

THE FUTURE IS NOW

FIRST WORLD CONGRESS
ON SPINA BIFIDA
RESEARCH AND CARE
MARCH 15-18, 2009



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First World Congress on Spina Bifida Research and Care:**

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- **National Institutes of Neurological Disorders and Stroke,
National Institutes of Health**
- **Spina Bifida Association**
- **Spina Bifida Foundation**

Funding for this conference was made possible in part by grant number 1R13SH017978-01 from the Agency for Healthcare Research and Quality (AHRQ), award number R13NS065517 from the National Institute of Neurological Disorders and Stroke, National Institutes of Health (NIH), and grant number 5U59DD000318-02 from the Centers for Disease Control and Prevention (CDC). The views expressed in written conference materials or publications and by speakers and moderators do not necessarily reflect the official policies of the U.S. Department of Health and Human Services; nor does mention of trade names, commercial practices, or organizations imply endorsement by the U.S. Government.

First World Congress on Spina Bifida Research and Care

March 15 - 18, 2009

Orlando, Florida

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Introduction

Earl Y. Cheng, MD

Associate Professor of Urology

The Feinberg School of Medicine at Northwestern University

David B. Joseph, MD, FAAP

Professor of Urology

University of Alabama Birmingham

Timothy Brei, MD, FAAP

Associate Professor of Clinical Pediatrics

Indiana University School of Medicine

The First World Congress on Spina Bifida Research and Care took place in March 2009 in Orlando, Florida. During this landmark meeting, researchers and clinicians from around the world gathered together to discuss clinical and basic science research aimed at improving the care of individuals with Spina Bifida. The roots of this meeting began in May 2003 in Washington, DC when over 100 experts met to discuss the development of a research agenda for evidence-based practice in Spina Bifida. The proceedings from this meeting were published by the Centers for Disease Control and Prevention (CDC) in what is now known as the Green Book.

Following the Evidence-Based Research in Spina Bifida meeting in 2004, the wheels were put into motion for the First World Congress. Members of the Professional Advisory Council (PAC) of the Spina Bifida Association (SBA) recruited several leading researchers in their respective fields to form the Program Planning Committee. This group worked diligently over several years to put this landmark conference together. The meeting was a tremendous success; more than 350 individuals from 30 countries attended. Over the course of three days, presentations included keynote talks, multidisciplinary panel discussions, and breakout sessions in eight separate disciplines including urology, neurosurgery, neuropsychology, orthopedics, developmental pediatrics, epidemiology/genetics/public health, transition, and nursing/allied health. A lifetime achievement award was presented to David McLone, MD. The format of the World Congress allowed further cross fertilization between individuals from numerous specialties and different research backgrounds to foster new collaborative research ideas for the future.

This publication summarizes the information from the plenary talks and abstract presentations from the First World Congress. Additional detailed information from the meeting can also be found on the First World Congress Web site (<http://medicalconference.spinabifidaassociation.org>).

This conference could not have taken place without the generous funding and support from numerous sources including the CDC, the Agency for Healthcare Research and Quality, the National Institutes of Health, as well as industry sponsorship. The collaborative efforts of these government agencies and industry is greatly appreciated and represents the framework that will support future advancements in Spina Bifida research in the future. The success of this meeting was also a direct result of the efforts of numerous individuals who worked tirelessly behind the scenes. This includes members of the Board of Directors from both SBA and the Spina Bifida Foundation, as well as the PAC, staff of the SBA, and Site Solutions Worldwide.

As we proceed forward from this meeting, it is our sincere hope that the seeds have been planted for the growth of new and important research agendas in the future. With continued support, these research efforts will certainly result in developments that will improve the lives of individuals with Spina Bifida and their families. We would like to take this opportunity to thank all of those individuals who attended this meeting and whose research comprises this volume. We look forward to working together with all our colleagues across the world to promote a worldwide Spina Bifida research agenda.

Program Planning Committee

Adult/Transition

Thomas S. Webb, MD, MSc (Cincinnati Children's Hospital)

Developmental Pediatrics

Timothy Brei, MD, FAAP (SBA Medical Director, Riley Hospital for Children)

Gregory S. Liptak, MD (SUNY Upstate Medical University)

Mark Merckens, MD (Oregon Health Sciences University)

Epidemiology/Public Health

Russell Kirby, PhD (University of South Florida)

Neurosurgery

Jeffrey Blount, MD (University of Alabama – Birmingham)

David McLone, MD, PhD (Children's Memorial Hospital – Chicago)

Jerry Oakes, MD (University of Alabama – Birmingham)

Neuropsychology

Jack Fletcher, PhD (University of Houston)

Nursing/Allied Health

Mary Jo Dunleavy, BSN (Children's Hospital of Boston)

Karen Rauen, MSN, RN, BCIAAC (Children's Hospital of Wisconsin)

Orthopedics

Luciano Dias, MD (Children's Memorial Hospital – Chicago)

John Sarwark, MD (Children's Memorial Hospital – Chicago)

Lee Segal, MD (University of Arizona College of Medicine)

Physical Medicine/Rehabilitation

Shubhra (Sue) Mukherjee, MD (Rehabilitation Institute of Chicago)

Urology

Earl Y. Cheng, MD (Children's Memorial Hospital – Boston)

David B. Joseph, MD, FAAP (Children's Hospital of Alabama)

Stuart Bauer, MD (Children's Hospital Harvard University)

John Brock, III, MD (Vanderbilt Children's Hospital)



Observations on the Evolution of Management of Spina Bifida across an Academic Career

Marion L. Walker, MD
Professor of Neurosurgery
Department of Neurological Surgery
Division of Pediatric Neurosurgery
University of Utah
Salt Lake City, Utah

I have had the rare opportunity to see many changes in the way we manage patients born with myelomeningocele. Over the past 37 years, including my time as a neurosurgical resident, pediatric neurosurgical fellow, and an attending neurosurgeon, the philosophical approach and surgical management of myelomeningocele have changed dramatically. Some of these changes are due to technical advances in diagnostic testing, imaging, surgical instrumentation, and surgical technique. However, significant improvement in the long-term outcome can be attributed to a change in our attitude regarding the importance of early and aggressive intervention.

Historical Perspective

In order to appreciate the changes in the management of myelomeningocele during the past 30 years, it is important to look at how our approach to patients has changed. Before the ventricular shunt was available, very few patients survived. Once hydrocephalus became treatable, the decision to treat was based on the general neurological status of the patient. Since this is such a complex problem and so many of these children have obvious brain impairment in addition to spinal cord involvement, treatment was denied to many. They were left without surgical intervention to see if they would survive. Shunts were implanted in the survivors at a later date to provide “custodial care.”

This approach might best be summarized by a lecture given by Dr. John Lorber in 1975. He was discussing the advances of medicine and technology and pointing out that many diseases, such as prematurity and meningitis, that were so difficult to treat before technical advances were now much more treatable. He commented, however, that, “Unfortunately, the indiscriminate use of advanced techniques of all types has also kept alive those who would have died but now live with distressing physical or mental

handicaps or both, often for many years, without hope of ever having an independent existence compatible with human dignity” [1]. As suggested by this statement, Dr. Lorber favored treating only those myelomeningocele patients that survived with minimal deficits. This became known as selection.

As time passed, many neurosurgeons felt a desire to treat patients who had more substantial deficits. Approaches to treatment began to take a more moderate approach in some centers, and after Dr. David McLone began his practice of pediatric neurosurgery at Children’s Memorial Hospital in Chicago in the mid 1970s, more focused management came to be more fully accepted. Dr. McLone’s strong interest in these patients led him to question the appropriateness of selection relating to treatment. He noted that the argument for selection had become a debatable issue, contingent on the answers to several questions:

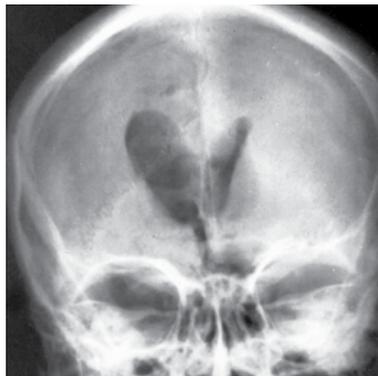
- Do valid criteria exist that accurately predict the patient’s outcome?
- What percentage of inaccuracy is acceptable?
- How many children will die to ensure that no disabled child will survive?
- Are there universally accepted criteria below which death is preferable?
- Can one person choose death for another [2]?

Dr. McLone made a commitment to treat all patients and kept excellent records on each to study outcomes. His first study involved a group of 100 consecutive nonselected patients with myelomeningocele. He has since published the long-term outcomes (20-25 years) on these patients [3]. It is now well established that most children born with myelomeningocele can have a meaningful and productive life. As complications from this disease have been minimized, further studies have shown that these patients do not deteriorate as they age solely because they have myelomeningocele. Instead, neurological deterioration is almost always due to a treatable cause [4]. In this section, I will discuss the approach to diagnosis and the ongoing assessment of these patients and point out how our approach to management has changed.

Diagnosis

In the 1970s, a diagnosis of myelomeningocele in a newborn was almost always a surprise to physicians and the family. An understanding of the importance of alpha fetoprotein (AFP) was only beginning, and ultrasound was in its early stages as a tool for fetal diagnosis. The understanding of associated brain anomalies and hydrocephalus was incomplete. Typically, physicians used air ventriculography to diagnose hydrocephalus, but visualization

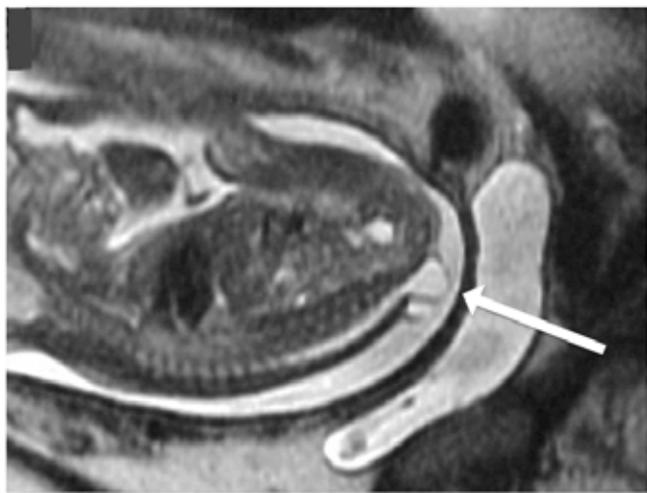
and understanding of the remaining brain anatomy were limited. It was necessary to perform a pantopaque myelogram to image the spinal cord, but this was impracticable in most cases because of the low-lying and tethered spinal cord. The computed tomography (CT) scanner became available in the mid 1970s, and although its use greatly added to our understanding of brain anatomy in these patients, it was not significantly better than conventional myelography when studying the spinal cord.



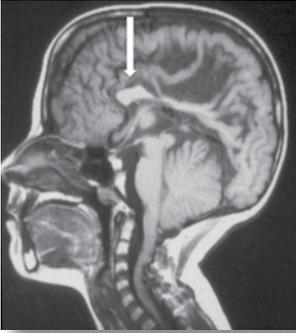
Detection of myelomeningocele today often begins early in utero. The quadruple screen maternal blood test, which is usually performed at about 16-18 weeks of gestation, can detect myelomeningocele in a significant percentage of pregnancies [5]. High levels of AFP suggest that the fetus has a neural tube defect. Prenatal ultrasound is also helpful and is very commonly done even for routine pregnancies. If there are questions regarding the accuracy of any of these tests, magnetic resonance (MR) imaging of the fetus can give definitive answers.

Imaging

Imaging patients with myelomeningocele has changed dramatically over the past three decades. Before the advent of CT scanning, imaging of the brain was accomplished through angiography, pneumoencephalography, or air ventriculography. Although we cannot discount the value of the imaging obtained during those times, the advent of CT scanning and subsequent development of MR imaging have dramatically improved our ability to



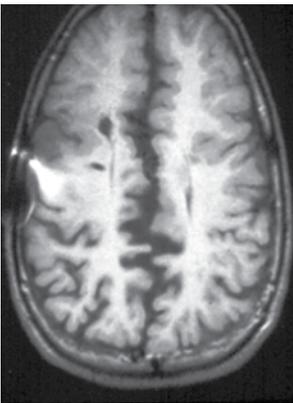
evaluate normal and abnormal states in the central nervous system (CNS). In the 1970s, the approach to imaging was generally to wait and see if there was a need to intervene.



Routine cerebral angiography was not recommended; nor was routine ventriculography.

Pneumoencephalography was difficult—if not impossible—in patients with myelomeningocele because of associated tethered spinal cord and scarring at the site of the closure of the myelomeningocele. Today, it is common to obtain baseline MR imaging of the entire CNS in patients with myelomeningocele. In our practice, we have often delayed baseline MR imaging until the

patient is approximately one year of age because the anatomical structures of the brain and spinal cord can more easily be appreciated after one year of age. This baseline imaging is used to document associated brain and/or spinal cord abnormalities, which may include the Chiari II malformation, with partial or complete absence of the corpus callosum, and hemispheric interdigitation. [6-8]



Early MR imaging of the spinal cord shows tethering, which is routinely present in patients because of closure of the open neural placode and resulting scarring. When we speak of “tethered spinal cord” in the patient with

myelomeningocele, we are generally referring to the clinical diagnosis associated with deterioration of neurological function and the accompanying damage to the distal portion of the spinal cord that may result from the scarring associated with the earlier surgery.

Neurosurgical Management

Neurosurgical management has likewise changed dramatically over the past 30 years. The closure of the placode at the time of the initial surgical procedure is far different than in the past; the management of hydrocephalus is changing; and the prospect of intrauterine surgery is bringing new promise to the management of myelomeningocele. Ongoing evaluation and observation of patients with Spina Bifida has changed significantly through the years.

Closure of the neural placode at the time of the initial closure of myelomeningocele is now performed routinely [9]. Prior to 1981, the open neural placode was generally left open; however, this allowed scar tissue to adhere directly to the placode and made future untethering more difficult for the

surgeon and risky to the patient [10]. Today, the placode is reapproximated into the neural tube. This restores a more normal anatomical appearance to the lower spinal cord and allows the surgeon to visualize more normal-appearing anatomy if a future untethering procedure is indicated.

Intrauterine Closure of Myelomeningocele

Preliminary clinical reports have demonstrated that intrauterine closure of myelomeningocele may be associated with a reduction in shunt-dependent hydrocephalus and the Chiari II malformation. [11-16] The Management of Myelomeningocele Study (MOMS) is an NIH-sponsored randomized trial of intrauterine closure of myelomeningocele being conducted in three different centers [<http://clinicaltrials.gov/ct2/show/NCT00060606>; accessed August 24, 2009]. The experimental arm involves fetal surgery to close the Spina Bifida defect prior to 26 weeks of gestation with delivery by Caesarean section at approximately 37 weeks of gestation. In the other arm of the trial, the fetus is delivered normally and undergoes postnatal closure of the defect when medically stable. The primary outcome measures of this study are (1) infant death or need for ventricular shunt by 1 year of life and (2) the mental development index on the Bayley Scales of Infant Development and functional-anatomical level of the lesion at 30 months of age. Secondary outcome measures are (1) the presence of Chiari II malformation, (2) neurodevelopmental status, (3) ambulation status, (4) presence of neuromuscular defects, and (5) grades of maternal, psychological, and reproductive functioning. To date, accrual is incomplete so no data are currently available for review.

Evaluation and Treatment

The initial evaluation of a newborn with myelomeningocele includes a general physical examination in addition to documentation of the neurological findings. Although associated birth defects are relatively uncommon, it is important to ascertain whether other systems are involved. In particular, patients are evaluated for their cardiac and pulmonary status and for kidney and urogenital abnormalities. Ultrasound and/or CT scans of the brain are performed as a baseline study. We include an echocardiogram, ultrasound of the kidneys, and a voiding cystourethrogram for all patients.

Management of Hydrocephalus

Despite improvements in other areas of care, shunts placed for the relief of hydrocephalus continue to fail at a very high rate [17], and hydrocephalus continues to be the most common serious long-term issue for myelomeningocele patients and the most common reason for surgery. The management of hydrocephalus is obviously not a new concern. The fact that shunts con-

tinue to fail at a high rate is only a part of the story of the difficulties of managing hydrocephalus from a historical perspective. Perhaps Leo Davidoff, a neurosurgeon early in the 20th century stated it best in 1929: “Hardly any other pathological condition has been accorded more determined attention on the part of the medical profession...than has hydrocephalus and in hardly a single other condition have cures been so elusive or so often wrecked on purely mechanical obstacles.”

Eighty years after Davidoff’s statement, treatment failure remains the rule, not the exception, in the management of hydrocephalus. Approximately 40% of new shunts fail within a year, 55% fail by two years, and approximately 80% fail within ten years [18]. In some large pediatric neurosurgical services the ratio of shunt revisions to new shunt placements is 2.5 to 1. Troublingly, hydrocephalus is the one condition for which we have made very little progress in the last 60 years [17]. With the invention of the shunt, many felt that hydrocephalus had been “cured,” but the long-term outcome for hydrocephalus and shunt survival is still similar to that of the early 1960s, when shunts became routinely available. Our focus seems to have been more on controlling cerebrospinal fluid flow through valves than on correcting the pathophysiology of the underlying hydrocephalus. Currently in the neurosurgical community, renewed focus is being placed on a better understanding of the underlying mechanisms of hydrocephalus and finding better treatment options [19-22].

Ongoing observation of shunt function is important and has changed dramatically over the past 30 years. We now take a more proactive approach to the patient with shunted hydrocephalus. Although ongoing baseline imaging is not necessary in most patients, a baseline image obtained after initial shunt placement and after shunt revision is important. We obtain images of patients several months after a shunt revision to establish a new baseline. This is extremely important when shunt failure occurs, especially when the patient may be under the care of a new physician or institution. Cognitive development is now recognized to be an important assessment of shunt function. Cognitive function, including an ongoing evaluation of the patient’s school performance, is now included as a part of the assessment of shunt function.

We now recognize that neurological deterioration of a patient with myelomeningocele is not a normal progression of the disease [4]. Thirty years ago this process was not well understood. We also recognize that neurological changes almost always have an underlying treatable cause. The most common cause is shunt malfunction. The second most common is tethering of the spinal cord.

Chiari II Malformation

When I entered neurosurgical practice in the 1970s, it was quite common to surgically decompress a Chiari II malformation when a patient showed neurological changes suggesting brain stem dysfunction. That approach has changed dramatically through the years. It is now well recognized that many symptoms suggestive of Chiari II deterioration are attributable to shunt malfunction. Even if a patient has stable ventricular size, a shunt revision will often result in neurological improvement. As a result, Chiari II decompression is rarely performed now [4, 7, 23].

We have learned through experience that symptoms attributable to the Chiari II malformation fall roughly into three groups. In patients born with symptoms suggestive of Chiari II malformation, surgery is not helpful. In patients with no early signs of Chiari II dysfunction who later develop brain stem dysfunction during infancy, surgery may be useful in a small percentage if the shunt is eliminated as the underlying cause of the dysfunction. For the older patient who has never had Chiari II-like symptoms and who has not improved after shunt revision, surgical decompression may have a better chance of success. The fact that shunt revision so often results in improvement of neurological status and Chiari II decompression has been much less successful has led to the mantra of the neurosurgery management of the patient with hydrocephalus: "It's always the shunt."

Tethered Spinal Cord

Tethered spinal cord is another neurosurgical condition that must be monitored on a long-term and ongoing basis in patients with myelomeningocele. Between 25% and 35% of patients will require surgical untethering of their spinal cord at some point [4].

The clinical presentation of the tethered spinal cord is quite typical in patients with myelomeningocele. Patients often complain of a very specific type of pain, usually in the lower back. The pain is worsened with activity, like walking, and improves with rest. When the patients rest, the pain will go away. Once they start walking again, the pain recurs until they rest, and the pain goes away. There is a neurologic claudication component to the pain. Compared to 30 years ago, we have a much better understanding of the pathophysiology underlying this clinical presentation [24]. Other symptoms and signs associated with tethered cord include progressive orthopedic deformities, scoliosis, changes in bowel and bladder continence, motor weakness, sensory changes, disturbances of gait, and, rarely, disturbances in upper extremity function.

Progressive orthopedic deformities may be a sign of tethering. Orthopedic surgical repair in patients who have spinal cord tethering may not be main-

tained. We have learned that repair of a deformity that “won’t stay fixed” is often an indicator of spinal cord tethering. Progressive scoliosis is also often related to tethering [4, 25-27]; however, scoliosis progression will often stop and the scoliotic deformity will often improved if untethering is performed early.

The classic “red flag” sign for tethered cord is a change in bladder continence [28]. Most patients who have myelomeningocele can be socially continent using intermittent catheterization techniques. Losing the ability to remain continent when compared with previous abilities is a strong indicator of tethering. These changes can also be documented with urodynamic testing and repeated for comparison purposes over time.

Knowing the history of the patient’s neurological status is important in assessing whether tethering is occurring. Changes in urological function over time are usually associated with some degree of tethered cord. We have learned that manual muscle testing is a very reproducible test that can be used in a clinic setting to monitor patients through time. We believe it is also important to include upper extremity testing. Progressive loss of strength in upper or lower extremities is a common sign of deterioration secondary to tethering.

Genetics

Genetics is an area in which we will see the most significant advances in the prevention and treatment of myelomeningocele. Our understanding of the neurulation process, including normal and abnormal states, has changed dramatically over the past two decades. Advances in molecular genetics now lead us to a better understanding of the underlying mechanisms of the abnormalities associated with myelomeningocele and may, perhaps, lead to earlier diagnosis and treatment.

Conclusion

I have had the rare privilege of seeing significant changes in the diagnosis and management of patients with myelomeningocele. These patients routinely bring abundant love and closeness to a family. They are brave. I see them face their neurological and associated problems with uncommon courage. I continue to be amazed at their positive attitudes and the way they face their trials. I feel certain that we will see much more progress toward an effective treatment and, someday, a cure.

Meeting of the Section of Neurological Surgery

The three core neurosurgical issues in caring for patients with Spina Bifida are hydrocephalus, tethered spinal cord, and the Chiari II malformation. Insights and advances have been made in each of these areas and central concepts have emerged. Yet much remains to be learned and ideas and approaches differ significantly between centers. The Neurosurgery breakout session centered on these ideas and incorporated two reports on the care of patients with Spina Bifida (SB) from around the world.

Hydrocephalus

Hydrocephalus and its multitude of manifestations in the patient with SB remain a great challenge. Eighty five percent of patients with myelomeningocele develop hydrocephalus and require a ventricular shunt. Ventricular shunts were developed in the early 1960s and have saved thousands of lives of patients affected by hydrocephalus. Despite the tremendous contributions of ventricular shunts, however, they have a high rate of failure that is unpredictable. Forty percent of shunts fail within a year of placement, sixty percent within two years and 80 percent within a decade of placement. Shunt infection rates vary widely in reported series but repeated numbers from large experienced centers are around 6% to 8%. Sadly these statistics are not significantly different from those reported 25 years ago, which indicates a discouraging lack of progress in this area of critical importance to the SB community. One of the reasons that little progress has been made is that shunt failure and shunt infection have multi-factorial etiologies. As such a large number of factors need to be controlled for in the construction of organized clinical trials. To date the neurosurgical literature has been remarkably sparse on organized clinical trials in hydrocephalus research. This in part results from a state of under-funding of hydrocephalus research in general and hydrocephalus in SB in particular.

The failure rate of ventricular shunts has prompted a search for alternate techniques to treat hydrocephalus. The endoscopic third ventriculostomy (ETV) is one such option. The principle of ventricular shunts is to give an alternative pathway for the spinal fluid to be shunted outside of the nervous system. The ETV by contrast opens an alternative pathway for the cerebrospinal fluid to flow to the outside of the brain where it is normally reabsorbed. Several papers addressed ventricular shunts and ETV at the First World Congress Neurosurgery breakout session.

Roger Bayston from Nottingham, United Kingdom, who has dedicated a career to studying CSF shunts and patterns and prevention of shunt infection, presented two papers. The first addressed the results of a survey obtained from patients with ventriculo-peritoneal (VP) shunts compared with those who have ventriculo-atrial (VA) shunts. VA shunts were developed first and direct the diverted spinal fluid directly back into the blood stream because the distal catheter is placed in the right atrium like a central venous line. VA shunts were essentially replaced by VP shunts over time as VA shunts were shown to have serious complications such as bacterial endocarditis, shunt nephritis (a cause of kidney failure) and thrombus propagation with risk for pulmonary embolus. The severity of these complications has prompted a rather sinister reputation within neurosurgery for VA shunts. Bayston's paper addressed whether this reputation was well founded in the current era. The authors surveyed 405 adult patients from a large public health service database with VA shunts and matched them with 405 patients with VP shunts. This retrospective survey found that the incidence of infection and obstruction were similar and that the severe problems of thrombus formation and nephropathy were extremely low. One limitation of this study is that if individuals with VA shunts had died already from complications, they would not be available for this retrospective evaluation. They concluded that VA shunts should remain a viable alternative when VP shunting is not possible. This is particularly important in the SB population as urologic procedures can make placement in the peritoneum untenable.

Bayston's second paper addressed the role that antimicrobial impregnation of shunt catheter material plays in the prevention of shunt infection. This topic has been extensively discussed and widely contested at neurosurgical meetings for several years because different groups from around the world have found widely different results when shunts are constructed of materials that have antimicrobials directly impregnated into them. Some groups have found little difference in shunt infection rates while others have found a pronounced reduction in shunt infection when antimicrobial materials are utilized. In this study, Bayston's group showed the effectiveness of the antimicrobial shunt catheter to kill three species of microorganism commonly implicated in shunt infection.

Outcome measures for children with SB who underwent shunting were reported by Robinson et.al. from Rainbow Babies Hospital in Cleveland. This study used a standardized, validated instrument (the Hydrocephalus Outcome Questionnaire) to survey outcomes of a group of 55 patients followed in the SB clinic. In this survey the 55 patients, who did not differ from non-study patients followed in the clinic, reported lower physical domain scores if they had symptomatic Chiari II malformations or required spinal cord untethering. Interestingly the 85% of patients who required a VP shunt did not have lower scores; however, shunt revision beyond the age of 2 years

was associated with lower scores in cognitive domains. Infections slightly lowered physical domains with no difference in other domains. Limitations included the small sample size, multiple management strategies for addressing the different issues and lack of detailed neuropsychological evaluation.

Blount and colleagues reported in a retrospective series from Birmingham, AL, that intradural spinal surgery was associated with an increased rate of CSF shunt failure. This group worked cooperatively with their orthopedic colleagues who surveyed similar data for extradural spinal surgery. Intradural spinal surgery was clearly associated with elevated shunt obstruction rates whereas extradural spinal surgery was not. The authors concluded that either the spine surgery was contributing to shunt obstruction via an unidentified mechanism, or patients with blocked shunts were presenting with signs and symptoms of tethered cord as their initial and singular manifestation of shunt insufficiency.

Dr. Wakhlu and colleagues presented a large retrospective series from India that supported the contention that insertion of the shunt at the same time of myelomeningocele closure in babies with open SB was not associated with increased shunt complications. This has been an issue of discussion and contention for some time and is particularly important in an era of increasing attention to resource utilization. A presentation by Dr. Jogi Pattisipu from Orlando embraced the importance of a standardized protocol, rigorous attention to detail and contemporary technologies such as endoscopy to manage the complex hydrocephalus that often accompanies SB.

The award for the best overall paper given at the First Annual Congress was awarded to Dr. Ben Warf, who presented outcome data on a series of patients treated with shunts who were compared with a matched group treated with endoscopic third ventriculostomy (ETV). The data were compiled from a large group of patients treated by Dr. Warf when he directed the neurosurgical program at CURE-the Children's Hospital of Uganda. These authors emphasize the importance of choroid plexus coagulation (CPC) at the same time ETV is performed. Prior reports from their group had established a high rate of effectiveness for the combined ETV-CPC procedure. A concern commonly expressed in the debate about ETV versus shunting is whether or not the larger ventricles tolerated after successful ETV are associated with cognitive impairments not seen with the smaller ventricles that typically accompany a successful shunt. Dr. Warf and team administered a standardized instrument of outcome (Bayley Scales of Infant Development) to three groups that they identified from their larger population. The first group required no treatment for hydrocephalus. The second underwent a VP shunt and the third group underwent ETV-CPC. No differences were found in outcome among groups. When these results were combined with prior findings from this group they concluded that ETV-CPC treats children

with hydrocephalus from SB with lower risk of failure and infection without compromising neurocognitive development. The paper stirred significant interest and debate. The central issue was whether or not these results could be reasonably extrapolated to other populations of patients with SB.

