National Spina Bifida Patient Registry and UMPIRE:  
inconstenting Care Nationally & Locally

Teal on the Hill  
April 27, 2020
Both Centers (JW & RB) participate in NSBPR and UMPIRE studies and receive grants from Centers for Disease Control and Prevention
In 2005, the Spina Bifida Association (SBA) sent a survey to spina bifida clinics in the US. The survey identified differences in treatment from one clinic to the next. SBA recommended the development of a registry. Congress authorized the creation of the NSBPR.
How does the Registry work?

10,319 participants

- CDC funds Spina Bifida Clinics to participate in the NSBPR
  2008: first 9 grantees
  2019: 14 grantees (5 year funding cycle)
- Some clinics participate in a self-funded capacity
  2019: 8 unfunded sites
- Patients in NSBPR clinics and their families can participate
- Participation is voluntary
- Data are gathered every year on patient visits
What is the purpose of the NSBPR?

To identify health care and clinic practices that are associated with the best outcomes for people living with spina bifida (SB)
What do we collect and why?

• History
  – Questionnaires
    • 2011 – 20 questions
    • 2019 – version 2.6.5 – 43 questions + 3 supplemental for skin breakdown
  – 2014 – Newborn Protocol to Preserve Renal Function – Urologic Management of Newborns and Infants (UMPIRE)
What Do We Collect and Why?

- Participant Characteristics, Interventions, and Diagnostics
- Review the Outcomes
- Utilize the NSBPR data to improve care for all people living with spina bifida
National Spina Bifida Patient Registry

• What has been published so far?

• Publication is important
  – Share what we are learning
  – Improve care for Americans with SB
  – Increase level of science in SB research
  – Prove to Congress & Federal Government that this is a worthwhile investment
Testing the Feasibility of NSBPR

- Findings from 2009-2011
  - First 10 funded clinics
  - Enrolled 2070 patients
  - Described initial demographic info
  - WE CAN DO IT!
The National Spina Bifida Patient Registry: Profile of a Large Cohort of Participants from the First 10 Clinics

Kathleen J. Sawin, PhD, CPNP-PC, FAAN\textsuperscript{1,2}, Tiebin Liu, MSPH\textsuperscript{3}, Elisabeth Ward, RN, MPH\textsuperscript{4}, Judy Thibadeau, RN, MN\textsuperscript{2}, Michael S. Schechter, MD, MPH\textsuperscript{5}, Minn M. Soe, MD, MPH\textsuperscript{6}, and William Walker, MD\textsuperscript{2}, on behalf of the NSBPR Coordinating Committee*
• Findings from March 2009 – June 2012
• 2172 patients at 10 clinics (72-411 pts)
• Age – mean 10 y; 85% under 18 y
• 54% of those > 2 y – community ambulators (45% of MMC)
• 88% & 95% of MMC ≥5 y had bowel & bladder impairment
• Older individuals more likely to:
  – Be continent
  – Have pressure ulcers
  – Not be community ambulators

• Non-Hispanics blacks – less continence

• All outcomes except community ambulation showed significant variation among clinics
ORIGINAL RESEARCH

Factors Associated With Pressure Ulcers in Individuals With Spina Bifida

Sunkyung Kim, PhD, Elisabeth Ward, RN, MPH, Brad E. Dicianno, MD, Gerald H. Clayton, PhD, Kathleen J. Sawin, PhD, CPNP-PC, FAAN, Patricia Beierwaltes, DNP, CPNP, Judy Thibadeau, RN, MN, National Spina Bifida Patient Registry
• Data from 3153 pts at 19 clinics

• 19% reported a skin ulcer at most recent clinic visit

• Risk factors include:
  • Level of lesion
  • Wheelchair use
  • Urinary incontinence
  • Presence of shunt
  • Recent surgery
  • Male sex
Factors Associated with Mobility Outcomes in a National Spina Bifida Patient Registry

ABSTRACT

• Data from 2604 pts aged 5+ at 19 clinics
• Community ambulation was associated with:
  – No shunt
  – Lower level of motor function
  – No history of hip or knee contracture release surgery
Factors Associated with Mobility Outcomes in a National Spina Bifida Patient Registry

