

# Urology Interagency Coordinating Committee (UICC)

Friday, June 26, 2015  
9:00 a.m. - 12:00 p.m.  
6707 Democracy Blvd. Room 701  
Bethesda, MD  
Meeting Minutes

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## Welcome and Introductions

*Rob Star, M.D.*

*National Institute of Diabetes and Digestive and Kidney Diseases*

Dr. Star welcomed participants to the urology interagency coordinating committee and welcomed participants to comment on any ongoing programs.

## Update on Congenitalism and Transitional Urology Perspectives from the Front-Line of Establishing a Transition Clinic

*Michael Hsieh, M.D., Ph.D.*

*Director, Clinic for Adolescent and Adult Pediatric Onset Urology (CAPITUL),  
Children's National Health System*

Dr. Hsieh began his presentation by discussing survival rates for patients with spina bifida. There is a transitional gap in care from pediatric to adulthood. Aging patients with unique biology require specialized care such as surveillance for bladder cancer. Challenges of transitional care include insurance coverage, sexual development, and psychosocial issues. In a society with only adult and pediatric urologists who are interested in treating their own patient subgroups, what should we be doing to “transition” patients during adolescence? Dr. Hsieh presented the following timeline:

### Recommended Health Care Transition Timeline

AGE:	12	14	16	18	18-22	23-26
	Make youth and family aware of transition policy	Initiate health care transition planning	Prepare youth and parents for adult model of care and discuss transfer	Transition to adult model of care	Transfer care to adult medical home and/or specialists with transfer package	Integrate young adults into adult care

Dr. Hsieh noted that Children’s National Hospital is focused on pediatrics, he sees patients up to age 22. The Clinic for Adolescent and Adult Pediatric Onset Urology (CAPITUL) developed by a joint venture of Children’s and the George Washington University to address transition issue. CAPITUL is the first dedicated transitional urology program in the mid-Atlantic region and will also see adults with pediatric onset urologic disorders. This clinic interfaces closely with other regional medical centers and practices.

In 2014, a landmark paper which detailed the fertility preservation perspectives of male pediatric cancers and their parents was published: Stein DM, Victorson DE, Choy JT, Waimey KE, Pearman TP, Smith K, Dreyfuss J, Kinahan KE, Sadhwani D, Woodruff TK, Brannigan RE. Fertility Preservation Preferences and Perspectives Among Adult Male Survivors of Pediatric Cancer and Their Parents. *J Adolesc Young Adult Oncol*. 2014 Jun 1;3(2):75-82. The themes most frequently expressed by survivors included concern regarding long-term treatment effects and a retrospective desire for fertility impairment to have been discussed when they were originally diagnosed with cancer. Parental themes included the same hindsight desire, as well as reliance upon the treating oncologist for direction in selecting the course of treatment, and an acknowledgment that input from a specialist in fertility preservation would have been beneficial. In conclusion, although future reproductive potential was not consistently reported as a source of apprehension when diagnosed with cancer, both survivors and their parents noted it to be a paramount concern later in life. Parents and survivors both reported that fertility preservation discussions should be routinely incorporated in the clinical context of a pediatric cancer diagnosis.

To address fertility preservation concerns and further research in this area, the testicular tissue cryopreservation (TTC) study was conducted to follow males prior and during therapy. The study concluded that post pubescent boys need assistance from andrologists on preserving fertility. A solution would be an umbrella program that includes cross-disciplinary such as patient navigators, physical therapists and social workers. In addition, a clearinghouse for transition policy guidelines and databases of adult regional caregivers, as well as the potential for transitional care fellowships is needed. An example of such a program is the transition medicine and surgery (TRAMS) at the George Washington University (GW) (this includes CAPITUL). Challenges include reimbursing for “extra effort”, establishing referral base, defining roles of attending in multiple urologist-involved cases, follow-up policies and assessing on how best to follow up patients. TRAMS offers potential for fertility preservation to boys undergoing gonadotoxic therapy for cancer.