Chiari II Malformation

The diagnosis and treatment of the Chiari II malformation has undergone significant evolution over the past 25 years. Initially this hindbrain abnormality was thought to arise as a result of insufficient space in the posterior fossa of the skull for the brainstem and the cerebellum. MRI images showed neural elements such as the medulla and pons to be caudally displaced and “pushed down” into the top of the spinal canal. The predominant concept initially was that insufficient physical space and crowding gave rise to the signs and symptoms of brainstem insufficiency that have become known as the symptomatic Chiari II malformation. In clinical terms these consist of altered swallowing, stridor (in infants), poor airway protection, poor secretion management and poor respiratory drive in very young infants. Aggressive surgical intervention aimed at making the physical space available to the brainstem and rostral spinal cord marked the earliest era of neurosurgical thinking about the Chiari II malformation. Results from that era were decidedly mixed and far less favorable than would have been anticipated if the pathophysiology of the Chiari II malformation were simply an issue of physical space for the neural structures.

This led to a gradual evolution in thinking about the Chiari malformation that holds that the brainstem in children with myelomeningocele is fundamentally malformed to a variable degree (between patients) and that this fundamental problem is at the cellular level and hence not correctable by surgery. As a result of this condition the compromised brainstem in these children is uniquely sensitive to any other physiologic stressor. In children with myelomeningocele the overwhelmingly most common stressor would be hydrocephalus or insufficient shunt function. Therefore a recent interest and gradual evolution has occurred toward aggressively addressing shunt function as the first and most significant intervention in managing the symptomatic Chiari II malformation.

Because of the centrality of hydrocephalus in the current thinking about the symptomatic Chiari II malformation, ongoing discussions about the Chiari II malformation occurred during the session on hydrocephalus. It is evident that significant differences between large experienced centers still occur in the use of posterior fossa decompression in the treatment of symptomatic Chiari II malformations. Dr. Trumble’s paper about management strategies for the symptomatic Chiari malformation served as a further forum in which these issues were debated.

Tethered Spinal Cord

The tethered spinal cord (TSC) is another issue of controversy in neurosurgery. While there is general agreement that all patients with myelomeningocele harbor some element of physical tether at the closure site due to normal routine scarring, differences occur among centers regarding the aggressiveness with which surgical untethering is pursued and in the techniques utilized once surgery is undertaken. Olavarria and colleagues presented a large retrospective series of 70 children with TSC who underwent operative untethering. Some patients had lipomyelomeningocele or dermal sinus tracts. Results were excellent, with 90% relief of pain and >70% demonstrating restoration of urologic function to preoperative levels. Less than 1% demonstrated neurologic decline. This series echoed several larger but older series in the literature and reflected what appear to be the most widely held beliefs within the neurosurgery community; namely that pain is largely relieved, neurologic decline arrested but not restored and operative risks of imparting severe lasting neurologic insult are low.

Meurrens from Leuven Belgium and Partington from Gillette Children's Hospital in St. Paul, Minnesota both addressed the challenging issue of adult TSC. Meurrens's group reviewed a series of 33 adult patients who were evaluated for TSC. Twenty-six patients underwent surgical release. Adults most commonly undergo TSC release for relief of pain and are often found to have multiple neurologic anomalies of varying subtlety. Partington emphasized the role of trauma (especially seemingly minor trauma) in the onset of symptomatic TSC in adults. Often repetitive lumbar flexion maneuvers were implicated. When pulsed steroids failed to provide relief, surgery was advocated with good relief of pain and stabilization of neurologic decline.

The paper that challenged conventional concepts the most was presented by Robin Bowman from Children's Memorial Hospital in Chicago. The paper was entitled "Does the 'Benign' Sacral Dimple Exist?" It addressed a common entity that neurosurgeons are called upon to evaluate, the coccygeal pit. These are small crevices that occur in the skin over the lower back and the top part of the gluteal crease. Current widely accepted concepts hold that these abnormalities are uniformly non-tethering lesions if they are (1) within the gluteal crease, and (2) not associated with any other abnormality of the skin such as excess hair, discoloration, hemangiomas or fat lumps. These lesions are known as "benign sacral dimples" or "coccygeal pits." Those lesions that have those additional characteristics are known to be at high risk of traversing the space from skin to nervous system and producing a tether associated with a lipoma, lipomyelomeningocele, dermal sinus tract, or more rarely a diastematomyelia. Most pediatric neurosurgeons evaluate dozens of such patients in a year and do not routinely image those with lesions characteristic of benign sacral pits.

Bowman's group at Children's Memorial obtained MRI scans of the lumbar and sacral spine on patients treated in their clinic with these diagnoses. Of 75 patients enrolled, MRI scans were obtained on 70. Radiographic abnormalities were found in an alarming number. Twenty three percent had fat in the filum (a marker that can be associated with tethering but is not pathognomonic per se). Ten percent had a low riding conus medullaris and 7% had either hydromyelia or an open canal. Those patients who had low riding cords were further evaluated with urodynamic studies; two-thirds were found to have sufficient functional abnormalities to warrant surgical exploration and release. These results were worrisome to the collected audience of neurosurgeons who have all embraced similar principles in clearing these patients. These principles have been developed over time and repeated observations between large centers of excellence. If the results from the Bowman paper are taken at face value it may appear that a significant number of such patients have been missed by conventional screening criteria. The strongest evidence against the assertion of the paper may be that there has not been any such wave of adults with sacral dimples and symptomatic TSC observed in clinical practice. If indeed 20% of patients with sacral dimples developed symptomatic TSC then there would have to be a huge number of adults requiring surgery. Clearly this study requires further attention and repetition at other centers and will undoubtedly be a topic of lively discussion in the future.

International Care

Finally we also gained some insight into the differences with which neurosurgical issues are managed around the world with presentations that discussed the care of children with SB from Slovakia and Japan. Horn and colleagues presented a fascinating though brief review that showed the evolution of thinking and approach toward the surgical treatment of children with open myelomeningocele in Slovakia. They performed a retrospective analysis of all children with meningomyelocele treated at their facility between 1988 and 2007 and defined groups as 'open defect' or 'closed defect,' and by the decade in which they were treated. In the earliest part of the study (1988-1997) 14/106 (13%) children born with an open defect did not undergo surgery to close the defect within the first month of life. By 1998 56/57 (98%) were operated on within the first month of life. Ventricular shunts were only placed in a total of 36 patients.

Inagaki and colleagues discussed the role in which ultrasound is used by the OB-GYN and neonatology community for pre-natal screening of severe congenital anomaly. Termination of pregnancy is considered an option until 22 weeks gestation and is often recommended if a severe anomaly is detected. The Japanese Society of Pediatric Neurosurgeons is investigating ways that these studies and their results can be pursued with families in a more multi-disciplinary fashion.

Conclusions

We had a wonderful seminar that featured 14 different presentations from 6 different countries and several continents. This was a remarkable showing given a steady decrease in the numbers of abstracts about Spina Bifida / myelomeningocele at recent neurosurgical meetings and the fact that this meeting was the first of its kind. The neurosurgical issues surrounding the care of the patient with myelomeningocele remain challenging. Principles have emerged based on organized observations of talented, experienced and disciplined observers.

Hydrocephalus remains a core problem for which shunts have been the principle solution. Shunts have saved thousands of lives but are fraught with problems and limited progress has been made in solving them. Endoscopic third ventriculostomy may offer an alternative solution but results are mixed and SB patients fare poorly with ETV in many series. There has been an evolution away from direct surgical decompression of the posterior fossa in symptomatic Chiari II malformation. Surgical release of the symptomatic TSC improves pain; arrests neurologic decline and poses limited risk for permanent neurologic injury. Each of these principles is both widely embraced and under frequent thoughtful challenge. Much is known but much more needs to be known.



Epidemiology/Genetics

Epidemiology: The scientific basis for creating political will to prevent Spina Bifida and improve the lives of persons with the condition

Godfrey P. Oakley Jr., MD, MSPM
Research Professor of Epidemiology
Rollins School of Public Health of Emory University
Email: gpoakley@mindspring.com

Disclosure Statement:

I am a co-inventor of a patent that covers adding folic acid to contraceptive pills. While at the CDC, compensation, if any, will be under the regulations of the CDC. I have been a paid consultant to Ortho McNeil/Johnson and Johnson on this issue and may in the future. I have not done paid consulting on this issue during the last two years.

Prevention is urgent!

I like the title of this conference—The Future Is Now. What we do—or do not do—now does create the future. I also like the statement “The Fierce Urgency of Now.” Nowhere is there more urgency than the global elimination of folic acid-preventable Spina Bifida [1-3].

As a Spina Bifida community, we have collectively failed to tell our story effectively enough to create the political will for the resources we need to provide the best quality of care for persons with Spina Bifida, and to maximize the prevention of new cases of Spina Bifida. Epidemiological research, the key to providing the scientific basis for the prevention of folic acid-preventable Spina Bifida, can be the basis for tracking global prevention or lack of prevention.

Epidemiological Studies

Epidemiological studies can also improve the lives of persons with Spina Bifida by showing us interventions that actually help. Epidemiologic research can improve the care of persons with Spina Bifida; several areas deserve immediate action:

1. Registry of all persons with Spina Bifida

The cystic fibrosis community established a registry of most persons with cystic fibrosis many years ago. Analyses of data collected suggested that some patients did remarkably better than others. Investigations of the care given in those centers where children did better (positive deviants) provided the basis for improvement in the lives of all persons with cystic fibrosis. While it is not possible to predict exactly what benefit a registry of all persons with Spina Bifida would have, it is reasonable that clues to improvement will come.

2. Registry of the cause of death in all with Spina Bifida

We know of too many deaths of young adults with Spina Bifida, but we have not documented them; nor have we determined the causes of these deaths. If we had a registry of all deaths of persons with Spina Bifida and if we knew the cause of these deaths, we could find scientific clues to prevent future deaths.

3. Randomized controlled trials of components of care for persons with Spina Bifida

Evidence-based medicine, i.e., using the best data to guide medical treatment, is currently in vogue. The best evidence comes from randomized controlled epidemiological studies. Unfortunately, there have been few randomized controlled trials about the components of care for persons with Spina Bifida. These studies are critical to improving the lives of persons with Spina Bifida by showing whether or not a particular intervention actually works.

Epidemiologic Research and the Prevention of Spina Bifida

1. The failure to prevent folic acid-preventable Spina Bifida

One of the most important research studies ever reported was the epidemiologic study that showed in a randomized controlled trial that folic acid would prevent the majority of cases of Spina Bifida. I had assumed that this amazing scientific contribution would be immediately followed by the near total prevention worldwide in a few years. However, after 20 years, only about 10% of the Spina Bifida that can be prevented has been prevented [1]. The total prevention of folic acid-preventable Spina Bifida remains a dream because of lack of political will to provide sufficient resources for prevention.

It is instructive to look at the Polio Eradication Program. When it began in the late 1980s, approximately the same number of annual global cases of polio occurred as the current numbers of children born with Spina Bifida

and Anencephaly. The political will was created to give CDC an annual appropriation of \$200 million to provide scientific leadership and technical assistance and science-based advocacy for the polio eradication program. That program has been highly successful, preventing more than 99% of polio in the world. The up-to-the-date status of the Polio Eradication Program can be found at <http://www.polioeradication.org/>. This web site shows the incidence of Polio in every country. For example, in the first six months of 2009, 89 cases were reported in India. A reasonable estimate of the number of cases of folic acid preventable Spina Bifida in India annually is 20,000. Folic acid-preventable Spina Bifida is as preventable as Polio, but the political will for the leadership and resources is lacking; therefore, the Spina Bifida epidemic continues every day.

A well-funded center whose mission is the total prevention of folic acid-preventable Spina Bifida is necessary. The CDC would be a logical institution, as it has shown it can provide the leadership to eradicate Smallpox and has provided considerable leadership and resources to bring about the near eradication of Polio. Given resources similar to those available for Polio eradication at CDC, the birth defects group at CDC could achieve within a decade the near global elimination of folic acid-preventable Spina Bifida. The political will to fund this center is lacking.

2. Science-based advocacy to prevent Spina Bifida caused by valproic acid

In the early 1980s, epidemiologic research produced the evidence to show that in utero exposure to valproic acid increased by 10-fold the risk of a woman having a baby with Spina Bifida. The drug remains on the market in many countries and in some countries it has been approved for new indications. Good data on the current global incidence of valproic acid induced Spina Bifida are lacking. Improved epidemiologic studies of the occurrence of valproic acid induced Spina Bifida would provide part of the science base to promote the prevention of these birth defects.

3. Maternal Diabetes associated Spina Bifida

Maternal Diabetes increases the risk that the baby will be born with Spina Bifida. It is possible that folic acid fortification prevents this increase, but it has not been studied. Women who have better control of their Diabetes have a lower prevalence of birth defects, suggesting that improved care of women with Type 1 diabetes would lead to a decrease in Spina Bifida. The prevention of the birth defects, including Spina Bifida, associated with Maternal Diabetes is an area rich in prevention possibilities; it is likely to be realized only when the political will is created to fund a center to maximize the prevention of birth defects associated with Maternal Diabetes.

4. Finding other causes of Spina Bifida

Although folate deficiency, the use of valproic acid during pregnancy and Maternal Diabetes are the causes for most cases of Spina Bifida, children continue to be born with Spina Bifida who do not have these risk factors. Epidemiological research, such as the research that found the folate deficiency, valproic acid and Maternal Diabetes associations should be conducted to find the other causes of Spina Bifida.

Conclusions

Epidemiologic research can improve the lives of persons with Spina Bifida by identifying the most effective forms of care and the preventable causes of young adult deaths. Ample opportunity exists for significantly decreasing the occurrence of Spina Bifida by preventing Spina Bifida associated with folic acid deficiency, prenatal valproic acid exposure, and Maternal Diabetes.

The pace at which the lives of persons with Spina Bifida can be improved and the pace at which the prevention of Spina Bifida can be improved require the identification of substantial, new resources devoted to these priorities. The CDC has shown that, when it had sufficient resources, it was able to provide the leadership and technical assistance to eradicate Smallpox and to nearly eradicate Polio. An annual CDC appropriation of 200 million dollars could be expected to increase dramatically the pace of the improvement in the lives of persons with Spina Bifida as well as increase the pace of the prevention of Spina Bifida.

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Meeting of the Section of Epidemiology, Public Health, and Genetics

This session consisted of presentations grouped in the following areas: epidemiology of Spina Bifida, outcomes for persons with Spina Bifida, public health initiatives concerning Spina Bifida, and the genetic basis of Spina Bifida. The session was moderated by Godfrey P. Oakley, Jr., MD, MSPM, whose keynote lecture remarks appear earlier in this report.

Joseph Mulinare, MD, provided an international perspective on efforts to prevent neural tube defects through flour fortification with folic acid. The presentation began with a brief overview of the epidemiology of neural tube defects (NTDs), and a discussion of guidelines for reaching women of reproductive age to prevent NTDs. Data from the US and globally on the impact of folic acid fortification and supplementation on prevention of NTDs were presented. Dr. Mulinare provided a brief perspective on issues about benefits and potential harms related to the use of folic acid.

From the perspective of primary prevention of NTDs in the US, a major challenge is how to reach Hispanic women. This group has both a higher risk of NTDs than other race/ethnic groups (WilliamsL et al. Pediatrics 2005) and lower folic acid (FA) intakes than white non-Hispanic women (Yang QE et al. Am J Clin Nutri 2008). Current public health strategies are focused on reducing the prevalence of NTDs and increasing FA intake of Mexican American women, modeling folic acid intakes for corn masa flour and evaluating the pros and cons of adding folic acid to corn masa flour.

The available evidence demonstrates that blood folate levels among women of reproductive age have increased substantially in the US since folic acid fortification. The decreasing NTD numbers are consistent with an increase in folic acid content in flour and foods. Current decreases in NTD prevalence do not necessarily reflect the full prevention potential of folic acid. Fortification of foods with folic acid is feasible, economical, and effective public health policy. At the present time, there are no proven adverse effects of folic acid fortification.

Yet, from the perspective of primary prevention and public health, the unanswered question remains. How will we know when we have reached the point at which all folic acid-preventable NTD have been eliminated? Will this be when NTD rates stabilize at some specified level, or when all women reach a specified consumption of folic acid or blood level for folate, or perhaps, when we fully understand the underlying biology of prevention?

Dr. Mulinare's presentation was followed by a series of four presentations examining patterns of Spina Bifida health care utilization and expenditures,

coordinated by Judy Thibadeau, RN, MN. This portion of the agenda began with an overview of the National Spina Bifida Program, which broadens the public health agenda for Spina Bifida in terms of treatment, health status and outcomes, and secondary prevention initiatives. The four presentations included data on the most frequent and costly outpatient medical conditions utilized by people with Spina Bifida stratified by age. They compared the percentage of people affected by these conditions among those with and without Spina Bifida, calculated expenditures for these conditions for people with Spina Bifida, and provided estimates of medical expenditures for Medicaid-enrolled children and adolescents with Spina Bifida in the United States. They compared these findings with previously estimated expenditures for privately insured children and adolescents with Spina Bifida (Ouyang et al (2007) using the 2003 MarketScan Commercial Claims and Encounters database. The Veterans Administration Spina Bifida Health Care Program Database was also described.

Melissa Danielson, MSPH presented an analysis of patterns of mortality associated with Spina Bifida in the United States for the years 1979 to 2005. Median age at death has increased among individuals with Spina Bifida during this period. Decubitus ulcers and conditions of the nervous and urinary system are much more frequent causes of death among individuals with SB than general population.

The oral session concluded with two presentations concerning the genetics of Spina Bifida. Michelle O'Byrne, MS, examined the association of folate metabolic pathway genes with meningomyelocele in humans. Allison Ashley-Koch, PhD, provided a summary of current knowledge of the genetic basis of Spina Bifida, addressing three general questions: are NTD's genetic, can animal models help to disentangle NTD genetics, and what specific genes are involved? Genetics research uses several approaches to focus investigations, including patterns of familial clustering, increased recurrence risks, twin studies, and patterns of NTD co-occurrence with chromosomal abnormalities and other genetic syndromes.

Approaches to identifying disease susceptibility genes include genomic screens, candidate gene analysis, and genomic screen and linkage analysis. Genomic screens begin with no a priori hypothesis, look across entire genome. Linkage analysis studies families that have more than one person with a disorder. Genome wide association studies (GWAS) examine families of all types, even subjects in case/control studies. Candidate gene analysis involves an a priori hypothesis that a gene is involved due to either biology or position. Persons with the disorder of interest are compared to people without the disorder. Study subjects may be unrelated, or individuals from the same family. Genomic screening with linkage analysis involves studying many families that have multiple family members with NTDs. It can take

years to collect families and months to complete laboratory work. Previous work has identified several promising leads for further analysis. However, because of the complexities involved with this approach, candidate gene analysis is currently the most commonly used strategy for research examining the genetics of NTDs.

Clearly many genes are involved in NTDs. NTDs are so complex, however, that some genes may cause certain types of NTDs, but other genes cause other types of NTDs. For example, it is probable that different genes cause anencephaly while others cause myelomeningocele. Similarly, different genes may be involved in thoracic (upper) myelomeningocele, lumbosacral (lower) myelomeningocele; genetic mechanisms may be different for closed NTDs (lipomyelomeningocele) and open NTDs (myelomeningocele).

Summary

Public health approaches must continue to focus both on primary and secondary prevention of NTDs. While folic acid holds considerable promise, additional research is needed to determine whether there are other etiologic pathways to primary prevention as well as to determine how to optimize the effectiveness of health promotion strategies aimed at improving pre-conceptual folate levels through fortification, supplementation and diet. Research into secondary prevention strategies will require additional study of treatment, co-occurring conditions, and long-term outcomes, focusing not only on infancy and childhood but across the lifespan. Studies of the role of genetics in NTDs should expand to consider genetics within the broader milieu of epigenetics and epigenomics.



Transition

Where to from here? Transition to Adult Health Care for Young People with Spina Bifida

S.M. Sawyer, MBBS, MD, FRACP

Centre for Adolescent Health, Royal Children's Hospital

Department of Pediatrics

The University of Melbourne and Murdoch Children's Research Institute

Melbourne, Australia

Email: susan.sawyer@rch.org.au

Introduction

The landscape of health and well-being for children with complex developmental disabilities such as Spina Bifida has dramatically changed over the past 50 years. Much of the knowledge and skills required to manage the challenging life and health issues facing children with Spina Bifida and their families has risen as a result of the critical mass that has accompanied the development of multidisciplinary clinics within specialist pediatric settings. When coupled with multiple technical advances in surgery, imaging, and pharmacology, the resultant reduction in mortality has been striking. In contrast to 50 years ago when over 90% of infants with Spina Bifida were not expected to survive childhood, the overwhelming majority of infants born in high-income countries can now expect to mature through adolescence to face the rigors of adult life [1].

What sort of future might this growing adolescent and young adult cohort face? Notwithstanding the impressive limitations in physical, cognitive, and emotional functioning that continue to be the hallmark of Spina Bifida, the challenge for families and health care professionals alike is to promote young peoples' capacity to live fulfilled, meaningful lives. A wider challenge for the health care system is to ensure that as young people mature they are equipped to look after their health as independently as they can, and that appropriate health care services continue to be available to manage the increasing complexity of health problems they experience with age [2].

Adolescent Development

Adolescence refers to the critical period of development that lies between childhood and adulthood. The World Health Organization considers adolescence as the time between 10 and 19 years, and youth as that period

between 15 and 24 years. While the term young person is commonly used without any particular definition, it formally refers to the combination of terms that encompass the age from 10 to 24 years. More recently, the term adolescent and young adult (AYA) is increasingly used; in relationship to specialist health services, this can include young adults up to the late 20s or even early 30s.

Adolescent development is commonly conceptualized around three developmental domains: physical, cognitive, and psychosocial. The physical changes of puberty have long been considered to mark the start of adolescence, while social milestones such as completing education, achieving financial independence, marriage, and parenting typically mark the end of adolescence and acceptance into adult life. Over the past 50 years, the changing social context of adolescence has resulted in an upward extension of the age at which many of these milestones have been achieved. This results in the 'end' of adolescence becoming less distinct. What is clear is that the onset of puberty in early adolescence signals a transition into greater health risk, whether from mental disorders (especially in girls [3]), from risky behaviors such as substance use and unsafe sexual behaviors, or from higher rates of participation in risky behaviors that increase the likelihood of accidents and injuries [4].

Recent developments in neuroimaging have provided a scientific explanation of those aspects of cognitive development that our grandparents and their grandparents have long recognized as typical of adolescence. It is now known that the brain continues to develop during adolescence with 'pruning' and myelination leading to more efficient processing and a greater capacity for executive functions (responsible for high level organization and planning skills) as well as emotional regulation [5,6]. These same processes are no doubt also responsible for the greater capacity of adolescents first, to imagine an ideal world (or body), and second, to more critically compare themselves with others as well as to this imagined ideal.

Psychosocially, middle to late adolescence is a time during which healthy young people become more focused on educational achievement with eventual transitions to the work environment and financial independence. During this time, peer relationships become more critical to the ways in which young people view themselves.

Adolescents with Spina Bifida

In addition to the usual developmental challenges faced by adolescents, young people with Spina Bifida face an additional set of challenges that relate to the presence of the developmental disorder and its associated comorbidities. For example, young people with Spina Bifida are more likely to

experience a disturbed trajectory through puberty; early onset (idiopathic precocious) puberty is far more common in girls with Spina Bifida than in girls without disabilities. While lack of physical dexterity can be challenging for menstrual management, broader repercussions relate to early puberty being a risk factor for poor mental health. A challenge for health professionals and families alike is not to assume that the various aspects of adolescent development occur synchronously. Thus, it cannot be assumed that the earlier physical maturity that accompanies precocious puberty is accompanied by equally mature cognitive and social functioning. Indeed, young people with Spina Bifida have been shown to be less socially engaged than their healthy peers [7]. This is likely to be related to the impact of motor disability on opportunities for recreational, sport, and other social relationships, as well as deficits of cognitive functioning, like non-verbal learning disability. These factors may also at least partially explain why young people with Spina Bifida and other childhood disabilities have over twice the rate of sexual abuse as children without disability [8].

Many aspects of Spina Bifida affect learning. The impact of cognitive deficits commonly becomes more pronounced in adolescence due to the greater importance of learning and independent problem solving expected at this time, and the more nuanced and sophisticated patterns of social interaction that occur [9]. In addition, youth with physical disabilities risk being treated by parents and by others as younger than their years because limitations in physical mobility and activities of daily living require greater parental support (e.g., managing continence). It is likely that lower social acceptance and lower romantic appeal in young people with Spina Bifida [10,11] is a reflection of many different factors including neurocognitive deficits, physical appearance, and disability, and a range of accompanying social challenges including social stigma.

Studies of mental disorder suggest that young people with Spina Bifida are at greater risk of depressed mood and low self-worth [12]. Some studies paint a picture of young people with chronic illness and disability struggling to come to terms with “normal” adolescent development through reduced opportunities for ordinary social engagement (e.g., schooling, recreation, and intimate relationships) [7]. However, increased family conflict and less emotional engagement by adolescents with Spina Bifida with their parents may also simply reflect normal adolescent development. Beyond this, family responses are equally critical in terms of balancing an understandable desire to protect young people against the need for young people to learn by doing. One challenge for parents and health professionals is appreciating that what in early and mid adolescence might initially feel reassuring (for example, that their child is not yet sexually active) becomes more concerning by late adolescence and young adult life if it reflects poor social skills, limited opportunities for social engagement, and greater emotional dependence on

family. It is sobering to appreciate that, in general, young people with special health care needs have more rather than fewer health risk behaviors and mental disorders [13].

Quality Primary Health Care for Young People with Special Health Care Needs

Since 1967, the American Academy of Pediatrics has promulgated the notion of a “medical home” as a framework for providing quality primary health care to children, young people, and increasingly to adults with special health care needs [14]. Responding to decades of research showing substantial unmet health care needs in children and youth with special health care needs (CYSHCN) [15], the attributes of care provided through an ideal medical home are that it is accessible, family centered, continuous, comprehensive, coordinated, compassionate, and culturally effective [14]. Endorsement of this approach, which arguably is the same as quality primary health care, has come from the American Academy of Family Physicians, the American Academy of Pediatrics, the American College of Physicians, and the American Osteopathic Association, with support from the Maternal and Child Health Bureau (MCHB).

The principles developed by the MCHB in support of the medical home that are most relevant to the care of young people with Spina Bifida are that:

- Families of CYSHCN are partners in decision-making and are satisfied with the services they receive
- CYSHCN receive ongoing comprehensive care within a medical home
- Families of CYSHCN have adequate private and/or public insurance to pay for the services they need
- Community-based services for CYSHCN are organised so families can use them easily
- Youth with SHCN receive the necessary support to make transitions to all aspects of adult life including adult health care, work, and independence [16].

A systematic review of the attributes of a medical home suggests that CYSHCN experience better outcomes when receiving care that incorporates aspects of the medical home [15]. More specifically, children who can identify a medical home are about half as likely to experience delayed or forgone care and to have unmet health needs [17]. Within this framework, questions

of particular interest are how youth can be supported to make the required transition to adult health care, and what linkages are required to support access to appropriate specialist health care.

Transition to Adult Health Care

Universally, multiple life transitions occur for young people in and around late adolescence and early adulthood. These include changes in social roles (e.g., greater expectations of independence, greater capacity for intimate relationships), changes in civic and legal roles (e.g., ability to vote, drive a car), and changes in economic roles (e.g., completion of education and training, expectations of financial independence). Just as important for young people with complex health conditions are the changes within the health care system that accompany the transfer from specialist child oriented to adult focused health care.

