SPINA BIFIDA ASSOCIATION

SBA Legislative Priorities

2023 Teal on the Hill

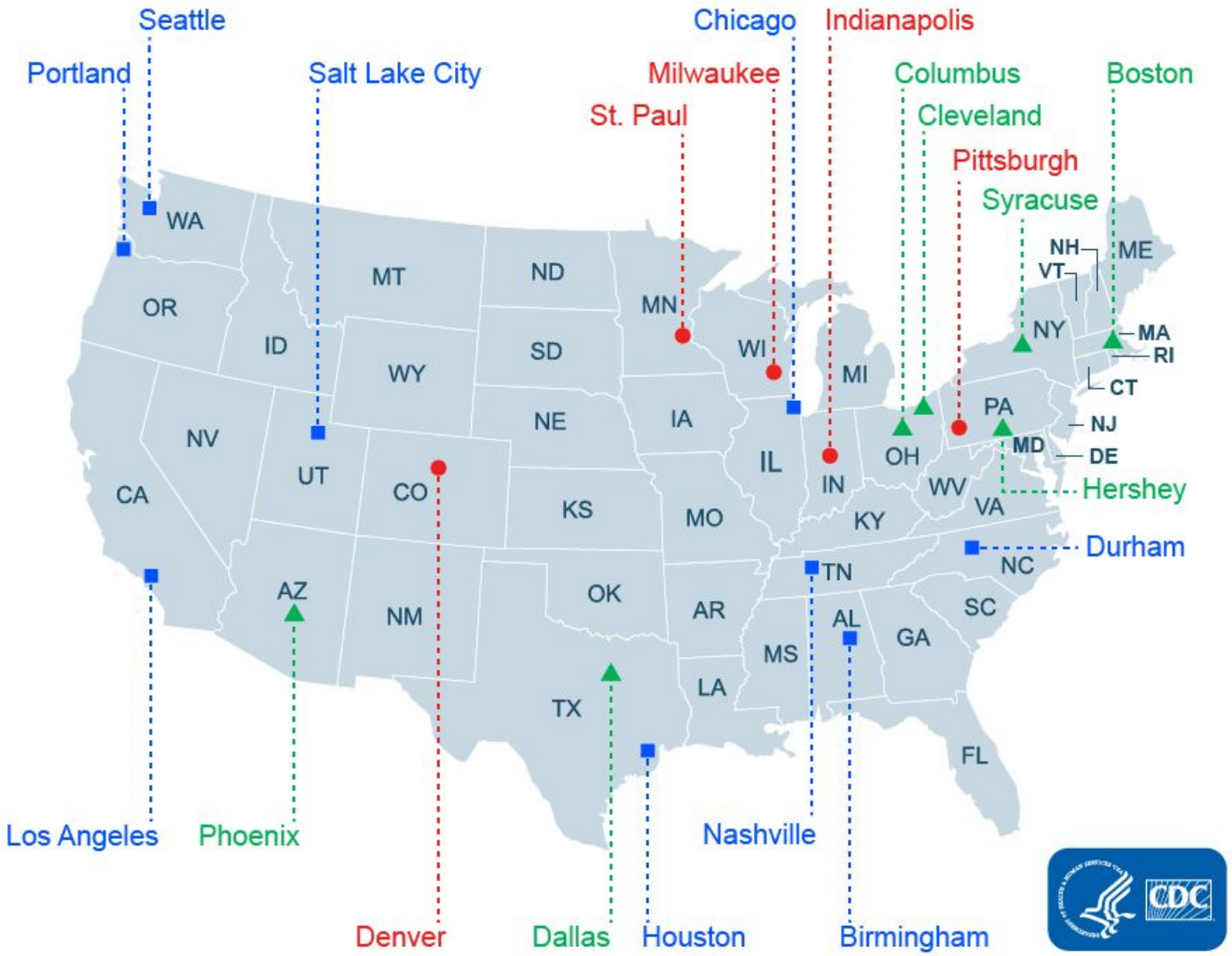
John Wiener, MD

February 26, 2023

To build a better and brighter future for all those impacted by Spina Bifida

Disclosures

- Grants from Centers for Disease Control and Prevention
- National Spina Bifida Patient Registry
- UMPIRE Study



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National Spina Bifida Patient Registry (NSBPR)

- History
  - Questionnaires
    - 2011 – 20 questions
    - 2017 – version 2.6. 45 questions + 3 supplemental for skin breakdown
  - 2014 – Newborn Protocol to Preserve Renal Function
    - Urologic Management of Newborns and Infants (UMPIRE)

National Spina Bifida Patient Registry (NSBPR)

- What has been published so far?
- Publication is important
  - Share what we are learning
  - Improve care for Americans with Spina Bifida
  - Increase level of science in Spina Bifida research
  - Prove to Federal Government that this is a worthwhile investment

National Spina Bifida Patient Registry (NSBPR)

Birth Defects Research (Part A) 97:36–41 (2013)

