cognitive challenges

Timeline
Two-to-Five years

Feasibility Assessment
The literature already contains investigations attesting to feasibility but further feasibility efforts may be needed for some sub-groups of pre-school children with autism.

Threats to implementation
Ensuring treatment fidelity (as well as collecting data to evaluate treatment fidelity)

Threats to study completion
Recruitment and retention of subjects
Requires agreement to participate by parents. Requires randomization to different treatment approaches.

Potential Threats to Generalization
Heterogeneity of population
May not generalize to older population

Comparative Effectiveness Research Proposal for Oropharyngeal Dysphagia

Nature of Problem or Research Question

Many patients with neurological conditions experience dysphagia as a result of neurologic illnesses or injuries such as traumatic brain injury, stroke or Parkinson's disease [1-3]. Speech-language pathologists (SLPs) trained in dysphagia management play an integral role in the evaluation and treatment of swallowing disorders for adults with neurologically induced dysphagia. The type of treatments provided by SLPs to improve swallowing function depends on the cause, type and severity of dysphagia as well as other factors such as the extent to which the patient can attend, follow directions, and comply with the SLPs instructions, especially when eating alone. Evidence concerning the efficacy of behavioral treatment approaches for individuals with neurologically-induced dysphagia is accumulating but much more research is needed to determine the best interventions for each of the various patient groups as determined by the etiology, dysphagia symptoms, and other case mix factors. Although at present, there are a limited number of studies, there is some positive evidence for the efficacy of these treatments on various swallowing outcomes. Treatment to improve disordered oropharyngeal deglutition has traditionally centered on behavioral interventions, with the intended purpose of facilitating safe and efficient oral feeding. Behavioral therapeutic approaches have been used clinically by SLPs trained in dysphagia management [4] for over 20 years and include posturing of the head and neck, physical maneuvers altering oral and pharyngeal physiology, tactile, thermal and electrical stimulation, oral and facial exercises, and diet modifications [5]. The goal of postural treatments is to alter the flow of the bolus by repositioning the body, head and/or neck prior to the onset of the pharyngeal phase of the swallow, with maintenance of the position until the swallow was completed. Postures included the *side lying* posture, *chin tuck*, or neck flexion posture, and the *head rotation* posture. Maneuvers were defined as volitional movement of the oral, pharyngeal, or laryngeal structures before or during the pharyngeal phase of the swallow that are intended to increase swallow force, or alter airway protection mechanisms. Maneuvers included in the present proposal include the *effortful swallow* maneuver, the *Mendelsohn* maneuver, *supraglottic* maneuver, and the *super supraglottic* maneuver. In constructing the clinical questions, various outcomes should be considered. Outcomes can be classified in terms of effects on *swallow physiology* (e.g. timing, efficiency, pressure and elimination of aspiration); *functional swallow ability* (e.g. oral feeding and quality of life); and *health* outcomes (e.g., weight and nutritional status, and the incidence of adverse outcomes such as aspiration pneumonia and immunocompromised health conditions).

To date, there have been a number of published guidelines and evidence-based systematic reviews (EBSRs) focusing on dysphagia within various populations and treatment settings [6-9]. The seven behavioral treatments being proposed as the focus of this comparative effectiveness proposal are three postural interventions (side lying, chin tuck and head rotation) and four

swallowing maneuvers (effortful swallow, Mendelsohn maneuver, supraglottic swallow and super supraglottic swallow).

The specific question to be addressed is:

For patients with neurological disorders and evidence of oropharyngeal dysphagia, what is the comparative effectiveness of postural techniques (i.e. the *side lying* posture, *chin tuck*, or neck flexion posture, and the *head rotation* posture) versus volitional swallowing maneuvers (i.e., effortful swallow, the Mendelsohn maneuver, supraglottic swallow or super supraglottic swallow) as delivered by SLPs trained in dysphagia management on swallowing physiology, functional swallow ability, and health outcomes?

Impact/Utilization

Common etiologies of dysphagia include cerebrovascular accidents (CVAs), traumatic brain injuries and degenerative neurological diseases. These conditions often cause oropharyngeal dysphagia and can lead to serious and life threatening consequences such as aspiration pneumonia, malnutrition and immunocompromised health. Data from the Agency of Health Care Policy and Research (1999) report an estimated 300,000 to 600,000 individuals each year exhibited some form of dysphagia as a result of neurological illnesses or injuries [1]. Kuhlemeier [11] reports that dysphagia is a frequent complication of cerebrovascular accidents. An incidence rate of 37% to 78% has been reported for this population [12]. Moreover, findings from the American Speech-Language-Hearing Association's (ASHA's) National Outcomes Measurement System (NOMS) indicate swallowing as the most commonly treated disorder for individuals with neurological diagnoses [13]. NOMS data reveal that 47.6% of patients receiving SLP intervention in healthcare settings are being treated for dysphagia secondary to neurological diagnoses; the majority of whom (66.8%) make measurable functional progress in swallowing ability after receiving SLP services [2]. The primary aim of SLP intervention is to reduce the risk of aspiration and improve swallow function for safe and efficient oral intake [14]. To do this, SLPs employ a number of behavioral therapeutic approaches, including the use of compensatory swallowing postures and/or swallowing maneuvers. Increasing our knowledge concerning *what works best for whom* is much needed to reduce the incidence of avoidable adverse effects associated with oropharyngeal dysphagia secondary to neurological conditions.

Nominated Interventions

<u>Postural techniques</u>

- *side lying* posture, *chin tuck*, or neck flexion posture, and the *head rotation* posture

<u>Volitional swallowing maneuvers</u>

- effortful swallow, the Mendelsohn maneuver, supraglottic swallow or super supraglottic swallow

Summary of Research Findings to Date

According to the five-phase model of investigating clinical outcomes for behavioral interventions developed by Robey [15] prior to introducing interventions as treatments for specific patient groups, it is necessary to establish the existence of an intervention effect, and determine if that effect is sufficient to warrant further testing. Establishing such an effect in the case of oropharyngeal dysphagia begins with defining the physiologic changes that occur during the treatment; this identifies the ability of the treatment to modify function, and establishes a knowledge base from which to formulate hypotheses regarding the potential effects the treatment may have on specific types of disorders. Physiologic changes can include changes in oral or pharyngeal pressures, duration and timing of swallow events, structural movement or displacement, and muscle activation.

A systematic search conducted by the National Center for Evidence-based Practice at the American Speech-Language-Hearing Association of the peer-reviewed literature published between 1985 and 2008 yielded 17 studies which met predetermined inclusion criteria (cite article in press). Of those studies, five examined postural techniques [16,17,18,19,20] and 13 examined swallow maneuvers [16,18-32]. Five studies provided data to address swallowing postures. Of those, three studies investigated the chin tuck [20, 22,23] and two examined the use of head rotation [18,19]. Thirteen studies provided data addressing swallowing maneuvers with the majority (62%, 8 of 13) investigating the effortful swallow intervention [22,25-28,30-32]. Three studies examined the Mendelsohn maneuver [21,24,29], three examined the supraglottic swallow [21,22,33], and three examined the super supraglottic swallow [20,21,33]. Physiologic variables which were addressed by these studies fell into one of four categories, including oral or pharyngeal pressures, duration and timing of swallow events, structural movement or displacement, and muscle activation. The body of literature included in this systematic review collectively indicates that there is physiologic evidence to support existing hypotheses regarding the role of behavioral interventions in treating specific aspects of oropharyngeal dysphagia.

Proposed Study Designs

- Single-subject cross-over designs to further investigate efficacy in specific populations not previously studied
- Group designs comparing these approaches to further investigate generalization in specific populations not previously studied
- Random assignment, matched control samples, double-blind clinical trial

Inclusion/Exclusion

Inclusion criteria: Patients with a diagnosis of oropharyngeal dysphagia secondary to a neurological condition.
Exclusion criteria: Patients with moderate or severe cognitive impairments affecting compliance.

Timeline
Two-to-Five years

Feasibility Assessment
The literature already contains investigations attesting to feasibility, but as not all settings and populations are represented, further feasibility study may be warranted.

Threats to implementation
Maintaining double-blinding
Ensuring treatment fidelity (as well as collecting data to evaluate treatment fidelity)

Threats to study completion
Recruitment and retention of subjects

Potential Threats to Generalization
Heterogeneity of population (even within a given diagnosis)

Comparing Physical Therapy Interventions for Treating Chronic Pain Among People with Disabilities

Nature of Problem or Research Question:

Chronic pain is consistently listed among the most common secondary conditions reported by people with mobility impairments (1-3). While treatment of pain conditions has a strong evidence base, there has been little comparative effectiveness research on evidence-based treatments for people with disabilities. One of the key strategies for treating chronic pain is physical therapy; however, long-term maintenance of chronic pain requires ongoing physical activity (4). This study will examine the incremental cost-effectiveness of providing regular physical activity following standard physical therapy to manage pain reported by individuals with mobility impairments.

Impact/Utilization: Study results will help to determine the value of providing access to regular physical activity services for people with mobility impairments.

Nominated Intervention (1): Physical Therapy

Physical Therapy including modalities such as ultrasound, heating and icing, massage and physical activity are standard components of pain management (4)(5).

Summary of Research Findings to date:

Physical therapy has consistently shown effectiveness in reducing pain (6, 7) across health conditions.

Nominated Intervention (2): Physical Therapy supplemented with physical activity.

Summary of Research Findings to Date: Clinical practice (8), correlational studies (9) and intervention trials all support the efficacy of physical activity (10, 11) for managing chronic pain among people with diverse health conditions.

Proposed Study Design: A multi site randomized controlled trial with repeated measures. Subjects will be randomly assigned to either physical therapy alone or physical therapy with a supplemental physical activity program.

Sample: People with disabilities ages 18-70

Exclusion: People with co morbid psychiatric conditions other than depression.

Timeline: 2- year cost-effectiveness study with 6-months post-intervention follow-up data collected.

Data Collection Plan: Self-report staggered baseline design with pre-, post-, and 6-month follow-up.

Data Analysis Plan Anticipated: Repeated measures analysis of variance

Feasibility Assessment: *Threats to Implementation* - Effective randomization and subject recruitment. *Threats to study completion*- subject attrition.

Potential Threats to Generalizability: Treatment protocols will be controlled for the study to detect any incremental effectiveness of proving physical activity. Hence, the degree to which the model reflects actual clinical practice will affect generalization of results.

Comparison of the outcomes and length of speech-language pathology services when benchmarked NOMS data or individualized estimates are applied to care planning

Interventions compared: The study compares the services needed and outcome achieved for Medicare beneficiaries when a speech-language pathologist plans goals and amount of services while using, or not using, the American Speech-Language Hearing Association's (ASHA) National Outcomes Measurement System (NOMS) predictive data to identify the services and outcomes for similar patients.

Background: The Adult Component of the NOMS collects communication or swallowing function measurements according to a series of seven-point scales called Functional Communication Measures (FCM). Speech-language pathologists (SLP) are certified to reliably administer the measures. Functional gain is determined by the difference in an FCM score from admission to discharge. These data, in turn, provide clinicians with national comparisons on which to base clinical decisions. However, utilization of the benchmarks in planning or treatment is thought to vary greatly across SLPs and facilities.

In 2005, The Centers for Medicare and Medicaid Services issued Benefit Policy Manual instructions for outpatient therapy services that required documentation of improvement during treatment.[1] The NOMS was the only tool that met the criteria for measuring speech-language disorders: established psychometrics, clinical utility, ability to use computer interfaces, acceptance by therapists, and ability to provide predictive data.

In 2007, the Centers for Medicare and Medicaid Services established a research project titled "Developing Outpatient Therapy Payment Alternatives" (DOTPA). The purposes of this 5 year project are to identify, collect, and analyze therapy-related information tied to beneficiary need and the effectiveness of outpatient therapy services. The ultimate goal is to develop payment method alternatives to the current financial cap on outpatient therapy services.

In 2008, CMS contracted with Computer Sciences Corporation for a study titled Short Term Alternatives to Therapy Services (STATS). Before October, 2010, this project is tasked to: Collect and analyze quarterly and annual claims data; partner with stakeholders in analysis of utilization, policies, and clinically appropriate limitations or guidelines that may be used to develop options for short term alternatives to therapy caps.

The proposed research project would complement both of these projects by using electronic data collection and by focusing exclusively on outcomes measurement. This study creates a platform for linking appropriate payment to necessary services, and for reporting quality measures.

[1] Medicare Benefit Policy Manual, Chapter 15, sections 220.3.

Impact/Utilization: If benchmarking information improves care planning, it would be likely to affect not only the estimated half million Medicare beneficiaries treated annually by speech-language pathologists, but all of their patients. Clinicians would be motivated by better outcomes to utilize this data and they could rely on the data to justify appropriate services.

NOMS is the only tool for speech-language pathology services that is approved by the National Quality Forum and is part of the National Quality Measures Clearinghouse. It is available without cost to speech-language pathologists. Since there is little financial incentive to the tool sponsor, this study addresses a question that is unlikely to be addressed through other funding mechanisms.

The comparison of NOMS outcomes to Medicare claims results will create a unique database with potential for valuable future research relevant to creation of patient registries, comparative study of the effect of treatment choices, and affect on utilization.

Nominated Intervention (1): National Outcomes Measurement System

Summary of Research Findings to date:

The Adult component of NOMS (the National Outcomes Measurement System) has been collecting data on over 220,000 patients since late 1998. Communication or swallowing function is measured according to a series of seven-point scales called Functional Communication Measures, which were endorsed by the National Quality Forum in 2008, and added to the National Quality Measures Clearinghouse in 2009.

The American Speech-Language Hearing Association has unpublished research data on this tool which they will share as requested.

In 2004, the NOMS was used to identify changes in patient care following the introduction of the Inpatient Rehabilitation Facility Prospective Payment System for Medicare beneficiaries.[2] The study found that following introduction of the IRF PPS

more patients with cognitive, communication, and swallowing disorders were discharged from inpatient rehabilitative care with less than adequate functional skill levels.

Nominated Intervention (2): Control Group tested with NOMS but treated without knowledge of the NOMS test results.

Nominated Intervention (3): Control Group treated without performance of NOMS.

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[2] Frymark, Tobi B., Mullen, Robert C., *Influence of the Prospective Payment System on Speech-Language Pathology Services.* Am.J. Phys. Med. Rehabil. December, 2004, Vol. 83, No. 12, Pg 1-10.

Proposed Study Design: Identify a sample of providers or suppliers of services who will utilize the NOMS, provide one group with benchmarking information to use in care planning. Identify a control group not utilizing NOMS. Match the patient characteristics. Analyze the NOMS groups for known group construct validity, sensitivity to change, responsiveness and feasibility (practicality, ease of use, frequency of use). Compare outcomes, service utilization and cost of treatment when NOMS is performed and benchmark information is utilized in planning to a similar group where benchmarks are not utilized. Compare services utilized and cost when NOMS is not performed.

Sample: Medicare beneficiaries age 65 and over with communication and swallowing disorders.

Inclusion/Exclusion

- Include all Medicare beneficiaries with SLP disorders in clinic in study groups

Timeline: 18-24 months

Data Collection Plan Anticipated

- Identify providers/suppliers who are using NOMS by using tool sponsor contact information.
- Obtain Data Use Agreements, extract processed claims data from CMS Data Repository.
- From the universe of Medicare therapy claims, identify controls with similar characteristics to those beneficiaries whose therapists utilized NOMS with benchmark information.
- Collect initial and discharge information using NOMS on both sample groups. Collect utilization and cost data from control group.
- Match Medicare claims data to clinical data.
- Develop chart review and interview procedure (for feasibility measure).

Data Analysis Plan Anticipated

- Compare the study to the control group for functional outcome, number of visits, number and type of services, episode length in calendar days, recidivism, allowed charges, paid amount.
- Determine differences in the type, and number of treatment techniques, value of tools to treatment planning, burden of tools.
- Prepare and present Report.

Feasibility Assessment:

Threats to Implementation

Therapists may have but not use the information on cohort expectations.

Threats to study completion

Difficulty obtaining cooperation of provider/suppliers who are NOT using the target tools without an incentive.

Potential Threats to Generalizability:

- The Medicare population, while large, differs in some respects from the geriatric population of the nation as a whole.

- Some of the therapists who have not utilized a tool to identify cohort expectations may have a level of training and expertise that allows them to effectively estimate expectations without use of the tool.