As children mature into adolescence, their health care generally continues to be coordinated within the specialist child health system. As the life expectancy of young people with Spina Bifida has improved, so too has the need to develop specialist health services within the adult health care system. Internationally, adult specialist services are now well developed for various disorders such as cystic fibrosis that equally require multidisciplinary care; as with Spina Bifida, until the last few decades little demand for adult services had been seen due to patterns of childhood mortality. Models of adult health care have been much slower to develop for young people with developmental disabilities such as Spina Bifida, for whom the added complexities of more pronounced functional disability would render the need for comprehensive specialist health services even more critical.

Making a successful transition to adult health care is increasingly recognized as an important health outcome for young people with chronic disease and disability. Regardless of where they are managed or by whom, the notion of transition to adult health care is one that embodies a change in place from pediatric to adult services and a change in orientation from family-centered to more individually focused services [18]. The American Academy of Pediatrics and the Society for Adolescent Medicine maintain that the goal of transitional care is to maximize lifelong functioning and potential through the provision of high quality, developmentally appropriate health care that continues uninterrupted as the individual moves from adolescence to adulthood [19,20]. This planned movement of adolescents and young adults with chronic conditions from child centered to adult orientated health care systems is distinct to the event of transfer, which simply refers to the physical move from pediatric to adult services [21].

A set of principles has been developed by the Society for Adolescent Medicine in support of transition to adult health care [20].

In summary, these are that:

- Health care services need to be age and developmentally appropriate
- Health care services need to be able to address the common concerns of young people, including growth and development, sexuality, mood, and other mental health disorders, substance use, and other behaviors
- Successful transition to adult health care will help increase personal responsibility and facilitate self-reliance
- Transfer of health care should be individualized to meet the specific needs of young people and their families
- A designated individual must be responsible for coordinating and streamlining transition to adult health care

More specifically, this consensus statement identified six critical first steps to ensure successful transition to adult health care [20]. These were:

- Assurance that all young people with specific health care needs have a designated health care professional who takes specific responsibility for transition in the broader context of care coordination and health care planning
- Identification of the core competencies required by health care providers to render developmentally appropriate care and health care transition, and assurance that these are taught to primary health care providers and are an integral component of their certification requirements
- Development of a portable, accessible medical summary to facilitate the smooth collaboration and transfer of care among and between health care professionals
- Development of up-to-date detailed written transition plans, in collaboration with young people and their families
- Assurance that the same standards for primary and preventive health care are applied to young people with chronic conditions as to their healthy peers
- Assurance that affordable, comprehensive, and continuous health insurance is available to young people with chronic conditions throughout adolescence and into adulthood

Models of Young Adult Health Care

Broadly, four models of health care for young adults with Spina Bifida are currently being used. The first model is for young people simply to remain within the specialist pediatric health care system, continuing to be managed by various child health specialists into their adult years. The experience from other conditions is that when more typically “adult” complications such as pregnancy or drug addiction arise, pediatricians risk precipitously transferring young people to specific adult services (e.g., obstetric or drug and alcohol services) without putting in place an approach for more comprehensive and coordinated care. While some may view continuing access to pediatric services as the only reasonable response to the absence of more appropriately specialized services for young adults, this “propping up” of adult specialist services equally risks downplaying the extent of demand for comprehensive, coordinated adult services. Furthermore, while this model may be manageable for medical professionals who consult privately with young adults, the intersection of increasing survival of young adults with the upper age policies of children’s hospitals will at some stage render this model unsustainable.

The second model is to transfer young people to an adult specialist service. This may be a specialty Spina Bifida service or, more commonly, an adult developmental disability or rehabilitation service. Within these services rehabilitation specialists such as physiatrists, rather than pediatricians or pediatric surgeons, are more likely to take the clinical lead. The key features of this approach are that (a) a critical mass of patients exists to ensure the acquisition of specialist skills, (b) access to allied health as well as medical care is available, and (c) care coordination occurs. Typically, transfer to adult health care is deemed most appropriate once secondary education has been completed [22]. The major challenge with this model in many parts of the world is a lack of capacity. Many adult hospitals and specialists lack interest in developing an appropriate clinical response to the complex health care needs of these young people. This commonly reflects limitations imposed by models of health care funding and health insurance, as well as a lack of training for adult health providers about chronic conditions of childhood onset and about adolescent health.

The third model is to transfer young people at an earlier age (commonly 15-17 years) to a young adult service, which may be based within either a pediatric or an adult program. This is effectively the same approach as model two but without the expectation of ongoing care beyond young adulthood, at which time transfer to an adult services (model 2) or primary care (model 4) is still required. In comparison to models 1 or 2, the goals of young adult services (which have most commonly been developed to support young people with diabetes and cancer) are more explicitly about facilitating healthy ado-

lescent development and managing complex medical issues. This is equally critical for young people with Spina Bifida and their families.

The fourth model is to transfer young people to primary care, with the primary care physician responsible for care coordination and linkage to specialist health care and allied health care. While each of the first three models implicitly includes the primary care provider as a member of the multidisciplinary team, the key difference in this model is that the primary care provider is the clinical lead and care coordinator. A challenge for this model is whether the primary care provider has sufficient time and capacity to undertake these roles. A further challenge for the system is whether a sufficient critical mass can be developed for any member of the adult “team” to gain the necessary understanding of the many different impacts of Spina Bifida, whether this is the primary care provider or any of the other medical, surgical or allied health specialists within the adult health care sector.

Almost no evidence is available to support one model over another. What is clear is that many of the acute and chronic health issues affecting young adults with Spina Bifida are at least to some extent preventable [23], and that lifelong coordinated care is strongly recommended. In this context, it is highly concerning that in one study, 76% of adults with chronic and complex disabilities of childhood did not have a primary care provider [24]. Equally concerning is that admission rates were nine times higher than in the general population in one study, [24] with medical expenditures for youth with Spina Bifida estimated to be 13 times higher than for healthy youth [25]. Data like these no doubt raise immediate concerns for health care funders about the cost of managing this cohort. However, that nearly half of all hospital admissions for this lifelong complex condition are potentially preventable [23] and that the cessation of a multidisciplinary clinic resulted in significantly worse (and more expensive) health outcomes [26], suggests the value of health economic modeling in determining the benefits as well as costs of comprehensive multidisciplinary health care for adults with complex and expensive conditions.

Equally critical is the need for health care professionals to think developmentally and to anticipate future health care needs. Disappointingly, in a population-based study of parents of 5,533 adolescents with special health care needs, only 50% reported their child’s doctor had talked about changing needs in adulthood, and only 21% had ever discussed transferring their child’s care to an adult provider in time [27]. Whatever future model of care is available, early and repeated discussions with the young person and family are required to build their support and increase both the young person’s and the family’s capacity to move forward with confidence into the adult health care sector. A stronger focus on supporting young people and families to acquire self-management skills that would enable them to function more

independently as they mature is required. Programs that help young people with chronic conditions learn from each other, whether by participating in peer support groups, through social networking sites, or through recreational activities such as camps, are but some of the ways of achieving this.

Measuring Health and Life Outcomes

As with other complex conditions, the nature of Spina Bifida and its impact on the individual and family change with age and development. A recent literature review of the health care needs of adults with Spina Bifida highlights the incredible range of health concerns, including both disease specific (e.g., neurological, bladder and bowel functioning, mobility and musculoskeletal function, sexual functioning) as well as generic (e.g., obesity, mental disorder, skin care, pain) [2]. Whether in relationship to the general principles supporting children and youth with special health care needs, the more specific principles supporting transition to adult health care, or the models of healthcare for young people with complex conditions, a broader requirement is the development of a framework for monitoring and evaluating both health and life outcomes for people with Spina Bifida. In the United States, the recent development by the Spina Bifida Association of a Spina Bifida Database Registry is a very positive step towards supporting a research and evaluation framework.

Currently, a distinct risk exists for young people with complex health and developmental needs that maturation beyond the purview of specialist pediatric services will result in reduced access to developmentally appropriate, quality health care. To date, determined individuals who appreciated the lack of access to future adult health care for their patients and who developed good relationships between themselves and adult colleagues have been largely responsible for the success of most services for adults with developmental disability around the world. However, the complexity of contemporary health care for young people with Spina Bifida means that more systematic approaches to the development of adult services and to approaches for supporting the necessary linkages between pediatric and adult services are required. Many children's hospitals and rehabilitation programs would benefit from hospital-wide transition policies to support these tasks. Many parts of the world would equally benefit from regional health planning that would help identify or prioritize the development of developmentally appropriate, accessible, quality health care for young adult with complex disabilities.

A further risk is that the absence of the critical mass that would result from the development of specialist adult services leads to a failure to invest in a research framework for adults with Spina Bifida. Among other things, one might expect that with time, we would have a better understanding of the changing health care needs with age and what models of care might work

best to support the ongoing health care needs of this complex population as they mature. Investment in a training agenda for those who work with adolescents and young adults also is required, whether in the pediatric or adult health care settings.

Failure to invest in systematic approaches to monitoring health outcomes, in research, and in clinical training would sadly mean that by the year 2020, we may have no better understanding of how to improve the health and well-being of adults with Spina Bifida than we do today. The health care needs of young people with Spina Bifida do not lessen with age. If anything, the reverse is the case. Investment in more systematic approaches to clinical service development, research and training are required in order to improve both the health and life outcomes of these young people.

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Meeting of the Section on Transition

Transitioning from Pediatric to Adult Medicine for Adolescents and Young Adults with Spina Bifida: Research and Programmatic Developments

Introduction

Since the 1970's, survival in Spina Bifida has steadily increased; currently over 85% of children with neural tube defects are expected to reach adulthood. With longevity comes the need for adult-oriented services and preparation for the transfer of care out of pediatrics. The Spina Bifida Association and affiliated multi-specialty clinics have been addressing these issues for the last decade. At the First World Congress on Spina Bifida in March 2009, researchers and practitioners presented twenty-five abstracts on adolescent-to-adult transition from the United States, Canada, Australia, and the Netherlands.

The abstracts were divided into two main categories: 1) research, and 2) programmatic development. Several research abstracts examined the relationships between the medical, cognitive, academic, executive functioning, and psychosocial characteristics of adolescents and young adults (AYA) with Spina Bifida and their self-management abilities, community living skills, and health-related quality of life (HRQOL). Other research examined barriers and facilitators to participation in social, community, and physical activity and how participation impacted HRQOL. The process of transition in certain multi-specialty programs was studied, examining patient/family satisfaction and how well the process is documented in the medical record. Finally, research was presented that evaluated the average timeline for transfer of self-management responsibility from parent to AYA.

Programmatic abstracts described pilot projects in lifespan training curriculums, adolescent transition services, adult multi-specialty clinics, camp-based training programs, and a teleconference mentoring program. Other presentations described assessment tools for transition programs and Internet-based care coordination and educational resources for healthcare providers, families, and AYA self-advocates.

Transition Research

Most of the research presented was from regional, convenience samples of fewer than 100 subjects; however, several multi-site studies were presented, which included subjects attending one of several multi-specialty Spina Bifida programs. Two national studies were presented: one from a self-selected sample of conference attendees, and one randomly-selected, nationally rep-

representative population. Most of the research included validated measures and the majority utilized the constructs of activity and participation from the World Health Organization International Classification of Function.

One measure under development, the Adolescent Self-Management Independence Survey - version II (AMIS-II), was used in several of the transition research studies. The development of this instrument has been presented at previous conferences and the current abstract evaluated the measure's psychometric properties using a sample of 135 parent-child dyads from four Spina Bifida clinics. The age range of the participants was 12 to 25 years. The 17-question AMIS-II demonstrates construct validity and reliability. The instrument has two factors: 1) Self-management: Independent Living, and 2) Self-management: Condition. The tool is administered by interview and measures the amount of independence across multiple domains, including personal care skills such as toileting and taking medications, and independent (community) living skills such as making and keeping appointments and refilling prescriptions. The AMIS-II was offered as a reliable tool to assess clinically relevant self-management skills in AYA with Spina Bifida [1].

Using validated instruments including the AMIS-II, a study of a convenience sample of 26 young adults and 13 parents attending a transition program in Wisconsin found most of the young adults (ages 18 to 35 years (mean 23 years)), needed supervision with managing their personal health needs and with independent (community) living skills. The greatest needs were in the areas of finances, vocational rehabilitation, health care, independence, and socialization [2].

Also using the AMIS-II, a convenience sample of 61 young adults was evaluated for the association between self-management skills and psychosocial functioning and health outcomes. Overall the young adults had greater independence of self-care skills directly related to their daily Spina Bifida needs than overall independent (community) living skills. Higher self-management skills were associated with community living outcomes, but not with being employed or having a higher quality of life. No association was found between higher self-management skills and health outcomes, such as having pressure sores, urinary tract infections, hospitalizations, or continence [3].

Cognitive ability and executive functioning were compared to independent living skills in a sample of young adults in Chicago. Scores on standardized scales of functional academic skills, cognition, and academic achievement were more likely to be significantly associated with money management, transportation independence, health and safety abilities, and memory than with parental report of the adolescents' independence skills. The number of shunt revisions was not associated with any of the outcomes [4].

Self-management ability, depression, and quality of life were compared to mobility status in a multi-site study of 61 young adults with Spina Bifida. Mobility status (full-time wheelchair, part-time wheelchair, ambulatory) was not associated with QOL or depression. Trends were found towards lower self-management scores and greater need for assistance for full-time wheelchair users compared to those with some ambulation [5].

The overall findings in these studies indicate an ongoing need for assistance with self-management skills into young adulthood particularly for community living skills. Physical factors such as shunt revisions and mobility status do not appear to impact self-management skills, but intellectual and executive function may affect independent living skills. Self-reported quality of life does not appear to be affected by self-management skills or mobility status.

The timeline for transfer of self-management responsibility was examined in two abstracts. A concordance study about who is responsible for medical tasks conducted with a convenience sample of adolescents and their mothers in Chicago revealed that transfer of responsibility generally begins at 12 to 13 years with significant increases in the adolescent taking charge at 16 to 19 years of age [6]. A survey of a convenience sample of 62 parents and 41 young adults measuring the time of transfer of responsibility for medical care revealed the majority of care is shared between adolescent and parent between 12 and 17 years of age; 2/3 transfer care during the young adult years. While most personal care management transferred to the young adult after age 18 years, medication management and appointments tended to stay with the parent. Nutrition and bowel management were more likely to remain shared with the parent by young adult [7].

The process of transition was evaluated in two abstracts. In a descriptive cohort study of a pilot transition program in Wisconsin 21 young adults and 12 parents were interviewed using a qualitative telephone questionnaire two weeks after their initial transition visit. Over 80% responded that the process, which included a pre-transition visit with the nurse, a tour of the adult health facility, and a transition visit with the psychiatrist and urologist, was "better than expected" and was reassuring. Persistent concerns included access to neurosurgical consultation and the need for non-medical transition services [8].

A retrospective chart review of one Spina Bifida clinic evaluated the extent to which transition plans were documented in the patients' medical records. Plans related to education, employment, and recreational activities were documented in more than 70% of the charts. Bowel continence and activities of daily living were documented in greater than 50% of the records. Less than half of the charts, however, had transition plans for social relation-

ships, health care, living arrangements, and urinary continence [9]. These studies indicate that a planned process that includes anticipatory guidance and coordinated visits alleviates patient and parental anxiety, but that more work is needed to help facilitate transition to adult community living.

Community participation, including post-secondary school education, employment, and transportation-independence were examined in several abstracts. A retrospective chart review of a Spina Bifida clinic in Ohio revealed that 45% of adolescents remain in high school past the age of 18 years. Sixty percent of 22+ year olds are still not working or attending any kind of training program despite 82% receiving vocational-educational rehabilitation services. Over 70% of these adults are receiving supplemental security (SSI) or disability income and over 60% have public health insurance. The majority of adults were not driving, which was associated with hydrocephalus status but not lesion level [10].

Barriers to community participation were studied in 101 adolescents and young adults in Chicago. Initially only 30% of the subjects participated in community activities at least weekly. After an intervention by a transition-coordinator a 17% increase occurred in those who participated in weekly activities, with the greatest impact on the 15 to 17 year age group. Subjects over 21 years who were not in school, not working, and initially had no community activities were least likely to increase their participation. Overall, two-thirds of the adolescents maintained their participation for 6 to 12 months. The most frequently reported barriers were language (non-English speaking) and the lack of motivation, information, and transportation [11].

Physical activity participation was examined in 51 adolescents and young adults in the Netherlands and compared to fitness, body fat percent, cardiovascular disease risk factors, social roles, social support, and health-related quality of life. Physical activity and fitness had positive effects on participating in daily activities and physical HRQOL but not body fat percent or mental HRQOL. Participation in sports activities did not depend on ambulatory status but was associated with greater perceived athletic competence, physical appearance, and family social support [12].

Based on the classification system and domains of the World Health Organization (WHO) International Classification of Function (ICF), a nationally representative sample of 129 youth with Spina Bifida were studied for barriers and facilitating factors for community participation. As an example, communication problems, taking medications, and upper extremity problems were significant barriers to social participation with friends. This study is a rare example of a randomly selected, national sample of individuals with Spina Bifida, which can provide needed insight about those individuals who are not followed by multi-specialty clinics or attending national conferences [13].

Overall, participation and independence in the adult group was low, but could be facilitated by an adult transition coordinator. Barriers included physical and medical constraints, but lack of motivation was a significant variable. More research into identifying barriers to community participation and effective interventions is needed.

Transition Programs

Several pilot programs to facilitate self-management skills, physical activity and community participation, and transfer of care to adult services were described. Multiple modalities including a lifespan approach, camp training programs, teleconferencing, and internet-based tools have been tried.

A program from the Spina Bifida Association in Cincinnati uses an “effective mentoring model” via nine weekly one-hour teleconferences with 10 to 12 participants. A syllabus that addresses self-care topics and negotiating the health care system has been developed, and includes guest moderators with expertise in Spina Bifida. The program has served 99 older adolescents and young adults of diverse backgrounds, but with an emphasis on those who are geographically isolated or financially challenged [14].

The Shriners Hospital in Chicago presented their first SPINABILITY Camp, which is designed for participants at least 13 years of age with independent transfer and bowel and bladder self-care abilities. The camp lasts one week and is staffed by an interdisciplinary team that utilizes a “challenge by choice” philosophy, meaning that campers participate at the level of their choice for each sports-oriented physical activity. The inaugural camp was well received and will be repeated [15].

Several presentations outlined the successes and challenges of regional transition programs, including those in Alabama, Illinois, Wisconsin, and Ontario, Canada. Important features of these programs believed to facilitate successful transition include a lifespan approach with specific training and mentorship for the child/adolescent; collaboration among pediatric and adult healthcare providers; family education and support; and dedicated transition coordinators to set developmentally appropriate goals, and to manage scheduling, transportation, as well as the transfer of health information from one team to the other. These pilot programs identified gynecology, family planning, bowel continence programs, adult surgical specialists, and quality health insurance as the greatest needs for an adult Spina Bifida clinic [16-19].

Gillette Lifetime Specialty Healthcare summarized five years of experiences with their adolescent and adult clinic, which uses an interdisciplinary team of urological, rehabilitation, and neurosurgical specialists to address the cognitive, socio-emotional and physical needs of their patients. The ages of their

clientele range from 16 to 74 years, with equal participation of males and females. They reported high patient satisfaction based on customer satisfaction surveys. Parents and youth prioritized different areas. Teens chose Medical and Jobs as first priorities and parents chose Insurance and Medical [20].

A state government funded program from New South Wales, Australia utilizes a case management system to help young adults negotiate the more complex adult health care system. A Clinical Nurse Consultant and an Occupational Therapist who coordinate transitions from the three pediatric multidisciplinary Spina Bifida clinics to the two adult Spina Bifida clinics or to regional adult rehabilitation service providers staff the Adult Outreach Service. The transition service has facilitated regular attendance at the adult Spina Bifida clinic, decreased hospitalizations for pressure sores, reduced the incidence of renal impairment, and increased community participation [21].

Several transition tools are being developed. The Center for Disease Control and Prevention is developing a Preparation for Adult Participation logic model based on the principals of the World Health Organization International Classification of Function and using input from Spina Bifida experts and consumers. The transition pathway has three domains: 1) Self-management/Health, 2) Personal and Social Relationships, and 3) Employment/Income Support. A web-based Transition Preparation Checklist will list key developmental milestones in each of these domains and offer patient educational materials and assessment tools [22].

Another assessment tool, Assessment of Spina Bifida Care for Adolescents and Youth, was piloted by six Spina Bifida clinics in New York, New Hampshire, North Carolina, Ohio, and Pennsylvania. This health-care team self-assessment tool is adapted from a validated quality improvement measure from the Institute for Healthcare Improvement and is based on the Chronic Illness Care Model. Completed by medical directors, nurse coordinators, allied health professionals, and surgical sub-specialists, this pilot study found no significant differences in ratings across centers or provider types. The mean overall grade for the five centers was a "C" [23].

An Internet-based, interactive care coordination tool, which includes a video-format curriculum, has been piloted by SUNY Upstate Medical University. The content of this website, www.HealthyTransitionsNY.org, is adapted from the University of Washington Adolescent Health Transition Project; the curriculum was prioritized by consumer focus groups. Several ADA-accessible formats are available, which can be used for self-study or by using a moderator guide. The website includes a transition readiness checklist, goal setting guides, a search engine, health care guidelines, a transition medical history form, and an interactive video that demonstrates health care encounters [24].

Overall, the transition presentations highlighted common issues across all Spina Bifida providers. Interestingly, challenges in access to adult providers were similar regardless of the health care system. Young adults are still dependent on parents for management of their medical condition, particularly for administrative tasks such as appointments, finances, and medication refills. Community participation remains low but can be facilitated by a transition coordinator. Multiple tools are being developed to help with the assessment, coordination, and patient/family education during the transition process. More research is needed in understanding how to maximize development of self-management independence and community participation.

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Nursing and Allied Health

Nursing and Allied Health Response to Families Living with Spina Bifida: Past, Present, and Future

Robin R. Leger, RN, PhD
Assistant Professor, Community Medicine
New England Musculoskeletal Institute
University of Connecticut Health Center
Email: rleger@uchc.edu

Introduction

This keynote address is delivered worldwide to nurses and allied health (AH) care professionals (physical therapists, occupational therapists, certified orthotists, social workers, recreational therapists, child life specialists, school health teams, and others) committed to a lifespan approach to improving the health-related quality of life of individuals living with Spina Bifida.

We have been called ... “Nurse, Sister/Brother, Doctor, Therapist, Auntie/ Uncle, Teacher, and Friend” in a multitude of languages. I think of all the journeys we have had with our patients and families, and share this as a colleague on the state of the art and science of the care provided today. I was brought up with a child with cerebral palsy and readily knew disability, braces, wheelchairs, etc. as a normal part of day to day life and “culture.” I started working with families with Spina Bifida in 1977 on the neurology/ neurosurgery unit at Boston Children’s Hospital. In 1981, with nursing colleagues, I formed the North Eastern Myelodysplasia Association (NEMA). Over the years, the highlight of my career has been collaborating with colleagues across the USA; visiting Dr. Menelaus at the Spina Bifida Clinic at the Royal Children’s Hospital in Australia in 1982; and more recently visiting others at programs in Italy, Chile, and India.

Nurses/AH who demonstrate a commitment to Spina Bifida care have an ability to grasp the complexity of this multisystem, and potentially progressive, disabling condition and facilitate or develop interventions that have both practical and long term effects that support patients across care environments. In short, they combine critical thinking with a passion for making a difference in the lives of their patients. While recently re-reading Mohandas K. Gandhi’s autobiography [1], I found that the following quotes resonated with both a feeling of familiarity and a regard for the spirit of the

nurses/AH world-wide who care for people living with Spina Bifida: “. . . like loyalty, an aptitude for nursing was also deeply rooted in my nature. I was fond of nursing people, whether friends or strangers [p.173]. My aptitude for nursing gradually developed into a passion. ...Such service has no meaning unless one takes pleasure in it. ... But all other pleasures and passions pale into nothingness before service which is rendered in a spirit of joy [p. 175].”

Past, Present, and Future

A historical context of disability, genetics and chronic illness is beneficial in understanding the complexity of providing care and impacting policy for families affected by Spina Bifida. A recent monograph, *Genetics and Ethics in Health Care* [2], is relevant to the issues of survival (morbidity and mortality), stigma, access to health care and social policy issues faced by families living with Spina Bifida. The following highlights a few critical documents and cases [3]:

- In the 1700s, Diderot edited a lexicon of congenital anomalies, titling the section “Monstre” or monstrosities in a supplement to *Encyclopedie* (France).
- In 1971 Lorber’s selection criteria to decide whether or not newborns with Spina Bifida are treated became controversial (England) [4]
- In 1983 “Baby Jane Doe” a neonate with Spina Bifida and hydrocephalus prompted further dialog and new regulations under Section 504 of the Rehabilitation Act forbidding discrimination based on handicap related for federal funds (USA)
- Recent documents addressed the scarcity of resources for individuals living with Spina Bifida in African and impoverished nations [5, 6]
- Currently in the USA and worldwide adults living with Spina Bifida are struggling to access health services and care coordination; many are failing to find providers interested and knowledgeable about their health care needs
- The 2008 signing of the Genetics Information Non-discrimination Act (GINA) should further reduce discrimination of health insurance coverage for individuals with disabilities (USA)

Although policies are changing, preventing disparities in health care across settings and the impact of stigmatization remain challenges for patients, the chronic health care needs and potential risks for secondary conditions in Spina Bifida [7-10] require additional diligence in supporting:

- A multidisciplinary, comprehensive team approach to care
- Continuity of services over time and episodes of care and care coordination [11]
- Family centered social support [2, 12-16]
- Transition services and transfer of care across the lifespan and from pediatric specialty programs and/or medical homes to adult providers and/or medical homes (this may take two or more years to accomplish in an individual) [17-24]
- Utilization of other chronic illness management models such as the Wagner Chronic Condition Model [25], and adaptation to the lifespan healthcare needs in Spina Bifida to challenge the disparities in health dollars and services
- Research, collaboration, evidence-based practice, guidelines, and dissemination [3, 20, 24, 26-29]

The Art and Science of Spina Bifida Care

The profession of nursing is described as both an art and science and includes a vast number of roles and practice domains that are needed in a lifespan approach in the care of individuals living with Spina Bifida and to meet the needs of their families and communities. Beyond language and cultures, roles include: bedside/hospital based nursing; counseling/support; outpatient care; care coordination and triage; advocacy; education and mentoring; school health [18]; public health; consultation; collaboration; research; policy development; program development and evaluation; and publication and dissemination of evidence. Nurses and therapists are critical in the ongoing assessments and education for family members in the habilitative techniques of: positioning, donning and doffing of bracing/orthotics and adaptive equipment, skin care, mobility and functional skills in the home and community, and skills and activities needed for recreational, social, vocational and sexual health [30, 31].

Select clinical competencies and domains of practice of nurse/AH clinicians working in Spina Bifida include

- Maintaining and providing safety (emergency management and prevention) and comfort—the #1 priority!
- Providing education on folic acid and strategies for optimizing daily intake for both females and males [32] of reproductive age across health and community environments
- Preventing secondary conditions [8, 9] while promoting health [33-35]
- Optimizing growth and development while minimizing the impact of disability
- Addressing neurological/neurosurgical issues, including hydrocephalus and signs of shunt malfunctions, seizures, tethered cord syndrome, and Chiari symptoms [36]
- Addressing orthopedic and mobility issues [37, 38]
- Addressing habilitation and rehabilitation [39-44]
- Managing bowel and bladder function to promote continence and hygiene; maintain renal and gastrointestinal health; promote skin care, nutrition; and prevent obesity [45-50].
- Continuing research into and preventing latex allergy. Latex allergy research and precautions were spearheaded by nurses working with Spina Bifida clinics and changed practice worldwide [26, 27, 51-59]
- Promoting health-related quality of life (HRQOL) in Spina Bifida [9, 60, 61].

