



SPINA BIFIDA
ASSOCIATION

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

Teal on the Hill

April 27, 2020

Disclosures

Both Centers (JW & RB) participate in NSBPR and UMPIRE studies and receive grants from Centers for Disease Control and Prevention

National Spina Bifida Patient Registry Origin

- 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

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

Testing the Feasibility of a National Spina Bifida Patient Registry

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

- 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^{1,2}, Tiebin Liu, MSPH³, Elisabeth Ward, RN, MPH⁴, Judy Thibadeau, RN, MN², Michael S. Schechter, MD, MPH⁵, Minn M. Soe, MD, MPH⁶, and William Walker, MD⁶, on behalf of the NSBPR Coordinating Committee*

February 2015



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, MSPH³, Elisabeth Ward, RN, MPH⁴, Judy Thibadeau, RN, MN², Michael S. Schechter, MD, MPH⁵, Minn M. Soe, MD, MPH⁶, and William Walker, MD⁶, 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 \geq 5 y had bowel & bladder impairment

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

April 2015

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

- **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**

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

August 2015

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 Beierwattes, DNP, CPNP,^{g,h} Judy Thibadeau, RN, MN,^g 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

Dicianno BE, Karmarkar A, Houtrow A, Crytzer TM, Cushman 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.

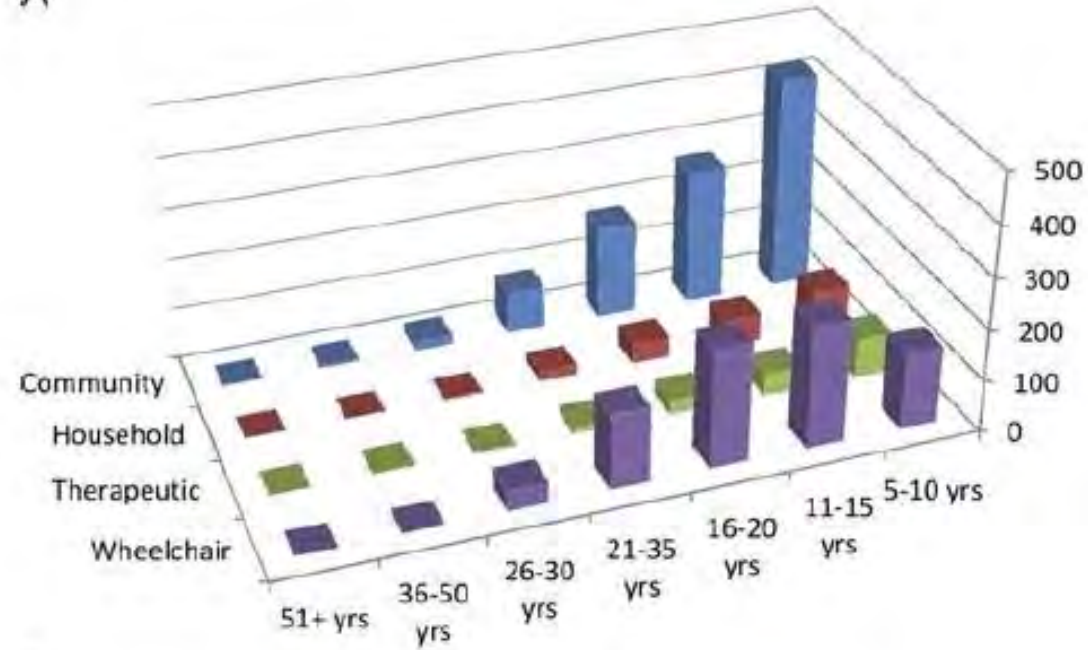
- **Data from 2604 pts aged 5+ at 19 clinics**
- **Community ambulation was associated with:**
 - No shunt
 - Lower level of motor function
 - No history of hip or knee contracture release surgery