Testing the Feasibility of a National Spina Bifida Patient Registry

Judy K. Thibadeau,[1,*] Elisabeth A. Ward,[1] Minn M. Soe,[1] Tiebin Liu,[1] Mark Swanson,[1] Kathleen J. Sawin,[2] Kurt A. Freeman,[3] Heidi Castillo,[4] Karen Rauen,[5] and Michael S. Schechter[6]

[1]Centers for Disease Control and Prevention, National Center on Birth Defects and Developmental Disabilities, Atlanta, Georgia
[2]Children's Hospital of Wisconsin Research Chair in the Nursing of Children, College of Nursing, University of Wisconsin-Milwaukee, Milwaukee, Wisconsin
[3]Institute on Development and Disability, Oregon Health & Science University, Portland, Oregon
[4]Division of Developmental & Behavioral Pediatrics, Cincinnati Children's Hospital Medical Center, University of Cincinnati College of Medicine, Cincinnati, Ohio
[5]Children's Hospital of Wisconsin, Milwaukee, Wisconsin
[6]Department of Pediatrics, Emory University School of Medicine, Children's Hospital of Atlanta, Atlanta, Georgia

Received 3 May 2012; Revised 21 September 2012; Accepted 25 September 2012

Testing the feasibility of NSBPR

- Findings from 2009 – 2011
  - First 10 funded clinics
  - Enrolled 2070 patients
  - Described initial demographic info

Birth Defects Research (Part A) 97:36–41 (2013)

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ORIGINAL ARTICLES

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The National Spina Bifida Patient Registry: Profile of a Large Cohort of Participants from the First 10 Clinics

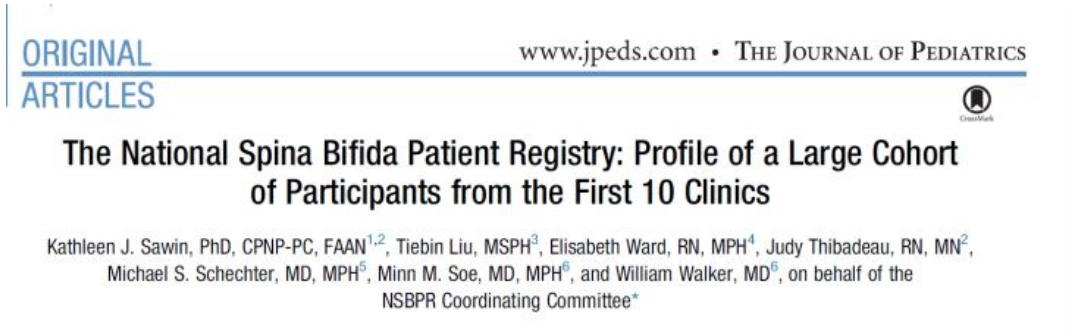
Kathleen J. Sawin, PhD, CPNP-PC, FAAN[1,2], Tiebin Liu, MSPH[3], Elisabeth Ward, RN, MPH[4], Judy Thibadeau, RN, MN[2], Michael S. Schechter, MD, MPH[5], Minn M. Soe, MD, MPH[6], and William Walker, MD[6], on behalf of the NSBPR Coordinating Committee*