An Invitation to Participate in Global Strategies

In this time of electronic highways and rapid communication we need to take advantage of our ability to develop partnerships and collaborate on research and policy development with people around the world. It is important to form these partnerships with community-based organizations, the individuals living with Spina Bifida and their families and to adopt community-based participatory research methods as a principal and methodology [10, 62]. We can develop the leverage to change policy, decrease disparities in health services and diminish gaps in care and research.

You are invited to share your best stories and exemplars of care; best practice scenarios and guidelines; case examples and successful strategies at advocating or policy formation. Invite others to visit your program, travel,

give grand rounds, consult, share your knowledge and be political (in the best sense of the word). Inspire and mentor the young professionals in your environment or the students you encounter, and support one another! Work to improve opportunities for all individuals with Spina Bifida; promote inclusive environments of acceptance and value, free of discrimination and stigma; facilitate and ensure the development of access to services and care coordination; protect those at risk for neglect or abuse; and know your value when discouraged.

We need to continue to “think outside the box” or re-visit programs that were effective but have fallen by the wayside. For example, many of our patients deserve to have a comprehensive rehabilitation program to gain independence skills similar to individuals with an acute/traumatic spinal cord injury. This could be accomplished at a camp, rehabilitation program, or in hospital for a week during their elementary school years and again in their teen years to further refine skills. Yet insurance coverage and systems of care have provided this in only a few rare cases.

Nurses/AH are often the architects and the bricks that build bridges to schools, communities, public health agencies, home health, employee health, recreational and sports opportunities, and if necessary palliative and hospice care. The nurse coordinator roles in Spina Bifida have been drastically reduced by health care redesign and by efforts to reduce costs. Many of us (nurses and allied health care professionals) have lost our positions or are required to take on other patient populations or roles that reduce our time and roles with patients who have Spina Bifida. At this First World Congress Meeting on Spina Bifida Care and Research let us renew our commitment, and support one another. You are welcome to join in and read the following [63].

The Nightingale Declaration for Our Healthy World

We, the Nurses [Allied Health Professionals] and concerned citizens of the global community, hereby dedicate ourselves to the accomplishment of a healthy world by the year 2020.

We declare our willingness to unite in a program of action, sharing information and solutions to resolve problems and improve conditions -- locally, nationally and globally -- in order to achieve health for all humanity.

We further resolve to adopt personal practices and to implement public policies in our communities and nations, making this goal for the year 2020 achievable and inevitable, beginning today in our own lives, in the life of our nations and in the world at large.

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Meeting of the Section on Nursing and Allied Health

The section on Nursing and Allied Health (NAH) includes nurses, social workers, physical therapists, occupational therapists, dietitians, and “interested others.” The NAH podium and poster presentations were placed into one of two categories, Childhood Transitions or Promoting Positive Outcomes for People with Spina Bifida and Their Families. Acknowledging that those categories frequently overlap, the following summaries combine the key themes of all the presentations.

General Themes

Understanding the perceptions of people with Spina Bifida and their families is key to helping healthcare providers develop proper teaching-learning interventions and programming. Most of the following information was generated by qualitative studies, starting with younger children and ending with the late teen/young adult aged person.

In Jean Brown’s description of self-management of the neurogenic bladder, she emphasized the importance of timely interventions based on the process model of developmental stages. This is a sequential “stepping process,” resulting in successful transition to independent social continence. Parents and healthcare providers need to provide opportunities for this sequential developmental process to occur.

When Pat Braun interviewed primary care givers about their perspectives on transitioning care of neurogenic bowel management to their pre-adolescent children with Spina Bifida, barriers and facilitators were identified. Those responses led to the development of a practice model to facilitate the transition process. When healthcare providers are knowledgeable about barriers and facilitators on an individual level as well as collectively, appropriate interventions can be planned and implemented, resulting in pre-adolescents assuming their own bowel care.

In a study reported by Kathy Sawin on adolescents’ perspective of how their family managed the chronic condition of Spina Bifida, it was concluded that adolescents were knowledgeable about the management behaviors needed by the family (skills, strategies for the routine as well as the non-routine). It was suggested that adolescents be included in family studies about chronic illnesses, as they are likely to contribute to the family’s management of the condition.

Melissa Bellin reported on the experience of self-management of adolescent females with Spina Bifida. Barriers to participating in self-management activities included limited opportunities to manage money, build job skills, and develop autonomy in self-care decisions. Adolescents with Spina Bifida require planned, incremental responsibility for decision-making relative to their self-management experiences.

Analysis by Christine Kinavey of commonalities in narrative accounts given by adolescents with Spina Bifida imply that the biopsychosocial challenges faced by children born with Spina Bifida may interfere with normative developmental processes, including self-identity. Greater efforts need to be put forth to maximize developmental opportunities and foster identity not only as an individual, but as a member of a family, the immediate community and the global community.

Teen mentoring projects reported by Karen Rauen emphasized that these projects can significantly contribute to the teen's socialization skills, independence and participation in the community. Group activities as well as one-to-one activities with an adult mentor foster the achievement of individual teen goals as well as group goals. Outreach efforts to teens living outside of metropolitan areas can also achieve these goals through creative programming.

Transitions

The summary themes that resulted from the combination of presentations and discussions regarding transitions are: start early, offer opportunities, use repetition, and celebrate successes.

More specifically, the group agreed to the following:

- Pre-natal counseling is important in terms of providing information and starting a trusting relationship. This type of counseling should be encouraged.
- Start early to transition to the next steps toward self-management. Transitioning often takes more time and more repetition for children with Spina Bifida.
- Help parents learn to “let go” and to see the benefits for their child regarding involvement in decision making. Parents need to involve the child in chores and raise the bar on expectations.
- While respecting different cultures, it is important that families are taught the consequences of not doing recommended activities, such as catheterizations.

- Help families understand the critical times of transition in growth and development, such as entering school and puberty; provide them insights on normal adolescent behaviors.
- Mentoring programs can facilitate teen transition into adulthood by increasing socialization skills, self-management skills, and community participation.

The quest to find better interventions based on scientific evidence for people with Spina Bifida and their families is perpetual. Reports on the following studies and interventions offer evidence of what can help promote positive outcomes for this population.

Two presenters reported on innovative pilot studies to promote the transition process. Rachel Neff Greenley presented a study of two tailored problem solving interventions focused on enhancing adaptive family communication and child involvement in decision making. Families completed pre- and post-intervention questionnaires. Over 70% of parents reported that the intervention was useful in improving key areas of functioning, including child self-management skills, problem solving skills, and family communication. Children's responses paralleled those of their parents.

Findings from a prospective controlled trial, the Transition Training Preparation (TPT) Program, were presented by Kathryn Smith. The study is examining whether the TPT cognitive-behavioral program in combination with Spina Bifida management will lead to improved transition of self-management as well as physiologic and psychosocial outcomes in adolescents. Specifically, pre- and post-intervention measures were collected on role mastery, well-being of relationships and self-care agency. Outcomes of the TPT data are being analyzed and may indicate the usefulness of similarly designed programs in facilitating the transition of teens to responsible adulthood.

Promoting Positive Outcomes for People with Spina Bifida and Their Families

Prevention of latex sensitization for people with Spina Bifida remains a major concern. Eric Levey reported on a study conducted to determine the prevalence rate of latex sensitization in children with Spina Bifida. Forty-four percent of those born before 1994 were found to be sensitized, while only 14% of those born after that time were sensitized. The substantially lower prevalence in sensitization is believed to be related to latex avoidance measures. While data continue to be analyzed, the question of continuing latex precautions for newborns with Spina Bifida remains.

Treatment methods to promote continence for the neurogenic bowel were presented. Use of the Cone Irrigation Enema was reported by Jean Brown as an acceptable alternative to other non-surgical methods, resulting in a high level of continence for many children and adults. Using a standardized tool that assesses functional independence, Drew Davis presented a study on the degree to which the Antegrade Continence Enema (ACE) promoted bowel management independence in children with Spina Bifida. Results indicated that children were more independent in their bowel programs following the ACE.

A study to determine the effects of chronic health conditions on families that have a child with Spina Bifida, a rheumatological condition, epilepsy or asthma was reported by Karen Rauen. Instruments were used that measured family conflict and family impact. Despite the visual aspects of Spina Bifida and its complexities, families with Spina Bifida reported less problematic impact on the family than those with epilepsy and asthma.

Several presentations focused on pressure ulcer development in people with Spina Bifida. In a retrospective chart review reported by Patricia Beierwaltes, 76% of the subjects with Spina Bifida had had a pressure ulcer (48% sacral; 45% foot). While 10% of the ulcers were caused by burns, the rest (90%) were caused by pressure. The researchers correlated ulcer development with incontinence and program adherence. They found that program non-adherence was the only significant variable related to ulcer development. It was concluded that developing ways to promote adherence with healthcare regimes is critical to the prevention of pressure ulcers and their associated complications and costs. Laura Gueron reported that a survey of Spina Bifida clinics regarding lower extremity assessments revealed that responders did not use any type of formal evaluation or educational tools. Subsequently, Gueron's group developed foot screening and foot evaluation forms, a teaching DVD, and a lower extremity education packet about skin care for patients. The instructional Skin Care DVD includes how to inspect, wash, and care for the lower extremities as well as proper sock and shoe selection and orthotic wear. Pre-tests were administered to subjects before they watched the DVD. Post-tests showed significant improvement in understanding the care needed for lower extremity health and pressure ulcer prevention.

Sexuality of adolescents and young adults (AYAs) is a topic rarely discussed with parents or people with Spina Bifida. However AYAs are interested in it and have many unanswered questions. Dealing with the sexual consequences of having a disability offers unique challenges. Susan Labhard reported that to address the "need to know," a Sexuality Workshop for Adults with Spina Bifida was developed and implemented in a Spina Bifida clinic and a transition camp. Topics included relationships and sexuality, options to

traditional sexual expression, useful references and the use of appropriate media. Labhard concluded that clinical research has demonstrated that young adults can improve their knowledge of socialization and sexuality by attending a one hour workshop on the topic.

The summary themes that resulted from the combination of presentations and discussions regarding interventions to promote positive outcomes are: develop useful tools—and test them, use what works best, find ways to motivate, and address all relevant topics.

More specifically, the group agreed to the following:

- Latex precautions need to be continued for people with Spina Bifida, with a common sense approach regarding sensitivity.
- Bowel continence is critical to medical issues (constipation, skin ulcers), mental health issues (self-esteem), and social issues (isolation).
- Use what works best. The Cone Irrigation Enema may result in bowel continence for some, while others do best to achieve that goal via the ACE.
- Develop a concrete plan for advancing self-management skills and independence that is really understood. Terminology can be barriers to success, and repetition and practice are essential.
- Recognize that sometimes parents and providers do all the right interventions to move a child toward greater independence, but the child's cognitive issues and need for external motivation may be the problem; acknowledge this and change the approach.
- When written tools and return demonstrations do not show understanding of a newly learned skill, a DVD that can be repeated many times may be helpful.
- Regarding sexuality and the person with Spina Bifida, families need to understand privacy issues, friendships, relationships, and the appropriateness of wanting to be sexually active. The person with Spina Bifida needs to be aware of the risk of sexual exploitation and how to prevent it from happening.

Future Directions for Research and Care

More research and care initiatives are needed in the following arenas:

- Helping parents of a child with Spina Bifida to see the benefits of
 - o Giving the child age-appropriate choices
 - o Involving the child in household chores
 - o Promoting self-care management
 - o Raising the bar on expectations
 - o Incrementally increasing the child's responsibility
 - o Seeing the child as a sexual being
 - o Involving the child in his/her larger community
- Participating in innovative health promotional/self-management opportunities and programs
- Evaluating latex sensitivity in the Spina Bifida population to determine which precautions are necessary and which ones are not
- Developing appropriate tools to address the sexuality-related educational needs of this population
- Identifying strategies to motivate the person with Spina Bifida to adhere to self- management and healthcare recommendations
- Encouraging greater sponsorship of multisite studies to increase subject numbers and, therefore, increase the generalizability of findings to the greater Spina Bifida community.

Urology

XIAO Procedure to Refunctionalize Bladder and Bowel for Patients with Spina Bifida

Chuan-Guo Xiao, MD
Department of Urology
Union Hospital
Tongji Medical College
Huazhong University of Science and Technology
Wuhan, China
Email: xiaocg@mails.tjmu.edu.cn

Acknowledgment:

Supported by grants from: The National Key Basic Research Program of China (2003CB515300); The Ministry of Public Health of China (Major Clinical Trial Grant); Natural Science Foundation of China (NSFC 39925033)

Abstract

An artificial somatic-autonomic reflex pathway procedure for restoring voluntary bladder and bowel control in patients with Spina Bifida was introduced. Clinical application and the results of the new procedure are reviewed in detail.

Introduction

Neurogenic voiding dysfunction is a major problem for children with Spina Bifida, which affects approximately one per thousand newborns [1]. Despite rigorous pharmacologic and surgical treatment, incontinence, urinary tract infections, and upper tract deterioration remain problematic. The combination of pharmacological agents, surgical bladder augmentation, and clean intermittent catheterization has limited success [2].

History

In 1989 we proposed to establish an artificial “skin-CNS-bladder” reflex pathway below the spinal cord lesion (or just above the lesion for sacral cord injury) as a means of restoring controllable micturition after spinal cord injury (SCI) [3]. The assumption underlying this work is that the motor axons of a somatic reflex arc may be able to regenerate into autonomic preganglionic nerves and thus reinnervate the bladder parasympathetic ganglion cells, thereby transferring somatic reflex activity to the bladder

smooth muscle. This reflex pathway, which is basically a somatic reflex arc with a modified efferent branch that transfers somatic motor impulses to the bladder, has been designed to allow patients with SCI to initiate voiding by scratching the skin.

The new concept was tested first in rats [4]. An artificial cross over “skin-CNS-bladder” reflex pathway was established by intradural microanastomosis of the left L4 ventral root to the L6 ventral root, which innervates the bladder and external urethral sphincter in rats, while leaving the L4 dorsal root intact as a starter of micturition. After axonal regeneration, 15 of the 24 rats with the new pathway underwent electrophysiological study. Single stimuli (0.3-3 mA, 0.02-0.2 ms duration) to the left L4 nerve resulted in evoked potentials (0.5-1 mV) recorded from the left L6 nerve distal to the anastomosis. The bladder detrusor contraction was very quickly initiated by trains of the stimuli and bladder pressures increased rapidly to levels similar to controls. Neural tracing study of the pathways with horseradish peroxidase (HRP) on six rats demonstrated that the somatic motor axons regenerated successfully into the pelvic nerve, and the bladder was reinnervated by the L4 somatic motor neurons. The bladder contraction could also be initiated by electro stimulation of left sciatic nerve as well as by scratching the L4 related skin. A new concept was derived from the skin-CNS-bladder reflex pathway: i.e., that the impulses delivered from the efferent neurons of a somatic reflex arc can be transferred to initiate responses of an autonomic effector [4].

Continued animal experiments were focused on the underlying mechanism of the somatic-autonomic reflex pathway for micturition. The skin-CNS-bladder reflex was established in the cat by intradural microanastomosis of the left L7 ventral root (VR) to the S1 VR while leaving the L7 dorsal root (DR) intact to conduct cutaneous afferent signals that could trigger the new micturition reflex arc [5].

After allowing 11 weeks for axonal regeneration, urodynamic, pharmacological, and electrophysiological studies were conducted in pentobarbital or chloralose anesthetized animals. Results showed that a detrusor contraction was initiated at short latency by scratching the skin or by percutaneous electrical stimulation in the L7 dermatome. Maximal bladder pressures during this stimulation were similar to those activated by bladder distension in control animals. Electrophysiological recording revealed that single stimuli (0.3 to 3 mA, 0.02 to 0.2 msec duration) to the left L7 spinal nerve in which the efferent axons had degenerated evoked action potentials (0.5 to 1 mV) in the left S1 spinal nerve distal to the anastomosis. In addition, increases in bladder pressure were elicited by trains of the stimuli (5 to 20 Hz, 5 seconds) applied to the L7 spinal nerve.

Urodynamic studies including external sphincter EMG recording demonstrated that the new reflex pathway could initiate voiding without detrusor-external urethral sphincter dyssynergia. Atropine (0.05 mg/kg, i.v.) or trimethaphan (5 mg/kg, i.v.), a ganglionic blocking agent, depressed the bladder contractions elicited by skin stimulation. The skin-CNS-bladder reflex could also be elicited after transecting the spinal cord at the L2-L3 or L7-S1 levels.

In summary, the cross-wired somato-autonomic bladder reflex was effective in initiating bladder contractions and coordinated voiding in cats with an intact neuraxis and could also induce bladder contractions after acute transection of the lumbar spinal cord. The new pathway is mediated by cholinergic transmission involving both nicotinic and muscarinic receptors. The somatic motor axons can innervate bladder parasympathetic ganglion cells and thereby transfer somatic reflex activity to the bladder smooth muscle [5].

Clinical Trials

A clinical trial of the artificial somatic-CNS-autonomic reflex arc procedure was started in 1995 with very promising results [6]. By the end of 2004, a total of 92 patients with SCI who had hyper reflexic or acontractile bladders in our hospital had been treated with the somatic and autonomic reflex arc procedure; 81 of them regained bladder control one year postoperatively [7]. The National Institutes of Health (NIH) at New York University Medical Center sponsored a clinical trial of the somatic-autonomic reflex arc for micturition after SCI; this has also produced similar satisfactory results for the first two SCI volunteers [8].

Encouraged by the ability to surgically establish a somatic-autonomic reflex pathway in patients with SCI with restoration of bladder storage and emptying, we started a trial of this procedure on 20 children with Spina Bifida and neurogenic bladder in 2000 [9]. The neurological principals underlying the procedure were the same as for the patients with SCI, though the specific details were different due to the neural abnormalities associated with Spina Bifida.

All the children underwent limited laminectomy and a lumbar ventral root (VR) to S3 VR micro-anastomosis. The L5 dorsal root (DR) was left intact to serve as the afferent branch of the somatic-autonomic reflex pathway after axonal regeneration. All patients underwent urodynamic evaluation before and after surgery. Preoperative urodynamic studies revealed two types of bladder dysfunction: areflexic bladder (n=14) or hyperreflexic bladder with detrusor external sphincter dyssynergia (DESD) (n=6). All children were incontinent. Seventeen of the 20 patients gained satisfactory bladder control

and continence within 8 to 12 months after VR microanastomosis. Twelve (71.4%) of the 14 patients with areflexic bladder showed improvement. In these cases, the bladder capacity increased from 117 to 208 mL, and the mean maximal detrusor pressure increased from 18.35 to 32.57cm H₂O. Five of the 6 patients with hyperreflexic bladder showed improvement with resolution of incontinence. Urodynamic studies in these cases revealed a change from detrusor hyperreflexia with DESD and high detrusor pressure to nearly normal storage and synergic voiding. In these cases, mean bladder capacity increased from 94.33 to 177.83 ml, post void residual urine decreased from 70 to 24 mL. Overall, three cases failed to exhibit any improvement.

Like the patients with SCI, the children who gained bladder control also gained bowel control. Five patients had signs of partial loss of the left L4 or L5 motor function after surgery, variable from slight muscular weakness to visible foot drop in two children. Otherwise, no short or long term complications or adverse events were observed. The most fascinating and unexpected result of this trial, however, was that the children who gained bladder storage and emptying functions also gained bladder sensory function. Pre-existing sensory infrastructure may have been activated by stretch as the detrusor tone and bladder storage function improved. Since spinal cord continuity was not interrupted as in SCI, the central nervous system at both spinal and supra-spinal levels may have plasticity to accommodate the artificial somatic-autonomic reflex pathway for micturition. Thus, it appears that the artificial somatic-autonomic reflex arc procedure is an effective and safe treatment for patients with Spina Bifida to restore bladder continence and reverse bladder dysfunction [9].

Thus far, we have performed the procedure for bladder and bowel control safely on 1520 children with Spina Bifida [10]. Of these, 640 have had follow-up with urodynamic testing at one year postoperatively. The “effective” rate is 87%, compared to the 85% for the first 20 cases reported. The criteria to determine “effectiveness” includes: 1) voluntary voiding without catheterization, 2) post void residual urine less than 100 mL, 3) No urinary tract infections (UTI), and 4) freedom from anticholinergics. A small portion of the patients did have mild stress incontinence at the one-year follow-up, but

they usually needed no more than one diaper per day. This kind of stress continence likely will disappear or be reduced within two years postoperatively, according to our experience with the first 20 patients who had had at least two-year follow-up. The rate of partial loss of left-sided L4 or L5 motor function after surgery, which had a rate of 25% in the first 20 cases, has been reduced to 5%; this decrease in the complication of weakness likely is due to a change in procedure in which only half the lumbar ventral root is used [10].

Neurogenic bowel disorders, another major problem for Spina Bifida patients, are also improved by the procedure. Follow-up of 640 patients at the one-year post surgery who had manometry testing showed an improvement rate of 87%. In general, those who gained bladder function also gained bowel with improvement in continence or constipation; also, both bladder and bowel sensory function were improved. Interestingly, most patients felt bowel function improvement one month earlier than bladder function [10]. In summary, the artificial somatic-autonomic reflex pathway procedure (XIAO Procedure) can improve bladder continence and reverse bladder dysfunction for patients with Spina Bifida effectively and safely.

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Meeting of the Section on Urology

Xiao Procedure in the United States

The Urologic session began with a provocative report by Kenneth Peters, MD from William Beaumont Hospital in Bloomfield Hills, Mich., describing his experience using the Xiao procedure for rerouting functioning lumbar motor nerves to sacral root motor neurons that innervate the lower urinary tract. Nine children underwent this procedure and were followed for at least one year. Their evaluation revealed that one child was able to volitionally void to completion with no incontinence or urinary infections. Six other children had bladder contractility and were able to initiate a reflex contraction by scratching or rubbing the appropriate ipsilateral sensory dermatome but neither could fully empty their bladder or totally control when urination occurred. In two children no improvement in bladder function occurred following this surgery. Dr. Peters raised concerns about the procedure because eight children experienced some degree of transient weakness in the ipsilateral lower extremity after surgery; however, this change resolved in seven children by 6 months postoperatively. One child, however, has a persistent foot drop at last follow-up.

Cancer in Adults

J. Christopher Austin from Iowa discussed an alarming finding of 10 adults with carcinoma in their augmented bladder at a mean interval of 11 years post surgery. All tumors were invasive cancers and all had local metastases at the time of diagnosis, revealing their aggressiveness. This was succeeded by a lecture from Richard Rink from Indiana who found an overall incidence of bladder tumor formation following augmentation cystoplasty with intestine or stomach in his patients of 3%. A discussion ensued regarding the best way to screen patients with a prior augmentation; although no consensus was reached, it was felt that at least a renal and 'bladder' ultrasound was necessary every year beginning five years after the cystoplasty.

Co-morbidity

Several reports dealt with co-morbidity factors and Spina Bifida. Dominic Fimberger from Oklahoma noted that body mass index (BMI) had a positive predictive value with regard to complication rates following surgery in children with Spina Bifida. Unfortunately, most patients with myelodysplasia are overweight or obese and this may contribute to their higher rate of complications.

In a study of factors leading to renal failure in children with neurogenic bladder dysfunction, Michele Torre from Genoa, Italy, found that vesicoureteral reflux and upper urinary tract dilation were most often associated with renal functional impairment in these children. John Weiner from Duke studied a nationwide sample of patients with Spina Bifida discharged from the hospital; 82% undergoing bladder rehabilitation surgery had an augmentation compared to 18% with an ileal conduit diversion. The majority of the former were female and younger than those having an ileal loop. In addition, the diversion group required fewer health care resources, shorter hospital stays and less use of home health care aids. In another study, Alum Williams from Nottingham, England, concluded that patients had better continence outcomes if a caecostomy was performed in conjunction with augmentation cystoplasty, because this enhanced the effectiveness of the postoperative bowel management program.

Basic Science

Dominic Frimberger from Oklahoma found that (1) canine small intestine submucosa (SIS) bladder constructs did better when incubated with hyaluronic acid poly(lactic co-glycolic) nanoparticles, (2) urothelial regeneration over SIS expressed normal proteins uroplakin and ZO-1 by two weeks postoperatively suggesting a normal urothelial barrier to urine infiltration, and (3) distal SIS tissue produced less inflammatory response than proximal SIS with more macrophage and fewer eosinophil infiltrates, producing less of a destructive reaction in the bladder constructs.

Several papers dealt with the new field of stem cell research and autologous bladder augmentation. Yusuf Kibar from Ankara, Turkey, assessed the feasibility of injecting bone-marrow-derived mesenchymal stem cells into rat bladders subject to increased outlet obstruction; they found that the decompensated bladders had increased muscle mass, contracted in response to M₃ receptor stimulation and showed improved urodynamic parameters for compliance and capacity.

Earl Cheng from Chicago, Ill., showed that bone marrow-derived mesenchymal stem cells grown in tissue culture expressed properties similar to smooth muscle cells and that epithelial progenitor cells grown in tissue culture had the ability to induce vascular in-growth. Thus, the combined harvesting of these “primitive” cells has the potential for being used in tissue engineering of neo-bladders in the future.

Surgical Techniques

New surgical techniques for improving urinary continence were proposed. Gregory Dean from New York successfully employed a polypropylene perineal sling for incontinence in males with neurogenic bladder dysfunction, and reported an 80% complete continence rate. The Oklahoma group (Dr. Fimberger et al) found that bulking agents injected into either the continent urinary or fecal stoma to relieve persistent incontinence after surgery was successful in 78%, thus reducing the need for an extensive re-operative procedure. Finally, this latter group noted an 85% continence rate with urethral lengthening to achieve dryness and only a 7% incidence of difficulty with subsequent catheterization.

Warren Snodgrass from Dallas, Texas evaluated the long-term effects of a bladder neck sling procedure on detrusor compliance. Initially he found only five children had improved compliance at one year post-surgery, but an additional six improved subsequently. Only one child's bladder compliance worsened with time. Thus, he discussed the need for early augmentation cystoplasty after bladder neck surgery because his observations suggest that it takes time for the bladder to adjust to increased outlet resistance. All the remaining children in his study had no change in their bladder compliance.

Timothy Bertram from Winston-Salem studied bladder constructs for trigone-sparing augmentation cystoplasty in juvenile canines. At two years following surgery he found that three distinct layers of "normal" tissue had developed in these augments over time along with good detrusor compliance and capacity. He concluded that the bladders grow as these animals mature into adults. All these research efforts are beginning to show progress in the ability of surgeons to replace severely diseased bladders with autologous tissue, thus reducing the need for bowel augmentation.

Antegrade Colonic Enema (ACE)

The Malone antegrade colonic enema (MACE) procedure was discussed by Anthony Balcom from Milwaukee, Wis., who evaluated various irrigating solutions for cleansing the bowel using the antegrade continent stoma. He found that baby shampoo mixtures, followed by polyethylene glycol solutions were the most efficacious in reducing "toileting time" while other additives led to increased seepage from the stoma. The site of the ACE (left versus right) did not affect this finding. Many confounding variables affected the response, however, including the state of hydration, degree of ambulation, type of diet, degree of motivation, as well as the number of co-medications.

Jonathan Kaye from Atlanta, Ga., described similar findings regarding left versus right-sided ACEs in 34 patients. No differences in toileting time, ease

of use, incidence of stomal stenosis or other complications were found when one side was compared to the other. John Park from Ann Arbor, Mich., performed 45 ACEs (20 right-sided and 25 left-sided) and again found no difference in amount of irrigation solution needed to achieve a good response. However, he did note a significant increase in toileting time, more abdominal colic and less effective emptying with the caecal versus the sigmoid ACE. In both instances he cautioned for the need to create a good continence mechanism due to troubling seepage from the stoma that may occur if this has not been attained.

Earl Cheng presented a simple yet effective way to prevent stomal stenosis using the “L-stent,” a shortened catheter tied into a knot to prevent seepage through its lumen but more importantly to create an “L” shape so it can be inserted into the stoma and easily taped to the surrounding skin when an individual is not using the ACE; 17% of stomas developed stenosis in his series, but following placement of the “L-stent” and a daily application of betamethasone cream to the surrounding tissue, 87% reported improved ease of catheterization with no further need for any corrective surgery.