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

ABSTRACT

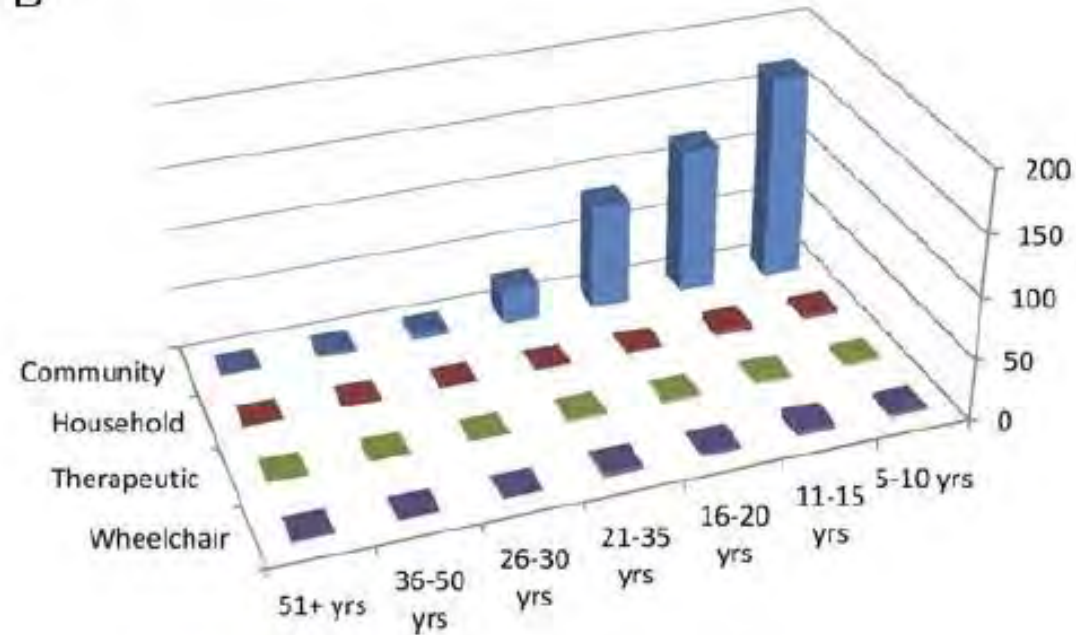
Dicianno BE, Karmarkar A, Houtrow A, Crytzer TM, Cushman KM, McCoy A, Wilson P, Chinarian J, Neufeld J, Smith K, Collins DM: Factors associated with mobility outcomes in a national spina bifida patient registry. *Am J Phys Med Rehabil* 2015;94:1015-1025.

A



MMC

B



Non-MMC

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

ABSTRACT
 Dicianno BE, Karmakar A, Houtrow A, Cryzler TM, Cushmanick 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.

Decompression for Chiari malformation type II in individuals with myelomeningocele in the National Spina Bifida Patient Registry

Irene Kim, MD,¹ Betsy Hopson, MHSA,² Inmaculada Aban, PhD,³ Elias B. Rizk, MD,⁴ Mark S. Dias, MD,⁴ Robin Bowman, MD,⁵ Laurie L. Ackerman, MD,⁶ Michael D. Partington, MD, MS,⁷ Heidi Castillo, MD,⁸ Jonathan Castillo, MD, MPH,⁸ Paula R. Peterson, MS,⁹ Jeffrey P. Blount, MD,² and Brandon G. Rocque, MD, MS²

- 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

Treated hydrocephalus in individuals with myelomeningocele in the National Spina Bifida Patient Registry

Irene Kim, MD,¹ Betsy Hopson, MHSA,² Inmaculada Aban, PhD,³ Elias B. Rizk, MD,⁴ Mark S. Dias, MD,⁴ Robin Bowman, MD,⁵ Laurie L. Ackerman, MD,⁶ Michael D. Partington, MD, MS,⁷ Heidi Castillo, MD,⁸ Jonathan Castillo, MD, MPH,⁹ Paula R. Peterson, MS,⁹ Jeffrey P. Blount, MD,² and Brandon G. Rocque, MD, MS²

- 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^{a,*}, Kristina D. Suson^b, Jonathan Castillo^c, Jonathan C. Routh^a, Stacy Tanaka^d, Tiebin Liu^c, Elisabeth Ward^{e,f}, Judy Thibadeau^c, David Joseph^g and National Spina Bifida Patient Registry

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^f*Carter Consulting, Inc., Atlanta, GA, USA*

^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

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)

January 2018

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

March 2018

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

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- **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^{a,*}, David B. Joseph^b, Tiebin Liu^c, Michael S. Schechter^d, Judy K. Thibadeau^e, M. Chad Wallis^e, Elisabeth A. Ward^{c,f}, and John S. Wiener^a

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^fCarter Consulting, Inc., Atlanta, GA, USA

March 2018

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)