#### Discussion:

- Dr. Gomez-Lobo discussed fertility preservation for females. This includes freezing eggs (just became standard of care 2013) and ovarian tissue freezing. Currently, there have been 30 patients who have successfully become pregnant using these freezing methods. Dr. Gomez-Lobo developed the female protocols for females to implement this process. (This information will be presented later during the meeting.)
- There are evolving principles of best practices. There should be a written policy for transition; the transfer of care is not the same as transition. There is interest across disciplines to aid transition such as gynecology and oncology.
- Two research questions: which patients develop bladder cancer and why? How do we monitor these patients and detect cancer early? Compliance is a big issue (catheter compliance and predictors of compliance) and how to engage patients through adolescence.

**Professional Grass Roots Perspective - Report on Urocongenitalism Work Group at American Urological Association Annual Meeting May 2015**

*Hadley Woods, M.D.*

*Cleveland Clinic Lerner College of Medicine*

Dr. Woods discussed the progress made over a year ago from the UICC. Education among societies such as the American Urological Association (AUA) and specialty societies is key; the merge of community and health systems within the nation is challenging, in part due to geography. The genitourinary congenitalism (GU) area is supported by such advocacy groups such as the AUA and the Society of Urodynamics and Female Urology (SUFU), but each advocacy groups has their own area of interest and there is limited cross talk. Dr. Woods discussed the 2015 AUA GU congenitalism working group at the AUA. The goal of this working group was to encourage crosstalk among pediatric and adult urologists through the discussion of three separate cases. There is little information about what happens to patients across the lifespan. Among the participants in the working group were gynecologists, pediatricians, pediatric/adult urologists and reconstructive surgeons. Dr. Woods discussed existing challenges:

- Meaningful inclusion of patients
- Transcending society “silos” to share experiences
- Better understanding of epidemiology
  - Basic: lifespan, causes of death, associated costs
- Better definition of outcomes measures
  - What is a “UTI”?
  - Estimating Glomerular filtration rate (GFR)?
- Systems: payment challenges, care coordination, community services. Systems for HHS and CMS and develop care pathways.
- Integrating “specialty” providers and community providers to improve access and quality of care

Next steps for the working groups include scientific and academic discourse at breakout sessions during SUFU and AUA meetings, as well as developing education/patient care guidelines. Dr. Woods discussed the missions and goals of HHS, CDC, NIH, and the Administration for Children and Families. Dr. Woods also discussed the function of Patient-Centered Outcomes Research Institute (PCORI). PCORI began funding research in December 2012 and maintains a sizeable and growing portfolio of projects designed to improve patient care and outcomes through patient-centered comparative clinical effectiveness research (CER). PCORI research funds are guided by the five National Priorities for Research and Research Agenda. The work under these priorities is managed by PCORI scientific programs, which track it and evaluate its effectiveness. PCORI is also charged with developing and improving the science and methods of CER that will lead to better healthcare decisions and, ultimately, to improved patient outcomes. The Methodology Committee within PCORI oversees work on methods. There is a strong emphasis on engaging patients and the broader healthcare community in all work as is evident in the criteria PCORI has developed for funded research. Lastly, PCORI also provides awards to encourage engagement of patients and other stakeholders in CER.

Discussion:

- Include patient perspective: critical outcomes for patients and outcomes should be studied. This is a large issue in bowel and bladder for incontinence.

- Regret was prevalent for patients; the perspective of regret for non-fertility preservation.
- Trade-offs for society and patient centered approaches.
- Patients with spinal cord injuries in rural areas are challenging to treat because there are limited or no providers in these areas.

**Report on Research Needs for Effective Transition in Lifelong Care of Congenital Genitourinary Conditions Meeting**

*Tamara Bavendam, M.D., M.S.*

*National Institute of Diabetes and Digestive and Kidney Diseases*

Dr. Bavendam discussed the conceptual framework for lifelong urologic care, in particular for spina bifida patients. Plenary efforts for the meeting included the invitation of multidisciplinary health professionals and pre-populated questions for the breakout sessions. The six breakout groups included:

- Spina bifida reproductive health: reproductive health
- Spina bifida: nonurologic
- Neuropathic bladder & exstrophy: LUT/bowel dysfunction
- Neuropathic bladder & exstrophy: nephrologic/metabolic issues
- Male genital reproductive health
- Female genital reproductive health

Working groups identified research needs by group:

*Epidemiology:*

- Basic cross-sectional and longitudinal epidemiology across the spectrum of conditions and related symptoms
  - Reproduction – sexual function, fertility and pregnancy outcomes
  - Renal function
  - Elimination functions – bladder and bowel
- Basic cross-sectional and longitudinal epidemiology of complications
  - Acute kidney injury
  - Urinary tract infections
  - Urolithiasis
  - Bladder cancer
- Understanding etiology (genetics/heritability, environmental impacts) of the conditions, symptoms, and complications

*Outcomes:*

High Priority

- Renal and Metabolic outcomes
- Bladder outcomes
- Bowel outcomes
- Defining UTI versus colonization
- Sexual function and reproduction outcomes
- Neuro-cognitive and behavioral outcomes

Medium Priority

- Systemic concerns (bone, pain, obesity)

- Broaden definitions of independence and interdependence
- Measurement of adequate hormonal support from puberty to adulthood

*Clinical Care:*

- UTIs and bladder colonization (i.e., patient education, provider integration, clinical pathways, patient impact)
- Nephrologic care (GFR estimation, metabolic assessment, role of AKI)
- Bladder/reservoir management into adulthood (i.e., provider & patient education, surgical intervention/timing, surveillance of reconstructed patients)
- Sexual and reproductive health (i.e., clinical screening, patient/family education, divergent priorities between patient/provider/ family, surgical intervention/timing, psychological impact, functional outcomes, fertility impact)

*Self-Management:*

- Psychosocial issues
- Body image, sexuality and intimacy
- Future fertility expectations/counseling
- Baseline skills and/or socioeconomic factors needed for transition
- Understand learning styles to promote self-management interventions
- Relationship between wellness programs, self-management and continence
- Effect of surgical options on self-management, sexual function, fertility and cancer
- Test behavioral interventions to improve self-management
- Use of mobile applications to increase self-management skills

*System Issues:*

- Learn as much as possible about the patients that fail to transition
- Education of primary care professionals
- Innovative payment models
- Comparative analysis of models of care

*\*Need to determine the best outcomes (e.g., cost, patient-centered outcomes, clinical outcomes) for assessing systems changes*

Dr. Bavendam noted that a meeting summary available at:

[http://www.niddk.nih.gov/news/events-calendar/Pages/Transition-Lifelong-Care-Congenital-Genitourinary-Conditions\\_02-2015.aspx#tab-minutes](http://www.niddk.nih.gov/news/events-calendar/Pages/Transition-Lifelong-Care-Congenital-Genitourinary-Conditions_02-2015.aspx#tab-minutes) (located under the “Minutes” tab).

*Discussion:*

- At Children’s Hospital, each provider bills separately.
- Medicaid is more innovative and flexible with managing patients financially.
- A large patient population is ambulatory. Patients who have less physical disabilities are more likely to have private insurance whereas the patients with more physical limitations are on Medicaid.
- Although there is a big push for multidisciplinary orgs, individual groups work in silos. Understanding social changes for adolescents is important as well as understanding motivators for providers to join clinic. An “accountable” care organization will insure. Motivation for providers to join clinics would be for the clinic/center to be a “center of excellence”.
- Technology based payment mechanisms would help not limit to office visits.
- Interdisciplinary clinics are ineffective.

## **Disorders of Sex Development – Translational Research Network**

*Veronica Gomez-Lobo M.D.*

*Children's National Health System/Georgetown University*

*Member, Disorders of Sex Development Translational Research Network*

Dr. Gomez-Lobo noted that she is a pediatric GYN who also treats adults. She noted that the central hypothesis for the Network is that evidence-based standardization of diagnostic and treatment (medical, surgical, and behavioral health) protocols will be associated with higher rates of definitively diagnosed disorders of sex development (DSD), reduced variation in clinical practice, enhanced patient/family healthcare-related experiences, and improved quality of life outcome.

Specific aims of the proposal included:

1. *Identifying novel pathophysiological mechanisms and improving the molecular diagnosis of DSD.*
2. *Standardizing radiological, biochemical, histological evaluations, descriptions of genital phenotype, and post-surgical appearance and function.*
3. *Identifying biological and social factors associated with variability in psychosocial, psychosexual, and quality of life outcomes in patients with DSD.*
4. *Building a sustainable infrastructure for translational research, including*
  - (1) designing and populating a scalable core registry to support a broad range of DSD-related inquiries
  - (2) ensuring rapid translation of new evidence into clinical practice by integrating standardized DSD diagnostic and treatment protocols and fostering transfer of best practices in healthcare delivery across network sites.