Table I. Context of Care: demographic and clinical characteristics

Characteristics	Total, n (%), N = 2172	By SB type		P value*
		MMC (n = 1763)	Non-MMC (n = 409)	
Demographic characteristics				
Age group, y				
Younger than 2	373 (17.2)	300 (17.0)	73 (17.8)	
2 to <5	346 (15.9)	265 (15.0)	81 (19.8)	
5 to <10	454 (20.9)	364 (20.6)	90 (22.0)	
10 to <13	252 (11.6)	207 (11.7)	45 (11.0)	
13 to <18	419 (19.3)	342 (19.4)	77 (18.8)	
18 to <22	210 (9.7)	179 (10.2)	31 (7.6)	
22 or older	118 (5.4)	106 (6.0)	12 (2.9)	.0364
Sex				
Female	1141 (52.5)	909 (51.6)	232 (56.7)	.0618
Race/ethnicity				
Non-Hispanic white	1377 (63.4)	1137 (64.5)	240 (58.7)	
Non-Hispanic black	144 (6.6)	128 (7.3)	16 (3.9)	
Hispanic or Latino	526 (24.2)	426 (24.2)	100 (24.4)	
Other	125 (5.8)	72 (4.1)	53 (13.0)	<.0001
Insurance (N = 2171)				
Any private	1010 (46.5)	788 (44.7)	222 (54.3)	
Nonprivate	1161 (53.5)	974 (55.3)	187 (45.7)	.0005
Site				
1	411 (18.9)	339 (19.2)	72 (17.6)	
2	271 (12.5)	233 (13.2)	38 (9.3)	
3	242 (11.1)	218 (12.4)	24 (5.9)	
4	247 (11.4)	201 (11.4)	46 (11.2)	
5	231 (10.6)	190 (10.8)	41 (10.0)	
6	255 (11.7)	175 (9.9)	80 (19.6)	
7	172 (7.9)	153 (8.7)	19 (4.6)	
8	196 (9.0)	132 (7.5)	64 (15.6)	
9	75 (3.5)	63 (3.6)	12 (2.9)	
10	72 (3.3)	59 (3.3)	13 (3.2)	<.0001
Education level				
Pre-elementary	857 (39.5)	676 (38.3)	181 (44.3)	
Primary/secondary	1205 (55.5)	993 (56.3)	212 (51.8)	
Technical school	8 (0.4)	7 (0.4)	1 (0.2)	
Some college	43 (2.0)	36 (2.0)	7 (1.7)	
College degree	7 (0.3)	6 (0.3)	1 (0.2)	
Advanced degree	4 (0.2)	2 (0.1)	2 (0.5)	
Other	48 (2.2)	43 (2.4)	5 (1.2)	.1523
Clinical characteristics				
Mobility status age 2 and older (n = 1782)				
Community ambulators	961 (53.9)	651 (45.0)	310 (92.3)	
Household ambulators	147 (8.2)	140 (9.7)	7 (2.1)	
Nonfunctional ambulators	145 (8.1)	137 (9.5)	8 (2.4)	
Nonambulators	529 (29.7)	518 (35.8)	11 (3.3)	<.0001
Functional LOL†				
Thoracic (flaccid lower extremities)	330 (15.2)	325 (18.4)	5 (1.2)	
High-lumbar (hip-flexion present)	211 (9.7)	204 (11.6)	7 (1.7)	
Mid-lumbar (knee extension present)	581 (26.7)	543 (30.8)	38 (9.3)	
Low-lumbar (foot dorsiflexion present)	393 (18.1)	343 (19.5)	50 (12.2)	
Sacral (foot plantar flexion present)	657 (30.2)	348 (19.7)	309 (75.6)	<.0001
Bowel function				
Impaired bowel function, total sample‡	1891 (87.1)	1618 (91.8)	273 (66.7)	<.0001
Impaired bowel function, ages 5 and older§	1200 (82.6)	1059 (88.4)	141 (55.3)	<.0001
Bladder function				
Impaired bladder function, total Sample**	1989 (91.6)	1693 (96.0)	296 (72.4)	<.0001
Impaired bladder function, ages 5 and older§	1299 (89.4)	1133 (94.6)	166 (65.1)	<.0001

Findings March 2009 to 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



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Sociodemographic Attributes and Spina Bifida Outcomes

- 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


Sociodemographic Attributes and Spina Bifida Outcomes

Michael S. Schechter, MD, MPH[a,b], Tiebin Liu, MSPH[b], Minn Soe, MD, MPH[b], Mark Swanson, MD, MPH[b], Elisabeth Ward, RN, MPH[b,c], Judy Thibadeau, RN, MN[b]

ACRM
AMERICAN CONGRESS OF REHABILITATION MEDICINE

Archives of Physical Medicine and Rehabilitation

journal homepage: www.archives-pmr.org

Archives of Physical Medicine and Rehabilitation 2015;96:1435-41

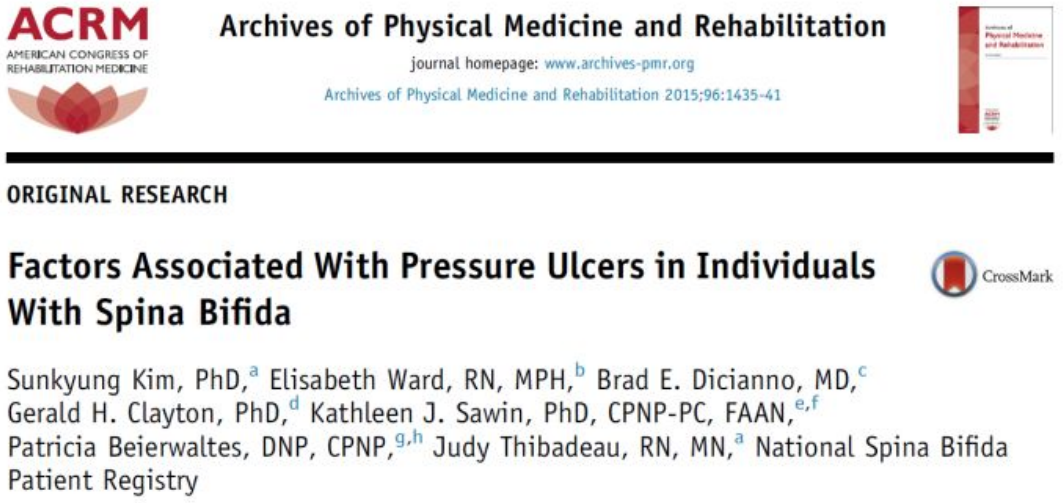
ORIGINAL RESEARCH

Factors Associated With Pressure Ulcers in Individuals With Spina Bifida

Sunkyung Kim, PhD,[a] Elisabeth Ward, RN, MPH,[b] Brad E. Dicianno, MD,[c] Gerald H. Clayton, PhD,[d] Kathleen J. Sawin, PhD, CPNP-PC, FAAN,[e,f] Patricia Beierwaltes, DNP, CPNP,[g,h] Judy Thibadeau, RN, MN,[a] National Spina Bifida Patient Registry