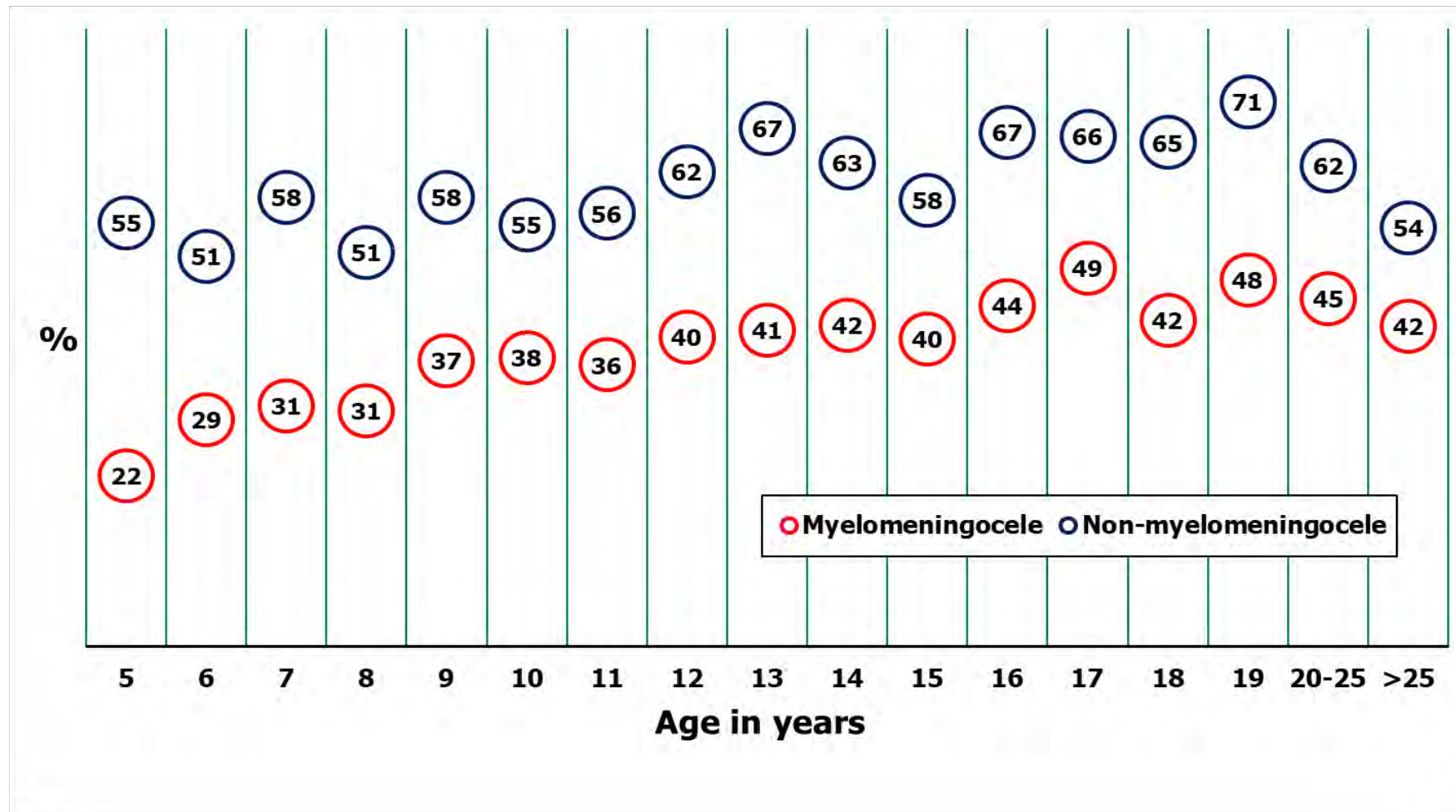
March 2018

Surgeries and Health Outcomes Among Patients With Spina Bifida

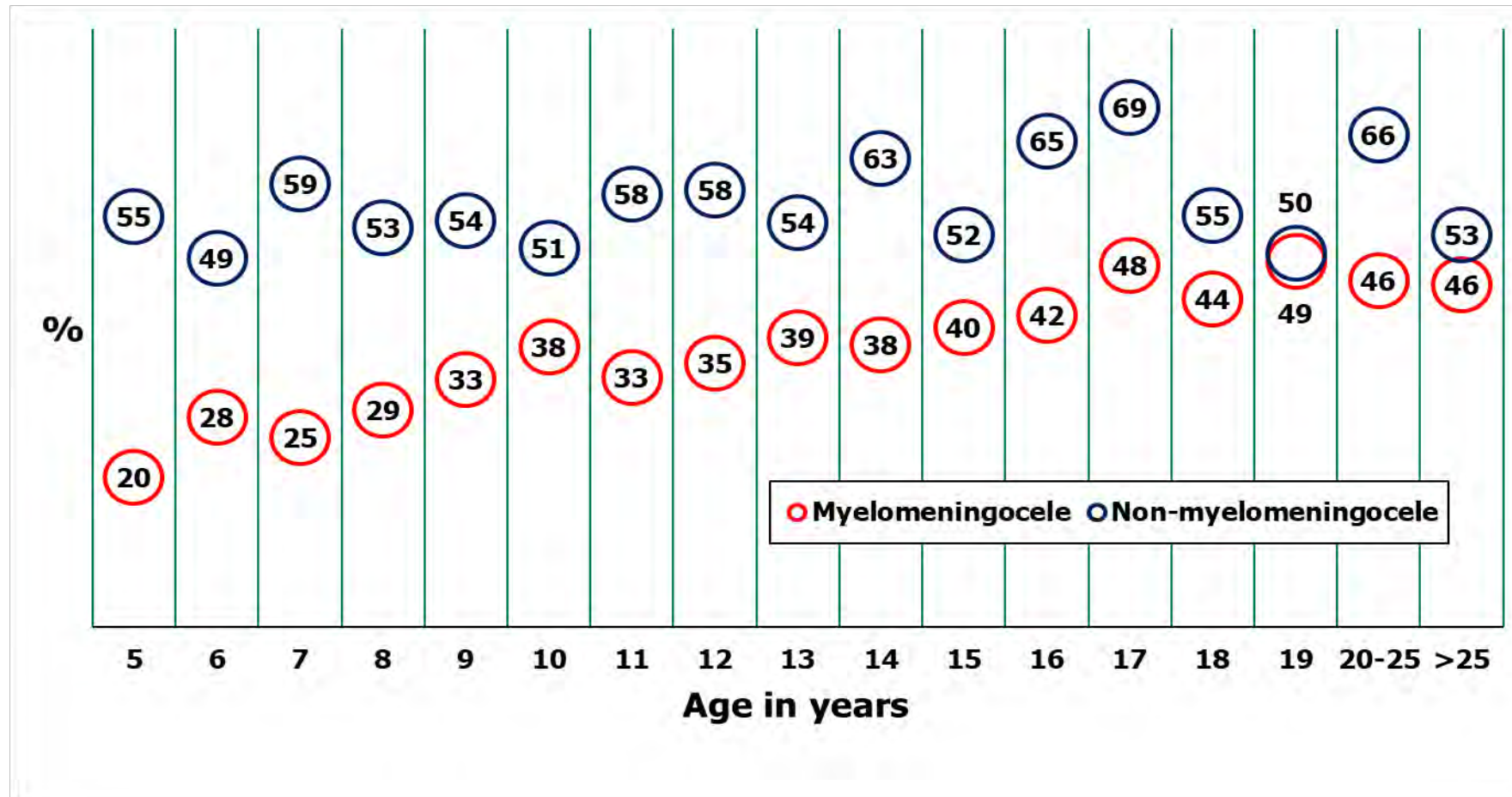
Noreen B. Alabi, MPH,^a Judy Thibadeau, RN,^a John S. Wiener, MD,^b Mike J. Conklin, MD,^c
Mark S. Dias, MD,^d Kathleen J. Sawin, PhD,^e Rodolfo Valdez, PhD^a