In June 2014, the DSD-TRN transitioned to the adult care workgroup and developed the following priority area outline:

- **General (covered mostly in <http://www.gottransition.org/youthfamilies/>) :**
  - Navigating transition process (from team to PCP+specialists)
  - Paying for care (Costs, Insurance (when to transition from parent insurance), etc)
  - General health and making efficient use of medical care
  - Self care & well being
- **DSD-Related (needing generalized informational DSD resource for late teens/adults & PCPs) More great options to template discussions here <http://www.gottransition.org/resources/index.cfm> :**
  - Navigating school and society (biology, outdated terms, unawareness, sensationalism in media)
  - General psychosocial care (understanding past care and condition, peer support, reviewing medical record with professional, counseling and therapy, depression/mental health)
  - DSD at the doctor's office and navigating new doctors (e.g. confidentiality, sensitivity, moving from teams to PCP+disparate specialists, resources to take to new doctors about DSD, care, and specific needs, finding experienced/informed clinicians in DSD, informed decision-making)
  - Sex & intimacy (with genital difference/ different anatomies)
  - Sharing with others (body, condition, fertility status, etc.)
  - Coping with clinical uncertainty (psychosocial)

- **DSD-Specific (needing specific map per condition for late teens/adults & PCPs + a customizable resource like this <http://www.gottransition.org/resourceGet.cfm?id=241> ):**

- Gonad management and uncertainty (surgical/gyne/endo/psychosocial)
- Hormone replacement (endo/psychosocial)
- Libido (endo+gyne+psychosocial)
- Bone health (endo+osteo)
- Dilation, vaginal treatments, and vaginal health (gyne/physical therapy/surgery/psychosocial)
- Genital health and treatments (clitoral/phallic surgeries), outcomes, and follow ups (sexual sensations, erections, strictures, urine issues, pain, epididymitis, discharges, surgical timelines) (surgical+psychosocial)
- Fertility management and family planning (gyne/urol/endo/psychosocial)
- Cancer risks (endo/surgical/psychosocial)

Goals for improving transition included:

- Educate general adult providers regarding issues to be aware of through review papers (Gyn, and other primary care providers)
- Educate affected individuals
  - Education materials to be guided by review paper;
    - » General transition resources: gottransition.org (e.g. changing providers, navigating insurance, and universal features of transitioning care).
    - » Resources for mid-tier or others mid-tier category or need (e.g. DSD and school, psychosocial care & DSD, navigating DSD care lifelong, sexual health/intimacy & DSD, sharing with others, dealing with uncertainty in care)
    - » Condition specific resources (e.g. dilation, fertility/infertility management, bone health, cancer risks, libido, hormone replacement genital health and treatments/surgeries)

Next steps for the work group include research on best practice in transition through the assessment value of transition tools created, assessment of tool for transition readiness and evaluation current transition strategies and assess for success (Benchmarking). For more information about the Network, please visit: <https://dsdtrn.genetics.ucla.edu/>.

### **Spina Bifida Registry and Standardization of Renal/Bladder Management (10 min)**

*Judy Thibadeau*

*Center for Disease Control*

Dr. Thibadeau noted that funding for this registry began in 2008. There are 6,000 clinic patient participants and 19 clinics. Published studies to date include:

- Testing the Feasibility of a National Spina Bifida Patient Registry (NSBPR).
- The NSBPR: Profile of a Large Cohort of Participants from the First 10 Clinics
- Socio-demographic Attributes and Spina Bifida Outcomes
- Factors associated with pressure ulcers in individuals with spina bifida

Dr. Thibadeau noted that the registry is used to capture information about transitional urology. Approximately 25% of patients are over age 18 and the registry does not include hospitalizations. In addition to the registry, the CDC developed the following programs:

- Spina Bifida End-Stage Renal Disease Project—This project looks at the connection between spina bifida and kidney failure (end-stage renal disease). CDC researchers are determining whether people with spina bifida are more or less likely to experience health problems and die from end-stage renal disease than people who are not affected by spina bifida. This study also looks at the medical and personal characteristics of people with spina bifida and end stage renal disease.
- Urologic Protocol for Young Children—CDC has worked with experts from across the country to develop a medical protocol that will safely and effectively monitor how well the bladder and kidneys are working in newborns and young children with spina bifida. Correctly monitoring the bladder and kidneys, with medical treatment as needed, may eliminate some or all kidney damage. Enrollment for this began in 2015 and assessment in the first 5 years will focus on UTI, renal function, renal scarring and clinical process improvements.