Quality of Life

In recent years there has been increasing interest in assessing quality of life in children with incontinence and Spina Bifida. Jenna Brand from Richmond, Va., conducted face-to-face interviews with 39 children (between the ages of 5 - 11 and 12 - 21 years) and their families. Parents and children had differing views about their quality of life. The degree of cognition and continence impacted positively on children, especially in the older aged group. Parents had more negative feelings about their child’s quality of life than their child did. In another study by Ann Neville-Jan from Pasadena, Calif., similar findings were noted: namely that urinary and fecal continence issues affected how older children performed in school and interacted with peers. Incontinence issues had a greater impact on teenagers than on pre-adolescents. The implications of using intermittent self-catheterization were not addressed in either study; however, this warrants further research.

Continence

Tera Bartelt from Milwaukee, Wis., evaluated the effectiveness of a continence program, which led to changes in both bladder and bowel function in children with Spina Bifida. She showed that efforts to improve continence in both systems simultaneously produced better outcomes than when either organ system was dealt with independently. The patient's level of lesion and extent of assistance needed, correlated positively with effectiveness of the response.

Urodynamic Testing

Andrew Coombs from Manhattan, attempted to improve the quality, reproducibility, and predictability of urodynamic studies in children with Spina Bifida by more accurately measuring detrusor leak point pressure (DLPP). He compared the DLPP (1) at the end of a cystometrogram with the catheter in place with DLPP (2) after the catheter was removed and reinserted, following cessation of all leakage. If the DLPP dropped below 40 cm H₂O with the second measurement, then upper urinary tract deterioration was substantially less likely to occur (15% of the sample) than if both pressure recordings remained above that level (44% of the sample). No child developed renal insufficiency in the former group whereas 11% did so in the latter group. In another study Mr. Coombs noted that at Columbia University, urodynamic studies are being performed at an earlier age than previously done in Spina Bifida children—primarily as a baseline to characterize lower urinary tract function and to treat children at risk proactively. On the other hand, children followed in community settings are being referred for lower urinary tract evaluation only after deterioration has occurred. The practice of utilizing urodynamic testing by community based physicians has not changed in the last 15 years and begs the question for developing standards of care for the Spina Bifida population.

Michael Carr from the Children's Hospital of Philadelphia presented the results of urodynamic testing in 54 children who had undergone prenatal closure of their myelomeningocele defect at 22 to 24 weeks of gestation prior to initiation of the MOMS trial. At five years of age, 10 children (18.5%) were voiding volitionally and were continent; two additional children were almost continent. As the assessment of these children is ongoing, it will be instructive to compare the findings in this group with children from the MOMS trial in the future.

Medications

Israel Franco from Long Island Jewish Hospital in New York evaluated the safety and efficacy of a new alpha-blocking agent, Alfuzosin, in children with Spina Bifida. At 0.2 mg/kg per dose, this drug proved to be safe and well tolerated without any drop in blood pressure while it lowered the leak point pressure, increased bladder capacity and improved compliance in most of the children who received the medication.

Paul Austin from St. Louis, Mo., reviewed the current literature on safety and efficacy of all drugs that affect lower urinary tract function in Spina Bifida. Most anticholinergic agents have a positive response in reducing over activity and increasing bladder compliance, but side effects are not uncommon. Some newer antimuscarinic agents (e.g., propiverine HCl) have greater selectivity towards detrusor muscle receptors with fewer systemic adverse effects; but these have not been tested extensively in children and are currently not approved by the FDA for use in the pediatric age group.

Summary

These exciting developments and the discussions that ensued during the breakout session speak to the high degree of interest as well as the need for better ways to manage the lower urinary tract in children with Spina Bifida and neurogenic bladder dysfunction. Overall, 43 abstracts were submitted, with several from centers outside the United States, including the United Kingdom and Turkey; 29 were given as either oral only or oral-poster presentations with the remaining 14 as poster only displays. The spectrum and the quality of the clinical, translational, and basic science research promoted at this meeting sparked thoughtful commentary and, hopefully, new avenues of research for future meetings.

Neuropsychology

Structural Changes and Dysfunction in the Hydrocephalic Brain

Marc R. Del Bigio, MD, PhD, FRCPC
Professor - Department of Pathology (Neuropathology)
University of Manitoba
Room 401 Brodie Centre
727 McDermot Avenue
Winnipeg MB R3E 3P5 Canada
Email: delbigi@cc.umanitoba.ca

Acknowledgment:

Dr. Del Bigio holds the Canada Research Chair in Developmental Neuropathology.

Conflict of Interest Disclaimer:

The author has received small contract fees from Codman and currently serves as a project consultant to Medtronic, both in relation to development of shunt technology.

Summary

In the context of Spina Bifida, hydrocephalus is usually caused by crowding of the posterior fossa with obstruction to cerebrospinal fluid flow from the fourth ventricle, and less often by malformation of the cerebral aqueduct. Some of the brain dysfunction is reversible by shunting, probably through restoration of cerebral blood flow and normalization of the extracellular environment. However, beyond a certain point, enlargement of the cerebral ventricles causes gradual destruction of periventricular white matter axons, especially the fimbria-fornix pathway and the corpus callosum. The clinical syndrome of hydrocephalic brain dysfunction is thus a syndrome of subcortical disconnection.

Meningomyelocele occurs in 3 to 7/10,000 live births with up to 50% of affected pregnancies terminated in some regions [1-3]. Hydrocephalus can be defined as a primary pathologic enlargement of the intracranial cerebrospinal fluid (CSF)-filled spaces. At birth approximately 2/10,000 live births have meningomyelocele with established hydrocephalus [4], and eventually 90% of individuals with meningomyelocele have enlarged cerebral ventricles and

50% to 80% develop hydrocephalus requiring surgical intervention [5-7]. In the context of meningocele, hydrocephalus is usually caused by crowding of the posterior fossa with obstruction to CSF flow from the fourth ventricle (i.e. the Chiari-II (a.k.a. Arnold-Chiari) malformation), and less often by malformation of the cerebral aqueduct [8].

Most CSF is produced by the choroid plexus while approximately 20% is water derived from the brain as product of glycolysis [9] and an unknown quantity by free exchange of water across the blood brain barrier [10]. Absorption of CSF is via arachnoid villi into the venous system and lymphatics associated with cranial and spinal nerves; the relative contribution of each is debated [11]. CSF has multiple functions including cleansing of potentially noxious byproducts of brain metabolism, a vehicle for molecular communication, and protection of brain by cushioning from vascular and extraneous shock waves [12].

The pathology of brain damage caused by hydrocephalus has been described in detail in several previous reviews [13-16]. Briefly summarized, the changes include destruction of the ependymal lining of the ventricle surface, stretching and compression of axons and small blood vessels in the periventricular white matter, secondary reactive changes including activation and proliferation of astrocytes and microglia, and destruction of axons with loss of connections between neurons. In humans and other species the major white matter targets are the fimbria-fornix, which connects the hippocampus to subcortical regions, and the corpus callosum. Long pathways extending to the spinal cord can also be damaged [17]. A threshold effect seems apparent and ventricular enlargement is tolerated to a limit; but the limit has not been well defined anatomically. The magnitude of damage is affected by the age of onset, the rate of ventricular enlargement, the size of the ventricles, the intracranial pressure (which in turn dictates the cerebral perfusion pressure), and coexisting pathological changes (especially cerebrovascular disease in older individuals [18]). In very immature brains with active cell proliferation, hydrocephalus can disturb development [19]. In infantile brains, ventricular enlargement can cause delayed myelination, which can recover after shunting [20-23]. However, axonal damage is not reversible.

The pathogenesis of the brain damage is multifactorial. At the most simple level, enlarging ventricles cause damage by mechanical stretching of axons. However, more complex processes are at work. In experimental animals, a reduction in the quantity of patent capillaries in the periventricular white matter occurs [24-26] with associated reductions in cerebral blood flow (CBF). CBF and related parameters have been measured in human hydrocephalus using a variety of techniques including positron emission tomography, transcranial Doppler, and radionuclide angiography; most have dem-

onstrated CBF reductions especially in white matter [27-29]. Improvement of blood flow after shunting correlates with clinical improvement in many studies [30-32]. Decreased blood flow is associated with local increases in glucose utilization [23], oxidative stress including lipid peroxidation and protein nitrosylation [33, 34], and activation of calcium-dependent proteolytic enzymes that destroy axons, similar to the molecular processes in stroke and head injury [35].

In addition to the cascade of damage caused by diminished blood flow, the alterations in extracellular fluid movements must be considered. Bulk flow of fluid occurs through interstitial spaces at a rate of 0.1 to 0.3 μ L/minute-gram of tissue [36]. Metabolic byproducts that cannot be recycled or extruded across the blood brain barrier flow to the ventricles from which they are washed away with CSF flow [37]. In hydrocephalus the extracellular spaces of gray matter are compressed [38, 39] and extracellular fluid flow is reduced [40, 41]. As a consequence, waste products accumulate in CSF and brain tissue [42, 43]. This imbalance of fluid outflow likely disturbs the delicate extracellular environment of neurons thereby impairing their function. However, with shunting this change is likely reversible.

Many studies have shown tissue changes in various neurotransmitters as a consequence of hydrocephalus [13, 44, 45]. Some of the neurotransmitter changes may be reversible by shunting [46]. However, when axons are lost, the density of synapses and the complexity of dendrites are reduced mainly in the cerebral neocortex but also in the hippocampus [47-57]. The upregulation of growth-associated protein (GAP-43) in hydrocephalus might be associated with synaptic remodeling [58]. Although neuronal changes in the striatum are not typically identifiable at the microscopic level, some imaging evidence supports functional changes in dopamine receptors [59].

Brain function is ultimately determined by the integrity, connectivity, and function of neurons, and hydrocephalus is associated with considerable impairments of function. This can be measured in humans and experimental animals electrophysiologically [60-70], by tests of learning and memory function [71-76], and by tests of motor function [68, 77-82].

The mainstay of treatment for hydrocephalus is CSF shunting, which is usually capable of reducing the ventricle size and may stop the progression of the damage [13]. However, the benefits of shunting need to be balanced with potential complications, particularly in premature infants and the elderly. Therefore, there may be a role for a pharmacologic supplement to treat hydrocephalus. The potential targets for pharmacologic intervention include reducing CSF production [83], enhancing CSF flow/absorption, "neuroprotection," axonal protection, improving blood flow, and improving recovery after shunting. For reasons previously discussed [15], reducing CSF

production might not be feasible. We have shown that the calcium channel antagonists nimodipine and MgSO₄ improve the behavioral and structural outcomes of juvenile rats with hydrocephalus, likely through improvement of blood flow [84-86]. Others have shown that antioxidant therapy is worth investigating at the experimental level [87]. However, given the lessons learned in the treatment of strokes, we must not rush into drug therapy without adequate preclinical investigation [88].

Future investigation of the pathophysiology of hydrocephalus will depend on the use of optimal animal models [89-91], proteomic studies of cerebrospinal fluid [42], and the use of advanced imaging methods [92]. Interdisciplinary cooperation will be the key to understanding and optimally treating hydrocephalus and Spina Bifida [93].

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Meeting of the Section on Neuropsychology

The Neuropsychology Breakout Session consisted of two oral presentations and a poster session. The first set of oral presentations was devoted to the application of contemporary functional and structural neuroimaging methods to Spina Bifida. The second presentation addressed psychosocial factors in Spina Bifida. The posters were predominantly neuropsychology and cognitive neuroscience studies.

Neuroimaging

In the first session, Dr. Roozbeh Rezaie discussed applications of magnetoencephalography to children with meningocele and age-matched controls. Dr. Rezaie described the assessment of brain functioning using magnetoencephalography, which is a whole head neurophysiological method that assesses the biomagnetic fields generated by electrophysiological changes in the brain. In the first study, Dr. Rezaie described a study in which cortical oscillatory rhythms were assessed in children with meningocele and shunted hydrocephalus who varied in the integrity of the corpus callosum. This study found a significant reduction in the power spectrum in posterior and temporal brain regions and not in anterior and central areas. Significant correlations occurred between the quantitative measurements of the corpus callosum and alpha spectral power. The results suggested reduced regional transcallosal connectivity in these children.

Dr. Rezaie then described assessments of touch perception in children with Spina Bifida. Magnetoencephalographic recordings were made in response to a vibrating stimulus held on the fingers. The results demonstrated that both the ipsilateral and contralateral components of this somatosensory evoked potential were present in all controls. Many children with meningocele showed only the ipsilateral component. Again, these results suggested reduced regional transcallosal connectivity in these children.

In the third study, Dr. Rezaie presented assessments of language and reading in children with meningocele and controls. On a word reading task, controls exhibited significantly greater engagement in the left hemisphere, whereas children with Spina Bifida demonstrated more bilateral activation. This difference was especially apparent in the middle temporal gyrus. There were no differences on a language task that required listening to words. Since children with meningocele are often proficient word decoders, these findings suggest that bilateral activation, even when it involves greater activation of the right middle temporal gyrus, is a compensatory pattern that supports proficient word recognition.

In the second neuroimaging discussion, Dr. Khadar Hasan addressed the application of diffusion tensor imaging (DTI) to children with Spina Bifida. He described how diffusion tensor imaging is conducted and analyzed, emphasizing that it is a method for assessing the diffusion of water molecules in the brain that is particularly sensitive to the integrity of cerebral white matter. Dr. Hasan presented new applications of DTI that permit assessments of regional brain volumes as well as gray matter structures. Dr. Hasan showed that in some gray matter structures, such as the caudate nucleus, fractional anisotropy is actually higher, which is suggestive of delayed or aberrant synaptogenesis. In addition, he showed reductions in both gray and white matter volumes. Dr. Hasan also presented assessments of several white fiber pathways including the corpus callosum and different association fibers, which demonstrated lower fractional anisotropy consistent with reduced myelination.

The third presentation by Dr. Jenifer Juranek discussed the application of voxel-based morphometry to MRI scans in children with Spina Bifida and controls. Dr. Juranek described a pattern of increased cortical thickness in the frontal lobes and reduced cortical thickness in the posterior regions. She also assessed cortical complexity in cortically thinned regions, showing that cortical complexity in the brains of children with meningomyelocele is markedly increased when thinning is greater.

In the discussion, Dr. Marc Del Bigio presented a neuropathologist's perspective. He noted the value of examining the brain from a quantitative neuroimaging perspective. However, he also noted that imaging only provides a window to the brain and that correlations of MRI assessments with post-mortem studies tend to be weak. Dr. Del Bigio emphasized the impact of hydrocephalus on the brain, noting that each time the ventricles expand and reduce, damage to the white matter likely occurs. He argued that in order to understand imaging changes, the complete history of shunt failure history is critical. He asked about the effects of ventricular enlargement on brain development, noting that enlarged ventricles in utero usually affects the germinal matrix and this affects on brain weight, synaptogenesis, and myelin production.

Psychosocial Factors

In the session on psychosocial factors in Spina Bifida, Dr. Grayson Holmbeck presented a series of studies that evaluated a biopsychosocial model of Spina Bifida. In presenting this model, he emphasized a developmental perspective on the adjustment of adolescents with Spina Bifida. He also discussed a multi-finality model that addressed why some children with Spina Bifida do well and others do more poorly when they have same starting point. He presented assessments of different models for psychosocial

adjustment in Spina Bifida, including a higher stress model, a strength of association model, an additional stress model, and differential developmental growth model. He also presented evidence in support of mediational models of adjustment (e.g., Spina Bifida status • neuropsychological functioning • social adjustment). The evidence supported a disruption-resilience model of adjustment in which family support ameliorated some of difficulties associated with Spina Bifida, but that lower social class was an exacerbating factor for some families of youth with Spina Bifida. Finally, he presented a model that is being tested in a new study that examines neuropsychological functioning and peer relations in youth with this condition.

Dr. Heather Taylor presented a study evaluating the impact of parenting and motor skills on the development of cognitive, language, and daily living skills in inference in children with Spina Bifida and controls from 6 to 36 months of age. Dr. Taylor reported that the development of motor abilities was associated with cognitive, language, and daily living skills, with children who showed better motor development scoring higher on all domains. In addition, higher quality of parenting was associated with higher levels of development and faster growth in cognitive skills.

Dr. Melissa Bellin addressed issues related to the transition of adolescents and young adults with Spina Bifida into adulthood. Over 40% of a transition-age group of young adults indicated psychological distress. Factors related to individual, family, and environmental measures explained variability in psychological health. These rates of self-reported psychological distress are much higher than population rates in young adults.

Posters

The poster session addressed a variety of issues related to neuropsychology and cognitive neuroscience in Spina Bifida. Dr. Jackson addressed the relation of the Chiari malformation to intellectual functioning. In a comparison of 30 patients with shunted hydrocephalus and 17 with no hydrocephalus, the people with no hydrocephalus scored higher on IQ and most cognitive measures. In addition, the people with no hydrocephalus generally performed within the average range.

Dr. Andrew Zabel presented a poster regarding methods for quick and efficient clinic-based screening and monitoring that included assessments of executive functions, adaptive functions, processing speed, and working memory. He emphasized that the collection of neuropsychological data using measures that involve questionnaires was effective for clinic-based screening.

Dr. Heather Taylor presented a study on early information processing in 18-month-old infants with Spina Bifida and controls. An assessment of visual attention skills showed that attention difficulties observed in other

studies of school-age children with Spina Bifida can be documented in younger children as well. In addition, the findings supported the hypothesis that more impairment occurs in attention processes involving stimulus control and orienting than in response control and monitoring.

Dr. Paul Cirino assessed different aspects of mathematics performance in children with meningomyelocele; significant differences occurred relative to controls in a number of mathematic abilities, but differences were most apparent on small sum problems. Math performance was accounted for by basic foundational skills involving fact retrieval as well as more conceptual aspects of math ability; the latter was closely related to reading ability. In a study of reaction time in children and adults with meningomyelocele, Dr. Maureen Dennis found that individuals with Spina Bifida were slower to respond than controls. The degree of difficulty increased as the reaction time test increased; no interactions occurred with age.

Altogether, these three sessions provided new results and approaches to the assessment of children and adults with Spina Bifida. Dr. Fletcher noted the importance of insuring homogeneity within the sample, observing that only meningomyelocele is associated in a systematic way with malformations of the brain. Differences within children and adults with meningo-myelocele also may be present depending on whether hydrocephalus is shunted. He highlighted Dr. Del Bigio's discussion of the effects of recurrent hydrocephalus on the brain, noting that even children with arrested hydrocephalus who have never been shunted tended to show motor and cognitive difficulties. However, these difficulties are typically not seen in children who have Spina Bifida without hydrocephalus. This session featured contributions from eight different disciplines, highlighting the value of multi-disciplinary approaches to research on complex neurogenetic disorders like meningomyelocele.

Orthopedics

The Orthopedic Care of Children with Spina Bifida: What Have We Learned in the Past 30 Years?

Luciano Dias, MD
Professor, Orthopedic Surgery
Feinberg School of Medicine
Northwestern University

Medical Director
Motion Analysis Center
Children's Memorial Hospital
Chicago, IL

Introduction

Without a doubt, the overall care of the child with Spina Bifida has changed considerably in the last 30 years, in particular, orthopedic care. Before we outline and discuss these changes, we should give credit to a number of outstanding pediatric orthopedic surgeons who established the basis for the care of Spina Bifida. I would like to give credit here to Drs. Mihran Tachdjian, Burr Curtis, Jack Banta, Earl Feiwell, J W Sharrard, Klaus Parsch, Malcolm Menelaus, Norris Carroll, Richard Lindseth and Douglas McKay. Their outstanding work for the last 50 years is well recognized.

Our involvement in Spina Bifida care started back in 1976 at the Children's Memorial Hospital in Chicago. The team has been together for the last 33 years. I would also like to recognize the leadership of Dr. David McLone in neurosurgery and Dr. William Kaplan in urology. In 1989, with the establishment of the Motion Analysis Center at Children's, the use of this technology became an important tool in the decision-making process for the management of the patient with Spina Bifida. Table 1 lists the areas in which motion analysis has helped in the care of individuals with Spina Bifida. We will be discussing the benefits of motion analysis and how much we were able to learn about the Spina Bifida gait pattern and pathology and how much the use of gait analysis influenced our decisions for functional procedures. The knowledge obtained from gait analysis in the last 20 years has significantly changed our approach to some of the orthopedic pathologies. At the orthopedic division of The Children's Memorial Hospital and at our Motion Analysis Center, an extensive number of research projects were undertaken resulting in the publication of numerous papers.

Functional Mobility Scale

In 2004, Kerr Graham introduced the Functional Mobility Scale for patients with cerebral palsy. We have been using this classification for the last 5 years and we find it to be quite useful in Spina Bifida. Basically it classifies the patient according to their ability to walk with support or without support over three different distances: 5 meters, 50 meters and 500 meters. The child's abilities are rated on a 6-point scale—see box.

For instance, if a child with Spina Bifida is classified as a FMS 3,2,1 this means that the child can walk with forearm crutches for 5 meters, uses a walker up to 50 meters and beyond that a wheelchair. If another child is classified as a FMS 6,6,6 it means that the child can walk without support for 5 meters, 50 meters and 500 meters.

Functional Motor Level

We feel that it is more appropriate and easier to classify patients with Spina Bifida into four main functional motor levels, rather than using specific vertebral levels (like L4).

- (1) The first functional level is the high lumbar-thoracic level in which the child lacks quadriceps strength, and may or may not have hip flexor power; this child will require a high brace such as a RGO or HKAFO for walking. In some instances, due to lack of trunk balance, walking ability is quite difficult and the high level child will then use a parapodium for standing. Most patients with a high level will be able to walk for short distances indoor and so would be classified as a FMS 2,1,1 or 2,2,1. Usually between the ages of 11 and 13 years, the child will elect to use a wheelchair for mobility because of the high energy costs of standing and walking. Exceptions, of course, are seen especially for the child without a ventricular shunt, or for a child who is not obese, has good upper extremity function and no joint contractures. These individuals can retain their walking ability for a longer time through adolescence and early adult life.
- (2) The second functional level is the low lumbar. Here hip flexors, knee extensors and medial hamstrings are present. The child can walk with AFO braces and forearm crutches. Most walk at a speed of between 55-60% of normal. As an adult they typically will be classified in the FMS system as a 3,3,1.

- (3) The third functional level is the high sacral in which the gluteus muscles are present, but weak; the gastrocsoleus strength is below grade 3. These individuals require AFOs to stabilize the ankle and foot; they walk without support with a gluteus lurch that will be more or less severe according to the strength of the gluteus. Their walking velocity is about 70-75% of normal. As an adult they would be in the FMS classification a 6,6,6 or 6,6,3. In our experience, about 94% of these children will retain good walking mobility as an adult.
- 4) The fourth functional level is the low sacral in which the gluteus muscles are strong; the gastrocsoleus is also strong, so they do not require AFOs. They walk without support and their gait is nearly normal or normal with the velocity being at 100%. Less than 5% of Spina Bifida children are classified as having a low sacral level.

Ventricular Shunt vs. No Shunt

Recent studies at our Motion Analysis Center have clearly shown that a child with Spina Bifida without a ventricular shunt walks faster and longer than a child with a shunt. Based on gait analysis parameters, the walking speed of the child without a shunt is 94.4% of normal; the walking speed of the child with a shunt, who has the same functional level, was 76.4%. This represents a 20% difference in speed, which is significant. We also found that the most important issue affecting the walking velocity is shunt infection and not the number of shunt revisions. When we examined sacral level children classified at the FMS 6,6,6 level, 71% were in the no-shunt group, while only 55% were in the shunted group. Again, these numbers are quite significant statistically. We believe that neurosurgeons need to have strong, precise indications for treating hydrocephalus with a shunt. Clearly, child who has Spina Bifida without a shunt functions at a higher level than those who are shunted.

Crutch-walking: 4 Point vs. Swing-thru Gait

We had been taught that the 4 point gait is more functional than a swing-thru gait. So, years ago we undertook a study at MAC; this study showed that the swing-thru gait is 33% faster than the 4 point gait and, what is even more important, the swing-thru gait is 33% more efficient (less oxygen cost) than the 4 point gait. In addition, studies done on upper extremity pain in adults with Spina Bifida have shown that the most common issues are shoulder problems related to patients using a wheelchair and not related with patients using crutches. In conclusion, the swing-thru gait is more efficient, faster and with lower energy cost.

The Effect of Trunk Movement and Valgus Stress at the Knee Joint

Because of gluteus weakness, the child with sacral level meningomyelocele has excessive pelvic and trunk movement, the well-known gluteus lurch. In a study done at MAC, we were able to demonstrate clearly that a significant amount of valgus stress in the knee joint is related to the abnormal trunk movement. The only way to decrease this abnormal stress, which can lead to arthritis and knee pain in adult life, is with the use of forearm crutches. In so doing, the excessive pelvic movements and the excessive trunk lurch decrease. Based on these studies we have attempted to introduce some crutch-walking for long distances in the child with sacral level at an early age, advising that the use of crutches will prevent problems with the knee joint during adulthood.

AFO Brace

About 95% of patients with Spina Bifida do not have enough strength in the calf musculature to stabilize their ankle-foot during gait. For this reason they will require the use of solid AFO braces. Studies done at MAC, as well as at other centers, have shown that the use of the braces allows the ground reaction forces to be more normal, passing in front of the knee joint and in so doing, prevent a crouched gait which, as we know from the energy standpoint, is quite ineffective. Previous studies have shown that an excessive external tibial torsion of more than 20 degrees will prevent the AFO from achieving full extension of the knee at mid-stance. For this reason, and others on which we will comment later, surgical treatment of external tibial torsion is indicated when greater than 20 degrees. Unlike the child who has cerebral palsy child, there is no place for hinged AFO braces in the child with Spina Bifida. The use of the AFO also allows a more normal firing of the musculature above the knee, in particular the vastus and rectus muscles.

As mentioned earlier in our presentation, the use of gait analysis has provided us with a major insight for the gait patterns in Spina Bifida, especially children with a low lumbar or sacral level. We have learned, for instance, that the excessive pelvic rotation is an attempt to increase stride length and any time that this pelvic rotation is decreased, it may affect the child's walking ability. Therefore, in patients with low lumbar and sacral levels, a spinal fusion down to the sacrum will affect the child's gait and, in some children, will take away their walking ability.

Another important lesson from the use of gait analysis concerns the muscles that generate power when walking, in particular the gastrocsoleus, gluteus

and hip flexors (iliopsoas). Since most children with Spina Bifida do not have good gastrocsoleus strength and the gluteus muscles are weak, they need to have strong hip flexors. Any surgery that takes away this flexor power will make gait more difficult. Thus, the iliopsoas transfer (the Sharrard procedure that was quite popular in the 70s) is a procedure that should not be used in Spina Bifida since it will considerably decrease the hip flexor power and make walking more difficult (Duffy).

Hip Dislocation and Subluxation in the Child with Low Lumbar Level

It is taught during orthopedic training that the treatment of a dislocated hip is surgical reduction with bone procedures at the pelvis and femur. So, for many years in the past hip dislocations in Spina Bifida were treated surgically and in some instances and, I must say, even quite frequently, with very poor functional results and somewhat fair radiological results. Back in the late 70s, Feiwell at Rancho Los Amigos emphasized that children with Spina Bifida with a dislocated hip did not require surgical relocation. It was more important to have a normal or nearly normal range of motion, i.e., a hip free of contractures. The work in Philadelphia by Ames also supported Feiwell's statements.

In the 90s at MAC we undertook a retrospective study of gait in individuals who had low lumbar level with a unilateral dislocation of the hip. We found that the gait symmetry was present when the dislocated hip had a nearly normal range of motion; however, gait asymmetry was present if the hip had either a flexion or adduction contracture. Clearly this study well documented Feiwell's statements in the late 70s, that the hip motion was more important than the dislocation of the hip. For the last 15 years we have taken a very conservative approach to the dislocated hip in the child with low lumbar level. If a contracture(s) is present leading to an asymmetrical gait, surgical correction of the contracture(s) is done, using a procedure such as an adductor myotomy or hip flexor lengthening and, occasionally in a more severe deformity, a valgus osteotomy of the proximal femur. However, we do not feel that there is a need to surgically relocate the hip. On the other hand, for the sacral level child who presents with a unilateral dislocation where the gait asymmetry is quite severe and the gluteus lurch on the located side also is quite severe, we feel, based on the knowledge from the gait analysis, that surgical relocation is indicated through pelvic procedures such as the Dega osteotomy and the varus derotation osteotomy of the proximal femur. However, it must be said that no papers have been published to substantiate this latter statement.