September 2018

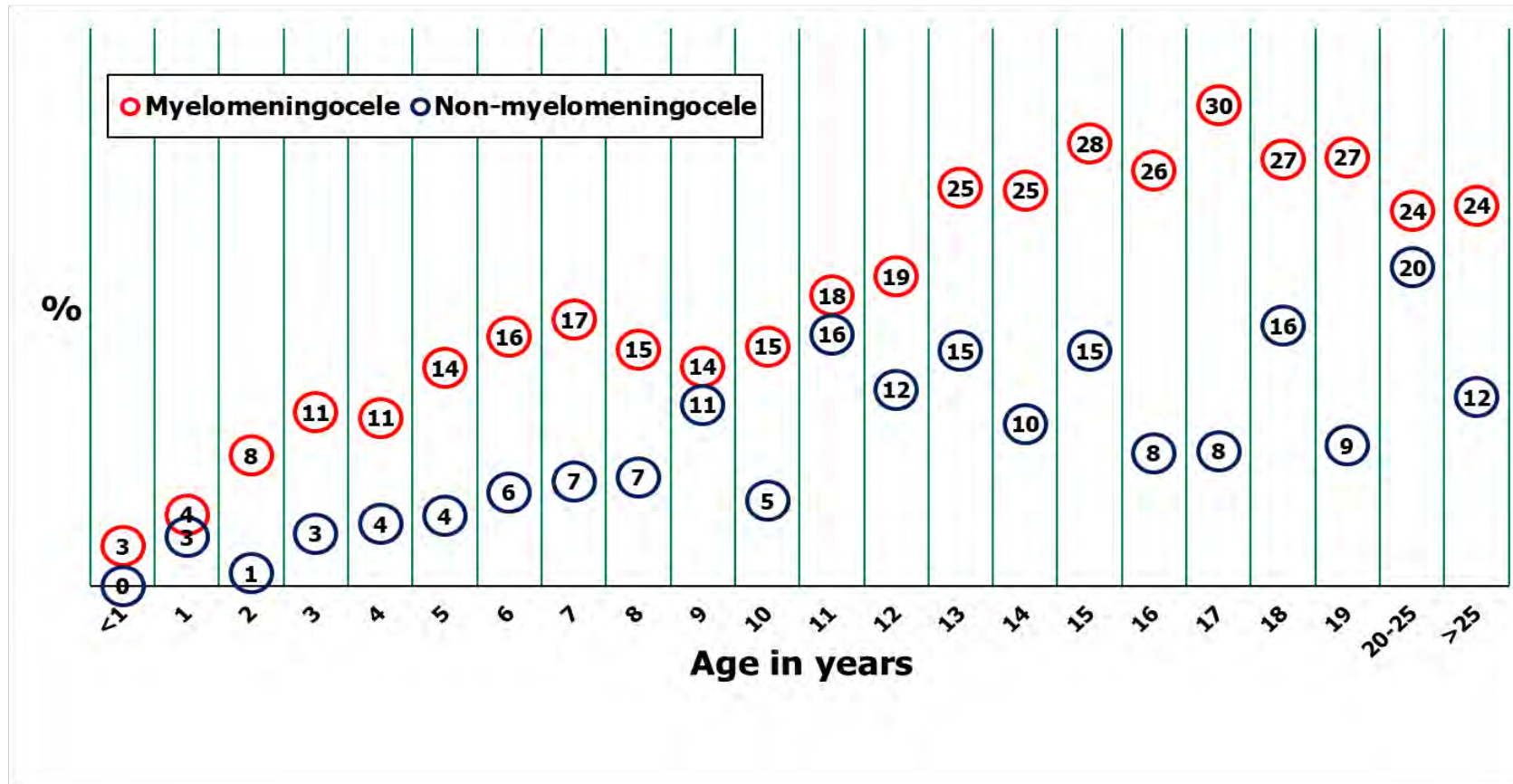
Prevalence of stool continence, by age and diagnosis, in spina bifida patients. The National Spina Bifida Registry, 2009–2013