Data from 3,153 patients 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



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CME ARTICLE • 2015 SERIES • NUMBER 12

Factors Associated with Mobility Outcomes in a National Spina Bifida Patient Registry

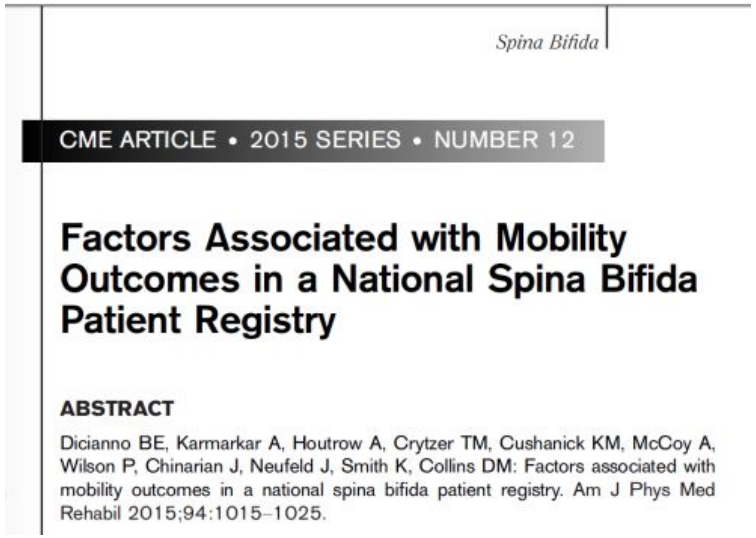
ABSTRACT

Dicianno BE, Karmarkar A, Houtrow A, Crytzer TM, Cushanick KM, McCoy A, Wilson P, Chinarian J, Neufeld J, Smith K, Collins DM: Factors associated with mobility outcomes in a national spina bifida patient registry. Am J Phys Med Rehabil 2015;94:1015–1025.

Factors Associated with Mobility Outcomes in a National Spina Bifida Patient Registry

Data from 2,604 patients ages 5+ at 19 clinics

- Community ambulation was associated with:
  - No shunt
  - Lower motor function level
  - No history of hip or knee contracture release surgery



Spina Bifida

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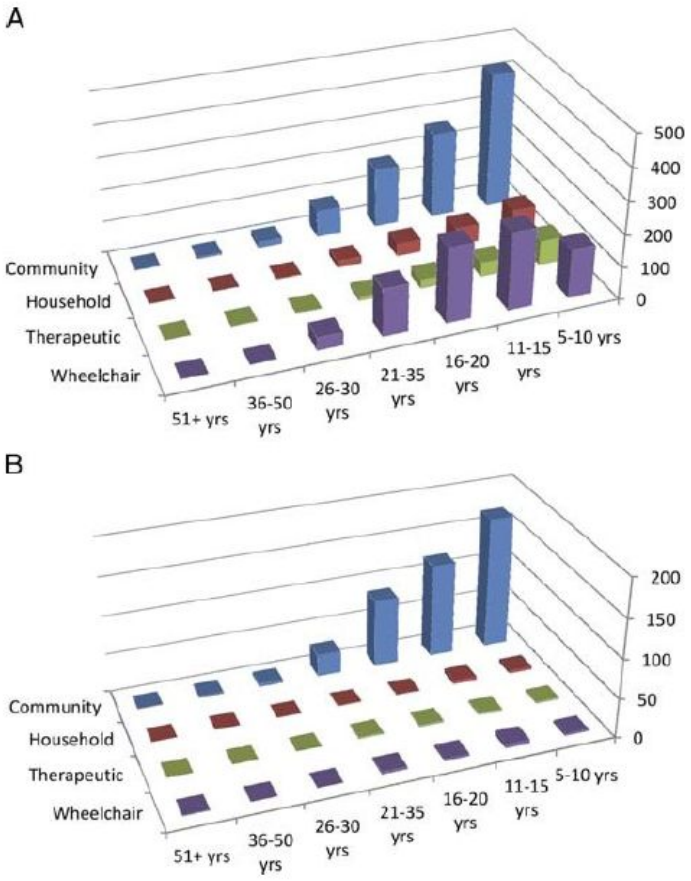

MMC

Non-MMC

Bowel Management in Adults with Spina Bifida

- 5,209 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
- Of all adults:
  - 14.0% worked full time
  - 18.1% worked part time
  - 20.9% were students
  - 27.9% identified as permanently disabled