Crouched Gait

A crouched gait is quite common in Spina Bifida secondary either to a combination of hip flexion and knee flexion contractures or to an excessive external tibial torsion (over 20 degrees). The knee flexion contracture in Spina Bifida develops gradually. Most of these children have weak gastrocnemius and weak gluteus muscles. They walk with a mild degree of knee flexion initially. With time, the posterior knee capsule gets tighter and shorter and the crouched gait becomes more severe, usually between the ages of 10 to 14 years. We found that the degree of flexion contracture at the knee on the examining table doubles when the patient is walking. For instance, if a patient has a knee flexion contracture of 20 degrees, during gait the knee will be in a flexed position at mid-stance at 40 degrees. A 40 degree crouched gait is very inefficient and the oxygen cost is quite high, usually 40% more than normal. Therefore, surgical treatment for the crouched gait is indicated when a knee flexion contracture is about 15 to 20 degrees or greater. A posterior capsulotomy is required in order to achieve full extension. The posterior cruciate ligament is left intact. If any contracture of the medial hamstrings or biceps is present, a selective lengthening is also indicated. A recent study at our Motion Analysis Center has shown an average improvement of at least 20 to 25 degrees when this surgery is performed. Occasionally, in an older patient who has a low lumbar or sacral level with a severe knee flexion contracture, a supracondylar osteotomy of the femur may be required in addition to the knee flexor release. We do not have any experience with the patellar advancement procedure that is used in patients with cerebral palsy.

Valgus Stress at the Knee Joint

Valgus stress at the knee joint in Spina Bifida is quite common in the low lumbar and sacral level, especially the sacral level child who walks without support. As mentioned before, the excessive pelvic/trunk movement is a major cause of this stress. The valgus stress is multi-factorial. A number of deformities can increase the stress including: (1) excessive pelvic rotation; (2) abnormal trunk movement; (3) femoral antetorsion; (4) knee flexion contracture; (5) external tibial torsion; (6) valgus deformity of the hindfoot. The use of gait analysis, especially the kinetics can demonstrate quite clearly this abnormal internal varus moment at the knee. When present, surgical treatment is indicated such as a derotation osteotomy of the tibia, a knee flexor release, and correction of the hindfoot valgus with a medial sliding osteotomy of the os calcis. For the excessive pelvic rotation and trunk movement, the only solution is the use of external support, i.e., crutches. Derotation osteotomy of the tibia for either excessive external or internal tibial torsion should be done over the age of 5 years. We recommend that the osteotomy be done in the distal third together with a fibular osteotomy; we also advise the use of internal fixation with a DCP AO plate. Complications

can occur such as delayed union and wound dehiscence. A very well planned surgical procedure and post-operative care is important.

Foot Deformities in Spina Bifida

To conclude, I would just like to give a few thoughts about foot deformities in Spina Bifida. About 95% of children with Spina Bifida will have some type of foot deformity. In some it is mild and does not require any surgery. Others will have severe deformities requiring surgical treatment. The main goal of our treatment for foot deformities is to have a flail, flexible, plantigrade and braceable foot. Whenever possible, any kind of fusion such as a subtalar joint fusion or triple arthrodesis should be avoided. For established, severe bone deformities osteotomies can be done in the hindfoot and midfoot area, while still preserving motion at the joints. For contractures, simple tenotomies with tendon excision can be performed. Rarely should a tendon transfer be done unless the child has a low sacral level and is walking without AFOs. The most common foot deformities are talipes equinovarus (TEV) and the calcaneovalgus foot. For TEV the procedure to achieve full correction is the posteromedial-lateral release through a Cincinnati incision. For the calcaneovalgus foot, the anterolateral release is a quite effective procedure. For the cavus or cavovarus deformity, which is more common in patients with lipomeningocele, bone procedures such as the Dwyer calcaneus osteotomy, the cuboid osteotomy and medial cuneiform osteotomy in association with soft tissue procedures can achieve a very braceable and plantigrade foot.

Ankle Valgus and Hindfoot Valgus

A common deformity seen in Spina Bifida is valgus deformity of the ankle joint; this is related to a gradual shortening of the fibula. With the lack of lateral support at the ankle, the distal tibial physis develops in a wedge shape. This leads to the development of a valgus deformity at the ankle. At the same time, quite frequently the valgus at the ankle is also associated with a valgus deformity of the hindfoot. A very effective procedure for correcting the mild to moderate degree of ankle valgus is the hemi-epiphysiodesis with a cannulated screw and, for the hindfoot valgus, the medial sliding osteotomy of the os calcis is the procedure of choice. In order to prevent pressure problems from the brace, we must have some flexibility at the joints, ankle, subtalar and midfoot joints. Again, this is why the arthrodesis will make the foot too rigid resulting in a higher incidence of pressure problems.

In summary, we have been able to outline most of the areas in which orthopedic care has changed in the last 30 years. I want to emphasize one more time the value of motion analysis for achieving a better understanding of

the Spina Bifida gait pattern and thus improved decision-making in the selection of procedures that will improve function and not just improve the radiological picture.

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Table 1.

1. Reciprocal and swing gait in Spina Bifida
2. Energy cost on distance of walking for the high level, low lumbar and sacral level children
3. Trunk kinematics and knee valgus stress
4. Knee valgus stress and its relationship with external tibial torsion
5. Crouched gait: surgical treatment and results
6. The effect of shunt and no shunt on the walking velocity and FMS classification
7. Rotational deformities of the femur
8. Rotational deformities of the tibia
9. The Shanz valgus osteotomy for hip dislocation in Spina Bifida
10. Unilateral hip dislocation in the low lumbar level
11. Results of surgical treatment for clubfoot deformities
12. Results of surgical treatment for calcaneus deformity: the anterolateral release
13. Surgical treatment for vertical talus
14. Talectomy: surgical results
15. Foot deformities in lipomeningocele
16. Fibular growth and ankle valgus
17. Surgical treatment for hindfoot valgus: the medial sliding osteotomy of the os calcis
18. Knee flexion contractures and surgical treatment
19. Knee extension contractures and surgical treatment
20. Hip dislocation/subluxation in the low lumbar level
21. Surgical treatment for external tibial torsion
22. Surgical treatment for internal tibial torsion
23. Shunt revisions and their effects on gait
24. Tethered cord: surgical results
25. Kyphosis: natural history
26. Surgical treatment of kyphosis
27. Surgical treatment of scoliosis

Meeting of the Section on Orthopedics

General Themes

The care of children with Spina Bifida and musculoskeletal problems continues to evolve. Major themes presented included the increasing use of motion or gait analysis, the need for improved evidence-based literature, and the awareness and prevention of musculoskeletal problems encountered by patients transitioning into adulthood. Presentations on gait analysis (including the plenary lecture) from two centers provided different perspectives on the role of gait analysis for clinical evaluation, decision-making, and evaluating outcomes of therapeutic procedures. The use of motion analysis in Spina Bifida as a research tool has provided objective assessment for our interventions. The body of literature using the highest grades of evidence-based interventions (levels of evidence and grades of recommendation) for musculoskeletal disorders such as hip dislocations and spinal deformities in meningocele remains limited. To date no randomized clinical trials for orthopedic interventions in Spina Bifida has been published. Spina Bifida is no longer a “disease of childhood” as this unique patient population transitions into adulthood. Coordination of care including education to promote independence must begin long before adulthood is reached. Orthopedic treatments during childhood have long-term implications for and impact upon quality of life issues for adults.

Historical Perspective

Recent advances in the orthopedic management of Spina Bifida are best understood in a historical context. This perspective was given in the presentation entitled, *The History of Orthopedic Care and Spina Bifida* by Jack V. Banta, MD. Innovations and advances in surgical care have depended on the close collaboration between orthopedists and neurosurgeons. These include the development of the ventricular shunt to control hydrocephalus and the recognition of neural pathology such as tethered cord and syringomyelia. Early treatment strategies for hip dislocation in Spina Bifida targeted the same techniques used for developmental dysplasia of the hip (DDH). However, lack of sensation in children with Spina Bifida led to poor results. Recognizing the relative importance of hip stability versus mobility has led to a reversal in surgical philosophy. Evolving from the polio era, treatment strategies such as arthrodesis of an insensate extremity resulted in Charcot changes in adjacent joints in children with Spina Bifida.

The early concept that the “quadriceps is the passport to ambulation,” based on the contributions of Williams and Menelaus from the Royal Children’s Hospital in Melbourne, Australia, is now recognized as only applicable to the first decade of life. The basic tenets of orthotics for ambulation include

low energy cost at a reasonable rate of speed (at least 30% to 60% of normal for age), independence in donning and doffing the braces, and independence with transfers. The priority of walking changes as children enter adolescence and the concept of efficient mobility (e.g., wheelchair ambulation being less energy demanding) takes precedence over independent ambulation. The treatment of spinal deformities in meningocele has made significant progress since the introduction of Harrington instrumentation fifty years ago. Advances in both anterior and posterior spinal instrumentation such as pedicle screw fixation have improved the outcomes for scoliosis and kyphotic deformities.

Gait Analysis

The routine use of gait analysis in meningocele is relatively new. In addition to the plenary lecture, Jeff Thomson, MD provided key points in the management of ambulatory patients in his presentation, Motion Analysis Today for Patient Evaluation and Decision-making. Gait analysis has been beneficial in surgical decision making, provides objective information on pre-operative abnormalities, and allows for the post-operative assessment of orthopedic interventions, including the use of orthotics and surgical procedures designed to improve a child's overall gait and energy efficiency. It is thought to be the best tool we currently have to measure outcomes. Hip abductor weakness impacts a person's ambulatory skills more than quadriceps weakness. Excessive motion of the pelvis in all three planes, as a result of hip abductor weakness, results in decreased function of the lower extremities and the knee in particular. Increased truncal lean produces a ground reaction force lateral to the knee, a net adduction knee moment and a kinetic knee valgus thrust that could result in premature degenerative changes. Ankle foot orthoses (AFO's) may increase transverse plane motion, and gait analysis has shown that the use of crutches and higher bracing above the knees sometimes are necessary in ambulatory patients. Gait analysis has been important in recent studies evaluating surgical procedures such as rotational osteotomies for dysfunctional lever arm, hip surgery, and tendon transfers about the ankle.

Physical Therapy

Colleen Krombach, PT, presented Physical Therapy: Advances in the Evaluation and Treatment of Meningocele. The importance of the manual muscle test (MMT) was emphasized in the presentation. Monitoring the motor examination annually can be invaluable in detecting any deterioration in CNS function, determining muscle imbalance and, potentially, identifying a tethered cord. Asymmetry in the MMT is suggestive of a tethered cord, whereas a symmetric decrease in the MMT is more consistent with shunt problems.

Surgical Procedures

The controversial subject of hip surgery and the lack of evidence-based guidelines for orthopedic interventions in Spina Bifida was presented in depth by James Wright, MD from the Hospital for Sick Children in Toronto. A thorough literature review failed to find a single randomized clinical trial (RCT) for orthopedic studies in Spina Bifida. The five levels of evidence and the four grades of recommendation used to evaluate peer-reviewed papers were applied to the current literature on hip and spine surgery in meningo-myelocele. The World Health Organization International Classification of Function provides a method of assessing whether treatments like hip and spine surgery improve function. The majority of studies were retrospective and not long-term. Based on studies with levels of evidence of one to three, the overall recommendations for both hip and spine surgery were grade B. Evidence was insufficient to determine if reduction of unilateral hip dislocations improved function. Also, an all pedicle screw approach for spinal deformity was found to have insufficient evidence to support its efficacy in the current body of literature.

Conklin et al. [1] reported on the incidence of ventriculo-peritoneal (VP) shunt failure following extradural spinal procedures. The authors found that four patients (20%) required a shunt revision within 12 months, while one patient required shunt revision within three months. Their data suggest that extradural surgery, unlike intradural surgery, is not associated with an increased rate of shunt failure. Martin et al. [2] reported a higher rate of complications (35%) and revisions (31%) than previously had been described in ambulatory patients with lumbar level MMC following tibial rotational osteotomies.

Hip Surgery

Emlinger and Mazur [3] presented a meta-analysis on the efficacy of treatment for hip dysplasia in ambulatory children with meningo-myelocele. Forty-seven papers published between 1964 and 2008 that had a level of evidence of grade 4 or higher were reviewed. In patients with motor levels of L3 or higher (thoracic/high lumbar), 33/37 papers recommended against treatment except for soft tissue release due to high complication rates; no difference in ambulation was found in dislocated vs. reduced hips, and energy expenditure for ambulation was high. In patients with L4 or below (low lumbar/sacral) 28/41 papers recommended hip reduction to help balance muscle forces, maintain reduction, and to develop more normal hip anatomy and mobility for ambulation. Thirteen of the published articles not supporting hip reduction cited the potential surgical risks and complications, the potential for further weakening hip musculature, and the finding that dislocated hips had little effect on ambulation compared to surgically

reduced hips. For unilateral hip dislocations, three papers advocated hip reduction as a way to reduce the subsequent risks of limb length discrepancy, pelvic obliquity, and scoliosis. Controversy still remains concerning the best approach for the management of hip dysplasia in patients with meningomyelocele.

Scoliosis and Kyphosis Repair

B. Stephen Richards, MD, presented advances in the management of scoliosis. A recent study by Wai et al. [4] discussed by both Dr. Wright and Dr. Richards, evaluated function and deformity following spine deformity in meningomyelocele. The benefits of surgery included improved sitting balance, fewer pressure sores, and, questionably, the avoidance of pulmonary problems. However, the paper also noted that no relationship existed between the Cobb angle and function, and that surgery did not improve overall function based on the Activities Scale for Kids (ASK) questionnaire. The paper put into perspective the need for careful patient selection, and the need to thoughtfully weigh the benefits vs. risks for each patient. Surgery should

Child's walking ability:

1. Uses wheelchair, stroller or buggy
2. Uses K-Walker or other walking frame without help from another person
3. Uses two crutches without help from another person
4. Uses one crutch or two calipers without help from another person
5. Independent on level surfaces: does not use walking aids or need help from another person
6. Independent on all surfaces: does not use any walking aids or need any help from another person when walking, running, climbing and climbing stairs

be avoided in patients who are functionally ambulatory with lumbar scoliosis, and individuals with extensive posterior scarring and rigid deformities with abnormal posterior elements. Pre- and intra-operative traction should be considered for rigid curves greater than 100 degrees. Also anterior fusion should be considered and may take on additional importance to maintain correction if the posterior instrumentation becomes infected and requires removal to control and eradicate the spinal infection.

The benefits of correcting sagittal plane spinal deformity in meningocele were addressed by John Sarwark, MD in his presentation, *Advances in the Management of Kyphosis*. Uncorrected kyphotic deformity may result in the reduction of the anterior abdominal wall that may make urinary diversion procedures more difficult. Severe kyphosis also may compress the abdominal contents upward, potentially limiting pulmonary function and result in the recently described secondary thoracic insufficiency syndrome. Costo-pelvic impingement may also occur from severe kyphotic deformities. The optimal age to consider surgery is when the children are younger, when the benefits outweigh the risks. The technique has previously been described by Nolden et al. [5] Preserving the thecal sac, decancellation of the apical vertebral bodies, instrumentation distally to the sacral alae with limited arthrodesis decreased morbidity and allowed continued growth of the thoracic spine.

Adult Care

As with many other conditions that we treat, it cannot be overemphasized that Spina Bifida is not a “disease of childhood.” Shubra Mukherjee, MD, emphasized the importance and timeliness of this statement in her presentation, *Coordination of Care and Transitioning of Children with Meningocele*. Education as part of care coordination should begin early before the critical adolescent phase. Preventive measures should be directed to minimize the risks of obesity, pressure sores, and to maximize functional independence. Children by the age of twelve years should be well versed in their own care and independence. A new concern noted among adult patients with Spina Bifida is lymphedema. The cause of lymphedema is unclear; no signs of deep venous thrombosis are present. Presently the treatment requires the careful use of compression stockings. Specific orthopedic issues for adults include achieving a plantigrade foot in non-ambulatory patients, and proper fitting orthotics to address valgus deformities of the knee and ankle in adult ambulatory patients.

Future Directions for Research and Care

1. Evidence-based treatment guidelines

Current treatment guidelines for musculoskeletal problems in meningomyelocele are based on only a few studies that have higher levels of evidence. The external demand for evidence-based outcomes and increased awareness of the need for well-designed clinical studies such as prospective multi-centered studies, should improve the quality of research.

2. Gait analysis as a clinical and research tool

Gait analysis remains the best objective tool to measure present and future therapeutic orthopedic interventions. Evidence-based studies using gait analysis provide better-defined treatment guidelines. Improvement in the reliability and validity of gait analysis should increase their acceptance for more widespread use. Functional analysis of alternative forms of mobility and movement used by children and adults (wheelchair ambulation, stair climbing, sit to stand) is an important future direction of research in meningomyelocele.

3. Transitioning to adulthood

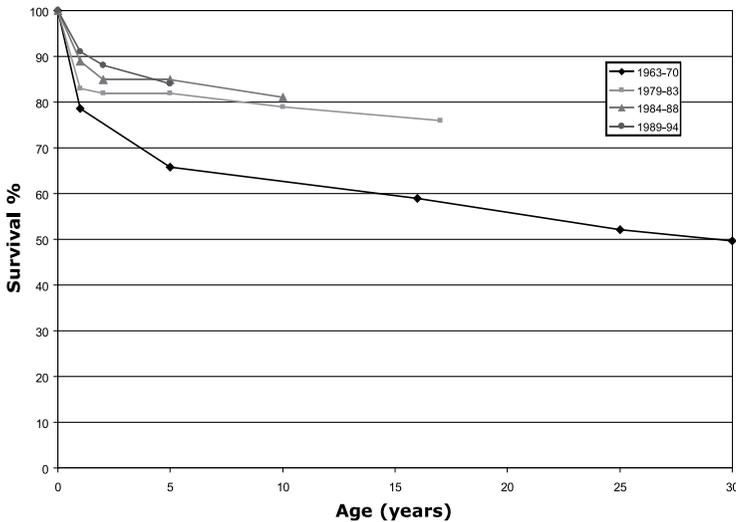
The body of literature addressing musculoskeletal issues in the adult patient with Spina Bifida is quite sparse. Future research should identify and define the musculoskeletal problems confronting adults, examine treatments used during childhood and their effects on adults, and develop strategies to prevent orthopedic problems in adults.

Developmental Pediatrics

Health Care and Spina Bifida: The Canary in the Coal Mine

Gregory S. Liptak, MD, MPH
Professor of Pediatrics
Director, Center for Development, Behavior and Genetics
Upstate Medical University
750 East Adams Street
Syracuse, NY 13210
Email: LiptakG@upstate.edu

Survival by year of birth



Introduction

This presentation was inspired by the deaths of two young adults with Spina Bifida that occurred in the past year, as well as the care of several adults in regional hospitals. The first death occurred in a 29-year-old man who had thoracic level meningomyelocele with shunted hydrocephalus. He graduated from high school, attended a community college for a year, and then lived with his parents. He developed a relationship with a young woman and moved away from home in an apartment that was supported by his family, his 'significant other' and payments from the SSI (Supplemental Security Income) program. His romance failed, though he continued liv-

ing in the apartment by himself after his companion left him. He became depressed and developed a decubitus ulcer that was not identified early. The ulcer became infected, he developed osteomyelitis, and subsequently died from sepsis.

The second death occurred in a 27-year-old woman who had thoracic level meningomyelocele, shunted hydrocephalus and obesity. She had sustained a pulmonary embolus after urological surgery when she was 19-years-old and was maintained on anticoagulants. She lived at home with her parents. She died from hemorrhage, presumably related to an adverse interaction between ibuprofen and warfarin.

Meanwhile, a urologist in a nearby community told a young adult that she does not screen adults with meningomyelocele for bladder cancer because “she does not believe that it is a major concern.” Around the same time, an adult with meningomyelocele was admitted to a community hospital with new onset headache with neck pain. The neurosurgeon interpreted the pain as radiculopathy and did not consider the possibility of shunt malfunction.

Canaries in Coal Mines

Coal mines are dangerous places, subject to gases like carbon monoxide and methane, which are potentially fatal but cannot easily be detected. From the mid 19th century to the mid 20th century (1986 in the UK [1]) canaries were brought down into coal mines in the United States and United Kingdom to warn the miners of unsafe conditions. Canaries are much more sensitive to carbon monoxide than humans [2]. The miners would observe the birds as they worked. If the canaries stopped singing, became unsteady on their perches or died, the miners immediately would evacuate the shaft.

Spina Bifida

Meningomyelocele (Spina Bifida) has been described as ‘the most complex birth defect compatible with life’ [3]. Individuals with this condition have impairments of the spine (e.g., scoliosis), spinal cord (e.g., neurogenic bowel and bladder, sexual dysfunction, paralysis and decreased sensation), and brain (e.g., hydrocephalus, Chiari II malformation with hypoventilation, seizures, strabismus, learning disabilities) [4]. Furthermore, children do not ‘outgrow’ these impairments or disabilities. As Woodhouse noted, “Sadly, nothing in Spina Bifida gets better with age.” [5] In an evaluation of adolescents and young adults (AYAs) with Spina Bifida, we [6] found that the percent reporting excellent health decreased from 29%, when their average age was 15 years (Wave 1), to 11% four years later (Wave 3); 44% of the sample who had reported excellent health at Wave 1 reported fair or poor health at Wave 3. These findings are troubling, especially since health and well-being

typically do not decline during adolescence and young adulthood in people without a chronic condition. Previous studies also reported declining health among young adults with Spina Bifida. For instance, Guarnieri and Vinchon [7] found that adults lost ambulation and continence and developed renal failure as well.

The thesis of this paper is that canaries in coal mines are analogous to people with Spina Bifida in the healthcare system. People with Spina Bifida are more sensitive to changes in the healthcare environment, and the current medical environment is dangerous and becoming more so. They are bellwethers for outcomes of care that will be coming to the rest of society.

Changes in the Care of People with Spina Bifida

In the 1950s an effective ventricular shunt system was developed by Holter and Spitz [8]. At that time the ileal conduit was the standard for urological care until Lapedes' [9] paper on clean intermittent catheterization. Prior to the 1960s, many newborns with Spina Bifida in the USA and UK were not treated surgically; many who were had complications including shunt infections and mental retardation. Because the prognosis was felt to be so poor, Lorber [10] recommended criteria for deciding who to treat and who to allow to die; this approach eventually was abandoned. Figure 1 shows survival by year of birth for four cohorts of children; the 30-year survival in the Hunt and Oakeshott cohort was only 50% [11].

Table 1 lists changes in the treatment of individuals with Spina Bifida since the 1970s. Significant technological improvements in care have improved survival, although the improvements seem to be smaller with every cohort (see Figure 1). Also, significant variations in the treatment of people with Spina Bifida exist, reflecting uncertainty and disagreement about standards of care. For example, Zegers et al. [12] reported, "The responses from 41 centers in 14 European countries confirm that . . . there is no consensus among European centers in terms of protocols for preventing, diagnosing and treating UTIs in children with NBSD [neurogenic bladder sphincter dysfunction] and for CIC [clean intermittent catheterization]." Also, the effects of care on quality of life, daily activities and social participation are not well established. For instance, studies examining the effects of enterocystoplasty have not been uniform or always of the highest grade. According to Scales et al. [13], "Almost all studies are retrospective, single institution case series of a relatively small number of patients. Few uniform or validated outcome measures for enterocystoplasty exist. . . . Interinstitutional variability in urodynamic measurements and in definitions of continence make comparisons of outcomes difficult."

Outcomes in adults with Spina Bifida continue to be less than optimal. For example, in a study of Dutch AYAs, where the mean age was 21 years, only 16% were living independently, more than one-third went to special secondary education, 53% of those who finished education did not have a regular job, and 71% did not have a partner [14].

Health Care in the United States

Health care in the United States has been described as medical care delivered through a patchwork of public and private entities and paid for by another patchwork of public and private insurers [15]. Insurance coverage is usually tied to the workplace or to government programs that often seem Byzantine in their intricacy. The current fee-for-service system is based on encounters with patients and favors volume; the focus is on illness not wellness. Acute care interventions and procedures are rewarded financially rather than prevention or longer chronic care interventions. According to Gladwell [16], “The United States has opted for a makeshift system of increasing complexity and dysfunction.”

Government insurance often pays less for care than private insurers. For example, according to the Wall Street Journal, “Currently, Illinois payments to such specialists are so low—estimated at 30% of what the care actually costs the doctors—that many pediatric specialists in Illinois refuse to treat indigent Medicaid children.” [17] In many communities it is extremely difficult if not impossible to get certain specialty care, like plastic surgery and gynecology, for children on Medicaid. [18].

Persons with disabilities who have health insurance often are unable to get equipment, supplies and services not covered by their plans. They experience more problems than others with follow-up care, availability of specialists, getting to doctors, and obtaining help during off hours. In order to save money, insurance plans may exclude individuals with disabilities like Spina Bifida, enrolling only healthier people. They may stint on care, denying necessary services to individuals who are enrolled. Both exclusion and stinting deny access to care for people with Spina Bifida.

Because of the complexity of Spina Bifida, a multidisciplinary care model that provides care coordination [19] has been recommended. Several organizations, including the American Academy of Pediatrics [20] and the Spina Bifida Association [21], have advocated a multidisciplinary approach to these individuals. Although this has not been studied formally, it appears that programs providing multidisciplinary care to children with Spina Bifida are shrinking in size as providers, especially surgeons, find that they cannot justify their salary by working in these clinics.

Even though the impairments of Spina Bifida do not disappear, adults with Spina Bifida get relatively less health care compared to adults without chronic conditions than is the case with children. For example, Ouyang et al. [22] compared health expenditures for people with Spina Bifida to expenditures for people without Spina Bifida; while the ratio of expenses was 13 in those from 1 to 17 years of age, it was only 6.3 in those aged 18 to 44 years. While AYA with Spina Bifida continue to need long-term follow-up by urologists, neurosurgeons, orthopedists, therapists, orthotists, and others, finding such experts who are willing and able to take care of adults is difficult [23]; finding programs that can coordinate their care is even more difficult.

Many countries currently are experiencing an economic recession; some hospitals are closing or laying off employees [24]. Because a multidisciplinary care program that treats people with Spina Bifida cannot make a profit or even break even, the threat to these programs, and to the health-care of the individuals treated by them, have been magnified by the recession. Furthermore, in March 2009 the jobless rate in the USA exceeded 8%, the highest it has been in 26 years [25]. Because health insurance coverage is tied to employment, increasing unemployment means that fewer people and families will be covered by insurance. Thus, like coal mines, the health-care environment is dangerous, with optimal care being increasingly difficult to find.

The Future

Table 2 lists some interventions that should improve the care of people with Spina Bifida in the near future. These interventions rely on technology and are not sufficient by themselves. A system of care is required to be able to deliver the complex care that people with Spina Bifida need and to integrate care within the community. An effective disease-management program [26] that uses evidence-based clinical practice guidelines and provides care coordination as well as integration with community systems of care is necessary to allow improvements in technical care to be delivered to people with Spina Bifida.

The population in the United States is aging [27]C:\Documents and Settings\adarnley\Local Settings\Temporary Internet Files\Content.Outlook\3S9FIZC9\[http:\www.ofm.wa.gov\pop\gma\default.asp] and individuals with Spina Bifida are surviving longer. However, more physicians are becoming subspecialists and hospitalists at the expense of general internal medicine [28]. Thus, it is not clear who will provide a medical home in the community for adults with Spina Bifida.

Conclusions

Canaries warned coal miners of the dangers in mines. People with Spina Bifida are medically fragile, and, like the canaries, are warning us of problems with our healthcare system. It is one thing for a canary to die to warn others that conditions are dangerous. It is quite another for a child or young adult to die to warn us about our healthcare system. Our current system is unable to provide adequate treatment for everyone with Spina Bifida, coordinate care and achieve high quality outcomes [29-31]. Adolescents and young adults have declining health; young adults are dying. Yet, unlike the national program established for people with cystic fibrosis [32] we do not monitor even the death rates of people with Spina Bifida. The ability of the healthcare system to provide optimal care to people with Spina Bifida is a critical test of the system. There is of course the human concern; there also is the concern that the care that people with Spina Bifida receive now will be an indication of the care that the rest of us will receive in the near future.