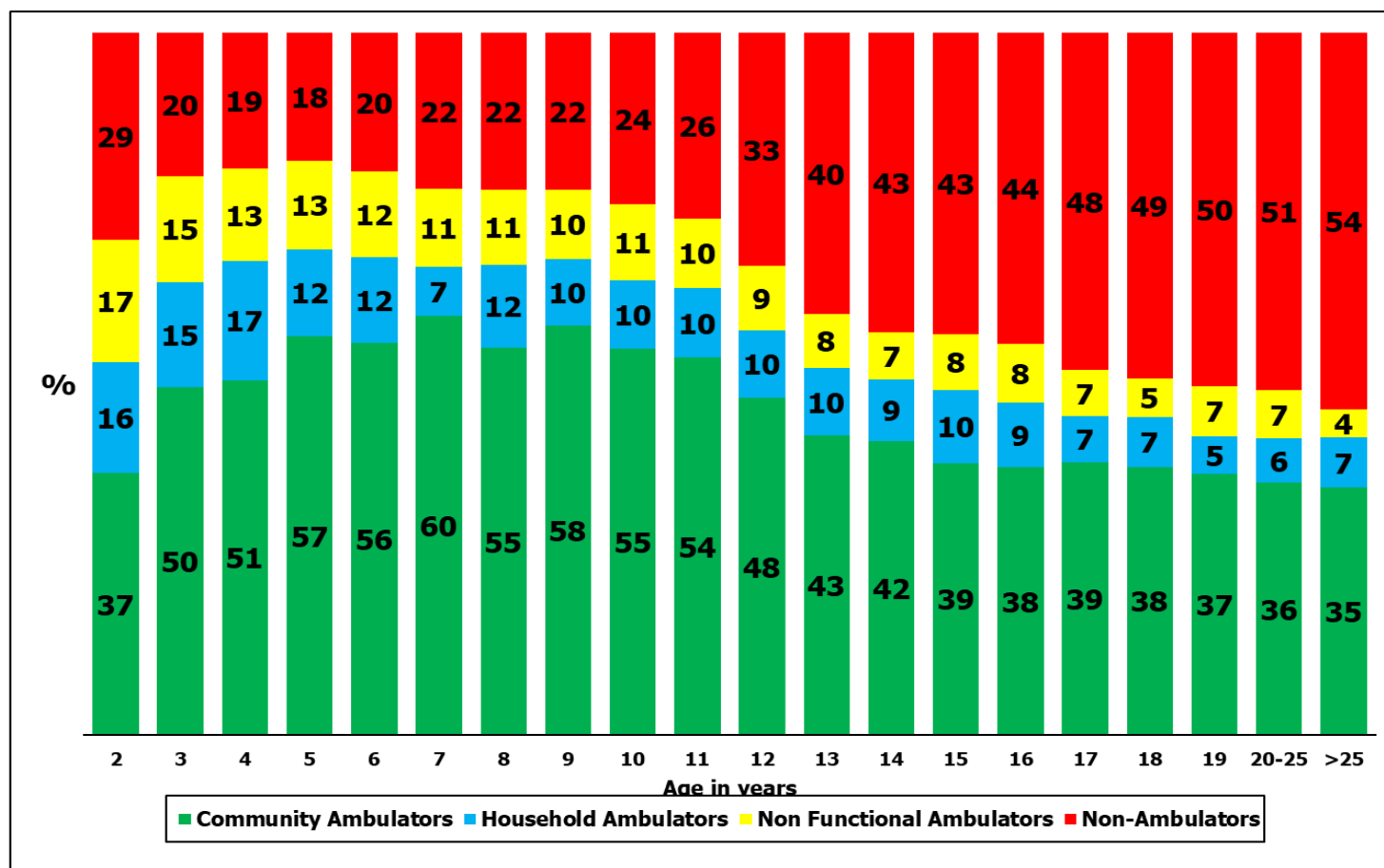
Prevalence of urine continence, by age and diagnosis, in spina bifida patients. The National Spina Bifida Registry, 2009–2013



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



› J Pediatr Rehabil Med. 2019;12(4):361-368. doi: 10.3233/PRM-180556.

Differences in Continence Rates in Individuals With Spina Bifida Based on Ethnicity

Kathryn A Smith ^{1 2}, Tiebin Liu ³, Kurt A Freeman ⁴, Cecily Betz ^{1 2}, Gerald H Clayton ^{5 6}, Heidi Castillo ^{7 8}, Jonathan Castillo ^{7 8}, Duong Tu ^{9 8}, Alexander Van Speybroeck ^{1 2}, William O Walker ^{10 11}

- **Hispanics were more likely to have bowel incontinence**

December 2019

J Urol. 2020 Mar 6;101097JU0000000000001010. doi: 10.1097/JU.0000000000001010. [Epub ahead of print]

Kidney Function Surveillance in the National Spina Bifida Patient Registry: A Retrospective Cohort Study.

Chu DI^{1,2}, Liu T³, Patel P⁴, Routh JC⁵, Ouyang L³, Baum MA⁶, Cheng EY¹, Yerkes EB¹, Isakova T^{7,8}.