Bowel Management in Adults with Spina Bifida

- Of 708 adults aged 25 years & older:
  - 19.5% had college degree
  - 27.0% had attended school after HS
- Of adults 25 years & older:
  - Bowel continence was not associated with
    - Gender
    - Health insurance status
    - Spina Bifida type or lesion level
    - Educational attainment
  - Bowel continence was associated with employment

Bladder Management and Continence Outcomes in Adults with Spina Bifida: Results from the National Spina Bifida Patient Registry, 2009 to 2015

John S. Wiener,* Kristina D. Suson, Jonathan Castillo, Jonathan C. Routh, Stacy T. Tanaka, Tiebin Liu, Elisabeth A. Ward, Judy K. Thibadeau, David B. Joseph and the National Spina Bifida Patient Registry

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 (STT), Nashville, Tennessee, Centers for Disease Control and Prevention (TL, EAW, JKT), and Carter Consulting, Inc. (EAW), Atlanta Georgia, and Department of Urology, University of Alabama at Birmingham (DBJ), Birmingham, Alabama

Bladder Reconstruction Rates Differ among Centers Participating in NSBPR

Data from 5528 individuals

- 20% had undergone bladder reconstruction
- Surgery rates varied 12-38% among clinics

Bladder Reconstruction Rates Differ among Centers Participating in National Spina Bifida Patient Registry

Jonathan C. Routh,*,† David B. Joseph, Tiebin Liu, Michael S. Schechter, Judy K. Thibadeau, M. Chad Wallis, Elisabeth A. Ward and John S. Wiener

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)

Variation in surgical management of neurogenic bowel among centers participating in National Spina Bifida Patient Registry

Jonathan C. Routh[a,*], David B. Joseph[b], Tiebin Liu[c], Michael S. Schechter[d], Judy K. Thibadeau[c], M. Chad Wallis[e], Elisabeth A. Ward[c,f], and John S. Wiener[a]

[a]Division of Urology, Duke University Medical Center, Durham, NC, USA

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[d]Division of Pediatric Pulmonary Medicine, Children's Hospital of Richmond at Virginia Commonwealth University, Richmond, VA, USA

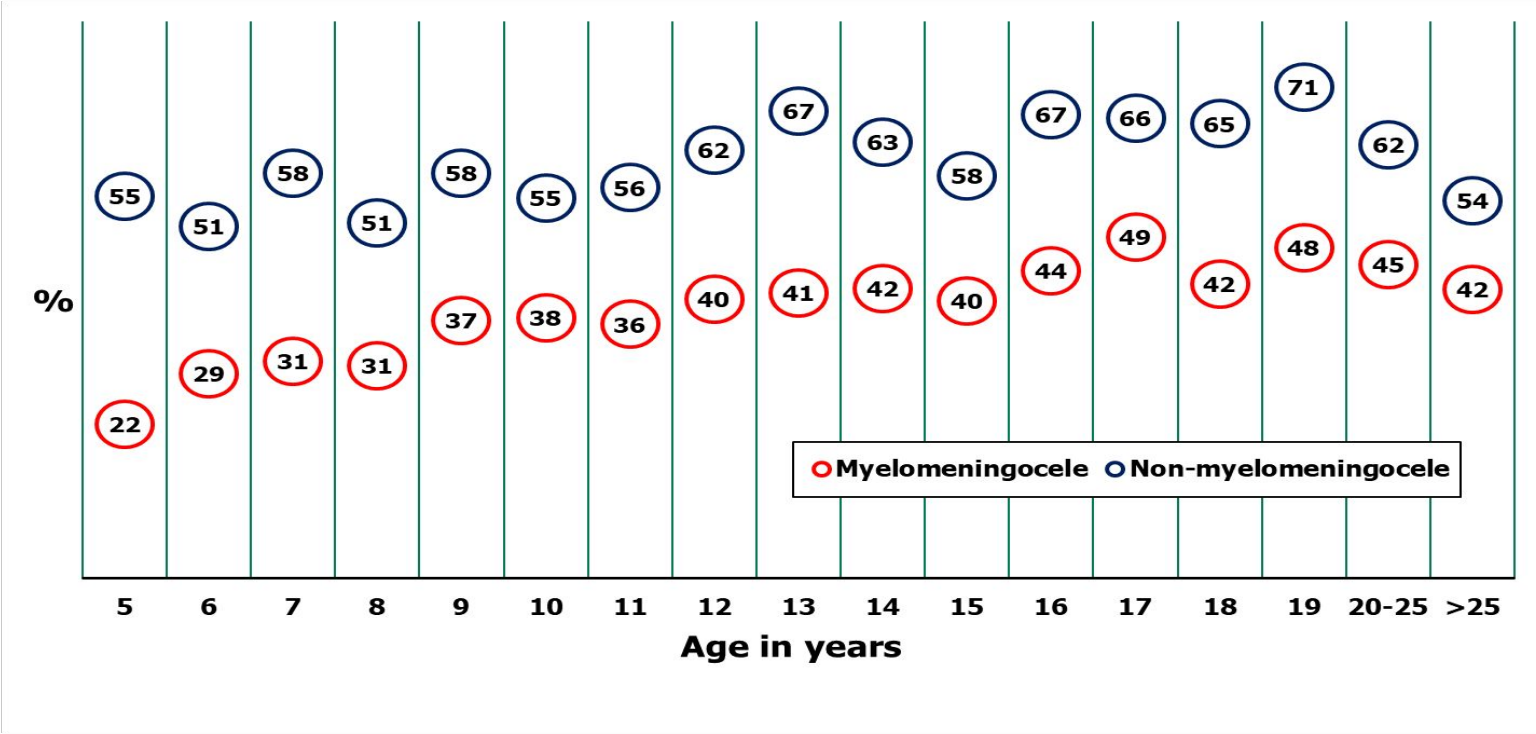
[e]Division of Urology, Primary Children's Hospital, Salt Lake City, UT, USA