### **GotTranstion.org**

*Marie Mann, M.D.*

*Health Resources and Services Administration*

Dr. Mann discussed updates to the Centers:

- Developed a standardized approach to the transition process in the clinical domain. This document has been downloaded by international organization and programs. It is being tracked how info is used.
- Developed four learning centers to address to address children with special health needs such as cancer, diabetes and without special health needs.
- Received the American Board of Physicians approval for the resolution to increase awareness about need for transition. Deputized college to develop initiatives.
- Initiated initial discussions with major payers: little interest but not sufficient.
- Developed a proposal and presented to CCMI to capture more data. One study has been funded that demonstrated cost savings.
- Developed a coding and reimbursement tip sheet.
- Discussed engagement of family and youth at the leadership level.

### **PCORI Perspective**

*Beth Kosiak, Ph.D.*

*Improving Healthcare Systems Program*

*Patient-Centered Outcomes Research Institute*

Dr. Kosiak began her presentation by noting that PCORI is tasked with 1) funding comparative clinical effectiveness research (CER) that engages patients and other stakeholders throughout the research process and 2) seeks answers to real-world questions about what works best for patients based on their unique personal characteristics, circumstances and concerns and gives clinicians the information they need to help their patients. According to statutory prohibitions, PCORI cannot fund cost effectiveness analysis, measures of the relative costs of care of two or more alternative approaches as the primary criteria for choosing the preferred alternative, development of clinical guidelines, or development of policy recommendations.

PCORI uses the following to define and evaluate comparative effective research:



- Comparators: Compares two or more real world options: diagnosis, prevention, treatment, health delivery. Each must be of proven efficacy (can be usual care, but not preferred). Can't be novel or developmental.
- Rigorous methods: Often RCT, but can be observational study; must have well-articulated design, powered to detect significant effects in primary outcomes
- Pragmatic: Conducted with real world populations in real life settings—not under controlled conditions
- Looks at key subgroups: Is the study powered to measure how the intervention works with different kinds of patients who have the disease or condition? (HTE)

#### A patient-centered PCORI:

- Actively engages patients and key stakeholders throughout the research process.
- Patients are partners in research, not just “subjects”. Includes patients with lived experience
- Researchers must prove that patients and caregivers find the research questions and outcomes important
- Must conduct research in real-life settings that entail choices and options patients and clinicians will actually face
- Includes diverse populations, especially hard to reach populations

#### Research Prioritization Criteria for Patient-Centered CER uses the following criteria:

- Disease incidence, prevalence and burden (particularly chronic conditions)
- Potential for new evidence to improve health, quality of and access to care
- Technical Merit/Scientific Rigor
- Generalizability
- Sustainable
- Patient-centeredness
- Patient and stakeholder engagement
- Relevance to patients and clinicians in decision making about treatment and delivery options
- Clear comparators

#### PCORI's Funding Vehicles and Funding Cycles:

- Competitive LOI screening for all funding vehicles
- Broad PFAs: 2 cycles per year, up to \$1.5 million in direct costs and 3 years in duration—Each of the 5 program areas (Addressing Disparities, Communication and Dissemination Research, Clinical Effectiveness, Improving Healthcare Systems and Methods) has 2 investigator initiated PFAs per year.
- Pragmatic Clinical Studies PFAs: 2 cycles per year, up to \$10 million in direct costs and up to 5 years in duration—Pragmatic clinical trials, large simple trials, or large-scale observational studies. The goal is to fund, real-world, impactful multi-site pragmatic studies.
- Targeted PFAs: One time opportunities that have ranged from \$15-\$30 million for one study (STRIDE Falls with NIA; HCV, Project ACHIEVE in Transitional Care) —Focuses on specific, high-impact topics selected in response to input from patients and other stakeholders through our Advisory Panels and public workshops. These are one-time opportunities.
- Engagement Awards: Start at \$50,000—Pipeline awards are designed to help investigators build the necessary patient/stakeholder structure to support a PCORI award.