ABSTRACT

• Data from 2009 – 2015
• 4448 participants
• 9.15% Chiari II decompression
• Higher the lesion level, increased risk for needing decompression
• Younger children more likely to also need tracheostomy
• Shift away from decompressions since 2005

December 2018
• 4448 participants born at or after 2005
• 79.9% VP shunt
• Range: 72 – 96%
• The higher the lesion level, the greater the risk of needing a VP shunt
• No change in treatment patterns before or after 2005
Bowel management and continence in adults with spina bifida: Results from the National Spina Bifida Patient Registry 2009–15

John S. Wiener\textsuperscript{a,\#}, Kristina D. Suson\textsuperscript{b}, Jonathan Castillo\textsuperscript{c}, Jonathan C. Routh\textsuperscript{a}, Stacy Tanaka\textsuperscript{d}, Tiebin Liu\textsuperscript{e}, Elisabeth Ward\textsuperscript{e,f}, Judy Thibadeau\textsuperscript{e}, David Joseph\textsuperscript{e} and National Spina Bifida Patient Registry

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\textsuperscript{g}Department of Urology, University of Alabama at Birmingham, Birmingham, AL, USA
Bowel Management in Adults with SB

- 5209 participants – 1370 adults (26%)
- Largest prior study – 225 adults
- Bowel continence reported by:
  - 58.3% of adults vs. 45.2% of 5-11 year olds
Bowel Management in Adults with SB

- Of all adults:
  - 14.0% worked full time
  - 18.1% worked part time
  - 20.9% were students
  - 27.9% identified as permanently disabled

- Of 708 adults aged 25 years & older:
  - 19.5% had college degree
  - 27.0% had attended school after HS
Bowel Management in Adults with SB

- Of adults 25 years & older:
  - Bowel continence was not associated with
    - Gender
    - Health insurance status
    - SB type or lesion level
    - Educational attainment
  - Bowel continence was associated with employment
Bladder Reconstruction Rates Differ among Centers Participating in National Spina Bifida Patient Registry


From the Division of Urology, Duke University Medical Center, Durham, North Carolina (JCR, JSW), Department of Urology, University of Alabama-Birmingham, Birmingham, Alabama (DBJ), National Center on Birth Defects and Developmental Disabilities, Centers for Disease Control and Prevention (TL, JKT, EAW) and Carter Consulting, Inc. (EAW), Atlanta, Georgia, Division of Pediatric Pulmonary Medicine, Children’s Hospital of Richmond at Virginia Commonwealth University, Richmond, Virginia (MSS), and Division of Urology, Primary Children’s Hospital, Salt Lake City, Utah (MCW).
Bladder Management and Continence Outcomes in Adults with Spina Bifida: Results from the National Spina Bifida Patient Registry, 2009 to 2015


From the Division of Urologic Surgery, Duke University Medical Center (JSW, JCR), Durham, North Carolina, Department of Urology, Children’s Hospital of Michigan (KDS), Detroit, Michigan, Department of Pediatrics, Baylor College of Medicine (JC), Houston, Texas, Department of Urology, Vanderbilt University (STI), Nashville, Tennessee, Centers for Disease Control and Prevention (IL, EAW, JKT), and Carter Consulting, Inc. (EAW), Atlanta Georgia, and Department of Urology, University of Alabama at Birmingham (DBJ), Birmingham, Alabama

March 2018
• Data from 5528 individuals
  – 20% had undergone bladder reconstruction
  – Surgery rates varied 12-38% among clinics
Variation in surgical management of neurogenic bowel among centers participating in National Spina Bifida Patient Registry

Jonathan C. Routh\textsuperscript{a, \dagger}, David B. Joseph\textsuperscript{b}, Tiebin Liu\textsuperscript{c}, Michael S. Schechter\textsuperscript{d}, Judy K. Thibadeau\textsuperscript{c}, M. Chad Wallis\textsuperscript{e}, Elisabeth A. Ward\textsuperscript{c, f}, and John S. Wiener\textsuperscript{a}

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Longitudinal Study of Bladder Continence in Patients with Spina Bifida in the National Spina Bifida Patient Registry

Tiebin Liu, Lijing Ouyang, Judy Thibadeau, John S. Wiener, Jonathan C. Routh, Heidi Castillo, Jonathan Castillo, Kurt A. Freeman, Kathleen J. Sawin, Kathryn Smith, Alexander Van Speybroeck and Rodolfo Valdez