Meeting of the Section on Developmental Pediatrics

Focusing on the common goal of improving the care provided to individuals with Spina Bifida, the Developmental Pediatrics session introduced both general and disease-specific concepts of chronic care management. Incorporating these strategies is currently difficult because of poor care coordination and limits on resources for health care utilization. In the past, the focus of discussion frequently was on acute management and the care of expected secondary conditions; insufficient emphasis was placed on preventive care or on care across the life span.

To address these broader issues, a series of questions were proposed as a framework to the panel of presenters:

- Is a disease management model applicable to Spina Bifida care?
- What can be learned from other conditions and how can these ideas be applied to this population and incorporated into their care?
- What collaborative steps can be taken to provide optimal care for individuals with Spina Bifida?

Disease Management

The central goals of the traditional disease management model are, in order, to decrease cost and to improve health care. Decisions made using this model, however, should be based on a scientifically derived base of evidence. Additional goals to improve outcome and adherence to care plans are also essential. The impact of co-morbidities must also be considered in the model. Ideally, what results is a model of care that is timely, efficient, evidence-based and continuous. In the case of complex lifespan conditions such as Spina Bifida, there is an inherent struggle between the need for specialization and the need for integration.

Within this care model, it is essential that individuals know how to manage their care for optimal results. Successful self management promotes autonomy and identifies the patient as the source of control for their health care. Optimal patient care includes integration of information from various sources and joint decision. Ideally, the resulting system is transparent, anticipatory and cooperative. Providing effective care requires developing a partnership with each patient, developing a care plan, coordinating

disparate systems to integrate their care, and providing patient education resources and delivery systems.

Common to disease management in any chronic condition are struggles with adherence to the plan of care, communication between provider and patient as well as between providers and outside sources. Knowledgeable providers need to evaluate environmental factors such as geographic location and issues related to transportation. Specific difficulties occur when implementing a disease management model for individuals with Spina Bifida. A decreasing number of programs adversely affects access. The patient population is aging; they are developing “adult diseases” that may be unfamiliar to pediatric providers. In addition the co-morbid conditions of their Spina Bifida (especially some of the cognitive differences) may be unfamiliar to adult providers.

One way to measure “success” within such systems of care is to look at specific outcomes through quality improvement initiatives: care delivery, access, hospitalization rates, complications, and mortality. Successful systems eliminate waste and are not solely focused on cost savings. Successful systems of care are inherently safe, having been designed to avoid errors, and promote early detection and prevention. They cannot be dependent only on the actions of individuals. Questions, however, remain: 1. Who will best monitor all of these activities? 2. Who decides what “better” care is? Other organizations (e.g., Cystic Fibrosis Foundation) have successfully advocated for assessment and implementation of care using specific criteria (process of care). Their structure includes the ability to measure and compare the results of changes made in providing care as well as who should provide such care. This systematic collection of data on a broad scale is in its very early stages for individuals with Spina Bifida through the ongoing Centers for Disease Control (CDC) Spina Bifida Association (SBA) Spina Bifida registry project.

Care guidelines have been developed for a number of chronic disorders. Many are based on expert opinion. Ideally, they should be accessible to providers, should direct inpatient and outpatient care, and should promote future research efforts. Such documents may be particularly important as individuals with chronic disease survive into adulthood. This component of an evidence based, disease management practice is already available to providers caring for individuals with Spina Bifida: Guidelines for Spina Bifida Health Care Services Throughout the Lifespan, which is available through the SBA.

Successful disease management requires appropriate coordination of care. Unfortunately, no single definition of care coordination exists. Some would say that “they know it when they see it!” Nor does agreement exist on whose responsibility it is. By default, it often becomes the families or the individu-

als themselves who takes on these tasks. Even in individuals with routine health care needs, care coordination is difficult. The current health payment system provides few incentives to coordinate care; increasingly providers are advocating for additional funding to provide this service. What may eventually make this possible for individuals with chronic health care needs and their providers is the concept of a Medical Home. To implement these ideas and meet these challenges, changes by patients and providers will be needed. However, change is hard. Effective communication and accurate outcome data is essential if this change is to be successful.

What Can We Learn from Others?

Specific clinics for children with Spina Bifida have existed since the early 1950's (Wilmington Children's Hospital). In many institutions, multidisciplinary clinics for children with Spina Bifida benefited not only those with Spina Bifida but also improved the quality and timeliness of care for other children with disabilities. Important innovations and observations originated in these Spina Bifida clinics. Many of these are now considered core care aspects including clean intermittent catheterization and avoidance of latex. Data collection and analysis on issues such as selective care of affected individuals and methods of delivery were the basis for better evidence based treatment decisions. The best example of a coordinated and evidence based project is the government sponsored MOMs study, which is evaluating the efficacy of prenatal surgery for meningomyelocele.

Cystic Fibrosis Foundation

In looking for ways to improve the multidisciplinary models and processes of care delivery for individuals with Spina Bifida, it is important to look at the history of these efforts and to integrate those lessons with those from other chronic disease management models (e.g., cystic fibrosis). Since 1955, the Cystic Fibrosis (CF) Foundation has served numerous roles: accreditation, registry maintenance, guideline development, professional and patient education, research, and care innovations. The practical outcome of this process is to help identify and suggest interventions for systemic problems by sharing what the best clinics are doing. The recent Spina Bifida Association (SBA) effort to describe the levels of clinical care available at different locations across the country is a first step toward a similar system.

The CF Foundation has also demonstrated the need and value of a registry and the importance of patient care guidelines. Their registry systematically collects valuable information and documents variations regarding natural history, effectiveness of new therapies / innovations and quality of care using factor analysis. For this to be successful, clinics have to believe that the data are valid; enrollment and data collection must be complete, timely and

of the highest quality. SBA and the Center for Disease Control are currently piloting a patient registry for Spina Bifida.

SBA Care Guidelines

The Spina Bifida Association recognized the importance of clinical care guidelines in 1990. With publication of the 3rd edition in 2006 of *Guidelines for Spina Bifida Health Care Services Throughout the Lifespan*, an effort was made to move away from opinion and toward data and levels of evidence as the basis of these guidelines. These are guidelines and not standards of care. The strengths of the current model include its brevity and standardized format, its functional and developmental orientation across the lifespan, its emphasis on continuity of care and its defining of functional outcome goals. A weakness is that it does not list the actual documents reviewed to make the recommendations.

For families, the guidelines could serve as an instrument to better understand the care delivery systems and the goals of that care. They could also be used as part of transition preparation and self care management training for adolescents and young adults. Primary care providers, especially adult focused providers, might use the guidelines as reference document for ongoing care. Numerous other proposed uses for the guidelines beyond clinical care have been suggested. Clinics might use them as a basis for seeking institutional funding or service support or as a basis for service payment. They could also be used for program evaluation: does a clinic follow the guidelines across the age ranges and does a clinic have all the necessary and recommended specialties?

The Medical Home

The Medical Home may be a viable model to provide continuous care and disease management for individuals with Spina Bifida across the lifespan. An excellent medical home effectively combines place, process, and people to allow a patient to receive effective care. It includes families, youth, and health care professionals in a system of care; it cannot successfully exist solely on the willingness of individual practitioners. Key requirements of this care model include communication and a family centered focus. In the medical home model, communication between primary care providers and specialists is emphasized. Specialty care should supplement and not supplant the primary care role. This model organizes assistance in a collaborative fashion and in accordance with each individual family's wishes, strengths, and needs. The family is the unit of attention.

Platform Presentations

The importance and contributions of successful centrally funded, multisite studies and advocacy by national organizations for Spina Bifida (SB) is clear. They allow implementation and assessment of care models based on objective measurements and data and not just anecdotal experiences. Quality care and management of individuals with Spina Bifida require the integration and cooperation of numerous specialties and a careful consideration of the entire individual and their family. Basic science and clinical research can each contribute to future improvements. Each of these core assumptions was quite apparent in the session's platform presentations. Data from several multisite studies examined topics as divergent as mental health and bowel and bladder continence.

Mental Health

Brei et al. examined mental health from the points of view of the adult caregiver/parent as well as the adolescent/young adult (AYA). Both groups reported poor mental health adaptation over time, which could certainly have implications for successful transitioning to adulthood. The adult reporters associated mental health adaptation with neuropsychological deficits and lower socioeconomic status while AYA associated lower mental health adaptation with less positive adolescent beliefs and low family resourcefulness. Because perceptions and the factors associated with mental health adaptation outcomes vary between parent and AYA report, clinicians promoting positive mental health adaptation need to evaluate both groups.

Bowel and Bladder Management

Urinary and bowel incontinence are nearly universal in individuals with Spina Bifida. Achievement of bowel and bladder continence is an important goal for children with Spina Bifida due to its far reaching impact on both physiologic and social outcomes. Continence is difficult to quantify and differences exist in definitions and acceptable degrees of wetness or numbers of accidents. Untreated, incontinence leads to significant limitations and participation in life activities. Achieving bowel and bladder continence enables full participation and significantly affects self-esteem and quality of life. Smith et al. utilized quantitative and quality of life qualitative methods to assess the effectiveness of selected bowel and bladder interventions on both physiologic outcomes and quality of life measures. A number of factors appeared to influence continence in their study: child and family participation in the prescribed program, environmental factors, medications prescribed, and surgical interventions. Freeman et al. found quality of life (QOL) scores were significantly lower for children with bowel incontinence

and, to a lesser extent, bladder continence based on parent report. Their results support the importance that continence status can have on participation in meaningful life activities for children with SB and how parental views of about continence are addressed. The structure and power of a multisite, centrally funded study allowed Merkens et al. to compare rates of bowel continence among medical and surgical treatment modalities, which has not been previously reported. Surgical treatment (antegrade enema instillation) was related to better functional continence. Children with bowel continence had higher QOL scores, as reported by either the child or parent.

Transition

As a life span disease, Spina Bifida includes a host of sequelae that adversely impact QOL by limiting participation in typical activities. In developing a life span care model, management strategies that support transition and promote independence are essential. Preparing for transition to adulthood is not intuitive. For families raising a child with a disability, everyday life demands often take precedence over future planning. Curran et al. presented Bloorview's Growing Up Ready framework [<http://www.bloorview.ca/resourcecentre/familyresources/growingupready.php>]. This evidenced-based, comprehensive and auditable framework recognizes the central role of families in the process of growing up. When successful, service providers, families and youth work together using a Shared Management Model to gradually shift responsibility for daily life activities and decisions to the youth as developmentally appropriate.

Independence

Jones et al. presented strategies from their program in Australia. Their aim is to facilitate the development of competent, independent adults who will fully integrate into all aspects of their communities. Success requires that decisions regarding goal planning be made in conjunction with the health team members, the child, the family and others. The value of their approach is that it also improves therapy methods, clinical reasoning and the drive toward best practice.

Hypertension

As a life span disease, Spina Bifida has some very specific sequelae that should also be considered. One example of this is hypertension. Children with Spina Bifida have many risk factors for hypertension (HTN) including neurogenic bladder, recurrent urinary tract infections, sedentary lifestyle, obesity and the presence of a shunt. Miller et al. identified 46% of their SB patients to be hypertensive, a rate significantly higher than the 8% prevalence in the NHANES controls making it a significant public health problem in this population. BMI was the only identified risk factor for HTN in their study.

Overuse Injuries

Gueron et al. reported that upper extremity overuse injuries in individuals with SB are not uncommon and their impact is significant. In addition to chronic pain and an inability to transfer independently, such injuries were also the cause of lost employment and independent housing. Individuals reported increased function and energy levels, with decreased U/E pain after receipt and use of their power chairs. While prevention of common overuse injuries is essential, the introduction of powered or power-assist mobility should be introduced early on, in the late teens or early twenties. In addition, regular performance of targeted stretching and strengthening exercises may help reduce pain and to prevent injury for patients with SB.

Basic Science and Primary Prevention

It is known that 60% of cases of neural tube defects are preventable by maternal folic acid (FA) supplementation prior to conception, but prediction in individual cases is not possible. A basic science study by Mayanil et al. used an animal model to study the de-methylation of histones H₃K₄ and H₃K₂₇ as a potential epigenetic marker for the underlying mechanisms of Spina Bifida. Identifying such markers might eventually lead to better predictive tools and the ability to identify potential therapeutic targets to treat this defect in utero.

The Management of Myelomeningocele Study

Elizabeth Thom PhD

*Eunice Kennedy Shriver National Institute of Child Health
and Human Development*

Maternal-Fetal Surgery Units Group

The Management of Myelomeningocele Study (MOMS) is a Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD) funded randomized trial of prenatal versus postnatal myelomeningocele repair that is ongoing at three clinical centers; the University of California San Francisco (UCSF), The Children's Hospital of Philadelphia (CHOP), and Vanderbilt University Medical Center (VUMC), with a central data and study coordinating center at the George Washington University Biostatistics Center. This report provides an update of the trial status in addition to a description of methods and policies in place to ensure that the study is of the highest standard scientifically and that the study results will be fully interpretable.

Repair of myelomeningocele in utero via hysterotomy began in 1997 based on evidence that neurologic function deteriorates during gestation and that in animal models similar surgery was associated with an improvement in neurologic function. Three centers, VUMC, UCSF, and CHOP, pioneered this technique and as of December 31, 2002, 234 women had received open fetal repair of myelomeningocele. The most striking result was the apparent reduction in placement of ventriculo-peritoneal shunt by one year of age: Of the 181 infants who had reached this age, 89 (49%) had had a shunt placed. This compared very favorably with the generally accepted rate of 80% to 90%. Supporting this result, both the Vanderbilt and the CHOP group demonstrated a marked improvement in the degree of hindbrain herniation following in utero repair.

While these results were promising they were not conclusive. Adequate control groups with which to compare the prenatally repaired infants were not available and the results were subject to bias and confounding. Follow-up was not complete and the outcomes reported were only short-term. Temper- ing enthusiasm for the procedure, concerns were raised regarding the risks to both mother and baby, particularly uterine rupture or adverse outcomes in future pregnancies for the mother, and prematurity with its sequelae for the infant. To determine definitively whether fetal repair of myelomenin- gocele, with its attendant maternal and neonatal morbidity, is warranted required a randomized trial. In 2002, the three maternal-fetal surgery centers and an independent data coordinating center, the George Washington University Biostatistics Center, were funded by the Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD) to

conduct a randomized controlled trial to compare the safety and efficacy of in utero repair of open neural tube defects with that of the standard postnatal repair. Centers other than these three that were interested in developing a maternal-fetal surgery program for in utero repair of spina bifida voluntarily agreed to postpone their efforts until the results of the trial were known.

Recruitment began in 2003, with a sample size goal of two hundred. Patients diagnosed with myelomeningocele are referred to the coordinating center for initial screening via telephone by a trained coordinator (currently a genetics counselor). After obtaining consent for screening, the counselor reviews the women's medical records. Even those who contact or are referred by a physician to one of the three clinical centers are required to pass through the initial screening process at the coordinating center. By this method we are assured of consistent counseling for the women regarding both spina bifida information, the logistical implications of participating in the trial, which are complex, the potential risks and benefits of prenatal surgery based on data published before the trial, and the concept and reasons for randomization. This additional step gives women time to process information and to consider their options at a most difficult point in their lives. We are also assured of consistency in applying the eligibility criteria.

Women at least eighteen years of age carrying a singleton fetus with a myelomeningocele lesion starting from T₁ to S₁ with evidence of hindbrain herniation, normal karyotype and whose pregnancy has not progressed beyond 25 weeks are potentially eligible for the trial. Women have to be resident in the USA, not necessarily a citizen, but with the ability to attend follow-up when their child is 12 and 30 months. Because prenatal surgery is associated with an increased risk of preterm birth, those with an already increased risk of spontaneous preterm delivery are excluded, e.g., those with a previous spontaneous preterm delivery and those with a short cervix less than 20 mm. Two other major exclusion criteria are contraindication to surgery, which encompasses conditions such as prior classical cesarean section, or open fetal surgery, and a body mass index of 35 or higher, again because of potential complications with surgery.

Those eligible and interested are assigned by the counselor to one of the three clinical centers where final detailed evaluation and screening is conducted over two days. Patients who still satisfy the eligibility criteria and consent to the study are centrally randomized via a secure website to prenatal or postnatal surgery. The randomization sequence is unpredictable and stratified by center. The advantage of this method is that the investigator cannot predict the next surgery assignment, which eliminates selection bias. If a patient is randomized to prenatal surgery, she receives the surgery within three days of randomization and once discharged stays in nearby

accommodation, with weekly visits to the MOMS maternal fetal medicine specialist until cesarean at 37 weeks if still undelivered. While this procedure is a potential burden on the patient, it ensures appropriate care in case of threatened preterm delivery or other problem. If a patient is randomized to postnatal surgery she returns home for prenatal care, and returns to the MOMS clinical center at 37 weeks of gestation for cesarean delivery and neonatal repair of the myelomeningocele defect by the MOMS neurosurgeon. Besides consistent neurosurgical repair for the postnatal group, delivering at the MOMS center ensures consistent neurosurgical and urologic management with respect to the need for shunt and clean intermittent catheterization for both groups, as well as complete and consistent data collection, including required radiological images. Costs for study procedures such as radiological imaging are covered by the study if the patient delivers at the MOMS center.

This trial has two main primary endpoints. The first is the need for ventricular decompressive shunting by 12 months of age defined by objective criteria. These criteria are communicated with the community neurosurgeons by the MOMS clinical center neurosurgeons, although actual management is of course in the hands of the infants' caregivers. All infants return for a follow-up visit at 12 months of age and their medical records are obtained. Of note, infants without a shunt who have died before twelve months are automatically considered to have satisfied the criteria for the outcome, since it is not possible to determine whether they would or would not have needed a shunt had they survived. This eliminates the potential bias in case there is a differential in survival between those assigned to prenatal or postnatal surgery. The records and images of all infants, including those for who no shunt has been placed, are reviewed by an independent committee of neurosurgeons, blinded to treatment assignment, to determine whether criteria have been met.

The second primary endpoint is based on a combination of neurodevelopmental outcome as measured by the Bayley Scales of Infant Development Mental Developmental Index (MDI) and motor outcome as measured by the difference between the anatomical lesion level and the motor function level. All infants return to the center for developmental testing and a physical examination. The Bayley score is determined at 30 months of age by an independent psychologist trained specifically for the study and blinded to treatment assignment. The difference between the anatomical and functional lesion levels is also determined by trained examiners. Since this is a difficult assessment, physical exams to determine motor function level are videotaped and reviewed by a single expert for consistency. Likewise radiologic images to determine anatomic level are reviewed by an independent committee of radiologists.

In addition, we received supplemental funding to determine whether prenatal surgery confers benefit on urological outcomes. For all infants whose mothers were enrolled after the start of funding, urinalysis, renal/bladder ultrasounds and video-urodynamics are conducted at discharge as well as the 12 and 30-month visits. The primary urological outcome is the need for clean intermittent catheterization (CIC) by 30 months of age, including recurrent urinary tract infections, as defined by objective criteria. Similar to the shunt outcome, if CIC is implemented without meeting criteria, this still qualifies as a primary outcome; thus it is important for urologists to use criteria. Again, an independent committee of experts, blinded to treatment assignment, determines whether criteria have been met. Major secondary outcomes include neonatal morbidity and mortality, Chiari II malformation and other radiological signs, as evaluated by the independent radiologists, other neurodevelopmental /motor testing conducted by the examiners, maternal reproductive outcome and an overall assessment of the child and family.

The common thread in the determination of outcome for this trial is the use of independent review committees and examiners. Since this is an unmasked trial, evaluation is potentially susceptible to both investigator and ascertainment bias. Even the perception of bias can be damaging. Investigator bias can occur when there is a lack of equipoise; thus the independence of the expert review is important. The review committees consist of experts at institutions that are not involved in the MOMS trial; indeed if a MOMS child is receiving care at the expert's institution the expert will recuse himself/herself from the review of the case. Ascertainment bias can occur when the group assignment is known to those evaluating the outcomes; therefore, the examiners and review committees are blinded to treatment assignment wherever possible. For example, the neurosurgeons reviewing the shunt outcome are not given actual neonatal head circumferences to review; instead the data are converted into percentiles. Thus small head circumferences related to prematurity, which is a common outcome with prenatal surgery, are not apparent.

As with any NIH-sponsored randomized clinical trial, a Data and Safety Monitoring Committee (DSMC) has been appointed by the NIH; each member has no conflict of interest and none has been involved in the care of a MOMS child. This is a multidisciplinary group with expertise in Biostatistics, Epidemiology, Maternal-Fetal Medicine, Neonatology, Pediatrics, Neurosurgery, Urology and Ethics; in addition a layperson sits on the committee. The committee is concerned not only with patient safety but also with ensuring that the trial's results are interpretable and that the study is conducted as well as possible. The committee makes recommendations to the NICHD based on a review of the interim data and has the authority to recommend stopping the trial before the planned sample size. Reasons for

stopping early include evidence of benefit or evidence of harm. In addition, if the trial were deemed to be 'futile,' i.e., if very little chance exists of showing a benefit given the data to date, a DSMC might recommend study termination. The committee also considers any external influences and events that alter the premises on which the trial was founded.

We projected that recruitment into the trial would take eighteen months. Unfortunately, this was overly optimistic, and after three years of experience, the projection was revised to six years. As of this writing, after a little over six years, 157 patients out of 200 have been randomized. Although many are eagerly awaiting the results and may not understand why interim results cannot be released, to preserve the integrity of the trial we cannot accede to this desire. Released interim data could induce investigators to make premature or incorrect inferences. Such data could affect how future participants are treated, which could bias the results or even inhibit the trial from finishing. Although this study is unmasked, we follow the procedures as though it were a double-masked trial, so that no outcome data are presented or discussed even with the MOMS investigators. Instead, the DSMC will make recommendations regarding any data that should be made available. In the meantime we continue our efforts to ensure that all women in the United States who are interested have access to the trial and encourage all with questions, comments as well as potential participants to contact us at 1-866-ASK-MOMS.

Appendix 1

Hal Pote Memorial Lecture

These remarks were delivered by Judy Woodruff on Wednesday, March 18, 2009 to attendees of the First World Congress on Spina Bifida Research and Care.

I'm deeply honored to have been asked to deliver this lecture in memory of a dear friend – the man who did so much to raise the profile of Spina Bifida, to raise money for research on Spina Bifida, to strengthen the Spina Bifida Association and to create the Spina Bifida Foundation.

Hal Pote was a banker and if he had been in charge, I'm confident the banking crisis would never have happened! He had incredible devotion to the cause that brings us all together – and remarkably, it was not because he himself was born with Spina Bifida, nor that he was the parent of a child with Spina Bifida. In Hal's case, it was through his extraordinary caring and compassion for his nephew. Hal always said because he was fortunate enough to have been healthy and done well in his life, he wanted to give back to others. He had a gift of boundless energy and he focused so much of that energy on Spina Bifida. I remember many conversations with Hal, his focus and his endless enthusiasm. We miss him terribly, but the light he brought to the work you do, shines on.

If Hal were here today, he would join me in congratulating you on this conference – on what I'm told is turning out to be a very successful first World Congress on Spina Bifida: Research and Care. Don't be modest about that! Someone said this morning how pleasantly surprised they were at the breakthrough work being discussed here. That is an exciting development. I wish I could have participated in more of your sessions than this morning. Just being here a short time reminds me of what a great group of people who work on Spina Bifida are! It is thrilling for me to know that you are learning from each other and that specialists from all facets of the Spina Bifida community have come together to compare notes and share ideas about research and about care. I know you have good reason to do this and I would just add that it certainly reflects the lives that individuals with Spina Bifida lead – they wake up every morning facing a complicated and cross-cutting set of challenges, ranging from how do they get around physically, how do they go to the bathroom (in my view, the most important question of the day!), to how do they deal with assorted but constant medical issues and a large network of physicians and other medical care providers.

I've built a career as a news reporter and interviewer – always trying to keep my personal views out of the stories I cover. But it probably won't come as a surprise there is nothing objective or dispassionate about my view of Spina Bifida. It's a deeply emotional subject for me, one I have lived with for the past 27 and 1/2 years, since our older son Jeffrey was born. Some of you have heard me tell the story of how my husband, Al Hunt, and I had never heard of Spina Bifida... when, in August of 1981, in the 8th month of my pregnancy with our first child, we scheduled a routine sonogram – to give us a better idea when the baby would be born. When the technician told us he saw something we should discuss with the doctor – enlarged ventricles in the baby's head (ventricles – another term we had not known) and the possibility of an opening in the baby's back – with spinal cord damage – they couldn't tell because my rib obscured the lower spine – we were overcome with worry and fear. What did this mean? The baby wouldn't be normal? He might need surgery? We had a million questions – and we spent the final month of my pregnancy in a state of high anxiety – still excited about the coming birth but dreading the news no parent wants to hear.

Jeffrey was born – on time – a beautiful bouncing baby boy – by C-section, at the doctor's request – with Spina Bifida, an opening in his lower back. We cried tears of happiness and privately, of sorrow, for the loss of what we didn't know and in fear of what lay ahead. When Jeff was 12 hours old, Dr. David McCullough (the late pediatric neurosurgeon and a wonderful human being) closed the opening in Jeffrey's back and did repair work that he explained and we didn't understand very well; it was all a blur. We came home from the hospital after a week; we settled into a dizzying routine of doctor visits, especially watching, as it turned out, his head because of those enlarged ventricles. Eventually Jeffrey's head became noticeably large and at 10 months, even though he was showing no developmental delays yet, Dr. McCullough recommended a shunt. I remember that hospital stay like it was yesterday – how scared we were and how quickly Jeff recovered. He was standing in his crib the day after the surgery: Mr. Personality!

Despite all the warnings we were given, Jeff began to walk on time at 13 months, and he ran (a little flat footed) and climbed and was the most active little boy you could imagine. If it weren't for that shunt, I would have forgotten about any limits on his mobility. But in the back of my mind was always a fear that he'd fall and hit his head. Jeffrey paid no mind; he rode a bike, fell and split his chin, went roller-skating with his mother's "Be careful" ringing in his ears – only to crash into the front door and cut his chin again – more stitches! He had no fear: climbed trees, got into all sorts of scrapes, learned to ski, was a natural! The only things he steered clear of were competitive ball sports, like soccer, after about age 6, because he noticed every other boy, and even the girls, ran faster than he could. It broke my heart, to watch a mandatory foot race at a school field day, when Jeff came in dead last. He

didn't show that it bothered him but it obviously did! He was a smart student, despite his learning disabilities– I actually brought him to two Spina Bifida national conferences to learn all we could about those frustrating “learning differences” – how hard math was, for example, and of course to find out anything we could about how to deal with the most embarrassing issue of all – bladder and bowel incontinence. Jeff catheterized himself starting at age 5, in pre-kindergarten. He was occasionally teased for being wet or having an accident and he mostly handled it remarkably well. But at a school where all the other children seemed 100% healthy though, he clearly felt different.

Still, he soldiered on, switched schools to get more academic support and grew increasingly interested in a career in medicine. He wanted to find a way to prevent Spina Bifida and he became fascinated with the study of infectious diseases along the way. He did junior internships at the CDC for a few weeks as a 14 year old. He interned at the NIH in a cancer lab as a 15 year old and had been hired for an internship at the FDA for the summer after 10th grade, when the dreaded recommendation came that he should get a shunt replacement. He hadn't had any problems in 16 years – so he was very reluctant to go through with the procedure. But he did.

And there were complications – complications that changed Jeff's life forever and the lives of all of us who love him.