[f]Carter Consulting, Inc., Atlanta, GA, USA

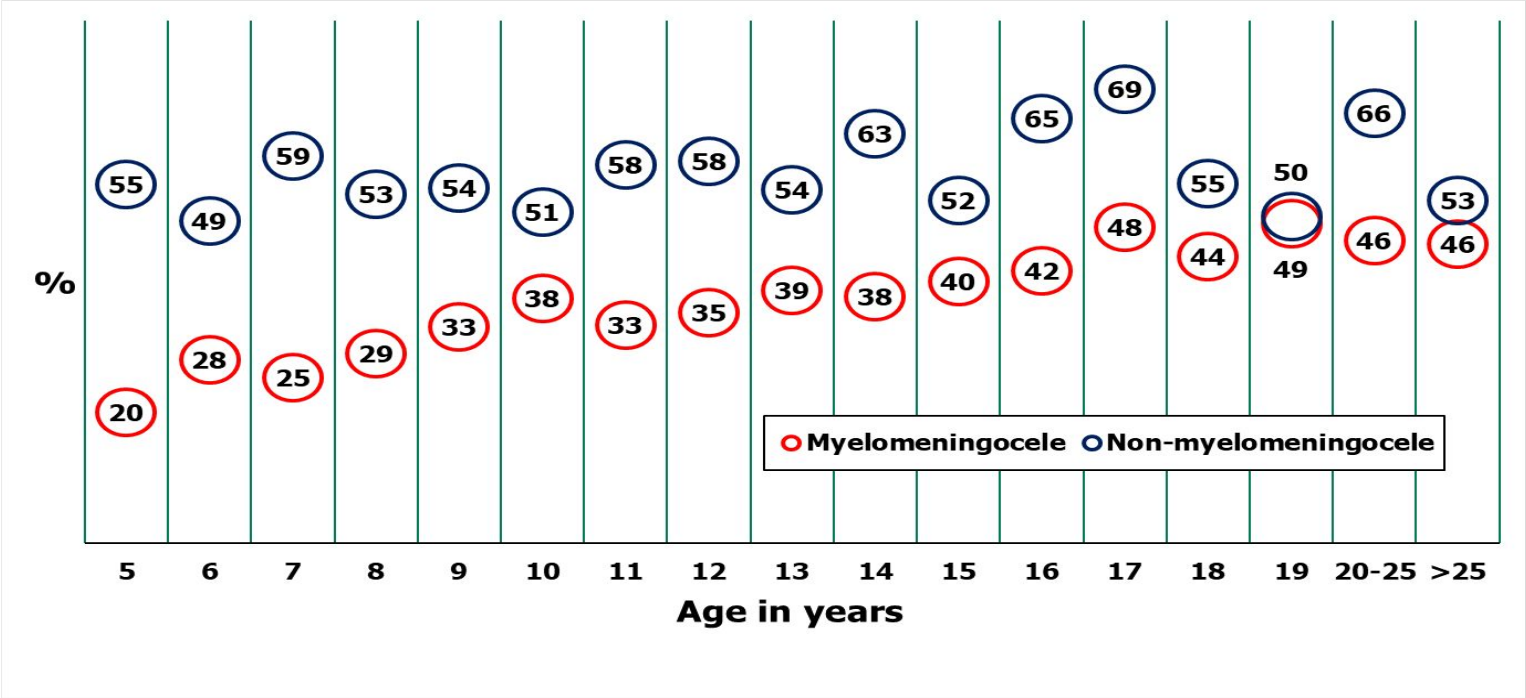
Surgeries and Health Outcomes Among Patients With Spina Bifida

Noreen B. Alabi, MPH,[a] Judy Thibadeau, RN,[a] John S. Wiener, MD,[b] Mike J. Conklin, MD,[c]
Mark S. Dias, MD,[d] Kathleen J. Sawin, PhD,[e] Rodolfo Valdez, PhD[a]