Comparative Effectiveness of Neuromuscular Hyperactivity Non-Responders Receiving Locomotor Training

Overview

The NeuroRecovery Network (NRN)* consists of specialized Centers at 7 rehabilitation sites in the U.S. that provide a standardized Locomotor Training (LT) program designed from scientific and clinical evidence for recovery of posture, standing and walking and improvements in health and quality of life in individuals with spinal cord injury (SCI). This program admits patients with incomplete SCI (AIS C and D) whose spasticity medications are titrated to only require an evening dose at bedtime. There have been a group of patients who demonstrate such high muscular activity ("non-responders") demonstrating dominant flexor, dominant extensor, or combined patterns where LT becomes difficult to provide consistently and with appropriate intensity. The question posed is whether treatment FES cycling in combination with LT compared to antispasticity medications in combination with LT would reduce the degree of neuromuscular hyperactivity and thus improve walking outcomes. The impact and utilization of providing evidence to answer this question would be improved walking outcomes for a greater number of the more severely affected patients. These patients are sometimes either too difficult to wean from anti-spasticity medications and consequently not admitted into the NRN program due to the physical challenges of rendering LT.

All patients would receive standardized LT as provided throughout the NRN and would be randomized into 1 of the 2 nominated treatments (described below). A standardized LT session includes *step training* that is comprised of task specific retraining for standing and walking on a treadmill using a harness to provide BW support with verbal and manual facilitation, *overground assessment* that transfers the current capacity in mobility, posture and walking skills to over ground and establishes priorities for further retraining, and finally *community integration* that provides instruction on daily activities in the home and community environments. Treatments follow the LT principles and are also progressed in a standardized way.

Nominated Intervention (1):

Functional electrical stimulation (FES) cycling will be initiated for 30 minutes before the LT session (1.5 hours) to total a 2 hour intervention 3-5 days a week. The frequency of LT is dependent on the level of independence of proximal to distal segments in producing functional activities. Research Findings to date: To date, evidence has supported the use of LT and FES cycling alone in single subjects and small samples and not in combination and not for individuals with moderate to severe spasticity. This study will be the first to evaluate the impact of cycling on the reduction of spasticity and improvement of waking outcomes.

Nominated Intervention (2):

Steady state anti-spasmodics (e.g. Baclofen) as opposed to the standardized LT selection criteria requiring patients to be weaned of these medications. Patients would continue to receive their existing level of anti-spasmodics or be placed on these medications if medically appropriate, throughout their LT program.Studies have implicated that anti-spasmodics may inhibit spinal neuroplasticity however not study to date has examined if these medications inhibit recovery of walking in combination with LT. Our proposed study will examine walking outcomes while spasticity medications remain.

Proposed Study Design:

This will be an RCT which will screen, select and enroll a minimum of 42 individuals with incomplete SCI (ISCI), AIS C and D. Subjects will be enrolled from all 7 NRN centers who demonstrate significant spasticity on the modified Ashworth scale (Grades 3 or greater) in at least 2 muscle groups bilaterally. Participants must have finished their rehabilitation and currently not receiving any physical rehabilitation. Subjects will be tested for walking outcome measures before the study intervention begins, every 20 sessions, at discharge and 6 months later. The walking outcome measures include the 6 minute walk, 10 M walk, step length and time, gait speed, and the SCI functional assessment inventory. Repeated measures ANOVA will be used to evaluate change in the walking outcome measures and covariates such as injury level, AIS level, time since injury, age and will be explored. The termination of treatment is based on a discrete discharge algorithm where no improvements in key areas require clinical discharge. If insurance support is denied, grant funds would be encumbered to allow patients to continue until no further change is evident.

Feasibility Assessment:

There may be some challenges to implementing this study in identifying patients willing to commit the time for LT in combination with the 2 nominated interventions. Therefore, the compliance may be somewhat diminished. Other challenges may include the physical demand of rendering LT if the 2 nominated treatments do not alter the spasticity. Recruitment may also be challenging however requiring only 6 patients/site/year should be a reasonable. The treatments proposed should be generalizable given clinics have access to supported walking and FES cycling equipment.

High-repetition doses of task-specific training to improve upper extremity activity and participation

Nature of Problem or Research Question:

Stroke and brain injury are major health problems in the United States. Nearly 800,000 new strokes occur each year, and 50% of stroke survivors have persistent dysfunction that disrupts their ability to participate in home and community life. As soldiers return home from conflicts overseas with traumatic brain injuries, and fewer people die from acute strokes or brain traumas as a result of improved acute care, the number of people living with disability after brain injury is rapidly increasing.

Innovative approaches to rehabilitation are needed to reduce the disabling consequences of stroke and brain injury. Neuroscience and rehabilitation findings are now converging to suggest that extended, task-specific practice is critical for producing lasting changes in motor system networks, motor learning, and motor function. Our recent work, however, shows that there is little use of the upper extremity after stroke and little task-specific practice during neurorehabilitation. In our observational studies, people with stroke or traumatic brain injury performed < 50 repetitions of task-specific practice during therapy sessions. In comparison, animal models of stroke and human motor learning studies employ 300-600 repetitions of task-specific practice per session. This discrepancy in the dose of task-specific practice is cause for concern because recent clinical trials suggest that dose of practice may be the key factor in optimizing motor recovery in a variety of neuromuscular conditions. If the remarkable plasticity of the nervous system is to be harnessed to improve motor rehabilitation, then we must provide an adequate stimulus (i.e. adequate dose of practice) to people with stroke and brain injury. We propose to translate the high-repetition doses of task-specific upper extremity training used in animal models to the human experience of stroke.

Impact/Utilization:

This project will contribute to a new understanding of the dose of movement practice that can be tolerated and if high doses of task-specific practice will stimulate better outcomes. A major advantage to our approach is that, if effective, it could be economically implemented in any setting in a very short period of time. Clinics would not need to purchase expensive equipment (e.g. robotics) and therapists would not need to undergo extensive training. This means that our approach could be implemented in all types of clinics, not just those affiliated with academic medical centers.

The long-term goal of this line of research is to improve functional outcomes in neurorehabilitation by determining optimal dosing of task-specific practice. As new advances in cell replacement therapies and pharmaceutical interventions for neurological injuries proceed, our work on investigation of dose will be critical. These new advances will not be beneficial on their own but will need to be paired with an optimal training program. We aim to develop this training program now, so that it is ready as new advances emerge.

The importance of understanding dosing transcends the upper extremity, the motor domain, and stroke and brain injury. Investigations into optimal dosing are needed for all movements and for all domains of neurorehabilitation. Our results will have profound implications for motor rehabilitation aimed at improving function and minimizing disability in people with other disorders/conditions, such as cerebral palsy, spinal cord injury, and multiple sclerosis.

Nominated Intervention:

The intervention is 300-400 repetitions of task-specific upper extremity training in one hour sessions, 3 days/week. The intervention is individually-tailored to each participant, so that practiced tasks match the activity and participation goals of the individual. The chosen activities are graded to challenge the capacity of the participant and difficulty is progressed according to established motor learning principles. As designed, the intervention can be provided within the current delivery system of outpatient neurorehabilitation services.

Summary of Research Findings to date:

Our pilot work indicates that this high-repetition intervention is feasible and beneficial in 10 people with chronic (> 6 months) stroke. The high number of repetitions of task-specific training is achievable, as indicated by average numbers of repetitions per session that were > 300 for all subjects. Participating in the intervention did not result in negative consequences such as pain (e.g. shoulder pain from doing large amounts of activity) or undue fatigue. Changes in upper extremity activity, as measured by the Action Research Arm test, were greater than the estimated minimal clinically important change in the majority of subjects and greater than the published average changes due to Constraint Induced Movement Therapy. More importantly, participation in daily life, as measured by the Activity Card Sort and the Canadian Occupational Performance Measure, had improved at the end of the 6 week intervention and at the 1 month follow-up.

Proposed Study Design:

We propose a single-blind, randomized, controlled trial with a repeated measures design. Benefits of high repetition doses will be compared to the benefits of standard rehabilitation care, where both groups will receive the same frequency and duration of therapy. We will recruit people with upper extremity paresis and upper extremity activity limitations due to stroke or traumatic brain injury. Potential subjects will be between 18-90 years of age and have experienced a stroke or brain injury in the previous 1-3 months. The time within the first few months after stroke and brain injury is within the critical period when this intervention could have its greatest impact on activity and participation. Data from our pilot project have informed specifics design parameters regarding sample size, inclusion/exclusion criteria, duration of the intervention, outcome measures, and clinically-meaningful changes on those measures. Subjects will be randomized to the high-repetition dose or standard care groups using an adaptive randomization scheme to minimize baseline differences. Therapy will be provided in 1 hour sessions, 3 times per week for 8 weeks. Post-intervention assessments will occur at the end of the 8 week intervention and 3 months later. A timeline for the project is provided in the table.

Activity		
Year 1	Q1	Hire & train personnel; finalize recruitment materials, protocol, data collection forms, etc.
	Q2	Enroll subjects
	Q3	Enroll subjects
	Q4	Enroll subjects
Year 2	Q1	Enroll subjects
	Q2	Complete subject enrollment, with any additional subjects to replace drop-outs

		as needed
	Q3	Complete interventions and follow-up assessments on enrolled subjects
	Q4	Data analyses, manuscript preparation, plan next phase

Our hypothesis is that high-repetition doses of task specific upper extremity training will result in greater improvements in activity and participation than standard rehabilitation treatment. We will test our hypothesis using well-established outcome measures. The primary endpoint will be the Action Research Arm Test score at the 3 month follow-up point. Secondary endpoints will include the Stroke Impact Scale, Canadian Occupational Performance, and Activity Card Sort scores at 3 months post intervention. Analyses will be done using mixed model repeated measures ANOVAs to look for differences between groups and across time. Additional data on the success of delivering the interventions (e.g. compliance with the intervention, repetitions achieved, fatigue, etc.) will also be collected and analyzed. Extensive statistical resources are available on our campus to assist with the randomization, data management, and data analysis processes.

Feasibility Assessment:

The biggest barrier to clinical trials is subject recruitment. Our partner outpatient rehabilitation facility treated over 300 people with stroke and brain injury in each of the last 3 years. In our pilot project we met our recruitment goals and even had a waiting list at one point. Thus, enrollment will be limited by the amount of personnel available and not by the availability of participants. We have previous experience with managing and organizing a multi-site observational study of rehabilitation post stroke and previous experience with stroke rehabilitation clinical trials. These experiences will help us overcome the expected and unexpected challenges of the proposed project. Furthermore, we have a strong track record of successfully completing and publishing results from funded projects.

Potential Threats to Generalization:

Our results will generalize directly to people with stroke and traumatic brain injury. Generalization beyond these populations will need to be explicitly tested in future studies. Unlike most studies evaluating motor rehabilitation interventions, we have included people in our pilot work who also have deficits in other domains, such as cognition and language dysfunction. We intend to include individuals with deficits in multiple domains in the proposed project because this is the reality for most patients with stroke and brain injury. Having a sample that is representative of what is seen in rehabilitation clinics will greatly improve the generalization of our findings to current stroke rehabilitation practice.

Further information regarding rationale, significance and detailed methodology for this project are available on request.

The comparative effectiveness and cost-effectiveness of SNF-, IRF-, and home health agency-based rehabilitation for individuals with hip fracture.

Nature of Problem or Research Question: What is the relative effectiveness, cost-effectiveness (or expenditure-effectiveness) of SNF-, IRF-, and HHA-based rehabilitation for individuals with hip fracture?

It would also be important to examine the effectiveness/cost-effectiveness of episodes of post-acute care by looking at various combinations of post-acute care since there is considerable evidence that hip fracture patients go on to use additional forms of post-acute care after leaving the initial post-acute setting.

Impact/Utilization: Individuals with hip fracture are one the fastest growing groups receiving post-acute rehabilitation care. Among all IRF patients, for example, they are the 3rd most commonly served group after those with stroke and joint replacement. However, there is little evidence that one setting of care is more effective than others. CMS and other payers want to know which setting is most effective and cost-effective for different cohorts of post-acute patients.

Nominated Intervention (1): SNF-based hip fracture rehabilitation

Nominated Intervention (2): IRF-based hip fracture rehabilitation

Nominated Intervention (3): Home health-based hip fracture rehabilitation

Summary of Research Findings to date:

Studies to date have had mixed results with neither setting providing a clear advantage over others. One of the more extensive studies on hip fracture rehabilitation is based on 1990s data prior to the implementation of the Medicare PPS for each of the 3 post-acute settings—SNFs, IRFs, and HHAs.

Proposed Study Design:

> **Design:** Retrospective observational cohort design
>
> **Sample (include target disability group, age group):** All Medicare hip fracture patients served in SNFs, IRFs, and HHAs in 2006 and 2007. May limit sample to those over 50 years of age.
>
> **Inclusion/Exclusion:** No exclusion criteria currently anticipated although there will be some exclusion criteria in the final study design.

Timeline: Study can be completed within 12 to 18 months. This study can be done relatively quickly since it can rely in large part on administrative data, namely on MedPAR and Medicare claims data.

Data Collection Plan Anticipated: Will use Medicare claims data from 2006 and 2007. These data become routinely available within 18 months.

Data Analysis Plan Anticipated: These data cannot address functional outcomes but can address outcomes such as hospital readmissions, institutionalization, and mortality since patients with hip fracture are at considerable risk for all three (compared to joint replacement patients where the incidence of these outcomes is quite low and therefore not as relevant when examining outcomes). We will use propensity scoring or instrumental variables to control for selection effects.

Feasibility Assessment:

Threats to Implementation: None anticipated.

Threats to study completion: This is a study that can be completed within 12 to 18 months. The main uncertainty is the timely negotiation of data use agreement with CMS and timely acquisition of Medicare claims data.

Potential Threats to Generalizability: This study would be limited to Medicare patients only and more specifically, fee-for-service Medicare patients. Nearly 20% of Medicare participants obtain their coverage through a private Medicare-sponsored plan under the Medicare Advantage program.

Cognitive deficits after TBI

Impact/Utilization: High

Nominated Intervention (1): Comprehensive cognitive rehabilitation (class I)

Summary of Research Findings to date: Retrospective comparison of this to alternative strategies suggests benefits, but controlled clinical trial in military population did not (Ciccerone et al., 2005)

Nominated Intervention (2): Psychosocial interventions (class I)

Summary of Research Findings to date:

Proposed Study Design:

Design – Phase III, multicenter RCT

Sample (include target disability group, age group) – adult

Inclusion/Exclusion

Timeline – postacute, chronic

Data Collection Plan Anticipated – battery of tests as recommended by the workshop on TBI Common Data Elements (2009) or NIH toolbox (available in 2011)

Data Analysis Plan Anticipated – something like a T-test but leave that to statisticians

Feasibility Assessment:

Threats to Implementation - none

Threats to study completion – competition with other ongoing TBI studies

Potential Threats to Generalizability: heterogeneity of TBI

The comparative cost-effectiveness of SNF- and IRF-based rehabilitation for individuals with hip and knee replacements.

Nature of Problem or Research Question: What is the comparative cost-effectiveness of SNF versus IRF-based rehabilitative care for individuals following a hip or knee replacement?

In 2008, acute care hospitals performed more than a million joint replacements, a doubling from 10 years ago. About 75% of these patients go on to use some form of post-acute rehabilitative care such as a SNF, and IRF, or home health. We are on course to do 3 million joint replacements by the Year 2030. This represents an enormous expense to the Medicare program.

Impact/Utilization: CMS has a strong interest in bringing the costs of hip and knee replacements under control by making sure that patients are channeled to the right post-acute setting.

Nominated Intervention (1): SNF-based rehabilitation

Nominated Intervention (2): IRF-based rehabilitation

Summary of Research Findings to date: Research shows that IRF care is only marginally more effective than SNF care, which leaves open the question of whether it is also more cost-effective since SNF-level care presumably costs less. A comparative cost-effectiveness study can quickly build on what has already been found with respect to effectiveness.

Proposed Study Design:

Design: Comparative observational cohort study

Sample (include target disability group, age group): Hip and knee replacement patients discharged from a cross-section of SNFs and IRFs from across the US.

Inclusion/Exclusion: All hip and knee replacement rehabilitation patients except:

1. Hip replacement patients who had their replacement following a hip fracture, i.e., non-elective hip replacements.
2. Those who died in the follow-up period (death unlikely due to hip or knee replacement or subsequent care). Cannot obtain follow-up data on these patients. Also, comparative expenditure data may be problematic for these patients.
3. Those who had a subsequent joint replacement and obtained their rehabilitation in a different facility other than the facility from which they obtained their initial rehabilitation.

Timeline: 2200 patients discharged from SNFs or IRFs in 2006-07

Data Collection Plan Anticipated: Use of two secondary data sources: (1) outcome data collected as part of an earlier observational cohort study and (2) Medicare claims data on same patients for 6 months following admission to a SNF or an IRF.

Data Analysis Plan Anticipated: Marry outcome data from earlier study with 2006-07 Medicare claims data. Adjust data for differences in case mix. Evaluate relative or comparative cost and expenditure effectiveness analyses. Also use stochastic frontier analysis to evaluate the comparative cost-effectiveness when considering two or more outcomes concurrently.