That was 10 years ago. Fast forward to today: Jeff is 27, attending his 8th year of college at a small school in North Carolina. He uses a wheelchair, needs help for all transfers, has the use of only one arm and has both vision and speech impairments, as well as major short term memory loss. He will always need companion care; his life is enormously circumscribed by his physical condition. But he has a spirit and a determination that are truly inspiring. He is determined to get a college degree and he is on track to do so after one more semester. He says his dream job would be to run a recreational and therapeutic facility for children with disabilities. Of course he would need considerable help to do that and we are starting to focus now on finding the right living arrangement for Jeff post-college that would allow him to pursue that dream or any other. And you won't be surprised to know how difficult that is; this country is dreadfully lacking in living and work opportunities for individuals with disabilities (I saw the 66% unemployment number for adults with Spina Bifida)! They are cute when they're young but a problem when they're older – that's the unspoken attitude. Having said that, there is something unstoppable about Jeffrey Hunt. And one way or another we want to make sure he has the fullest life he possibly can. We thank him for opening our eyes to the world of those with disabilities. That is the greatest gift he's given us, other than himself.

Our journey with Jeffrey – a journey that has involved (as all such journeys do) our entire family, including his younger brother and sister, has been – and continues to be – a long and winding road. Jeff has borne the toughest burden of all. And when I have allowed myself to slip into feeling sorry for us – to question, “why Jeff?” – I’ve realized there is no answer of course.... just as there is no answer for any of the thousands of parents who ask – why my child?

But Jeffrey himself doesn’t dwell on this. And we quickly figured out it does no good for ANY of us in our family to dwell on why and what might have been. It doesn’t get any of us very far, and it doesn’t serve the needs of these children, young people and adults, who live with this maddeningly complicated birth defect over the course of a normal life span.

We owe them much more than any backward look, and that’s why the research and other work you and your colleagues in the scientific and medical community do is so important.

It’s also why – not as a journalist who can’t take sides in these debates but, as a mother – I am able to celebrate the proposed increases in medical research in the new federal budget, and the funding that was part of President Obama’s economic stimulus package which Congress recently passed.

Despite the findings that public and private financial support of biomedical research increased dramatically over the decade from 1994 to 2004, there is no denying that over the past 6 or 7 years, since the early 2000s – the nation has seen a flat-lining in government investment in health-related research and development.

As recently as 2007, the overall trend in funding for research to improve health across the board was stagnant. What small spending increases there were, were eaten up by inflation. In 2007, the United States invested only 5 and 1 1/2 cents of every dollar spent on health care, in research.

So it was noteworthy that President Obama proposed and Congress approved \$10 billion for the National Institutes of Health in the Economic Recovery and Reinvestment Act – better known as the stimulus package.

And it’s believed the budget proposal for 2010 that President Obama sent to Congress a couple of weeks ago, will ultimately include a strong commitment to medical research, although there are few hard numbers available at this early stage of the legislative process.

Still, even as we celebrate these positive developments – and the fact that since 2002, when the National Center on Birth Defects and Developmental Disabilities was established, that it has received more than \$750 million for its work – still, the amount of money for research into Spina Bifida has been

comparatively small: some \$50 million in NIH funding over the past 5 years; another \$24 million for the National Spina Bifida Program, since it was established by Congress five years ago, and housed at the Centers for Disease Control. And to clarify, that money is devoted to, not only research, but also work on public awareness and outreach. Separately, another \$10 million over the past five years has been spent on Folic Acid related work – again, research, outreach, and other programs.

I had a first-hand glimpse of just how much more MIGHT be done, at the basic level of research, when I visited a lab recently at the Medical Center at the University of Colorado, Denver – the Anschutz Campus, in the Pediatrics Department. A scientist, Dr. Lee Niswander, and two graduate students were engaged in a fascinating three-pronged study.

To sum it up roughly, it involves: first, studying the normal process of neural tube closure to understand how the cells interact with one another, using dynamic imaging. Second, trying to understand the genes that are required for neural tube closure by identifying genes that cause neural tube defects. And third, trying to understand how genes interact with the environment, especially folic acid, in the prevention of neural tube defects.

Dr. Niswander told me it had been surprising to her to learn how little directed research had gone into understanding Neural Tube Closure and Neural Tube Defects. She said, it had instead been a rather haphazard or random mutation found in one lab or another without a specific interest in the subject. She said that was why she and her colleagues decided about seven years ago to try to tackle this more systematically by directly screening for mutations that affect Neural Tube Closure in the mouse embryo. They generated mutations in the mouse genome, and then looked for Neural Tube Defects. Using a process of working backwards, they've generated a large collection of mice, and have had considerable success in identifying the mutant genes, as well as figuring out how these genes work to allow closure of the neural tube. They have also discovered novel genes not previously recognized for their role in this process.

I was particularly interested in the research team's use of dynamic imaging to understand the closure process in mouse embryos. Previously, they said, as far as they knew, it had been studied only in non-mammals. Their early findings confirm that different parts of the neural tube come together in different ways – some by a process like “zippering,” and other – higher up – by sending out cellular “feelers” reaching across the gap between the neural folds. They told me this suggests that perhaps specific genes control closure differently from one region to another. I've already gone WAY beyond my understanding of what I saw, but what was important, was their effort to understand and identify all the genes involved – and building on that – to

understand eventually what therapies, in addition to folic acid supplements, may help prevent neural tube defects. We know that most, but not all NTD's, can be prevented by folic acid.

These are uncertain times in Washington. The financial crisis and the economic slide have come on fast, and have policy-makers scrambling for answers.

Our new President and the team he's put together are working non-stop to deal with collapsing financial institutions, toxic assets in the surviving biggest banks, rising unemployment, home foreclosures and a growing credit freeze. Just to name a few of the problems on their plate.

It's gotten ugly. Fingers are pointed in every direction over who is to blame.

But as someone who has covered Washington for over 30 years, even though I've not covered a recession quite like this one, I believe the worst of this mess will get sorted out. The financial landscape will look different of course; not every institution will survive and many have already gone under.

The argument that President Obama makes that this crisis presents an opportunity – a moment to address several other festering problems on the national agenda, including health care, is being challenged by critics who say he should focus single-mindedly on the economy. There's no doubt in all our minds that the economy is number one, but for the long-term well-being of our society, of the American people, it is hard to believe it's a good idea to delay yet again addressing questions of access and affordability of health care and yes, the need for research. From a parochial standpoint, as the parent of a child with Spina Bifida, and as a citizen, it's hard to accept the argument that delay makes sense.

That's why the work you do at this Congress, which goes on despite the recession, is so important. On behalf of family members everywhere – all those with a loved one who has suffered because of Spina Bifida – for all of us who would give anything to know no one else had to live with this terrible complex birth defect and all its complications – on behalf of all of us, thank you for all you do and all you will go on to do from here.

Appendix 2

Participant List

Jennifer Abrams MSW, LSW
Shriners Hospital for
Children-Chicago

Karen Rodrigues Abreu
AACD

Elizabeth Adams
OHSU

Caryline Adkins
Le Bonheur Children's

Kayleen Ala RN
Arnold Palmer Medical Center,
Orlando, FL

Ann Alriksson - Schmidt
CDC/NCBDDDD

Haluk Altioek
Shriners Hospitals for
Children - Chicago

Sheila Amoedo
SARAH Network of
Rehabilitation Hospitals

Brice Antao MD
Leeds Teaching Hospital NHS Trust

Mimi Ardis
Spina Bifida Clinic/Peoria, IL

Karen Arena RN
Spina Bifida Association of
Western PA

Brian Armour MD
Centers for Disease Control
and Prevention

Sherry Arvin
SBA

Christopher Austin MD
University of Iowa

Kim Baker RN
Gillette Children's Specialty
Healthcare

Anthony Balcom MD
Medical College of Wisconsin

John Banta, M. D. MD
Connecticut Children's
Medical Center

Mathilde Barbeau
CRDP-CA

Tera Bartelt BSN
Children's Hospital of Wisconsin

Peter Bartoò
Children's Faculty Hospital
Bratislava Slovakia

Roger Bayston PhD, FRCPath
University of Nottingham

Patricia Beierwaltes CPNP
Children's Hospital of Michigan

Melissa Bellin PhD, LCSW
University of Maryland School of
Social Work

Caryn Benline
Advocate Medical Services

Timothy Bertram PhD
Tengion Inc

Jeffrey Blount MD, FACS, FAAP
University of Alabama/ Children's
Hospital of Alabama

Rebecca Boudos MSW
Children's Memorial Hospital

Robin Bowman MD
Children's Memorial Hospital

Jenna Brand MSN, RN, CPNP
VCU Health System

Patricia Braun RNC, CPNP, PhD
Loyola University Medical Center

Timothy Brei MD, FAAP
Indiana University School of
Medicine

Angela Brentrup
University of Muenster, Germany

Virginia Briggs PhD
University of Massachusetts
Medical School

Lyndsey Bristow
Spina Bifida and Hydrocephalus
Association of Southern Alberta

John Brock, III MD
Vanderbilt Children's Hospital

Jean Brown MS, APRN
Shriners Hospitals

Cindy Brownstein
Spina Bifida Association

Karin Buchanan
LeBonheur Children's Hospital

Laurien Buffart PhD
Erasmus MC, Department of
Rehabilitation Medicine

Robert Burke
Hasbro Children's Hospital

Ryan Callaway

Cynthia Camille FNP
Duke University

Vince Campbell
CDC/NCBDDD

Michael Carr MD, PhD
Children's Hospital of Philadelphia

Maria Cartmill
Nottingham University Hospital
NHS Trust

Laura Caruso
Codman, A Johnson & Johnson Co.

Ana Laura Chang
AACD

T Debuene Chang
NIH/NDDK/KUH

Earl Cheng MD, FAAP
Children's Memorial Hospital,
Chicago

George Chiang
San Diego Children's Hospital

James Chinarian
Children's Hospital of Michigan

Anna Christison CTRS
Shriners Hospitals for Children

Paul Cirino PhD
University of Houston -
Texas Medical Center Annex

Jerry Clayton Ph.D.
Children's Hospital Denver

Bruce Cohen
VitaPath Genetics

John Collins
The Ottawa Hospital

Andrew Combs RPA-C
Columbia University Medical Center
Morgan Stanley Children's Hospital

Michael Conklin MD
Children's Hospital of Alabama

Charles Curry
Kennedy Krieger Institute

Andrea Cvitkovic

Melissa Danielson
CDC/NCBDDD

Amanda Darnley
Spina Bifida Association

Drew Davis MD
Children's Health System

Musa Murat Dayanç
Gulhane Military Medical Academy

Gregory Dean MD
Temple University

Marvalyn DeCambre MD
Rady Children's Hospital

Betsy DeCesare
Children's Hospital of Alabama

**Marc DelBigio MD, PHD,
FRCPC, BSc**
University of Manitoba,
Department of Pathology

Maureen Dennis PhD
The Hospital for Sick Children

Katie Devine
Loyola University Chicago

Catherine deVries
Pediatric Urology

Luciano Dias MD
Rehabilitation Institute of Chicago

Brad Dicianno MD
University of Pittsburgh
Medical Center

Julie Dicker RN
The Children's Hospital at West-
mead Sydney NSW Australia

Irene Dietz
Metrohealth Medical Center

Nienke Dosa MD, MHP
Center for Development,
Behavior, and Genetics,
SUNY Upstate Medical University

Mary Jo Dunleavy RN, BSN
Children's Hospital Boston

Brian Elmlinger MD
Nemours Children's Clinic

Michael Erhard
Nemours

Carolina Facciani
Astra Tech

Andrea Fairman
University of Pittsburgh

Ana Maria Fernandez Lauretta

Ann Flanagan PT, PCS
Shriners Hospitals for
Children - Chicago Unit

Jack Fletcher PhD
University of Houston

Ann Forster
MetroHealth Medical Center

Suzanne Fortuna CNS, FNP
Rainbow Babies And Children's
Hospital

Israel Franco MD
New York Medical College

Kurt Freeman
Oregon Health & Science University

Dominic Frimberger MD
University of Oklahoma Health
Sciences Center

Jacki Frost
Shriners Hospital for Children
Tampa

Herbert Fuchs
Duke University Medical Center

Michael Gallant M.D.
All Children's Hospital

Selenge Gantumur

MCS LLC

Kimberlee Garver LCSW, BCD

SUNY Upstate Medical University

Carla Garwood RN

University of Michigan

Bishoy Gayed MD

University of Pittsburgh Medical
Center

Timothy George

Pediatric Surgical Subspecialists

Roslyn Gleeson RN, BC, MSN

Spina Bifida Resource Network, NJ

Marianne Gorzkowski

Shriners Hospital Chicago

Eileen Gray

Salve Regina University

Roseann Greene RN

Children's Memorial Hospital

Adriane Griffen MPH, CHES

Spina Bifida Association

Judy Griffey

Nationwide Children's Hospital

Laura Gueron PT, MPH

Gillette Lifetime Specialty
Care Clinic

Rochelle Haas MD

AI duPont Hospital for Children

Dorothy Harris

Thames Valley Children's Centre

Khader Hasan PhD

U of Texas Health Science Center at
Houston

Daniel Herz

Children's Hospital at Dartmouth

Adonis Hijaz

Upstate Medical University

Vickie Hobensack

Nationwide Childrens Hospital

Judy Hodge

SUNY Upstate

Grayson Holmbeck PhD

Loyola University Chicago

Yves Homsy MD

Children's Urology Group

Amy Houtrow

UCSF

Cynthia Hulse

Penn State Hershey

Takayuki Inagaki MD

Kansai Medical University

Jared Jackson MA

Loma Linda University

JoLinda Jackson

Childrens Hospital of the
Kings Daughters

Alison Jones

The Children's Hospital at
Westmead

Joyce Jones

SBA

Susan Jordan

The Walter E. Boehm
Birth Defects Center

David Joseph MD

UAB Pediatric Urology

Jenifer Juranek PhD

UT Houston

Olena Kakhnovets

Optica

Jerie Beth Karkos MD

Children's Hospital of Wisconsin

Evan Kass

Beaumont Hospital

Jonathan Kaye MD

Pediatric Urology, Emory University
School of Medicine

Heidi Kecskemethy RD, CSP, CDT

duPont Hospital for Children

Stephanie Kelleher BSN RN

Medical University South Carolina

Jessica Kelly

Coloplast

Lizabeth Kelly PT

Pediatric Physical Therapy, Inc

Yusuf Kibar MD

Gulhane Military Medical Academy,
Ankara, Turkey

Christine Kinavey PhD

Kaiser Oakland Medical Center

Shelly King MSN CPNP

Indiana University

Russell Kirby PhD, MS, FACE

USF College of Public Health

Kristi Kirschner

Rehabilitation Institute of Chicago

Gloria Krahn

CDC/NCBDDD

Susan Krinsky RN, CRRN

Children's Urology Associates

Colleen Krombach

Children's Memorial Hospital

Brad Kropp M.D.

University of Oklahoma Health
Sciences Center

Julia L'Abbe RN, BScN

Bloorview Kids Rehabilitation

Susan Labhard MSN, RN

Shriners Hospital for Children

Chantal Ladouceur

Spina Bifida and Hydrocephalus
Association of Southern Alberta

Yegappan Lakshmanan

Children's Hospital of Michigan

Jennifer Langton

Kerry Lavan

The Children's Hospital

Cynthia Lazzaretti

UCSF Medical Center

Lynette Lee

Concord Hospital

Robin Leger RN, PHD

University of Connecticut
Health Center

New England Musculoskeletal
Institute

Paul Levett

Liberator Medical Supplu Inc.

Eric Levey MD

Kennedy Krieger Institute

Julian Lin

University of Illinois

Ronna Linroth CT, MA

Gillette Lifetime Specialty
Healthcare

Gregory Liptak MD, MPH

University of Rochester;
Medical Center

Dennis Liu

University of Toledo/St. Vincent
Mercy Children's Hospital

Lori Lowe MSN, RN, CPNP-PC

Riley Children's Hospital

Michelle Lyon

Astra Tech

Mary MacBlane MS, PNP-BC
SUNY Upstate Medical University

Amy Maggio
SBA of Illinois

Bryan Malas MHPE, CO, CPed
Children's Memorial Hospital

Sanjay Mardolkar MD
St. Vincent Mercy Children's
Hospital

Keith Marosek
Coloplast

Shelden Martin MD
Loyola University Medical Center

Mary Mastel-Smith
VA Health Administration Center

Scott Maurus
Hollister, inc.

C. Shekhar Mayanil PhD
Children's Memorial Medical Center

John Mazur
Nemours Children's Clinic

Lynnette Mazur
Shriners Hospitals for Children

Rhachelle McCraw
Spina Bifida

Suzanne McKee RN, BSN
Arnold Palmer Medical Center,
Orlando, FL

Kyle McLean NP
UWMF

Deborah McLeish
American Family Children's Hospital

Catherine McMillin OTR/L
Gillette Lifetime Specialty
Healthcare

Michelle McNeill
London Health Sciences Centre

Jose Medina-Bereciartu
Asociación Venezolana de la
Espina Bífida

Kathleen Meehan-Farrar RN
Children's Medical Services

Hal Meltzer
Rady Children's Hospital

Marcie Mendelsohn
Cincinnati Children's Hospital

Mark Merkens MD
Oregon Health & Science University

Tom Meurrens MD
UZ Leuven

Jennie Mickelson
Children's Memorial Hospital

Rosalia Misseri MD
Riley Hospital for Children

Victoria Moerchen PT, PhD
University of Wisconsin-Milwaukee

Abd Rashid Mohamed
College of Social Work

Penelope Molina
Associação Médica de Bento
Gonçalves

Shubhra Mukherjee MD, FRCPC
Rehab Institute of Chicago

Joe Mulinare MD
Ctrs for Dis Ctl & Prev

Catherine Muth
University Urology, PLLC

Vitale Myrtha
Sociedad Argentina de medicina
Física y Rehabilitación

William Nantau B.Sc, CNIM
William Beaumont Hospital
Royal Oak

Rachel Neff Greenley PhD
Medical College of Wisconsin

Carol D. Nelson LSW
Gillette Lifetime Specialty
Healthcare

Ann Neville-Jan PhD, OTR/L
USC/CHLA

Mike O'Brien
Royal Children's Hospital

Michelle O'Byrne MS
The University of Texas
Medical School

Colleen O'Toole RN
IWK Health Center

Godfrey Oakley MD
Rollins School of Public Health;
Emory University

Greg Olavarria MD
Childhood Neurosurgery

Martina Oneko MD
Kilimanjaro Christian
Medical Centre

Laura Orgeron
Astra Tech

Kathryn Ostermaier
Texas Children's Spina Bifida Clinic

Lijing Ouyang PhD
CDC/NCBDDD

Ann Paal MD
Estonian SpBH

Fernando Palacio
UroMed, Inc.

John Park MD
University of Michigan

Michael Partington MD, FACS
Gillette Children's Specialty
Healthcare

Lorna Patanella
University of Rochester

April Pate BA, ATP
Gillette Lifetime Specialty
Healthcare

Jogi Pattisapu MD
Childhood Neurosurgery and Center
for Hydrocephalus and Arnold
Palmer Hospital for Children

Kenneth Peters MD
Beaumont Hospital

Paula Peterson PNP
Primary Children's Medical Center

Leslie Phillips CPNP
Children's National Medical Center

Trish Piascik CRNP, PNP-BC
St. Christopher's Hospital for
Children

Daniel Preudhomme MD
University of South Alabama

Ann Price MD
Vanderbilt University School of
Medicine

Taophee Rabi MD
UCH, Ibadan, Nigeria

Norman Ramirez
Hospital La Concepcion

Gail Rampersaud MS, RD, LDN
University of Florida

Ravindra Rao
Loma Linda University
Children's Hospital

Karen Ratliff-Schaub
Nationwide Children's Hospital

Karen Rauen MSN, RN,
BCIAC-PMDB
Children's Hosp. WI

- Veena Raykar**
St Joseph's Hospital Auburn
- Roozbeh Rezaie PhD**
University of Texas Health Science
Center at Houston
- Cosimo Ricciardi**
Self employed
- B. Stephens Richards MD**
Texas Scottish Rite Hospital
for Children
- Shenandoah Robinson**
Rainbow Babies & Children's
Hospital
- Philippa Rodie**
BPNA
- M. Victoria Rodriguez-Casero**
Royal Children's Hospital
- Irina Rogaleva**
Association Spina Bifida and
Hydrocephalus
- Melanie Rolli MD**
Novartis Pharmaceuticals
Corporation
- Susan Ropacki**
- Samuel Rosenfeld**
CHOC
- David Sacco**
Neurosurgeons for Children
- Piali Samanta**
Novartis Pharmaceuticals
Corporation
- Dino Santacroce MD**
University of Toledo
- John Sarwark MD**
Children's Memorial Hospital,
Chicago, IL, USA
- Kathleen Sawin DNA,
CPNP, FAAN**
UWM and Children's Hospital
of Wisconsin
- Susan Sawyer MBBS, MD, FRACP**
Royal Children's Hospital
- Ben Scanlan**
Spina Bifida Association of Arizona
- Diana Schwab RN**
Spina Bifida Association
of Western PA
- Sharon Sellet BAAE**
Spina Bifida Association of
Cincinnati
- Wan Tew Seow**
KK Women's & Children's Hospital
- Jeff Shiflett**
All South Services for
World Congress
- Mikyong (Meekie) Shin DrPH**
NCBDDD, CDC
- Howard Silberstein**
University of Rochester
- Edwin Smith MD**
Emory University School
of Medicine
- Kathryn Smith RN, MN**
USC University Center for Excellence
in Developmental Disabilities
- Warren Snodgrass MD**
University of Texas, Southwestern
Medical Center/ Children's Medical
Center in Dallas
- Fabio Sparapani**
Universidade Federal de Sao Paulo
- Camie Spieker RN**
Seattle Children's Hospital

Anne Stanton
University of Kansas Medical Center

Craig Stevens
UroMed, Inc.

Ben Stewart
All South Services for World
Congress

Janet Stewart
Children's Hospital Denver

Janice Stilkey
Maine Medical Partners

Mark Swanson
Centers for Disease Control and
Prevention

Parvin Tajik MD
Tehran University/Medical Sciences

Heather Taylor PhD
University of Texas Health Science
Center at Houston

Julius Teague
Greenville Hospital System

Patricia Thaker
University of Wisconsin Madison

Judy Thibadeau RN, MN
Center for Disease Control
and Prevention

Elizabeth Thom PhD
The George Washington University
Biostatistics Center

Katherine Thoman-Godshalk
Cincinnati Children's Hospital
Medical Center

Sherri Thomas
Nationwide Children's Hospital

Jeffrey Thomson MD
CCMC Faculty Practice Plan

Michele Torre MD
G. Gaslini Institute, Genova, Italy

Edwin Trevathan
National Center on Birth Defects &
Developm. Disabilities, CDC

Eric Trumble MD
Neurosurgeons for Kids
Disney Children's Hospital
at Florida Hospital

Noel Tulipan
Vanderbilt University

Gayle Tyerman
Shriners Hospital for Children

Amrish Vaidya
KDA Hospital

Christopher Vance
SBAA

Vijaya Vemulakonda
Washington University School
of Medicine

Carla Verpoorten
University Hospital Leuven

Ashish Wakhlu MChir.
King George Medical University

Marion L. "Jack" Walker MD
University of Utah, Primary
Children's Medical Center

William Walker Jr., MD
Seattle Children's Hospital

Chad Wallis
Pediatric Urology

Julian Wan
University of Michigan

Benjamin Warf MD
A I duPont Hospital for Children

William, III Waring MD
Children's Hospital of Wisconsin

Thomas Webb MD, MSc
Cincinnati Children's Hospital

Beth White
St. Vincent Mercy Children's
Hospital

John Wiener MD, FAAP, FACS
Duke University

Alun Williams FRCS
Nottingham University Hospitals,
Nottingham, UK

Lisa Wilsford PA-C, MPH
Shriners Hospitals for Children

Pamela Wilson
The Children's Hospital

Judy Woodruff
Senior Correspondent,
The NewsHour with Jim Lehrer

Alison Wray
Royal Children's Hospital
Melbourne Australia

James Wright MD
Hospital for Sick Children

Kushtrij Xhiha

Chaun Xiao MD
Union Hospital of Tongji Medical
College Huazhong University
of Science and Technology

Elizabeth Yerkes MD
Children's Memorial Hospital

Ruthie Youssefi
Pediatric and Adolescent
Urology Inc.

T. Andrew Zabel PhD, ABPP - CN
Kennedy Krieger Institute

Donna Zahra PhD, ARNP
Nemours Children's Clinic

Kathy Zebracki
Shriners Hospital for Children,
Chicago

Appendix 3

Meeting Agenda

Monday, March 16, 2009

- 6:45 – 8:00 am Continental Breakfast
- 7:30 – 8:00 am Opening Remarks
Scott T. Price, Esq.
Earl Y. Cheng, MD
Edwin Trevathan, MD, MPH
Roger Bayston, PhD, FRCPATH
- 8:00 – 8:40 am Neurosurgery Keynote
Observations on the Evolution of
Thinking about Spina Bifida in Neurosurgery
across an Academic Career
Marion “Jack” Walker, MD
- 8:50 – 9:30 am Epidemiology/Genetics Keynote
Insufficient Progress in the Global Progress of Folic
Acid-preventable Spina Bifida
Godfrey Oakley, MD, MSPH
- 9:40 – 10:00 am MOMS Update
Elizabeth Thom, PhD
- 10:00 – 10:15 am Morning Break
- 10:15 – 10:55 am Transition Keynote
Transitioning Adolescents and Young Adults
with Spina Bifida to Adult Care
Susan Sawyer, MD
- 11:05 am – 12:30 pm Transition Panel
Nienke Dosa, MD, MHP
David G. McLone, MD, PhD
Shubhra Mukherjee, MD, FRCPC
Donna Zahra, PhD, ARNP

- 12:30 – 1:45 pm Lunch
- 1:45 – 5:30 pm Concurrent Breakout Sessions
 Topic areas:
- Developmental Pediatrics
 - Neuropsychology
 - Nursing/Allied Health
 - Urology

Tuesday, March 17, 2009

- 6:45 – 8:00 am Continental Breakfast
- 7:30 – 8:00 am Overview from Previous Day
Timothy Brei, MD, FAAP
- 8:00 – 8:40 am Nursing/Allied Health Keynote
 Nursing Response to Families Living with
 Spina Bifida: Past, Present, and Future
Robin Leger, RN, PhD
- 8:50 – 9:30 am Urology Keynote
 Lower Urinary Tract Refunctionalization by
 Somatic/Autonomic Nerve Root Transposition in
 Children with Spinal Cord Abnormalities
Chuan-Guo Xiao, MD
- 9:40 – 10:00 am SBA/SBF Update
Cindy Brownstein and Timothy Brei, MD, FAAP
- 10:00 – 10:15 am Morning Break
- 10:15 – 10:55 am Neuropsychology Keynote
 Structural Changes and Dysfunction in
 the Hydrocephalic Brain
Marc Del Bigio, MD, PhD
- 11:05 am – 12:30 pm Tethered Cord Panel
Jeffrey Blount, MD, FACS, FAAP
Robin Bowman, MD
Gregory Olavaria, MD
Elizabeth Yerkes, MD

12:30 – 1:45 pm	Lunch
1:45 – 5:30 pm	Concurrent Breakout Sessions Topic areas: • Epidemiology/Genetics • Neurosurgery • Orthopedics • Transition
6:00 – 8:00 pm	Reception

Wednesday, March 17, 2009

6:45 – 8:00 am	Continental Breakfast
7:30 – 8:00 am	Overview from Previous Day <i>David B. Joseph, MD</i>
8:00 – 8:40 am	Orthopedics Keynote The Orthopedic Care of Children with Spina Bifida: What Have We Learned in the Last 30 Years? <i>Luciano Dias, MD</i>
8:50 – 9:30 am	Hal Pote Memorial Lecture <i>Judy Woodruff</i>
9:40 – 10:00 am	Award Papers
10:00 – 10:15 am	Morning Break
10:15 – 10:55 am	Developmental Pediatrics Keynote Health Care and Spina Bifida: The Canary in the Coal Mine <i>Gregory Liptak, MD</i>
11:05 am – 12:30 pm	Closing Remarks <i>David Joseph, MD</i>
2:00 – 5:00 pm	Post Graduate Course (for registered attendees only)

Full abstracts of the work presented during the
First World Congress on Spina Bifida Research and Care are available at
<http://medicalconference.spinabifidaassociation.org>.