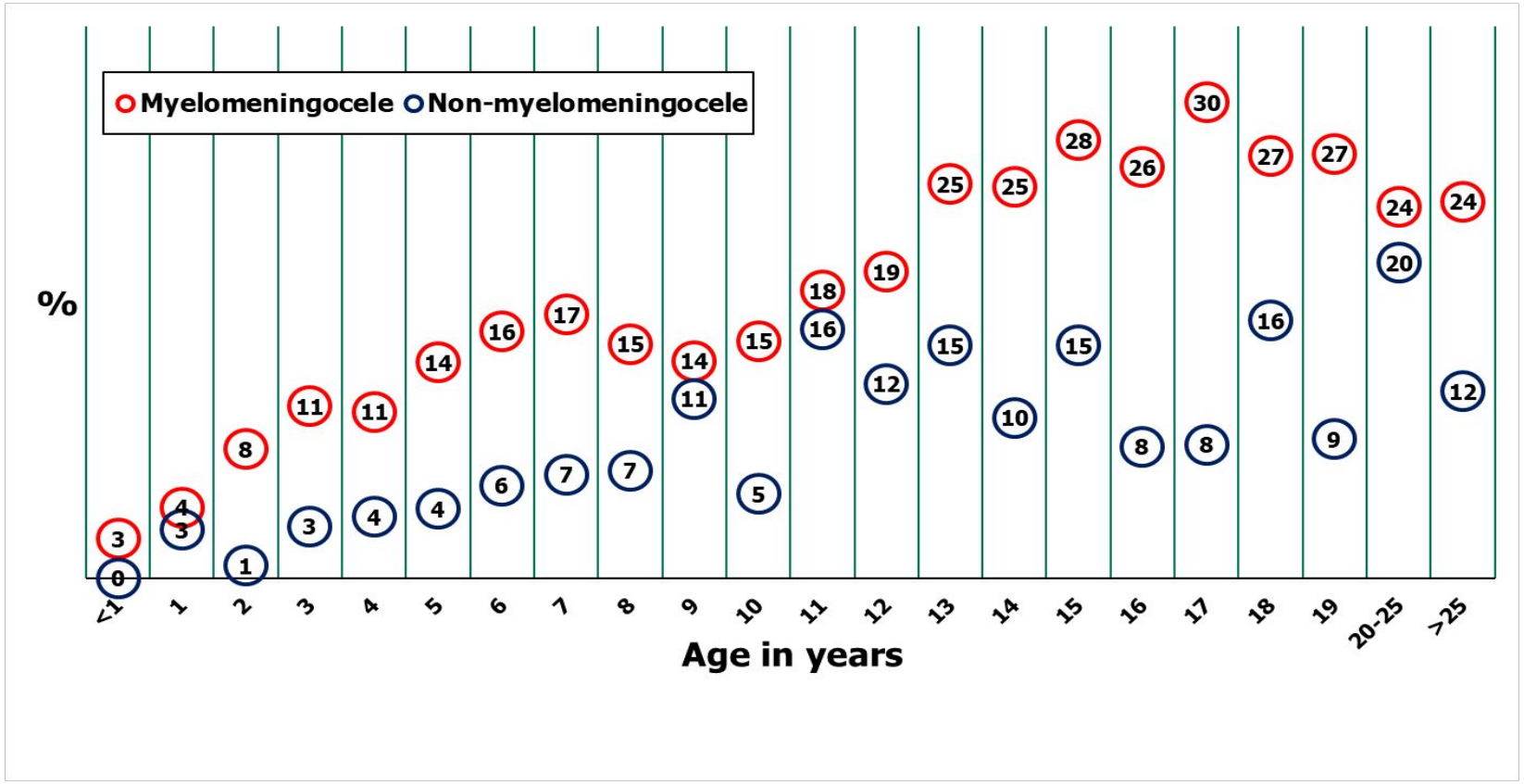
Prevalence of stool continence, by age and diagnosis, in Spina Bifida patients. NSBPR, 2009–2013