Feasibility Assessment:

Threats to Implementation:

1. There are few if any.
2. Possible low representation in select case-mix groups.

Threats to study completion: This is a study that can be completed within 12 to 18 months. The main uncertainty is the timely negotiation of data use agreement with CMS and timely acquisition of Medicare claims data.

Potential Threats to Generalizability: Participating 20 facilities are a self-selected. Smaller SNFs and freestanding IRFs are underrepresented due to facility selection criteria but study sample does represent geographic diversity with each major region of the nation well represented.

Improving the benefits of rehabilitation for those with paralysis of and impairment to lower limbs.

Impact/Utilization: 5.6 million people are paralyzed to some degree

Nominated Intervention (1): Standard rehabilitation is carried out at hospitals and rehabilitation facilities with little effort made on returning the patients to full participation in their communities.

Summary of Research Findings to date: High rates of unemployment, high health services use, high rehospitalization rates, high rate of secondary conditions, high rates of informal care provider injuries, high divorce rates, high rates of institutionalization, low income, low quality of life and low community participation.

Nominated Intervention (2):

Wheelchair skills assessments and training

Skills for community mobility (wheelies, up/down slopes/curbs, etc.)

Seating evaluation and recommendations (pressure mapping, provision of air cushions with pressure alert systems

Exercise evaluation and ongoing experience in learning how to use different types of adapted equipment for functions

Transfers, mobility device propulsion, lifting, reaching, driving

Enrollment into physical exercise and wellness programs

Evaluation of and training provided for personal assistance needs

Informal family member, link to paid personal assistants and training informal and formal personal assistants

Evaluation for and introduction to recreational opportunities

Competitive sports, nature trails and parks sand travel

Evaluation of and experience in community participation

Assess 20 sites in the home communities and travel with participant to the sites and make recommendation for site changes in receptivity

Evaluation of and experience in computer skills

Software options, voice entry - Naturally Speaking, keyboard adaptations, social networking via internet

Summary of Research Findings to date: No published studies in this country

Proposed Study Design:

Design: Controlled treatment, multicenter trials

Sample (include target disability group, age group):

Paralysis of upper and/or lower limb impairments, over18

Inclusion/Exclusion:

Paralysis of upper and/or lower limb impairments exclusion of individuals with minimal loss of movement function due to paralysis

Timeline: 2 yr planning, 3 yr implementation

Data Collection Plan: functional outcomes, recurrent hospitalization, health care utilization, secondary conditions, health and well being, quality-of-life and community participation

Data Analysis Plan: Inferential statistics

Feasibility Assessment:

Threats to Implementation:

Requires innovative collaborations with health insurance plans, rehabilitation centers, community agencies

Threats to study completion:

Provider acceptance, consumer acceptance, funding mechanism that require cost sharing between traditional medical based and community based service providers.

Potential Threats to Generalization:

May not generalize to non-paralyzed populations

Comparative Effectiveness Research Proposal for Newborn Hearing Screening Loss to Follow-Up

Nature of Problem or Research Question

Congenital, permanent childhood hearing loss affects 2%–4% of infants who spend time in neonatal intensive care units (NICUs) and 1–3 of every 1,000 infants in well-baby nurseries (Baroch, 2003, CDC, 2008). In an attempt to improve outcomes for children with hearing loss and their families, the National Institutes of Health and the Joint Committee on Infant Hearing (JCIH) in 1993 and 1994, respectively, and the United States Preventive Services Task Force (2008) endorsed the goal of universal detection of hearing loss in infants. The American Academy of Pediatrics (AAP) Task Force on Newborn and Infant Hearing (1999) and JCIH (2000, 2007) endorsed universal newborn hearing screening (UNHS) and the early hearing detection and intervention (EHDI) goals of screening no later than 1 month, confirmation of hearing loss no later than 3 months, and receipt of appropriate intervention no later than 6 months of age. Today, it is estimated that newborn hearing screening is provided to 92%–95% of babies born in the United States and its territories (CDC, 2008; National Center for Hearing Assessment and Management [NCHAM], 2007).

In order to maximize the effectiveness of services and prevent negative developmental impact on children with hearing loss, a positive screening result must be followed by timely diagnostic confirmation and initiation of services. Of infants born in the United States in 2006 who did not pass their newborn hearing screening, it is estimated that nearly half were lost to follow-up (CDC, 2008).

There is very limited research on the effectiveness of different approaches to limiting loss to follow-up. Therefore, the specific question to be addressed is

> **For parents or caregivers of newborns with a positive screen for hearing loss at birth, what is the optimal timing and nature of interventions by health care professionals to increase the likelihood of timely follow-up for a diagnostic evaluation and, if indicated, intervention.**

Impact/Utilization

Previous research has indicated that delays in the diagnosis of and intervention for hearing loss are associated with subsequent delays in children's receptive language development. A 2008 systematic review sponsored by the Agency for Healthcare Research and Quality concluded that "Children with hearing loss who had UNHS have better language outcomes at school age than those not screened." Specifically, children with hearing impairment confirmed by ≤ 9 months of age had significantly better age-adjusted scores than those confirmed later on 2 tests of receptive language and 1 of 2 tests of expressive language but not on the speech scale (USPSTF, 2008).

Nominated Intervention

Approaches to parental education and the timing and content of educational materials vary widely across the country. Most parents learn of newborn screening programs while in the hospital, not prenatally (Arnold et al., 2006). Through a series of focus groups and interviews, Arnold and colleagues found that stakeholders (i.e., parents of infants experiencing the newborn hearing screening (NHS) process, parents of children with hearing loss, audiologists, technicians, nurses, PCPs) preferred having communication about the newborn hearing screening process occur before birth and preferred that user-friendly patient education materials be used. A 2006 survey of parents by Alexander and van Dyck found that parents preferred to be informed prior to the screening of what the screening entails, the urgency of early diagnosis, and what the follow-up process will be (Alexander & van Dyck, 2006).

It is recommended that research be undertaken on the optimal timing of the parental education (pre-natal versus pre-screening versus post-screening) and whether written materials, oral communication, or a combination is most effective in promoting follow-up.

Summary of Research Findings to Date

A 2008 systematic review (ASHA, 2008) found virtually no scientific evidence that could be used by clinicians, administrators, or policy makers to identify the infants at highest risk of loss to follow-up or of the effectiveness of different approaches to promoting follow-up. While there were a small number of studies related to risk factors for loss to follow-up, vague definitions of terminology, absence of experimental controls and other manifestations of problematic study quality inhibited the drawing of any strong conclusions. No studies at all were found relating to follow-up from newborn hearing screening to diagnostic evaluation or to intervention. The authors then searched for studies on interventions designed to promote follow-up from initial hearing screenings to re-screenings, and identified three studies in the peer-reviewed literature. One found no difference in follow-up rates among mothers who had received individual versus group counseling, and a second found no improvement in follow-up among parents who had watched a 20-minute video on hearing screening during pre-natal classes. The third study found a significant increase in follow-up in an experimental group who received written materials, individual counseling, computer tracking of compliance, and reminder telephone calls compared to a control group who just received the written materials. That study did not attempt, however, to discern the relative contributions of each of the specific components of the "bundled" interventions.

Proposed Study Designs

Random assignment, matched control samples, double-blind clinical trial

Inclusion Criteria
Families or caregivers of infants with a hearing screening at birth suggesting possible hearing loss.

Exclusion Criteria
Family history of hearing loss
Infant death or medical complications making follow-up more difficult.

Timeline
1- 3 years

Feasibility Assessment
The literature already contains investigations establishing feasibility, but further study may be warranted.

Threats to implementation
Ensuring treatment fidelity (as well as collecting data to evaluate treatment fidelity)

Threats to study completion
Recruitment and retention of subjects

Potential Threats to Generalization
Heterogeneity of population, settings

Comparative Effectiveness Research Proposal for Intensity of Language Intervention for Adults with Acquired Brain Injury

Nature of Problem or Research Question

Intensity of treatment has been a topic of interest for some time in aphasia treatment studies (Poeck, Huber, & Willmes, 1989) and treatment for language disorders due to traumatic brain injury. Findings from Robey's (1998) meta-analysis of the aphasia literature reported large effect sizes (ESs) associated with treatment provided for 2 or more hr per week. In a review focused on intensity and outcomes, Bhogal, Teasell, and Speechley (2003) reported better treatment outcomes in studies that provided intensive treatment schedules. On average, the more intensive treatment schedules equaled 8.8 hr per week for 11 weeks, compared with the less intensive schedules of 2 hr per week for 23 weeks of treatment. Although both reviews and several efficacy studies included in those reviews have examined the impact of the intensity of service delivery, large scale comparative effectiveness studies have yet to be accomplished.

> **Compare the effectiveness of 30 hours of language intervention delivered over 3 weeks as compared to over 10 weeks on the rate of acquisition, response generalization (across language tasks and communication settings), and maintenance of targeted language processing skills six weeks after therapy is teminated.**

Impact/Utilization

Approximately 700,000 people in the United States survive cerebral vascular accidents (CVA), or strokes, per year, and approximately two-thirds of these stroke survivors require subsequent rehabilitation for a number of impairments including motor deficits, cognitive deficits, and speech and/or language deficits (e.g., NIH, 2006). Specifically, approximately 1,000,000 individuals in the United States suffer from aphasia, with the majority of these cases resulting from stroke (Holland, Fromm, DeRuyter, & Stein, 1996, ASHA, 2004). In a large prospective study involving over 1000 participants with a diagnosis of CVA, aphasia was observed to occur in 38% of the sample, with the incidence rising to 40% when only participants with left-hemisphere lesions were assessed (Pedersen, Jorgensen, Nakayama, Raaschou, & Olsen, 1995). Furthermore, Pedersen and colleagues found that of the participants with aphasia who survived the stroke, 44% completely recovered by the time they were discharged from the hospital. At a six month follow-up, 50% of participants with an initial diagnosis of aphasia continued to present with aphasia; that is after six months of recovery time, only an additional 6% of participants with aphasia had completely recovered their language function. Knowing whether the intensity of service delivery has an impact on outcomes for individuals with acquired brain injury would provide a rather simple solution to enable providers to adjust their methods of

delivering services to achieve more effective outcomes without altering the economic burden of these services.

Nominated Intervention

There are many different intervention approaches that have been used for adults with language impairments secondary to acquired brain injury. For the purposes of this comparison, the type of language intervention delivered can vary and would be determined by the speech-language pathologist in consultation with the patient and family. Programs differ in how goals are prioritized and the techniques used to target goals. Some programs rely heavily on singular strategies, while others are more comprehensive or eclectic. Most important to the goals of this proposal is that the intensity of the service delivery be systematically varied such that half of the cohort enrolled would receive 30 hours over 10 weeks (spaced) and the other half over 3 weeks (massed).

Summary of Research Findings to Date

A systematic search of the literature was conducted (Cherney, Patterson, Raymer, Frymark, and Schooling, 2008) to identify studies that directly investigated intensity of language intervention for individuals with acquired brain injury and directly compared conditions of higher and lower intensity treatment. Of the 10 studies that met inclusion criteria for the systematic review, 5 studies investigated treatment intensity (Basso & Caporali, 2001; Denes, Perazzolo, Piani, & Piccione, 1996; Hinckley & Carr, 2005; Hinckley & Craig, 1998; Raymer et al., 2006). Five of these studies contained sufficient data for calculation of treatment ESs. The effect of intensity in Denes et al. (1996), Pulvermuller et al. (2001), and Hinckley and Carr (2005) was derived from between-group comparisons for groups receiving intensive and nonintensive treatment. The effect of intensity in Study 3 of Hinckley and Craig (1998) was derived from within-group comparisons of the pre- and post difference scores from each intensive 6-week training session compared with the nonintensive 6-week training session. In Raymer et al. (2006), the effects came from within-subject comparisons across the individual participants. Four group studies used impairment outcome measures for which eight effect sizes (ESs) were calculable, including seven large ESs, all in favor of more intensive treatment. In the single-participant design of Raymer et al. (2006), ESs were larger in the more intensive condition for picture-naming acquisition and larger in the less intensive condition for word/picture verification. ESs could not be calculated for Basso and Caporali (2001), who described case studies of three pairs of individuals. In summary, individuals receiving more intensive treatment showed greater gains on language impairment tasks than did the comparison individuals who received a less intensive schedule. Thus, the language impairment outcome measures favored more intensive treatment for all language measures.

Proposed Study Designs

- Group designs comparing these approaches to further investigate generalization in specific populations not previously studied
- Random assignment, matched control samples, double-blind clinical trial

Inclusion Criteria
Adults with language impairments secondary to acquired brain injury

Exclusion Criteria
Pre-morbid history of psychiatric, neurological, and/or communication impairments

Timeline
Two-to-Five years

Feasibility Assessment
The literature already contains investigations attesting to feasibility but further feasibility efforts may be needed for some sub-groups of individuals with acquired brain injury.

Threats to implementation
Ensuring treatment fidelity (as well as collecting data to evaluate treatment fidelity)

Threats to study completion
Recruitment and retention of subjects

Potential Threats to Generalization
Heterogeneity of population
May not generalize to all etiologies of acquired language impairment

High rates of HIV infection among individuals with psychiatric disabilities.

Impact/Utilization: Rates of HIV among individuals with psychiatric disabilities are documented at 5-75 times the rate of the general population. In the published literature, studies of individuals with psychiatric disabilities indicate that testing prevalence, access to testing, knowledge about HIV testing, and other relevant correlates are lacking and that additional research is warranted specifically for this population. In fact, comparing different types of HIV testing among individuals with psychiatric disabilities has been noted as a focal area for comparative effectiveness research (Senn & Carey, 2009). Findings from research identifying effective models for HIV testing among individuals with psychiatric disabilities have the potential to assist in the early detection of HIV, subsequently resulting in earlier engagement in treatment to reduce illness progression and mortality, prevention of transmission to others, and reductions in overall costs of HIV-related health services to treatment systems.

Nominated Intervention (1): Rapid HIV testing.

Summary of Research Findings to date: A rapid HIV test is an enzyme-linked immunosorbent assay (ELISA) test (OraQuick®); however, rather than being analyzed in larger batches with other individual tests, rapid tests are analyzed alone. For a rapid HIV test, a finger stick sample of blood is collected and transferred to a vial. This sample is mixed with a developing solution. The test device, resembling a "dipstick," is inserted into the vial. In as little as 20 minutes, the test device will indicate if HIV–1 antibodies are present in the solution. These are standard procedures tested, outlined, and endorsed by the Centers for Disease Control & Prevention (CDC).

Nominated Intervention (2): Traditional, Blood Assay HIV Testing.

Summary of Research Findings to date: Traditional HIV tests (ELISA) use blood to detect HIV infection. In all of these tests, a small amount of blood is drawn from the arm and sent to an outside laboratory for evaluation. The traditional ELISA test takes approximately one week to complete, depending on where the test is performed. With a positive result, a confirmatory Western Blot assay is performed. These also are standard testing procedures documented by the CDC.

Proposed Study Design:

Design Randomly assign 300 individuals with psychiatric disabilities to rapid HIV testing versus traditional HIV testing and follow them for 9 months after testing.

Sample (include target disability group, age group) Individuals with psychiatric disabilities who are 18 years or older.

Inclusion/Exclusion Inclusion: Being 18 years or older, have a psychiatric disability, willingness to be tested for HIV; Exclusion: younger than 18 at time of study entry, already diagnosed with HIV/AIDS.

Timeline This could be a 2-year project with a 9-moth follow up. However, the project could be extended to 3-4 years with an additional 12- or 18-month follow up. Both are longitudinal desnigns and would provide valuable information regarding testing methods.

Data Collection Plan Anticipated: HIV risk assessment data will be collected at in-person meetings. Separate research interviews also will be conducted longitudinally at study entry, and again at 3- and 9-months after testing to examine other co-factors to HIV risks, including health beliefs and psychiatric symptoms. Testing outcome data also will be collected to examine overall rates of HIV infection within this population, but also to examine rates at which testing participants receive the test outcomes based on testing modality (i.e., rapid vs. traditional).

Data Analysis Plan Anticipated: Given the nature of the data to be collected, rates of HIV testing will be compared by modality using nonparametric analyses (e.g., chi square); longitudinal differences among outcomes including psychiatric symptoms, health beliefs, and other co-factors will be evaluates using repeated measures analysis of variance as well as randomized regression analyses.

Feasibility Assessment:

Threats to Implementation: Implementation threats include individuals' unwillingness to engage in HIV testing, as well as the potential that HIV positive individuals who are randomly assigned to the traditional testing group may not return to learn their results.

Threats to study completion: A threat to study completion would be attrition post-HIV testing, regardless of testing mode, by participants who are not interested in participating in the two follow up research interviews.