From the Rare Disorders and Health Outcomes Team, Division of Human Development and Disability, National Center on Birth Defects and Developmental Disabilities, Centers for Disease Control and Prevention, Atlanta, Georgia (TL, LO, JT, RV), Duke University Medical Center, Durham, North Carolina (JSW, JCR), Texas Children’s Hospital, Houston, Texas (HC, JC), Oregon Health and Science University, Portland, Oregon (KAF), Children’s Hospital of Wisconsin and University of Wisconsin, Milwaukee, Wisconsin (KJS), and Children’s Hospital Los Angeles, Los Angeles, California (KS, AVanS)
Surgeries and Health Outcomes Among Patients With Spina Bifida

Noreen B. Alabi, MPH, Judy Thibadeau, RN, John S. Wiener, MD, Mike J. Conklin, MD, Mark S. Dias, MD, Kathleen J. Sawin, PhD, Rodolfo Valdez, PhD
Prevalence of stool continence, by age and diagnosis, in spina bifida patients. The National Spina Bifida Registry, 2009–2013
Prevalence of urine continence, by age and diagnosis, in spina bifida patients. The National Spina Bifida Registry, 2009–2013
Prevalence of pressure sores, by age and diagnosis, in spina bifida patients. The National Spina Bifida Registry, 2009–2013
Percent distribution of ambulation status among patients with myelomeningocele, by age. The National Spina Bifida Registry, 2009–2013
Hispanics were more likely to have bowel incontinence
Only 62% of patients get “proper” surveillance of kidney function yearly.
UMPIRE PROTOCOL

• UROLOGICAL
• MANAGEMENT FOR
• PRESERVATION OF
• RENAL FUNCTION
Design and Methodological Considerations of the Centers for Disease Control and Prevention Urologic and Renal Protocol for the Newborn and Young Child with Spina Bifida


391 participants
At birth, only 4% have concerning US, 16% have reflux, & 8% have abnormal renal scan.
UMPIRE Protocol

Keeping bladders & kidneys safe!
This is what medical progress looks like:

- Largest database for SB in the world & 1st prospective urologic protocol

- Big data creates statistical power to show differences and effects

- These scientific publications prove:
  - The registry & protocol both work
  - Both can teach us new information
  - Both can impact care
  - Continuation is crucial to help to define standards of care for SB
This is what medical progress looks like:

• These scientific publications prove:
  – The CDC is making a difference with a small amount of federal research dollars
  – Your tax money is making a difference for individuals with SB
  – Your tax money is encouraging more talent to devote their careers to improving the lives of Americans with SB
  – More doctors are talking about SB at scientific meetings
So what?

• How are the NSBPR and UMPIRE studies translating into better care for Americans with SB?
NSBPR makes me better

• We spend more time asking about:
  – Education
  – Employment

• Our urodynamic studies have been modified to meet higher standards

• We have more powerful data to share with parents of newborns & young children
  – 76% of adolescent & adults cath bladder!
  – Less than ½ of all patients are continent!
  – 23% of adults have bladder augmentation
NSBPR has improved my clinic

• We now ask about skin breakdown

• We address bowel issues earlier and more thoroughly
  – We added another NP to help with this

• We ask about things the same way and at every visit

• We track no shows more closely
NSBPR has improved all clinics

- All now check creatinine blood test to monitor kidney function
- Skin care & ulcers regularly addressed
- We are studying variations in care to see why some clinics do more or less surgery than others
- Why do patients at some clinics or in some groups have better outcomes?
Power in numbers

• 166,000 Americans living with SB
• Twice as many as Sickle Cell Disease
• Four times as many as Cystic Fibrosis
• We can make a difference
• We are already making a difference
  – Largest database in the world
  – First prospective urologic protocol
Power in money

• Funding for NSBPR and UMPIRE has not increased 2011-2024

• Costs – personnel & administrative - increase

• Patient numbers increase
  – 2011 – 0 patients enrolled in Chicago & Durham
  – 2020 – 1165 patients enrolled in Chicago & Durham

• CDC needs more money to continue current sites & fund more centers
You

$\xrightarrow{\text{research}}$

SB Research & Care