- PCORNet and Patient Powered Research Networks Demonstration Projects (Phase II): \$2.5 million for up to 3 years—In Phase I of PCORnet, PCORI awarded funds for the development of 11 clinical data research networks (CDRNs), based in large health systems; 18 PPRNs, which are operated by patient-led groups; and one coordinating center ([www.pcornet.org](http://www.pcornet.org)).

PCORI's traditional care portfolio includes:

- \$65 million total for 12 awards (Project ACHIEVE--\$15 million and Duncan \$14 million) for 3 years
- Studies are primarily focused on transitions from the hospital, ED, Rehab centers, etc. to home for specified patient populations (stroke, children)
- ACHIEVE: evaluates multi-component care transitions being implemented across the US to determine which service clusters work for which populations in different healthcare contexts..
- Duncan, PI: In 50 North Carolina hospitals, compares post-acute stroke care to usual care in an RCT

In other traditional care portfolio developments, there are the following efforts:

- PFA on Evidence to Action Networks to facilitate collaboration and strengthen the research base across investigators in the field
- Topic Brief on pediatric to adult transitions was developed under the auspices of the AD Program, but not pursued.

### **Group Discussion**

*Improving quality of healthcare for persons with congenital abnormalities of urogenital system. Using resources to "Divide and Conquer"*

- Dr. Kosiak noted that ARC and PCORI are charged with dissemination of findings. PCORI has the lead on comparative effective research. ARC works to generate data for health systems.
- Dr. Star noted that NIDDK would be interested in answering specific urology questions or the use of particular models in an RFA. Dr. Moxey-Mims noted an RFA on adherence in nephrology and urology that was recently released. Also, there is PA on self-management. Urology applications are welcome.
- Dr. Star noted there is an uro-epidemiology initiative at a urology center.
- Rose noted MOM study focus on repair vs. MOM2 will have rich data end.
- Dr. Mann noted that there is very little information on population after age 18. Future direction efforts should include developing a mechanism to look at this population. Patients have been requesting this information. Judi noted use of electronics and specific questions. She noted that they have data on age range 18-70.
- NICHD is ready to roll out a "my pregnancy" website that is designed for broad population. This might be useful model for this type of request.
- Dr. Star noted that NIH cannot fund long term registries.
- Beth noted CDRNs can look at data.
- Rose noted that now that spina bifida field is growing as patients are living longer.
- Dr. Star asked participants how successful transition be evaluated. Dr. Gomez noted her definition included the components of self care, coordination with physicians, and psychosocial support.
- Dr. Wood noted time dependence is importance; what is important at 18 may not be at 33.

- Dr. Mann noted quality of life will be a dominating factor. At whatever age, patient living to fullest potential is relative. Provider must be knowledgeable about congenital conditions and transitioning the individual into adult care.
- Beth noted that there is a different model of care that are coming out. To address geographical issues, training for Project ECHO was launched in an effort to educate more primary care providers (PCP). Preliminary results show that PCPs provided care as well as specialists.
- Dr. Star noted interest in a methods study. Talk to patients and figure out outcomes and measure them.
- Beth suggested looking at patient engagement awards. There are some innovative ways to engage patients.
- Dr. Gomez noted that multidisciplinary teams work well in pediatric urology, but not adult urology. Spina bifida patient's providers local to them and PCPs have worked well. Give PCPs tools for support and referrals for surgery. Also, consider what behavioral incentives are there for the PCPs? The issue with tools for providers is that they are overburdened.
- CDC noted that transition really begins at birth and family should prepare at infancy.
- Kim Konkel noted that she is a social worker and noted medical social workers are trained very well and know what boundaries are; these professionals are excellent clinical care guides and patient navigators.

Dr. Bavendam thanked participants for their feedback and attendance. Information for the next UICC meeting and meeting minutes from this meeting will be sent by email shortly. Dr. Bavendam adjourned the meeting.