Potential Threats to Generalizability: One threat to generalizability would be that the population in this study may not be representative of a national sample of individuals with psychiatric disabilities. Despite the high-impact of HIV infection in the population, more rural and non-metropolitan areas may be differentially affected by the HIV epidemic. Likewise, areas with more concentrated representation of people from diverse ethnic and cultural backgrounds than the Chicago-area may demonstrate different rates of participation. A larger, national multisite study would strengthen the external validity of this proposal.

Comparative Effectiveness Research Project Proposal for Family-centered Interventions in Autism

Nature of Problem or Research Question

A philosophical mandate for family-centered practices has permeated both health care and educational fields. This philosophy offers a foundation for effective family—professional collaborations in assessment, diagnosis, and treatment of individuals with ASD (Prelock, Beatson, Bitner, Broder, & Ducker, 2003). Family-centered practices include careful attention to family priorities and concerns in planning interventions (e.g., Marshall & Mirenda, 2002), as well as to learning about the family system that includes an individual with autism, and developing contextualized assessments and interventions that respect the family system and preferences (Hecimovic, Powell, & Christensen, 1999; Moes & Frea, 2000). Families of individuals with ASD have assumed increasingly important roles in promoting a broader-based awareness and understanding of the disorders, and in the search for effective treatments through their collaborations with professionals to set a national research agenda, ensure the availability of research funding, and encourage participation in research (e.g., Anders, Gardner, & Gardner, 2003; Hollander, Robinson, & Compton, 2004.

Given the nature of autism and the needs of individuals with ASD, families often become teachers and interventionists (NRC, 2001). Family involvement in teaching children with ASD has been documented since the 1960s (Turnbull, Turnbull, Erwin, & Soodak, in press), though some families today place less importance on their roles as teachers and instead want more information on varying topics (Turnbull, Blue-Banning, Turbiville, & Park, 1999). Most comprehensive programs for individuals with autism offer parents training (National Research Council [NRC], 2001).

Families are consistent communication partners who should be provided with opportunities to give information about their child, to learn new skills, and to receive information about available resources. How and what families are taught have been influenced by a shift from the "expert" model of parent education, in which the professional directs the parents, to a more collaborative model, in which family individuality is recognized and families define their own needs and level of involvement (Becker-Contrill, McFarland, & Anderson, 2003; Turnbull et al., in press).

Although research indicates that having families play a critical role in the intervention process is an important part of effective programs for children with autism, research is not available yet to indicate which services and support strategies or what combination is most effective (NRC, 2001). Concerns, priorities, and perspectives of the family need to actively shape educational planning. All of the comprehensive intervention programs with the best treatment outcomes include a strong family component. Family members should be supported to be effective members of the educational team and provided with the opportunity to learn strategies for teaching their child new skills and reducing problem behaviors (NRC, 2001). Sources of support

may include teachers, other interventionists, formal support groups, informal networking with other caregivers of persons with ASD, and families, friends, and neighbors (NRC, 2001).

Geographic location (R. L. Koegel, Symon, & Koegel, 2002) and lack of financial resources (NRC, 2001) can be constraints on access. In a study of Medicaid-eligible children with autism, for instance, Mandell, Literud, Levy, and Pinto-Martin (2002) found that African American children received diagnoses 1 year later than Caucasian children, on average, with a mean age of diagnosis of 7.9 years for the African American children with autism. Although this study did not include a comparison group of higher income children, the relatively late mean age of diagnosis for all the Medicaid-eligible children included in the Mandell et al. study suggests that few children in low-income families received services during their preschool years, regardless of race.

Other cultural and linguistic factors may play roles in families' access to or use of services (Dyches, Wilder, Sudweeks, Obiakor, & Algozzine, 2004; Wilder, Dyches, Obiakor, & Algozzine, 2004). For example, there is variability in the rate at which children from racial and ethnic minority groups are served under the label of autism in the public schools (Dyches et al., 2004). This variability may be due to complex interactions between the values of families from different cultural backgrounds, and linguistic and cultural differences, which may contribute to an over- or under-identification of ASD among certain groups. Ultimately, the diagnostic label of an individual will influence the information and resources that will be offered to families or that the families will seek on their own. When a diagnosis of ASD is given, families will have different understandings of what the diagnosis means, views of etiology, attitudes toward the disability, and motivations regarding accessing services. Families with limited English proficiency may face linguistic barriers to navigating information and service systems in the United States. In addition, families of individuals with ASD may choose alternative forms of treatment based on individual values or cultural background. For example, one study reported that Latino families were more likely to access complementary and alternative medical treatments for their children than were Caucasian or African American families (Levy, Mandell, Merhar, Ittenbach, & Pinto-Martin, 2003

Families of individuals with autism benefit from support beyond the learning of new skills. They benefit from formal and informal supports as well (NRC, 2001). Formal supports emerge from collaborative partnerships between families and professionals, while informal supports include support groups, informal parent networks, and family members and friends (NCR, 2001). Support for families is an ongoing process that takes different forms with different families based on their individual concerns, priorities, and interests (Blue-Banning, Summers, Frankland, Nelson, & Beegle, 2004; Dunlap & Fox, 1999; Sandall, Hemmeter, Smith, & McLean, 2005). Activities such as learning intervention strategies or working with the child in an intervention program are associated with reports of decreased stress by mothers of children with ASD (Bristol, Gallagher, & Holt, 1993; R. L. Koegel, Bimbela, & Schreibman, 1996). Stress also is

alleviated by perceived social support from both informal networks and formal support systems (NRC, 2001).

Do family-centered services and support strategies improve social communication outcomes for preschool children with autism? Contrast standard services with and without family-centered services on social communication outcomes.

Impact/Utilization

Comparative effectiveness research involving families of children with autism will demonstrate how cultural, linguistic, and socioeconomic factors affect families' access to or use and selection of services. Effective practices that involve families can incorporate family preferences and address family priorities.

Nominated Interventions

- Single-subject cross-over designs to further investigate efficacy in specific populations not previously studied
- Group designs comparing these approaches to further investigate generalization in specific populations not previously studied
- Random assignment, matched control samples, double-blind clinical trial

Summary of Research Findings to Date
In progress

Proposed Study Design
Randomly assigned, matched control samples. Single-subject designs may be provide evidence of efficacy or effectiveness through multiple replications (Odom, Brown, Frey, Karasu, Smith-Canter, & Strain, 2003).
Inclusion/Exclusion
Preschool children with ASD and their families from diverse cultural backgrounds
Timeline
Two-to-Five years
Data Collection Plan Anticipated
In progress
Feasibility Assessment
In progress
Threats to implementation
In progress
Threats to study completion
Recruitment and retention of subjects
Potential Threats to Generalization
In progress

Optimal organization and delivery of post-acute care for hip fracture patients

Impact/Utilization: 500,000 new hip fractures cases/year

Nominated Intervention (1): Acute care followed by conventional rehabilitation

Summary of Research Findings to date: Rehabilitation improves hip fracture outcomes but excess mortality of 15% in first year

Nominated Intervention (2): Bundled acute and rehabilitation care with rehabilitation setting determined by need

Summary of Research Findings to date: unknown

Proposed Study Design:

Design RTC

Sample (include target disability group, age group) hip fracture 65+

Inclusion/Exclusion all hip fractures secondary to fall or trauma, exclude pathological fracture

Timeline 2 yr planning, 3 yr patient accrual

Data Collection Plan Anticipated functional outcomes, recurrent hospitalization, health care utilization, mortality, quality-of-life

Data Analysis Plan Anticipated intention-to-treat analysis

Feasibility Assessment:

Threats to Implementation requires collaboration w/CMS, requires innovation by providers

Threats to study completion provider acceptance, requires randomization

Potential Threats to Generalizability: may not generalize to younger populations

Optimal organization and delivery of post-acute care for stroke patients

Impact/Utilization: 750,000 new stroke cases/year

Nominated Intervention (1): Acute care followed by conventional rehabilitation

Summary of Research Findings to date: Rehabilitation improves stroke outcomes

Nominated Intervention (2): Bundled acute and rehabilitation care with rehabilitation setting detrmined by need

Summary of Research Findings to date: unknown

Proposed Study Design:

Design RTC

Sample (include target disability group, age group) stroke ages 65+

Inclusion/Exclusion exclude other co-existent neurological diseases

Timeline 2 yr planning, 3 yr patient accrual

Data Collection Plan Anticipated functional outcomes, recurrent hospitalization, health care utilization, mortality, recurrent stroke, quality-of-life

Data Analysis Plan Anticipated intention-to-treat analysis

Feasibility Assessment:

Threats to Implementation requires collaboration w/CMS, requires innovation by providers

Threats to study completion provider acceptance, requires randomization

Potential Threats to Generalizability: may not generalize to younger populations

Optimal organization and delivery of post-acute care for complex medical patients

Impact/Utilization: may help to revise treatment guidelines

Nominated Intervention (1): Acute care followed by SNF/home care

Summary of Research Findings to date:

Nominated Intervention (2): Intense inpatient rehabilitation followed by home care

Summary of Research Findings to date: unknown except for very select populations e.g., metastatic disease to spine with paralysis where clear improvemtns in quality of life demonstrated

Proposed Study Design:

Design RTC

Sample (include target disability group, age group) multiple organ transplant patients, severely decondtioned patients following cancer treatments

Inclusion/Exclusion TBD

Timeline 1 yr planning, 1 yr patient accrual

Data Collection Plan Anticipated functional outcomes, recurrent hospitalization, health care utilization, mortality, quality-of-life

Data Analysis Plan Anticipated intention-to-treat analysis

Feasibility Assessment:

Threats to Implementation requires collaboration w/CMS, requires innovation by providers

Threats to study completion provider acceptance, requires randomization

Potential Threats to Generalizability: will depend on diagnostic criteria

Enhancing Motor Training with Transcranial Direct Current Stimulation

Traumatic Brain Injury (TBI) is a major world-wide public health problem. The Center for Disease Control and Prevention (CDC) estimates that 235,000 people in the U.S. alone are hospitalized annually with a TBI and survive. Approximately 80,500 of TBI survivors are left with long-term disability. Another 10,000 or more who sustain a TBI, but are not hospitalized, are estimated to become disabled each year. Long-term disability after TBI includes problems with motor control (weakness, spasticity, and instability), cognition (thinking, memory, and reasoning), sensory processing (sight, hearing, touch, taste, and smell), communication (expression and understanding), and behavior or mental health (depression, anxiety, personality changes, aggression, acting out, and social inappropriateness). The CDC estimates the prevalence of disability resulting from TBI in the U.S. to be 5.3 million. The annual direct and indirect costs including those due to work loss and disability have been estimated at $60 billion. These costs recently increased very significantly due to the high number of veterans who require treatment because of TBIs they sustained during the war in Iraq. There is therefore an urgent and ongoing need for better strategies to minimize motor impairments as a consequence of TBI and promote the recovery of function in TBI survivors.

Upper extremity impairment and related functional limitations are important targets of the rehabilitation of individuals who suffered a TBI. Recent literature emphasizes the need for studies assessing the benefits of interventions aimed at improving motor function in TBI survivors. Only a small number of studies has been focused on assessing motor gains associated with rehabilitation in TBI survivors. The few studies focused on the use of traditional rehabilitation techniques have shown that limited motor gains are associated with traditional interventions. Recent research has explored the use of rehabilitation approaches based on high intensity and specificity of targeted movements (such as constraint-induced movement therapy) in TBI survivors. Preliminary results are very encouraging as they show that clinically significant gains can be achieved via intensive motor therapy. Based on these considerations, our research team recently carried out a pilot study that combined non-invasive electrical stimulation of the brain (i.e. transcranial direct current stimulation, tDCS) with robotic motor training aimed at upper extremity motor recovery in a group of TBI survivors. *Figure 1* shows the setup we utilized in our preliminary investigation. The combination of tDCS and robotics was motivated by recent scientific evidence that points at the benefits of these technologies.

Based on the outcome of our pilot study, we propose to perform a randomized sham-controlled clinical trial to assess the clinical and neurophysiological effects of therapy that combines motor training using a robotic device (ARMEO, Hocoma AG) specifically designed for upper extremity rehabilitation, which allows one to perform therapeutic exercises based on an interactive gaming environment, and the use of noninvasive brain stimulation achieved via tDCS. The proposed study will allow us to perform a comparison of therapy based on the above-mentioned

technology and traditional physical therapy as currently delivered in an outpatient setting. The project will be carried out over a period of two years. During Year 1, we will focus on comparing robotic therapy combined with tDCS versus robotic therapy alone. During Year 2, we will compare traditional physical therapy with physical therapy augmented by the above-mentioned technologies. The decision of whether we will use robotic therapy alone or a combination of robotic therapy and tDCS will be made based on the results of Year 1 of the project.

Parkinson's Disease Medication Management

Parkinson's disease affects about 3% of the population over the age of 65 years and more than 500,000 US residents. The characteristic motor features of the disease include tremor, bradykinesia (i.e. slowness of movement), rigidity (i.e. resistance to externally imposed movements), and impaired postural balance. Current therapy is based on augmentation or replacement of dopamine, using the biosynthetic precursor levodopa or drugs that activate dopamine receptors. These therapies are successful for some time, but most patients eventually develop motor complications. Complications include wearing-off, the abrupt loss of efficacy at the end of each dosing interval, and dyskinesias, involuntary and at times violent writhing movements. Wearing-off and dyskinesias produce substantial disability, and frequently interfere with medical therapies. Furthermore, fluctuations in the severity of symptoms and motor complications (referred to as "motor fluctuations") are observed during dosing intervals.

Currently available tools for monitoring motor fluctuations are limited. In clinical practice, information about motor fluctuations is usually obtained by asking patients to recall the number of hours of ON (i.e. when medications effectively attenuate tremor) and OFF time (i.e. when medications are not effective). This kind of selfreport is subject to perceptual bias (e.g. patients often have difficulty distinguishing dyskinesia from other symptoms) and recall bias. Another approach is the use of patient diaries, which can improve reliability by recording symptoms as they occur, but does not capture many of the features useful in clinical decision-making.

Over the past few years, we have developed a wearable monitoring system that tracks changes in the severity of symptoms and motor complications in patients with Parkinson's disease. The system is equipped with wireless body-worn sensors that can gather data continuously over a period of up to 5 days. We have developed algorithms that identify ON-OFF periods and estimate UPDRS (Unified Parkinson's Disease Rating Scale) scores on the basis of the analysis of sensor data (i.e. accelerometer data) recorded during performance of motor tasks such as pronation/supination movements of the forearms, reaching movements, walking, sitting, etc. We have recently augmented the capability of our system by developing a web-based portal that provides clinicians with remote access to the data and videoconferencing capability so that a patient examination can be performed via the Internet. Preliminary results we have gathered over the past few years in a pilot study on about 20 patients with late stage Parkinson's disease indicate that the tools we have developed and tested could facilitate and improve medication management in this patient population.

We propose to perform a comparative effectiveness study aimed at assessing whether medication management can be improved in patients with late stage Parkinson's disease by relying upon the tools described above. Patients recruited in the study will be randomized to one of two groups: 1) receiving standard clinical services by which medication management is achieved via clinical visits and patient's report of his/her satisfaction with medication effectiveness, and 2) undergoing field monitoring to assess the severity of symptoms and motor complications during

motor fluctuation cycles via the use of the system we have developed and tested over the past few years as described above. The study will be carried out over a period of two years. During the first six months of the study, we will focus on the deployment of the technology in the field. We have extensive experience with the use of this technology and we are confident that we can address all the challenges of deploying the system based on our experience and our collaborations with Dr. Matt Welsh, who serves as Director of the Harvard Sensor Networks Laboratory, and Mr. Doug McClure, who serves as Corporate Manager of the Partners Center for Connected Health. The remainder of the study will be focused on the proposed comparative assessment of the anticipated clinical impact of the technology we have developed. We have extensively collaborated with Dr. John Growdon, Director of the Motor Disorders Center at Massachusetts General Hospital, and Dr. Dan Tarsy's team at Beth Israel Medical Deaconess Center. We will rely on these collaborations to achieve the goals of the proposed study.

Comparison of two outcome measurement tools providing benchmark predictive data to identify the utilization patterns for physical or occupational therapy rehabilitative services.

Nature of Problem or Research Question: To compare the psychometric properties of two outcome measurement tools. Also to compare the use of these tools for evaluating changes in utilization patterns among therapists when benchmark predictive data is, or is not provided for planning outpatient physical and occupational therapy treatment.

Background: Section 4541 of the Balanced Budget Act of 1997 (BBA) (Pub.L. 105-33) imposed financial limitations on outpatient therapy services and requested development of payment alternatives. In an effort to reduce errors in therapy claims, in 2005, the Centers for Medicare and Medicaid Services issued Manual instructions for outpatient therapy services that required documentation of improvement during treatment. The transmittal recommended, but did not require, measurement tools that address physical and/or occupational therapy services. Two of those tools, Focus On Therapeutic Outcomes, Inc. (FOTO), and Boston University's AM-PAC (administered by CRE Care) have extensive psychometric research, are widely used, and have amassed large data sets. Since the tools were developed using different patient data and manage the information obtained in different ways, they may address the needs of therapists for use in patient care in different ways.