- **Only 62% of patients get “proper” surveillance of kidney function yearly**

UMPIRE PROTOCOL

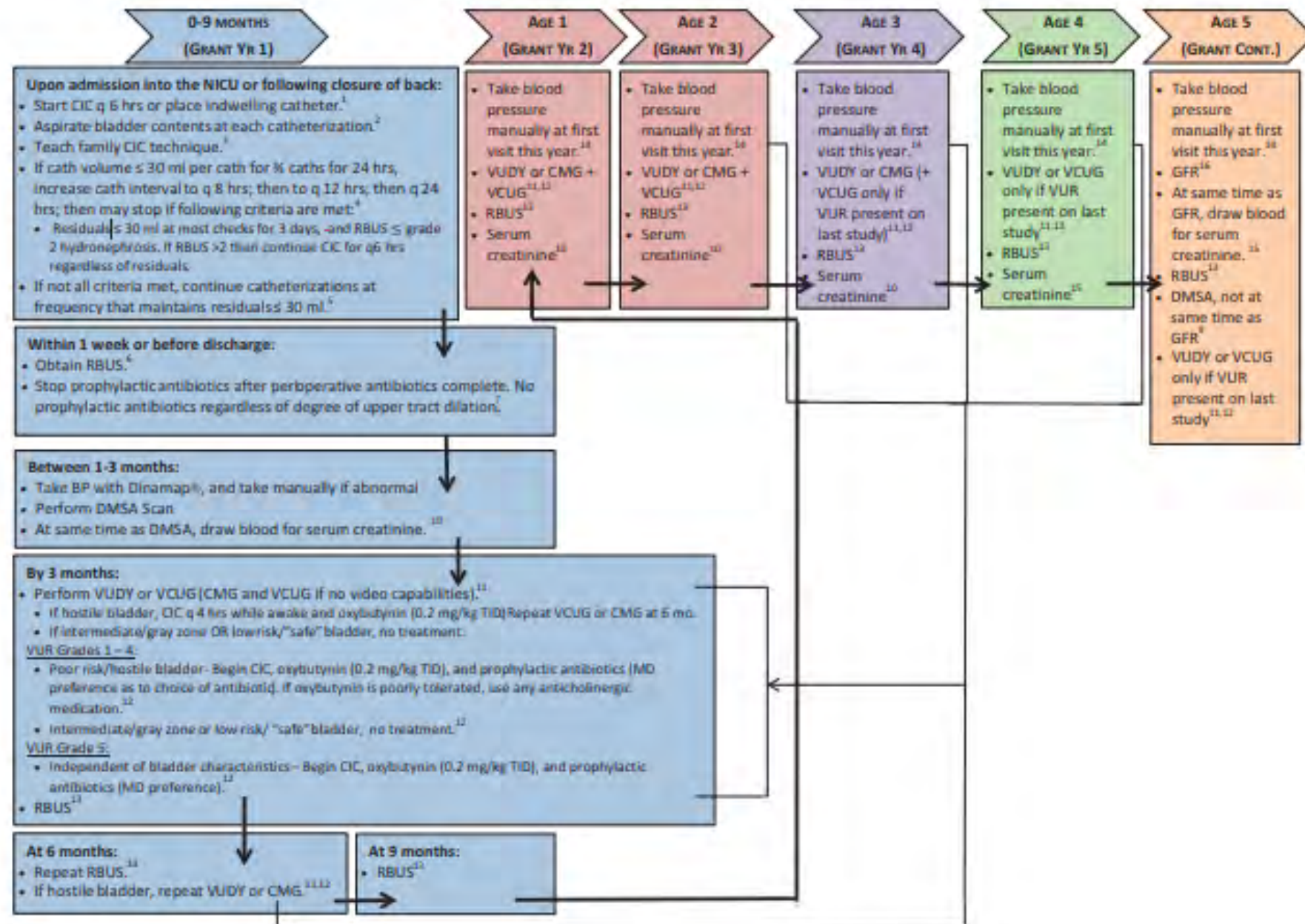
- **U**ROLOGICAL
- **M**ANAGEMENT FOR
- **P**RESERVATION OF
- **R**ENAL FUNCTION

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

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

391 participants

December 2016



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



Stacy T. Tanaka, Pangaja Paramsothy, Judy Thibadeau, John S. Wiener, David B. Joseph, Earl Y. Cheng, Duong Tu, Christopher Austin, Chester J. Koh, M. Chad Wallis, William O. Walker, Kathryn A. Smith, Jonathan C. Routh and Michelle A. Baum*

- **At birth, only 4% have concerning US, 16% have reflux, & 8% have abnormal renal scan**

June 2019

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 1/2 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



\$



\$



**SB
Research
& Care**