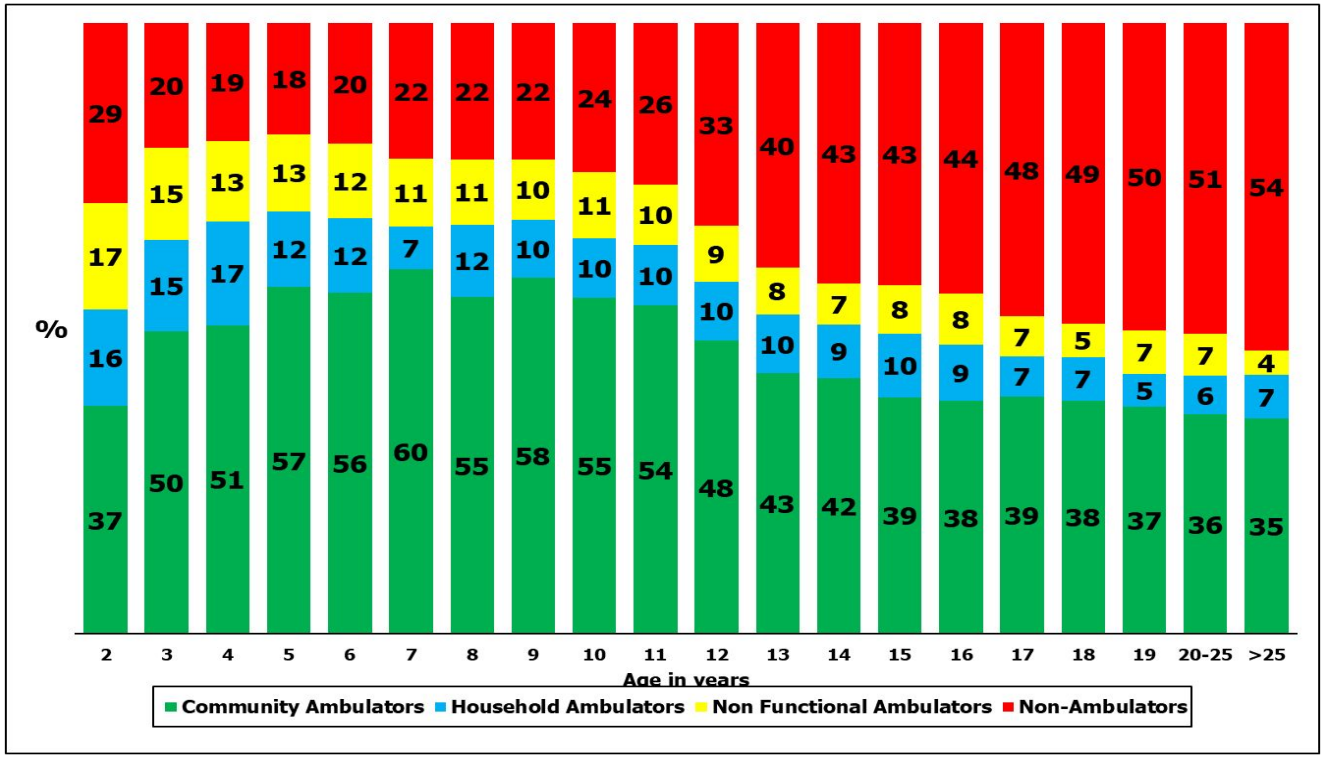
Prevalence of urine continence, by age and diagnosis, in Spina Bifida patients. NSBPR, 2009–2013



Prevalence of pressure sores, by age and diagnosis, in Spina Bifida patients. NSBPR, 2009–2013



Percent distribution of ambulation status among patients with myelomeningocele, by age. NSBPR, 2009–2013



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Pediatric Urology

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

Jonathan C. Routh,* Earl Y. Cheng, J. Christopher Austin, Michelle A. Baum, Patricio C. Gargollo, Richard W. Grady, Adrienne R. Herron, Steven S. Kim, Shelly J. King, Chester J. Koh, Pangaja Paramsothy, Lisa Raman, Michael S. Schechter, Kathryn A. Smith, Stacy T. Tanaka, Judy K. Thibadeau, William O. Walker, M. Chad Wallis, John S. Wiener and David B. Joseph

J Urol. 2019 Feb 5:101097JU0000000000000141. doi: 10.1097/JU.0000000000000141. [Epub ahead of print]

Baseline Urinary Tract Imaging in Infants Enrolled in the UMPIRE Protocol for Children with Spina Bifida.

Tanaka ST[1], Paramsothy P[2], Thibadeau J[2], Wiener JS[3], Joseph DB[4], Cheng EY[5], Tu D[6], Austin C[7], Koh CJ[6], Wallis MC[8], Walker WO[9], Smith KA[10], Routh JC[3], Baum MA[10].

This is what medical progress looks like

- Largest database for Spina Bifida in the world & 1st prospective urologic protocol
- Big data creates statistical power to show differences and effects
- These 12 scientific publications prove:
  - The registry and protocol both work
  - Both can teach us new information
  - Both can impact care
    - Continuation is crucial to help to define standards of care for Spina Bifida

This is what medical progress looks like

- These 12 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 Spina Bifida
  - Your tax money is encouraging more talent to devote their careers to improving the lives of Americans with Spina Bifida
  - More doctors are talking about Spina Bifida at scientific meetings

Momentum is building!

So what?

How are the NSBPR and UMPIRE studies translating into better care for Americans with Spina Bifida?

NSBPR makes me better

- I spend more time asking about:
  - Education
  - Employment
- My urodynamic studies have been modified to meet higher standards
- I have more powerful data to share with parents of newborns and young children
  - 76% of adolescent & adults cath bladder!
  - Less than ½ of all patients are continent!
  - 23% of adults have bladder aug

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

Power in numbers

- 166,000 Americans living with Spina Bifida
- 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 at Duke
  - 2019 – 462 patients enrolled at Duke
- CDC needs more money to continue current sites and fund more centers

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You →

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$ → SB Research & Care

SPINA BIFIDA ASSOCIATION

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