In 2007, the Centers for Medicare and Medicaid Services established a research project titled "Developing Outpatient Therapy Payment Alternatives" (DOTPA). The purposes of this 5 year project are to identify, collect, and analyze therapy-related information tied to beneficiary need and the effectiveness of outpatient therapy services. The ultimate goal is to develop payment method alternatives to the current financial cap on outpatient therapy services.

In 2008, CMS contracted with Computer Sciences Corporation for a study titled Short Term Alternatives to Therapy Services (STATS). Before October, 2010, this project is tasked to : Collect and analyze quarterly and annual claims data; partner with stakeholders in analysis of utilization, policies, currently available measurement tools to develop clinically appropriate limitations or guidelines that may be used to develop options for short term alternatives to therapy caps.

The proposed research project would complement both of these projects by using electronic data collection and by focusing exclusively on outcomes measurement. This study creates a platform for linking appropriate payment to necessary services, and for reporting quality measures.

Impact/Utilization: During CY 2007, Medicare paid $4.37 billion for outpatient therapy services. If providing benchmark data to therapists improves quality and controls costs, 4.4

million beneficiaries who utilize therapy services annually would benefit[3] . Specific outcome measurement items with strong scientific credentials for specific patient conditions would be useful in identifying appropriate payment for quality services. It is unlikely that comparison of these two proprietary tools using Medicare claims would be feasible without federal support. This study will serve as a base from which future comparative effectiveness research questions may be formulated, for example, using the most appropriate tool to compare treatment options for specific groups of patients.

Nominated Intervention (1): Focus On Therapeutic Outcomes, Inc.

The Functional Outcome Score of FOTO is based on 2.4 million patient episodes obtained over 17 years. The outcomes instruments are currently being administered in over 2,000 clinics nationally and over 70 clinics in Israel. FOTO measures have been approved by the National Quality Measures Clearinghouse, given time-limited endorsement by the National Quality Forum, and used in the CMS funded (2006) a pay-for-performance study.

Nominated Intervention (2): AM-PAC

The AM-PAC's psychometric properties have been extensively evaluated in inpatient as well as outpatient post acute care patient patients with major medical, neurologic, as well as major orthopedic impairments. The AM-PAC has demonstrated a high degree of reliability, known groups and construct validity, as well as shown a high degree of sensitivity to change across all three functional domains across.

The Basic Mobility and Daily Activity scales have been given time-limited endorsement by the National Quality Forum.

Proposed Study Design:

Design: To compare the psychometric properties of the AM-PAC and FOTO measures, the study will identify a sample of providers of physical or occupational therapy outpatient services who are interested in utilizing both tools. Data will be collected at initiation of treatment and discharge using the two instruments simultaneously for all patients with a variety of neuromusculoskeletal conditions. Results for the two tools will be compared for the psychometric properties of test-retest reliability, validity (known group construct validity), sensitivity to change, responsiveness, usability (practicality, ease of use, frequency of use) and feasibility. To compare the effect of the knowledge of benchmark data from a similar cohort, provide two group of therapists outcome and benchmark data for one tool each to use in treatment planning. Compare outcomes, cost, the ability of the tools to classify clinics by effectiveness (based on outcome), and efficiency (based on utilization of time or resources to

[3] Amy Kandilov, Ph.D., Brieanne Lyda-McDonald, M.S., Edward M. Drozd, Ph.D., RTI International "Developing Outpatient Therapy Payment Alternatives (DOTPA): 2007 Utilization Report" Date 2009

achieve outcomes) for both groups. Also compare the cost and utilization of services for similar patients whose therapists do not utilize either tool.

Sample: Medicare Part B beneficiaries age 65 and over with a variety of neuromusculoskeletal disorders.

Inclusion: All Medicare Part B beneficiaries in the cooperative clinics who are receiving physical or occupational therapy.

Exclusion: Patients without cognitive ability or surrogate to participate. Patients with less than 3 therapy visits in an episode.

Timeline: 18-24 months

Data Collection Plan Anticipated

Identify providers/suppliers who are using the target tools by using tool sponsor contact information. Collect initial and discharge information using both tools on a sample group.

Extract processed claims data from CMS Data Repository. From the universe of Medicare therapy claims, identify controls. Match claims data to clinical data. Develop and execute chart review and interview procedure for clinical feasibility.

Data Analysis Plan Anticipated

Describe the functional status change, number of visits, number and type of services, episode length in calendar days, recidivism, allowed charges and paid amount.

Compare the differences in the tools psychometrics and compare instrument usability/practicality, feasibility, burden on patients/staff and other differences that arise.

Feasibility Assessment:

Threats to Implementation: Lack of cooperation among tool sponsors is very unlikely, but possible. Difficulty finding beneficiaries whose interventions were similar except the tools is possible, but also unlikely due to the huge universe of Medicare claims.

Threats to study completion: Recruitment of providers, unless incentive to participate is offered or burden is low (such as focusing upon therapists who already use the instrument).

Potential Threats to Generalizability: The Medicare population, while large, differs from the general population in race, and possibly in socioeconomic level.

Therapists who already have adopted these tools may be different than therapists who have not adopted these tools. There is no evidence or theory, however, that the differences in willingness to adopt a given assessment tool would affect the sensitivity of the tool.

APPENDIX B

Examples of CER in the area of Assistive Devices and Technologies

Comparative Effectiveness of two approaches to treating footdrop in post-stroke population

Nature of Problem or Research Question:

Footdrop or the inability to adequately clear the toes/forefoot during the swing phase of gait is a major rehabilitation problem following stroke. The standard of care in the US for the treatment of footdrop is the ankle-foot-orthosis (AFO) which constrains movement by preventing relative plantar flexion. However, emerging evidence indicate that motor recovery is activity dependent; specifically, repetitive movement therapy that is novel, functionally relevant and applied early during recovery is effective in facilitating motor recovery following UMN lesions. Thus, although an AFO clearly provides functional benefit, it may also hinder motor recovery. An important alternative to an AFO is the peroneal nerve stimulator (PNS) which actively dorsiflexes the ankle during the swing phase of gait and may facilitate motor recovery. However, these approaches have not been directly compared during the critical early post-stroke phase with respect to their effect on motor recovery (1-12 weeks).

Impact/Utilization:

Nominated Intervention (1): Articulated AFO.

Summary of Research Findings to date:

The standard of care for post-stroke foot drop is an AFO. Approximately 20% of stroke survivors discharged from acute inpatient rehabilitation are prescribed an AFO. [1, 2] Options include off the shelf plastic AFO, double upright metal AFO, solid ankle custom molded AFO and the articulated custom mold AFO. There are no studies that compare the relative efficacy of these devices. However, the community consensus appears to be the articulated custom molded AFO.

There is now sufficient evidence demonstrating the efficacy of AFO relative to no device in enhancing the functional mobility of stroke survivors.[3-7] However, there are no randomized clinical trials with long-term follow-up demonstrating their effectiveness. Most studies utilized cross-sectional design that randomly assigned the AFO condition vs no AFO condition. Nearly all studies evaluated chronic stroke survivors with acute stroke survivors evaluated only rarely.[8]

While an AFO is effective in enhancing functional ambulation relative to no device, the constraints of an AFO, even an articulated AFO, might inhibit neurologic recovery. Two studies that evaluated the effect of AFO usage on motor activation of the ankle dorsiflexors seem to support this concern.[4, 5] In agreement with prior studies, both demonstrated the functional benefit of an AFO. However, both studies also reported reduced activation of the ankle dorsiflexors (tibialis anterior) during gait. One of these studies concluded "The study…supports the functional benefit of a rigid AFO in hemiparetic subjects…However, the reduced activity in the

tibialis anterior muscle may lead to disuse atrophy and hence long-term dependence on the orthosis."[4]

Nominated Intervention (2): Surface peroneal nerve stimulator

Summary of Research Findings to date:
In 1961, Lieberson and associates[9] described the first single channel surface PNS to provide ankle dorsiflexion during the swing phase of gait. Burridge and associates[10] reported the only randomized clinical trial of surface PNS compared to no device and demonstrated that the treatment group exhibits significantly greater increases in walking velocity than the control group. Since then numerous case series have reported similar improvements in gait parameters based on a variety of commercially available surface PNS, including the Odstock Dropped Foot Stimulator,[11-13] the tilt sensor based WalkAide[14-16]and the wireless Bioness L300.[17-19] Several evidence based reviews concluded that there was strong evidence that PNSs improve hemiplegic gait parameters.[20-22]

Researchers investigating PNS have long understood that the primary barrier to clinical implementation in the US is the AFO. Accordingly, several studies compared the functional benefits of PNS to an AFO.[19, 23-25] For the most part, the two devices were similar with respect to functional ambulation.

In contrast to the AFO where there is concern regarding inhibiting motor recovery, the PNS may facilitate motor recovery by providing novel, repetitive movement therapy in the context of the functionally relevant task of walking. Lieberson and associates were also the first to describe an apparent "carry-over" effect after use of a PNS. Some participants who previously did not exhibit ankle dorsiflexion were able to volitionally dorsiflex the ankle after using the PNS.[9] This initial observation of an apparent motor relearning effect has now been corroborated by several case series.[12, 14, 26] These studies showed after a period of use of the PNS, some stroke survivors experience modest improvements in gait parameters even when not using the PNS. However, there are no longitudinal RCT to confirm the presence and clinical relevance of PNS mediated motor relearning effect. Further, all studies were conducted during the chronic phase of stroke when the environment for influencing substantial motor recovery is far from optimal.[27]

Proposed Study Design:

Design: Single-blinded RCT; PNS vs AFO during acute/subacute phase with 3-mo FU for pilot trial and 6-9-mo FU for full trial.

Sample (include target disability group, age group): Adult strokes (45-75)

Inclusion/Exclusion:

Inclusion	Exclusion
• 45-75 yrs old	• LE edema or skin breakdown

• Hemorrhagic or nonhemorrhagic • Medical stability • Unilateral hemiparesis • Presence of footdrop during ambulation • Minimum ambulation ability of standing and stepping within the parallel bars with or without an assistive device • Ankle dorsiflexion to neutral with PNS while standing	• LMN lesion of the peroneal nerve • Severely impaired cognition • Significant visual-spatial deficits • Aphasia with impaired comprehension • DVT • Potentially life-threatening cardiac arrhythmias • Demand pacemakers or defibrillators, or other implanted electronic device. • Pregnancy

Timeline: This depends on whether the study is a pilot or a large scale trial. A pilot can be 3 yrs; a full trial will likely require 5-yrs.

Data Collection Plan Anticipated: Outcomes that span the WHO continuum of impairment (e.g. gait speed, quantitative gait analysis, EMG, fMRI, metabolic cost), activities limitation (e.g. laboratory based measures of functional mobility) and participation (e.g. real life measures of mobility, measures of stroke specific QOL). For a pilot study, outcomes should be assessed at baseline and monthly thereafter for 3 mo. For a full trial, outcomes should be assessed at baseline and at 2-3 mo intervals for 6 to 9-mo, respectively.

Data Analysis Plan Anticipated: longitudinal analysis using linear mixed models

Feasibility Assessment:

Threats to Implementation:

- Medical and neurological instability of acute stroke survivors
- Confounding effect of multiple therapies: PT, OT, speech

Threats to study completion:

- Loss to follow-up
- Noncompliance
- Poor recruitment

Potential Threats to Generalizability:

- Use of a PNS requires high level of support from skilled personnel in order to ensure proper electrode location, reduce or minimize electrical stimulation mediated discomfort and enhance overall compliance. In a clinical trial this support is provided. However, in real life this may be difficult to maintain and thus study results may not easily translate to the real world.

- Although surface PNS is FDA approved, it is not CMS approved. Thus even if this study demonstrates significant benefit of PNS over an AFO, the lack of 3rd party reimbursement may render the study clinically irrelevant. On the other hand, the study results may influence CMS decisions.

Children with disabilities (e.g. autism, Down syndrome, mental retardation, cerebral palsy) often also have co-morbid hearing loss.

Some of this group of children will have moderate to profound hearing loss and may be candidates for cochlear implants. Although both cochlear implants and amplification devices (e.g. hearing aids) are used in this population, the question of which is preferable related to efficacy and broad issues of cost and benefit is not known.

Impact/Utilization: High impact disorders of low frequency.

Nominated Intervention (1): Cochlear implants

Summary of Research Findings to date: limited in this population although perhaps 30-50% of children who received cochlear implants have an additional disability.

Nominated Intervention (2): Amplification devices

Summary of Research Findings to date: limited in this population

Proposed Study Design:

Design descriptive

Sample (include target disability group, age group) children with disability 5yrs of age of less

Inclusion/Exclusion: progressive neurological disease

Timeline: 1 year of amplification followed by offer of cochlear implant if slow probress

Data Collection Plan Anticipated Anticipate the spectrum of speech and language, cognitive, social adaptive, and quality of life funciton.

Data Analysis Plan Anticipated determined by statistician

Feasibility Assessment:

Threats to Implementation finding comparable children

Threats to study completion being able to control for cochlear implant intervention

Potential Threats to Generalizability: each child with a disability is unique

Improving the benefits of assistive technology use by those with lower limb impairments.

Impact/Utilization: Over 7 million people use mobility devices

Nominated Intervention (1): Mobility devices are distributed at rehabilitation hospitals/centers or by durable medical supply dealers using medical benefits and brief assessments of functional loss as criteria for distribution of devices.

Summary of Research Findings to date: Nonuse rates ranging from 12% to 80% depending on the type of mobility device and the method of device acquisition.

Nominated Intervention (2): Acquisition of mobility device based on functional and participation benefits in environments where mobility devices are used including home, work and community frequented sites.

Summary of Research Findings to date: No published studies in this country

Proposed Study Design:

Design: Controlled treatment, multicenter trials

Sample (include target disability group, age group):

Lower limb impairments, over18

Inclusion/Exclusion:

All individuals with lower limb impairments who use mobility devices

except those who use canes, crutches or walkers

Timeline: 2 yr planning, 3 yr implementation

Data Collection Plan: Nonuse of mobility device, functional outcomes, recurrent hospitalization, health care utilization, secondary conditions, quality-of-life and community participation

Data Analysis Plan: Inferential statistics

Feasibility Assessment:

Threats to Implementation:

Requires innovative collaborations with health insurance plans,

rehabilitation centers, community agencies. Requires removal of in-home CMS rule for use of mobility devices

Threats to study completion:

Provider acceptance, consumer acceptance, funding mechanism that

require cost sharing between traditional medical based and community based service providers.

Potential Threats to Generalization:

May not generalize to younger populations

Improving Immediate Responses to In-Home Falls

Improving the response to in-home falls in the elderly population is of paramount importance. Falls are the sixth leading cause of death in elderly people in the United States. A key factor to minimize the severity of fall-related injuries is to promptly detect the fall event and alert clinical personnel. During the past few years, a number of devices for fall detection have been introduced on the market. These devices are based on body-worn units (e.g. pendants, wrist-watch units) equipped with an accelerometer. The units are programmed to detect falls based on the analysis of accelerometer data and to send an alarm message to a caregiver. Unfortunately, the potential benefit of these systems is limited by poor compliance likely because subjects are overwhelmed by the large number of false fall detections (i.e. false positives) that mark commercially available systems.

We propose to develop a new system for fall detection that combines home robotics with the use of body-worn units and to compare the newly-developed system with a commercially available device (i.e., the Philips Lifeline system). In the proposed system, the body-worn unit will send a message to the robot (see *Figure 1*) when it detects a fall event and the robot will respond by acquiring and processing video and audio information to assess whether the subject actually fell. When the subject does not wear the body-worn unit, the robot can utilize additional sensors to detect fall events (e.g. via detection of vibrations of the floor). Although we anticipate that the combined use of the body-worn unit and the robot will be superior to the use of the robot alone, we believe that it would be unrealistic to assume that subjects will use the body-worn unit all the time. The proposed system has great potential in providing effective monitoring and prompt interventions in the prevention of fall-related complications.

The system will rely on wireless units that we have recently developed in collaboration with Intel Digital Health and researchers at Harvard University's School of Engineering. The wireless units can transmit data via an IEEE 802.15.4 protocol or using Bluetooth. Additionally, the units are equipped with a microprocessor of the MSP430 family that allows one to derive features from the accelerometer data and to estimate the likelihood of a fall. Patterns of accelerometer data associated with a fall will be established based on an existing biomechanical model.

We plan to recruit a group of elderly individuals who report frequent falls. We will compare the proposed system and the Philips Lifeline product. Subjects will be given two weeks to familiarize themselves with each of these technologies. They will be tested with both technologies via a cross-over design. The order in which the technologies are presented to the subjects will be randomized. A questionnaire will be completed for each technology and results concerning wearability of the systems, subject's compliance with the use of the system, level of acceptance and perceived usefulness of the system, and obtrusiveness of the system will be compared for the newly-developed technology and the Philips Lifeline product.

Year 1 of the project will be focused on finalizing the development of the above-described system. As part of the development of the system, we will carry out extensive biomechanical simulations related to falls detection. Year 2 of the study will be devoted to collecting data in the field and carrying out extensive data analyses. Simulated fall conditions will be analyzed using receiver operating characteristics to determine operating points of the algorithm for fall detection to be implemented on the body-worn units. Simulated falls will also be analyzed on the robotic platform to test the ability of the robot of identifying false positives without compromising the sensitivity of the system. These simulations will include video and audio data. Questionnaires will be gathered from individuals participating in the study and analyzed to compare the two technologies undergoing assessment.

We have already performed a preliminary evaluation of the robotic platform that we propose to use in the study. However, we still plan to perform an extensive assessment of the robot shown in *Figure 1* and opt for a different platform if necessary. Home robotics is a fast growing field and there are a number of platforms that we could rely upon if the one manufactured by iRobot is deemed to be inadequate. The proposed study will allow the development of a new system and its comparison with an off-the-shelf system for fall detection in the home environment.

Using Interactive Tabletop Technology to Direct Home Rehabilitation

The development of tabletop and interactive surfaces has revolutionized human-computer interaction. Tabletop and interactive surfaces are ideal for the implementation of interactive games. The physical interaction of a single user or multiple users with the interactive surface is particularly appealing in rehabilitation. Reaching movements and the manipulation of objects are essential elements of therapeutic interventions aimed at improving motor functions in individuals with mobility-limiting conditions such as cerebral palsy. The use of interactive surfaces provides an unprecedented opportunity to motivate the subject to reach for virtual objects and manipulate them on the screen. Interactive gaming has been utilized extensively in rehabilitation to motivate subjects to perform motor tasks that are important in rehabilitation. An example of the use of this technology in rehabilitation is the use of the Nintendo Wii, which has elicited a great deal of interest in the rehabilitation community. The use of interactive gaming is particularly appealing in the pediatric population where traditional therapeutic interventions have failed in engaging the child. This limits the benefits possible with the therapeutic exercise undertaken. On the contrary, children will likely respond well to stimuli provided within an interactive gaming context with the potential for significant therapeutic benefits.

Presently interactive gaming platforms (like the Nintendo Wii) are not totally suitable for the implementation of rehabilitation interventions. This is because interactive gaming platforms are not designed for rehabilitation and therefore do not provide control of the type and quality of movements performed by patients. For instance, the tennis video game on the Nintendo Wii platform allows patients to play either with limited movements of the wrist (i.e. waving the Wii Remote) or properly swing the arm with a large range of motion at the shoulder. In a standard therapeutic scenario, clinicians need to have control of the type and quality of movements performed by patients and assign the patient to specific exercises that target the execution of shoulder and elbow movements and other exercises that are specific of wrist and hand movements. Recent advances in miniature sensor technology have the potential to address the above-summarized limitations of existing interactive gaming platforms. Specifically, wearable sensors are currently available that allow one to track movements of the body and determine the type and quality of movements performed by patients.

In the proposed project, we plan to utilize tabletop and wearable technologies to implement therapeutic interventions based on interactive gaming. The project will be carried out over a period of two years. During Year 1, we will focus on assessing the suitability of the above-described platform for the implementation of games aimed at improving motor functions in children with cerebral palsy. This part of the study will be focused on fine-tuning the platform to maximize efficacy of the tools we are developing. We will rely on games that children can play on their own as well as games aimed at improving their interaction with others, including the therapist, their parents, and other children. During Year 2 of the

project, we will focus on a comparative assessment of interventions based on tabletop and wearable technologies and standard physical therapy interventions. Spaulding Rehabilitation Hospital has a large pediatric program overseen by Dr. Donna Nimec with whom we have been collaborating on clinical projects over the past six years. Dr. Nimec works very closely and provides pre-surgical clinical gait evaluations for the surgical team at Children's Hospital, Boston. About 1000 children with cerebral palsy receive clinical services through the unit directed by Dr. Nimec. We will work with Dr. Nimec to recruit children with cerebral palsy showing impaired reaching and hand dexterity. A group of children will undergo a rehabilitation program based on the use of tabletop interactive games. A second group will undergo physical therapy in the outpatient setting. Functional outcomes will be compared in the two groups to test the hypothesis that improved function can be provided via the intensity of motor training delivered by using interactive gaming tools. Future studies will explore the use of these tools for home-based therapy.

APPENDIX C

Examples of CER in the area of Health Promotion and Wellness Interventions for People with Disabilities

A Comparison of Traditional Community Based Mentoring Interventions and Efficacy- Based Wellness Coaching in Promoting Healthful Physical Activity and Nutrition for Overweight/Obese Adolescents with Disabilities

Nature of Problem or Research Question:

The prevalence of overweight and obesity in children and adolescents has increased dramatically in recent decades. Data from the National Health and Nutrition Examination Surveys (NHANES) collected in 2003–2004 indicate the prevalence of overweight individuals by ages 2–5, 6–11, and 12–19 are 13.9%, 18.8%, and 17.4%, respectively. Examination of historical NHANES data reveals that the prevalence of childhood obesity has approximately tripled during the past 30 years, mirroring the increased prevalence among adults. Our current research at the University of Illinois at Chicago (NIDRR Grant No. H133A060066) examined the prevalence of obesity and obesity-related secondary conditions in a national sample of 662 youth with disabilities ages 12-18 years. Prevalence of obese youth with disabilities was found to be significantly higher (17.5%) compared to youth without disabilities (13.0%), and more than 70% of the participants reported having at least one secondary condition including gastrointestinal problems, sleep apnea, asthma, depression, low self-esteem, and fatigue. Youth with disabilities are also more likely to live sedentary lifestyles than their non-disabled peers and there is compelling evidence showing an association of physical activity, sedentary behavior, and overweight/obesity (Zoeller, 2009). Obesity is a major public health issue among youth with disabilities. Despite this urgent need for interventions, there is a void in the literature on successful interventions for overweight youth with physical disabilities.

Impact/Utilization:

Adolescence is an important developmental period during which youth with and without disabilities develop much of the self-concept, attitudes and behaviors they will carry into adulthood. Effective, evidence-based health promotion interventions during this developmental period are direly needed so that youth and their families can establish the requisite self-management skills and health behaviors that will promote good health and reduce the risk of chronic and secondary conditions in adulthood.

Nominated Intervention (1):

Effectiveness of a one-to-one, community-based youth mentoring program to increase physical activity and promote healthier nutrition among overweight youth with disabilities.

Summary of Research Findings to date:

One-to-one youth mentoring programs such as *I can do it-You can do it* and *Kids Enjoying Exercise Now (KEEN)* have been shown to have a significant and positive impact on young people's lives, particularly for those youth found to be at highest risk (Catalano, et al 2004; Beir, et al 2000). Despite the popularity of such programs, few existing programs have been evaluated with the necessary methodological rigor to determine efficacy of program practices. Well-controlled, carefully designed comparative effectiveness studies are needed to establish the utility and efficacy of these programs within the broader health promotion programming context. A preliminary evaluation of the *I can do it-You can do it* program has shown mixed results and indicates the need for further research (Final Report on the Evaluation of the I Can Do It, You Can Do it Health Promotion Intervention, 2007).

Nominated Intervention (2):

Effectiveness of a telephone-based personal health behavior coaching intervention (Personalized Exercise/Nutrition Prescription or "PEP") to increase physical activity and promote healthier nutrition among overweight youth with disabilities.

Summary of Research Findings to date:

Findings from adherence and motivational research indicate that participation in health promotion (i.e., physical activity and nutrition) is far more likely when the programs are customized to address the unique needs and concerns of the individual user. Our previous research has shown that an intensive telephone-based personal health behavior coaching intervention can empower severely obese participants with mobility disabilities to make substantial increments in physical activity and improved nutritional habits, resulting in a significant reduction in BMI. The strength of person-centered programming lies in being able to develop recommendations for the individual that are realistic and achievable within the context of his/her circumstances and environment.

The proposed PEP + youth wellness coaching intervention uses information technology to provide wellness coaches with rapid access to evidence-based strategies for increasing physical activity, improving nutritional habits and improving the overall health status of participants. The PEP+ approach focuses on empowering youth to self-manage key health behaviors through positively focused steps toward developing greater self-efficacy for these behaviors (Rimmer & Rowland, 2007).

Proposed Study Design:

The proposed randomized controlled trial will assign participants to one of two physical activity and nutrition intervention conditions: (1) a traditional community-based mentoring approach such as *I Can Do it – You Can Do it*, or 2) *PEP+ Youth Wellness Coaching*.

Sample: Stratified random sampling will be employed in assigning participants to the two treatment conditions to assure the groups are comparable in terms of type and severity of disabilities represented. Recruitment size will depend on the amount of funding allocated for this comparative effectiveness study.

Inclusion/Exclusion: Participants must also meet the following eligibility criteria: (a) age 14-18 yrs; (b) have written permission from their physician to participate in the study; (c) have the ability to use hands and arms independently to exercise; (d) Percent Body Fat $\geq 85^{th}$ percentile based on triceps skinfold measurement; (e) have the ability to converse in English and complete activity monitoring report forms; (f) not be currently enrolled in a health promotion program; (g) have a parent or guardian sign the agreement to support recommendations of the wellness coach and; (h) have a sedentary lifestyle over the past 6-months as measured by the module on moderate and vigorous physical activity from the CDC Youth Risk Behavioral Surveillance System (YRBS).

Data Collection Plan Anticipated: Primary Outcome measures will include physical activity levels, nutrition intake, and self efficacy to exercise. Secondary outcome measures include quality of life and participation. Other data collected include barriers to physical activity and healthy eating, medications, and demographics. Data will be collected at the following points: screening, pre-testing, post-testing, and follow-up.

Data Analysis Plan Anticipated: To test the hypotheses concerning the effects of the intervention on primary and secondary outcome measures, a series of 2 (treatment 1 vs. treatment 2) by 2 (pre-test vs. post-test) mixed factorial analyses of variance (ANOVA) will be performed, employing Type III sums of squares. Significance will set at the .05 level. Any significant interaction effects will be evaluated through post-hoc t-tests. Adherence to and success of the intervention condition will be assessed using select criteria that are based on questions asked during the follow-up interview related to their participation in physical activity and adopting healthy eating behaviors.

Feasibility Assessment:

Threats to Implementation: A threat to implementation of this research would include any issue that would prevent or inhibit the recruitment of study participants or the ability to reach participants for data collection or coaching calls.

Threats to study completion: Threats to study completion may include any problems with participant retention or an inability to complete follow up measures with participants.

Potential Threats to Generalizability: Generalizability, or external validity, may be threatened on the ecological or population level. Threats to population validity could include the possibility that our youth with disabilities are misrepresentative of the general population of youth with disabilities. Potential threats to ecological validity include the possibility that the intervention is

affected by factors related to the period of time (historical, seasonal, etc.) in which the intervention takes place, by personal attributes of the staff implementing the intervention, or by effects related to the act of participating in a study itself, such as the Hawthorne effect or testing sensitization.

Comparing two established health promotion interventions; the Chronic Disease Self-management program and the Living Well with a Disability Program

Nature of Problem or Research Question:

The barriers people with disabilities encounter in maintaining and improving their health status are numerous and interrelated (1, 2). For these individuals, functional loss leads to unique self-management needs even as it limits opportunities for health improvement. Even more, ability to participate in the vast array of community activities enjoyed by most people who achieve and maintain good health status is limited for those with disabilities. Hence, the reinforcement contingencies to develop and maintain a healthy lifestyle are less salient and available to people with disabilities. Lorig et al. (3) reported those who did not complete the Chronic Disease Self-Management Program reported significantly fewer minutes of aerobic exercise per week and higher levels of activity limitation, pain/physical discomfort, fatigue, and health distress than completers.

The benefit of developing a healthy lifestyle is essentially two-fold. First, healthy lifestyles can reduce and even eliminate symptoms of chronic disease and permanent injury. Second, improved health status improves an individual's ability to fully participate in community. This research project will compare two evidence-based health education programs; the Chronic Disease Self-Management Program (CDSM; 3) and the Living Well with a Disability Program (LWD; 4-6). The CDSM program focuses primarily on symptom reduction while the LWD program addresses both symptom reduction and improved participation. The study will compare the effectiveness of each program on the health status of people with a disability compared to those without a disability.

Impact/Utilization: Study results will lead to a better understanding of how disability interacts with health behavior change. For people with a disability, a Living Well with a Disability may be more effective than the Chronic Disease Self-Management Program.

Nominated Intervention (1): Living Well with a Disability

The Living Well with a Disability health promotion program is a health education intervention that was developed from the premise that people with functional loss will be more apt to make healthy behavior choices when those choices are organized to facilitate achievement of specific important long-term goals.

Summary of Research Findings to Date: In a randomized staggered baseline design, workshop participants (i.e. people with mobility impairments) reported numerous statistically significant changes including a 13% reduction in limitation due to secondary conditions, a 13% improvement in health related quality of life (i.e. symptom days), a 5% increase in healthy

behavior and a 67% reduction in healthcare costs during the intervention period. Many of these effects were maintained over 12 months (5). Comparing these outcomes to individuals not receiving the intervention, workshop participants were three times more likely to be below the median on limitation from secondary conditions (AOR = 1.94 (1.03, 3.67)) twice as likely to be below the median of unhealthy days and (AOR = 3.05 (1.33, 7.01)), twice as likely to be below the median for health care costs (AOR = 1.96 (0.91, 4.26)) than those who did not receive the intervention (4). These results on secondary conditions mirrored those of a separate study (6).

Nominated Intervention (2): The Chronic Disease Self-Management Program is a health education program designed to address common symptoms of chronic disease. The intervention utilizes self-efficacy theory to guide interventions that elicit health behavior change.

Summary of Research Findings to Date: "As compared with controls, the treatment group demonstrated significant improvement in four health behavior variables ($P < 0.01$; number of minutes per week of stretching/strengthening and aerobic exercise; increased practice of cognitive symptom management; and improved communication with their physician). They also demonstrated significant improvement in five health status variables (self-rated health, disability, social/role activities limitation, energy/fatigue, and health distress; $P < 0.02$). No significant differences were demonstrated for pain and physical discomfort, shortness of breath, or for psychological well-being. The treatment group, as compared with the control group, had fewer hospitalizations ($P < 0.05$) and spent, on average, 0.8 fewer nights in the hospital ($P = 0.01$)" (3).

Proposed Study Design: A randomized controlled trial with repeated measures. Subjects will be stratified by disability status and randomly assigned to either the CDSM or the LWD program. Outcomes will be collected using known outcome measures to examine health behavior change, health outcome, health related quality of life and healthcare utilization and life satisfaction.

> ***Sample:*** People with chronic illness or permanent injuries ages 18-70 stratified by disability defined by regular use of mobility equipment.
> ***Exclusion:*** People with co morbid psychiatric conditions other than depression.
> ***Timeline:*** 2- year cost-effectiveness study with 6-months post-intervention follow-up data collected.
> ***Data Collection Plan:*** Self-report staggered baseline design with pre-, post-, and 6-month follow-up.

Data Analysis Plan Anticipated: Repeated measures analysis of variance with between subject factors to include disability status and intervention group

Feasibility Assessment: *Threats to Implementation* - Each of the interventions have been implemented successfully in both research and dissemination frameworks. Subject recruitment

will need to be addressed with significant staff time devoted to recruitment and maintenance of the study sample. *Threats to study completion*- slow rate of subject recruitment.

Potential Threats to Generalizability: Treatment fidelity and sample recruitment will be key to assure results are not merely a reflection of the most motivated community-dwelling adults who receive a standardized treatment within a research protocol. Each intervention uses facilitator training and a curriculum to maintain program fidelity.

Low employment rate of individuals with psychiatric disabilities

Impact/Utilization: The development of effective models to help individuals with psychiatric disabilities enter the workforce has the potential to enhance their economic security, quality of life, and community inclusion. The labor force participation of such a sizable group of individuals on the SSI/SSDI roles would also stimulate our nation's economy in the form of economic contributions through federal and state income taxes as well as sales tax paid on purchases, and growth of the economy's sales sector through an increased ability to make purchases.

Nominated Intervention (1): Supported Employment (SE)

Summary of Research Findings to date: SE is an evidence-based practice in the field of psychiatric disability, supported by numerous single randomized controlled trials (RCTs) as well as one large national multi-site study called the Employment Intervention Demonstration Study or EIDP (http://www.psych.uic.edu/eidp/).

Nominated Intervention (2): Customized Employment (CE)

Summary of Research Findings to date: CE is a promising practice developed by the USDOL, ODEP and evaluated in a national demonstration program with a non-randomized, pre-post design.

Proposed Study Design:

Design - Randomly assign 300 subjects with psychiatric disabilities to SE vs. CE and follow them for 1 to 2 years.

Sample (include target disability group, age group) – Individuals with psychiatric disability age 18-55 (or older)

Inclusion/Exclusion – Desire to work, willingness to participate in the research, willingness to allow access to service utilization data, earnings data, spending patterns, and clinical data

Timeline – This could be a 2 to 3-year project with a 12 month follow-up; or a 3 to 4-year project with a 24 month follow-up. Either would be valuable.

Data Collection Plan Anticipated – Vocational outcome data would be tracked weekly via telephone of employment, using the EIDP protocols. Services could be tracked on a monthly basis via telephone or electronically if service data are available and subjects provide consent. Changes in psychosocial outcomes (self-esteem, recovery) and behavioral changes (monthly spending, taxes paid) could be tracked through semi-annual interviews.

Data Analysis Plan Anticipated – Given the nature of outcome variables (interval level measures such as earnings and job tenure, and ordinal measures such as employment status and job benefits), longitudinal random regression analysis would be the appropriate statistical technique for use with these data.

Feasibility Assessment:

Threats to Implementation – Implementation threats include the unwillingness of subjects to allow access to sensitive mental health clinical data; fear of loss of SSI/SSDI and other benefits and entitlements; and hesitation to allow access to private earnings data and information regarding job loss. Money would have to be made available to fund the SE and CE service delivery and some level of assurance would need to be provided that employment services and supports would be ongoing following the completion of the research study.

Threats to study completion- Completion could be threatened by the uncertain economy and high unemployment rate in many parts of the U.S.

Potential Threats to Generalizability: Unless this was a national study with a larger sample size, the results would only be generalizable to the local area from which the sample population is drawn. A multi-site study would ameliorate this somewhat, although it would still not constitute a nationally representative sample.

Comparative Effectiveness of Work on Improving Health Status and Quality of Life for Low Income Persons with Disabilities Insured by Medicaid

Nature of Problem or Research Question: Poverty and unemployment for persons with disabilities are much higher than that of the general population, at least in part because increased employment can jeopardize individuals' federal disability cash benefits, health care coverage and health status through Medicare and/or Medicaid. Medicaid Buy-In programs allow people with disabilities to work, accumulate assets, and maintain Medicaid coverage. In 2007, nearly 106,000 people with disabilities participated in Medicaid Buy-In programs in 34 states. Although enrollment in these Buy-In programs has consistently grown over the years, little is known about how integrated employment affects health outcomes. Many researchers have documented the relationship between poverty and poor health status, but little research has focused on poverty in combination with disability. Our research question is: does working improve or diminish health status and quality of life for low-income people with disabilities who are insured by Medicaid?

Impact/Utilization: Because of the existing dearth of evidence related to the effectiveness of work programs for persons with disabilities, findings from this research will impact the way in which front line service providers and medical practitioners support work efforts of low income people with disabilities. Results can be shared with service providers and medical practitioners through dissemination to relevant professional organizations and databases both in this country and around the world, where many industrial economies are grappling with similar issues related to integrating their disability populations into the modern workforce. It is unknown at this time how significant the joint effects of insurance and work are on the health of low-income people with disabilities.

Nominated Intervention (1): Integrated employment for low income persons with disabilities being insured with Medicaid

Summary of Research Findings to Date: Liu, Ireys, and Thornton (2008) reported profiles of Medicaid Buy-In participants in 27 states, finding that Buy-In participants tended to be older than other persons with disabilities insured with Medicaid, and that about one-third had mental illness. No studies to date have utilized a comparison group analysis in order to understand social determinants such as age, gender, disability type, work history and attitudes, education level, self esteem, and quality of life factors as they relate to participation in a Buy-In program. Nor have any studies compared health outcomes of Buy-In enrollees with non-enrollees. Preliminary findings among persons enrolled in the Kansas Buy-In, Working Healthy, indicate that participation not only allowed for increased income, but more consistent access to Medicaid coverage and services (Hall & Fox, 2004; Hall, Fox, & Fall, 2009). Participants' average annual earnings, while still very low at under $8,000, increased over time and contributed to a sizable

increase in state and federal payroll taxes. But the degree to which work enriches health status and quality of life for persons with disabilities who are insured by Medicaid remains unknown.

Nominated Intervention (2): Traditional Medicaid insurance coverage for low income persons with disabilities that does not include integrated employment.

Summary of Research Findings to date: Hanson, et al., 2003 documented the hardships faced by persons with disabilities who have no insurance. They also identify the unique challenges that low income persons with disabilities face in assuring adequate medical care, even if they have Medicaid, because so few providers are willing to accept Medicaid payment. Once working-age people with disabilities have qualified for Medicaid, they are subject to strong incentives to remain poor, being forced to avoid working at gainful employment levels to remain eligible. Weiner (2003) suggests that working may put persons with disabilities at higher risk for adverse health outcomes. This line of reasoning suggests that not working while receiving Medicaid benefits could enhance health status and quality of life for persons with disabilities.

Proposed Study Design:

Design: Quasi-experimental, longitudinal case-comparison study

Sample: Data will be collected for the entire enrolled population of Working

Healthy (approximately 1,100 people as of April, 2009) and data for a comparison group of 1,200 individuals who are working age, disabled, and dually-eligible for Medicaid and Medicare.

Inclusion/Exclusion: The sampling frame includes all persons eligible for enrollment in the Kansas Medicaid Buy-In program (*Working Healthy)*, so that persons who enroll (cases) and persons who do not enroll (comparison group) are selected.

Timeline: We will access four years of historical and one year of current data, giving us the ability to examine longitudinal trends in health care utilization and costs as well as earnings.

Data Collection Plan Anticipated: We will utilize both administrative and self-reported data to fully understand the effect of enrollment in Working Healthy on health outcomes for low income individuals with disabilities. The International Classification of Functioning (ICF) framework for disability classifications will guide our selection of specific data elements in order to produce findings that are comparable to other disability research. Baseline and follow-up surveys of both enrollees and non-enrollees will address respondents' self-reported levels of self-esteem, quality of life, work attitudes, health status, and various demographics including gender, age, race, ethnicity, disability type(s), number of disabilities, employment history, earned and unearned income, and educational level. Some health status and quality of life items will be drawn from the SF-12v2 and WHO-QOL instruments (Bonomi & Patrick, 1997). These items will be added to an existing annual survey of the Working Healthy participants and incorporated into a new survey instrument for the comparison group. Various state and federal administrative

data including Medicaid, Medicare, as well as income tax and unemployment compensation records will be obtained through a business associate relationship with the state Medicaid agency and interagency/data use agreements with the Region VII office of the Centers for Medicare and Medicaid Services (CMS), and the Kansas Departments of Revenue and Labor. Data will include Medicare and Medicaid utilization and expenditure levels for outpatient, inpatient, and, for Medicaid records, pharmaceutical services, and gross income levels and earnings levels.

Data Analysis Plan Anticipated: We will use mixed model analyses. The mixed model framework will allow differences in initial levels of the dependent variable as well as differential change over time between groups to be modeled. The quality of life outcome variables of interest with respect to health care utilization over time are relative disease burden; inpatient, outpatient, and emergency department use; co-morbidities; and overall costs. Baseline scores will be used as covariates in the models with group membership and time as the primary independent variables. Relative disease burden will be calculated using Johns Hopkins Adjusted Clinical Group (ACG) Case-Mix System software, version 8.2 (2009). We will use mixed models to compare adjusted gross income (AGI) and earned income for both sample groups over time, using tax and unemployment compensation information as dependent variables, baseline scores as covariates, and group membership and time as independent variables. Logistic regression will be used to identify disparities in social determinants of health.

Feasibility Assessment:

Threats to Implementation: Integration of data from multiple sources has historically posed a major barrier to this type of comparative effects research. Past research either has been confined to using Medicare, Medicaid, self-reporting, or income data (such as Social Security or unemployment compensation data) to capture items such as health care services or monthly income. By linking these data sources to follow participants' health and personal experiences over time, we hope to address many of the shortcomings typically encountered when using selected administrative data sets for persons with disabilities. We will build upon our previous work in this area. More general difficulties associated with the use of administrative data for health services research are well known. They include issues related to confidentiality, linkage technology, costs, uniformity of and access to data, among others (Black & Roos, 1998; Roos et al., 1999).

Threats to study completion: None.

Potential Threats to Generalizability: While efforts will be made to describe the population and adjust for all measurable cofactors, there may be limitations to generalizability based on our one state sample.

Comparing established weight-loss interventions with a promising alternative

Nature of Problem or Research Question:

Research has shown that people with IDD have poorer health than peers without disabilities (Horwicz, Kerker, Owens, & Zigler, 2001; U.S. Office of the Surgeon General, 2002). In the past decade, government initiatives such as Closing the Gap: a National Blueprint to Improve the Health of Persons with Mental Retardation (2002) and Call to Action to Improve the Health and Wellness of Persons with Disabilities (2005) have helped to set the policies later described in Healthy People 2010. A recent review of work to date on translation of these policies into practice with people with IDD (Krahn & Drum, 2007) indicates that health promotion programs have been effective in improving quality of life, especially in the self-reported lifestyle behaviors. One area that has not shown improvement, however, is the teaching of good nutrition choices of persons with IDD, especially those living in group homes. Our observations lead us to conclude that many persons with IDD want to lead a healthy lifestyle, including eating and drinking in a healthier manner. Their environment makes choosing healthy alternatives difficult however. As a result, adults with IDD have more than 1.5 times the prevalence of obesity than in the general population (Rimmer & Yakima, 2006). Krahn and Drum conclude that in order for future health promotion strategies to be effective, environmental factors must be considered. This study will compare the effectiveness of two programs that promote weight loss, one through environment change and teaching ( ), and another through teaching along (usual care).

Impact/Utilization: Funding and Implementation of this proposal will have the following known and possible impacts:

1. Over a 2-year project, infuse hundreds of thousands of dollars into rural and generally depressed areas of Kansas.

2. Anticipated results, based on our pilot data include:

   a) significant weight reduction by 85%or more of IDD participants in Intervention 2.

   b) an increased empowerment of the IDD participants in Intervention 2 to be responsible for and in control their energy consumption

   c) reductions in costs for medical services and medications under Medicaid for the participants in Intervention 2.

3. Promote a “sea change” in the attitudes of those most invested in supporting people with IDD with regard to what individuals with IDD want in the way of healthier lifestyles and what they are willing to do to achieve them.

Nominated Intervention (1): Using the principles of volumetrics, the diet approach (Pictorial 5-3-2) involves teaching and coaching the participants about better nutritional habits, and changing the environment by using portion control and by replacing unhealthy foods available with health alternatives. This study has been studied extensively with typical adults (cites) and we recently conducted a pilot study of 77 individuals, funded by the Kansas Council on Developmental Disabilities and the U.S. Administration on Developmental Disabilities.

To match the level of understanding of the participants with IDD, the instructions for following the diet were modified from their usual printed form to be nearly entirely pictorial. Pictures were used in materials that were intended to guide the dieter in food planning, purchase and preparation. Pictures also were used in materials that participants used to record what they consumed each day. Where pictures were not relevant, we used extensive use of color-coding to guide the dieters (e.g., individual weight charts).

Summary of Research Findings to Date: Weight loss in this pilot averaged 6% of baseline weight at 6 months. Thru March, 2009, some early enrollees have completed 18 months in the project and others 12 or 9. The current data are shown in the table below. Starting average Body Mass Index (BMI) was 37.0. BMI is calculated as weight (kg)/height (m^2) and a healthy, normal BMI is considered to be under 25.

Time in diet	**Percentage loss from baseline weight**		
	Mean	Median	Range
6 mo (N=77/77)	6.1%	6.09%	0 - 19.55%
9 mo (N= 56/77)	9%	7.71%	0 - 27.08%
12 mo (N=30/77)	9.3%	8.18%	0 - 28.05%
18 mo (N=18/77)	12%	11.53%	0 - 28.5%

As part of our pilot, we provided a questionnaire that is required by all projects funded by the Administration on Developmental Disabilities. The results were:

- "I was treated with dignity and respect during the project activity." Yes=100%; No=0%
- "I have more choice and control as a result of this project activity." Yes = 98%; No=2%
- "I can do more things in the community as a result of this project activity." Yes=92%; No=8%

- "My life is better because of this project. Strongly Agree=72%; Agree=26%, Strongly Disagree=2%

Nominated Intervention (2): The Usual Care (UC) diet, as recommended by the National Heart Lung and Blood Institute (National Institutes of Health) (1998), should be compared with any novel approach because it is the standard diet recommended by health promotion programs for all people, including those with IDD.

Summary of Research Findings to Date: To date, little data on the effects of these programs on weight loss with individuals with disabilities have been published.

Proposed Study Design: This study will use a randomized controlled trial with repeated measures. Subjects will be stratified by type of residential accommodation and randomly assigned to either Intervention 1 or 2. Outcomes will be collected using known outcome measures to examine weight loss, changes in health (e.g, disease status, medication change, etc.), and changes in community participation.

Sample: People with disabilities who are overweight and between ages 18-70

Exclusion: People with cancer, heart disease, or metabolic disorders

Timeline: 2- year cost-effectiveness study with 6-months treatment and comparison of 12 month pre-treatment health care utilization data (Medicaid) with 12 months post-treatment follow-up.

Data Collection Plan: Monthly measurement for 18 months

Data Analysis Plan Anticipated:

Weight loss after 6 month weight loss intervention	• Descriptive statistics • Two sample t-tests comparing difference between Pictorial 5-3-2 and UC diet groups • Multiple linear regression to assess impact of covariates
Weights measured at 6, 12, and 18 months after intervention	• Linear mixed model to evaluate weight change over time • Mixed linear model to assess impact of covariates
SPARC score indicating level of community participation	To analyze pre and post- intervention data: • Wilcoxon-Mann-Whitney U test • Independent samples t-test
Using Medicaid claims data, measure change in disease prevalence and health care utilization, including pharmaceutics, lab, in patient, and out	• Descriptive statistics, Chi2 and t-tests, as appropriate • Multivariate models to assess impact of covariates

patient pre- and post-intervention	

Feasibility Assessment: *Threats to Implementation:* None; study has already been completed with pilot sample with considerable success. Current waiting list for future funded projects. *Threats to study completion*- slow rate of subject recruitment.

Potential Threats to Generalizability: The pilot achieved equal success with both genders, individuals with varied diagnoses, individuals on medications with weight gain as a known side effect, individuals with and without physical disability, individuals with and without diagnosis of mental illness, and individuals from various types of residential accommodations.

Nomination Form for Tobacco Control Comparative Effectiveness Research Project

Nature of Problem or Research Question:

Individuals with a range of disabilities experience differential levels of health and health-related quality of life compared to the general population. McGinnis, Williams-Russo, and Knickman (2002) reviewed U.S. research on five key factors (genetics, social circumstances, environment, individual behaviors, and access to medical care) that contribute to overall health among the general population. Their estimates of the contribution of each factor included: genetic predispositions (30%); social circumstances (15%); environmental conditions (5%); access to medical care (5%); and individual behaviors (40%). Although McGinnis makes a reasonable case for these estimates as applied to the general population, it is unlikely that they are accurate estimates for persons with disabilities. Nevertheless, individual behaviors, such as tobacco use, are likely to have similar or greater effects on the heath of persons with disabilities.

Cigarette smoking remains the leading preventable cause of death in the United States,[1] accounting for approximately 1 of every 5 deaths (438,000 people) each year.[2,3] An estimated, 20.8% of all adults (45.3 million people) smoke cigarettes in the United States.[4] Despite widely recognized negative consequences associated with tobacco use, there are higher rates of cigarette smoking among disability populations compared to the general adult population. According to the Centers for Disease Control and Prevention (CDC) using data from the Behavioral Risk Factor Surveillance System (BRFSS), approximately 30% of people with disabilities are smokers.

Although tobacco control has been a major health promotion focus and includes a number of evidence-based interventions, little research has been conducted to determine the effectiveness of tobacco control health promotion measures when tailored toward individuals with disabilities compared to tobacco control interventions that do not tailor activities to persons with disabilities.

Impact/Utilization:

Tobacco use accounts for more than $190 billion annually in direct and indirect medical costs, and at least 8.6 million Americans are living with at least one serious illness caused by tobacco use. Furthermore, exposure to secondhand smoke causes premature death and disease in nonsmokers, with costs in the United States estimated at $10 billion per year (CDC, 2007). Of the approximately 54 million adults with a disability, extrapolating from CDC prevalence estimates, over 16 million are smokers. Reduction in smoking rates among individuals with disabilities should result in decreases in medical costs and reduction in mortality.

Nominated Intervention (1):

State based comprehensive tobacco control programs (TCP) encompass coordinated efforts to establish smoke–free policies and social norms, promote and assist tobacco users to quit, and work to prevent initiation of tobacco use. The four components of TCP are:

- Population-based community interventions
- Counter-marketing
- Program policy/regulation
- Surveillance and evaluation

Summary of Research Findings to date:

Greater investments in state tobacco control programs are independently and significantly associated with larger and more rapid declines in adult smoking prevalence, according to the CDC. According to a CDC report ("The Impact of Tobacco Control Programs on Adult Smoking," also published in the February 2008 issue of the *American Journal of Public Health*) using data from all 50 states and the District of Columbia, declines in adult smoking prevalence among individual states were directly related to increases in state per person investments in tobacco control programs, independent of price increases (CDC, 2007). These results re similar to reports issued in 2007 from the Institute of Medicine, the National Institutes of Health, and the President's Cancer Panel that all concluded that comprehensive state tobacco control programs are effective public health investments (CDC, 2007).

Nominated Intervention (2):

State based comprehensive tobacco control programs that include disability issues in their initiative.

Summary of Research Findings to date:

No published reports that include people with disabilities as a target group, the State of Oregon obtained disability data in developing their TCP but no results are available.

Proposed Study Design:

Design- Embedded multi-site case study with matched comparisons

Sample (include target disability group, age group)- 2 to 6 states.

Inclusion/Exclusion- inclusion factors for matched comparisons could include population size, racial and ethnic factors, SES, and type of disability.

Timeline- Because of the time lag between TCP initiation and measurable results, this most feasible as a five year project or longitudinal.

Data Collection Plan Anticipated- Baseline and annual comparisons of matched states.

Data Analysis Plan Anticipated- The CDC has developed a range of recommended surveillance (health status, quitline data sets, etc), evaluation (e.g. process and outcome measures, etc), and expenditure measures for TCP. Qualitative assessments of disability

enhanced initiatives should also be conducted. Case reports should include comparisons between all data elements.

Feasibility Assessment:

Threats to Implementation- Although all 50 states and DC receive CDC funds for tobacco control, only a handful of states' total funding is at levels recommended by the CDC. States would have to agree to include disability as a target group.

Threats to study completion- Decreases in TCP funding due to economic issues or re-allocation of resources.

Potential Threats to Generalizability:

Use of case study approach.

Exergame cycling compared to standard exercise cycling.

Nature of Problem or Research Question: Diabetes mellitus is a major clinical and public health problem. This research aims to investigate what are ethnic group differences in physical activity, functional mobility and self-management among older women with type 2 diabetes, and if these can be mitigated with an innovative exercise strategy versus traditional strategy.

Impact/Utilization: Regular participation in moderately intense physical activity is associated with a substantially lower risk of type 2 diabetes and improved functional outcomes (Jeon et al 2007).

Nominated Interventions: Exergame cycling compared to standard exercise cycling.

Summary of Research Findings to date: The *Games for Health* Initiative is a project that applies cutting edge games and game technologies to develop a community and best practices platform for the numerous games being built for health care applications (www.rwj.org).

Proposed Study Design:

> **Specific Aim 1:** To characterize ethnic group differences in physical activity and functional mobility among older African American women and non-Hispanic white women with Type 2 diabetes.
>
> **Specific Aim 2:** To characterize potential psychosocial and sociocultural contributions to ethnic group differences in physical activity, functional mobility and diabetes self-management among African American and non-Latino White women ages 50-75 years.

Study Setting and Number of Subjects. Measures of physical activity, functional mobility and self-management will be conducted.

Sample. Wmen aged 50-75 years of age with doctor-diagnosed type 2 diabetes will be recruited from two ethnic groups (African American women and non-Hispanic White women).

Data Collection Plan. Physical Activity (Measure of clinical pain). Acute exercise provides an

experimental model for manipulating naturally occurring pain (Cook et al 2004). Measures of clinical pain, physical activity and functional mobility will be assessed using two types of light cycle fitness activities; exergame cycling (cycling while playing an on-screen video game), and standard cycling (cycling without playing the game activity) for approximately 15 minutes each; a total of 30 minutes. Healthy People 2010 recommends physical activity for at least 30 minutes, 3 times per week. Cycling activity will be standardized across participants to achieve mild to moderate levels of exertion. After each 15 minutes exercise period, participants will rate pain intensity from 0-100 using a Visual Analog Scale (VAS). Participants will have two 15-minute rest periods. During the rest periods, participants will provide VAS ratings (0-100) of pain every

five (5) minutes for 3 resting pain scores (rps). Each participant will engage in both types of cycling activity. In one exercise, participants will ride on the stationary bike for up to 15 minutes. In the other exercise, participants will ride the same stationary bike for up to 15 minutes, but will be playing a video game while exercising. This video game will be shown on a TV in front of the bike and will work with the bike to make the exercise more like a game. Which ride the participant will do first will be randomly selected. Participants will practice on the cycle before beginning the exercises.

Blood Pressure and Heart Rate. Blood pressure levels, heart rate readings will be recorded during each resting period. A wrist or arm mounted automated blood pressure device will be used. To assess hear rate from cycling activity, a wrist or chest-attached, heart rate monitor will be used.

Distance and Time. A record of the distance in miles and amount of time that the individual pedaled will be collected.

BioPsychoSocial Questionnaires/Inventories: Several biopsychosocial questionnaires will be used: (1) Diabetes Care Profile (DCP), (2) Diabetes Attitude Questionnaire (DAQ)—(U of Michigan, 2000), (3) Diabetes Knowledge Test (DKT), (4) Brief Pain Inventory (BPI), (5) McGill Pain Questionnaire (MPQ), (6) Michigan Neuropathy Screening Instrument (MNSI-Part I), (7) Multi-Ethnic Identity Measure (MEIM).

Data Analysis Plan: Specific Aim 1: To characterize ethnic group differences in physical activity and functional mobility among older African American women and non-Hispanic white women with Type 2 diabetes. A series of analyses of variance (ANOVA) will be used to test for differences between the ethnic groups in physical activity and functional mobility. **Specific Aim 2:** To characterize potential psychosocial and sociocultural contributions to ethnic differences in physical activity, functional mobility and diabetes self-management. A series of ANOVAs will be used to test for differences between the groups on each of the psychosocial variables. Pearson product-moment correlation coefficients will be calculated to determine associations among clinical pain, pressure pain thresholds and the psychological variables. To determine whether psychological variables mediate ethnic group differences in pain perception, for each pain measure on which group differences emerge, psychological variables that are significantly correlated with that measure will be used as covariates in a series of analyses of covariance

(ANCOVAs).

Feasibility Assessment: Threats to study completion: Recruitment and retention of participants

Potential Threats to Generalizability: May be generalizable to other rural and other ethnic groups.

Timeline. three year study

The Effectiveness of Currently Used Interventions for Language and Cognitive Development for Children with Hearing Impairments

Nature of Problem or Research Question: Historically there has been much debate about the most efficacious methodology to employ with infants, toddlers, children and youth with some form of hearing impairment that results in communication competence in English and academic achievement that culminates in employability upon high school graduation. Heated debates have occurred over decades espousing disparate methodologies including speech only, speechreading, audition, hearing aids, cochlear implantation, various forms of sign language, American Sign Language (ASL) only, and a Bi-lingual – Bi-cultural approach that encompasses emersion into the Deaf culture and ASL. Adherents of a given method steadfastly support it and there is a paucity of outcome data on what works best with which types of hearing impaired children and their families. Well-controlled comparative research studies are needed to identify them.

Impact/Utilization: Hearing impairment is an expensive disability for a child to have. The disability accounts for a significant portion of education dollars for residential schools, public school programs, interpreters, SSI benefits, vocational rehabilitation services, Medicaid billing, and public assistance funds across the lifespan. The identification of the most effective communication methodology to implement with specific categories of children given the methodology selected and implemented by their family is critically needed. Factors within the child, parents, and family impact successful outcomes. These specific variables need to be identified within each methodology to optimize their effectiveness. Research is needed to identify which communication modes are the most effective with hearing impaired children with which characteristics in which familial and contextual circumstances.

Nominated Interventions: It is proposed that a prospective population based epidemiological study be undertaken within an entire state in which there are extant data on all hearing impaired children served within the state over a 10 year period. All interventions implemented with the children would be studied. These would include: speech only, speechreading, audition, hearing aids, cochlear implantation, various forms of sign language, American Sign Language (ASL) only, and a Bi-lingual – Bi-cultural approach that encompasses emersion into the Deaf culture and ASL. Random assignment to groups would be accomplished by parental choice of the methodology implemented with their child.

Summary of Research Findings to date: To our knowledge, such a population study has not been undertaken to date to evaluate the effectiveness of a plethora of interventions with hearing impaired children. The extant research is comprised of studies with small samples of deaf and hard-of- hearing children within various intervention modes. Hofmeister (2000) identified ASL as an adjunct to reading comprehension among deaf children attending a residential school. There were no comparison groups with children instructed in a non-signing communication

mode, or children educated within the mainstream with a sign language interpreter. Much of the outcome research on the efficacy of ASL in the acquisition of English literacy has been reported on Deaf children and youth 12 years of age and older. Not surprisingly, an inverse relationship between length of exposure to ASL and English literacy has been identified among middle school and secondary level Deaf students (Strong & Prinz, 1997). Moeller (2000) found that early intervention in language development, be it auditory/oral or an English-based sign language system, was critical in the acquisition of English. Yoshinaga-Itano (2003) also reported that the combination of early intervention with either a sign language or oral/aural approach resulted in language competence. Tomblin and colleagues (1999) at the University of Iowa compared language achievement between children using hearing aides and those with cochlear implants. There were no sign language users as a comparison group. Knutson (2006) found that parental monitoring of cochlear implant usage and appropriate behavior management of the child were critical factors in speech and language development. Again, there were no comparison groups with deaf and hard-of-hearing children using other communication modalities. Furthermore, factors that help support children and families and lead to better outcomes also need to be considered in research studies. What has not been accomplished is a comparative effectiveness study of multiple communication modalities within a total population of hearing impaired children that includes family and contextual factors in analyses of communication outcomes.

Proposed Study Design:

Design: The project design would encompass a total population of hearing impaired children in a given state and encompass the collection of aggregate data on their language competence and academic achievement. Groups of children receiving each communicative mode implemented with hearing impaired children in the state would be compared for the effectiveness of the communication modes implemented with them. Outcomes would be English language competence, academic achievement, and, for older children, employment or college attendance after high school graduation.

Sample (include target disability group, age group): The entire population of children in a given state dating from 2000 to 2007 with a diagnosed hearing loss and who received educational intervention would be the subjects in the study. The children would range in age from 2 to 19 years.

Inclusion/Exclusion: Only children with a hearing loss would be included in the study. The hearing loss criteria would be bilateral, sensorineural ranging from moderate to profound.

Timeline: Two years is the typical time allowed in stimulus research. This would be sufficient time to conduct the archival record review to gather data on the variables of interest and conduct the statistical analyses.

Data Collection Plan Anticipated: Extensive archival record review of data on the children's respective communication methodology, resulting language competence, and academic achievement would be collected, coded, and stored for analyses.

Data Analysis Plan Anticipated: Logistic regression would be implemented to identify moderators and mediators of outcomes which would be communication competence, academic achievement, and employability or college entrance after high school graduation.

Feasibility Assessment:

Threats to Implementation: Conducting the study would depend on the cooperation of state agencies in possession of the archived extant databases and allowing researcher access to them. The research would be exempt from informed consent because all data gathered would be archival in nature and reported in aggregate form. Furthermore, all children within a given state with a hearing impairment for which they received some type of communication related intervention would be included and permit the use of aggregate data without name identifiers in the research.

Threats to study completion: The project would be labor intensive and require extensive archival record review and reliability checks of the data obtained. The two year time frame could potentially be a threat to completing such a study. However, with sufficient research assistants and timelines for work completion, it can be accomplished.

Potential Threats to Generalizability: The data would be generalizable to children with hearing loss in similar settings within the same state in which the study is located.

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CER Project on Residential vs. Family Based Interventions with Children and Youth with Behavioral Disabilities

Nature of Problem or Research Question: National attention was afforded the state of Nebraska when its legislature extended to 18 the age of children who could be dropped off at hospitals by parents without being charged with neglect or abandonment. This opened a floodgate of children and youth from not only Nebraska but other U.S. states. Almost 40 youth were left at hospitals within Nebraska and the majority had serious emotional and behavioral problems. Their parents were desperate because of an inability to obtain mental health services, ineligibility for such services, and refusal of treatment agencies to admit their child. The problem was magnified by the notion that the youth needed to be separated from the family in order to receive help and that the youth could be cured in the residential setting and returned to the family with all problems solved. Nebraska also received national attention when a 19-year-old youth fired an AK47 at a shopping mall early in the Christmas season of 2007. He had been removed from his home and attended multiple residential treatment centers before aging out of the system. Although a rare base-rate event, it illustrates a critical need for efficacy studies of the treatment methods afforded youth in residential placements.

Impact/Utilization: Residential mental health treatment encumbers a large amount of healthcare dollars and in spite of widespread claims of evidence based practice there is a paucity of generalizable research results that demonstrate the efficacy of residential treatment across settings with variable groups of children and youth including those with disabilities (Sullivan, 2009). Evidence-based interventions need to be validated across the settings in which they are delivered. There is a scarcity of mental healthcare dollars and research is needed to identify which treatment modality characteristics are efficacious with which types of children and youth and the contextual factors in which they reside. Removal from the family and placement in a residential setting is the most drastic intervention that can occur in the life of a child. Whether this solves a problem or makes a bad situation worse for a given child needs to be determined. Certainly contextual factors that mediate therapeutic outcomes need to be identified to guide prevention and intervention efforts.

Nominated Intervention (1): Residential Treatment Facility that employs physical and chemical restraint.

Summary of Research Findings to date: Recent reviews of the extant behavioral intervention research with children and youth by Kazdin (2008), Weisz and Grey (2008), and Boxer (2007) have lamented weaknesses in the extant research that is called evidence-based when it has not been replicated in multiple settings with youth with various types of behavior problem. Furthermore, seclusion and restraint can escalate to abuse and are controversial treatment methods that need rigorous evaluation in well controlled studies.

Nominated Intervention (2): Outpatient treatment with intensive family involvement

Summary of Research Findings to date: Chamberlain and colleagues (2002) at the Oregon Social Learning Center have demonstrated the benefits of including parents and foster parents as well as school personnel in interventions of preschool age children with behavior problems. This methodology needs to be replicated with older children and youth. There is emerging work that purports MAOA plays a role in aggressive behavior among males. A 'warrior gene' among males has been touted as a contributing factor in gang involvement among males by researchers in North Carolina. Much more research is needed in this area to identify specific environmental events that contribute to this finding.

Proposed Study Design:

Design: A randomized controlled study with the behavior disordered youth randomly assigned to residential or family-based treatment is a critical component of the research design along with close supervision and scrutiny of the dispensing of the treatment protocol to ensure adherence to it.

Sample (include target disability group, age group): The target disability groups would be children and youth between 8 and 18 years of age with behavior disorder designations. Given that many youth with behavior disorders have concomitant learning disabilities, these youth would also be included and analyzed as a separate group to determine if the additional learning disability is a factor in response to the treatments. Ideally, this would be a multi-site study with at least 60 youth per intervention group.

Inclusion/Exclusion: Children and youth with behavior disorders and children and youth with behavior disorders and concomitant learning disabilities would be included in the research.

Timeline: 2 years is the typical timeframe structure of stimulus funding. In reality, more time would be needed to test the efficacy of each treatment intervention and to determine if the treatment effects are time sensitive.

Data Collection Plan Anticipated: Data would be collected prior to treatment, immediately after treatment, and at 6 month intervals post-treatment for 1 year to determine if the therapeutic effects lasted over time.

Data Analysis Plan Anticipated: Logistic regression would be implemented to analyses and to identify mediators and moderators of therapeutic outcomes. For youth with significant violent aggression, a gene x environment interaction protocol would be an exciting component to include.

Feasibility Assessment: A major threat to such a study is having agencies that provide the intervention evaluate themselves. It is imperative that an independent entity conduct the research and evaluate the efficacy of a given intervention. Indeed, it would be efficacious if state social

service agencies were required to contract the evaluation of mental health treatment of the youth they serve to an outside university or other research entity that is not providing mental health services. This would ensure that providers are not evaluating themselves and would foster confidence in the validity of the outcome results.

Threats to Implementation: The research design requires random assignment to treatment groups. Parental or social service agency desire to determine which intervention was given to their child would contaminate the study results. Random assignment is an essential component that cannot be negotiated with parents or social service agencies.

Potential Threats to Generalizability: Self-evaluation by a treatment provider and lack of random assignment to treatment groups are also real threats to the generalizability of outcome results.

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