Descriptions of Select Medicine X | ED Sessions Covering PCOR/CER Topics

Summary:
Presented below are descriptions and details of select sessions and presentations that occurred during Medicine X | ED 2017 covering topics in PCOR/CER. This selection includes presentations in both the PCOR/CER abstract tract, as well as a selection of presentations relevant to our high level community-building effort around advancing patient-centered outcomes in medical education.

Title: Oral Presentation: Patient Centered Outcomes Research and Comparative Effectiveness Research Panel
Track: PCOR
Speakers: Ruth Bush, PhD; Joseph Gulfo, MD, MBA; Seth Ginsberg; William Palva
Moderators: Larry Chu, MD, MS; Alicia Staley, ePatient Coordinator
Description: This mainstage panel was moderated by patient Alicia Staley and Dr. Larry Chu, aimed at understanding the landscape of patient-centered outcomes research and comparative effectiveness research. Below you will find individual descriptions of presenters from the session.

Subsession 1: Patient collaboration: Re-imagining adolescent transition at an academic medical center

Speaker: Ruth Bush, Associate Professor, University of San Diego. Dr. Bush received her bachelor’s degree, cum laude, from Princeton University; her master’s degree from the University of London while on a Rotary International Fellowship; and her Master’s degree from the Graduate School of Public Health at San Diego State University, where she received the Outstanding Epidemiology Student award. She received her PhD from UCSD/SDSU Joint Doctoral Program in Public Health, where she was awarded dissertation funding from an NIH National Center for Research Resources M01 grant (5M01RR000827-25). Her dissertation research focused on assessing the effect of physical activity on bone mineral density among a cohort of women who had been varsity athletes.

Her extensive clinical research experience spans multiple health care settings, including military
Adolescents, academic hospitals, specialty and community-based clinics, and pediatric programs. She has led the development and initiation of clinical research studies as well as providing statistical consultations for projects in research areas as varied as patient-centered outcomes, women’s health, traumatic brain injury, and birth defects. She has served as a consulting member of Electronic Health Record (EHR) implementation teams.

**Description:**

**Background:**
Adolescents with spina bifida (SB), the most common neural tube defect in the United States, which annual affects 1500 to 2000 live births, require complex, multidisciplinary coordinated care for optimum health and development. Adolescent patients affected by chronic childhood disease often have problems finding adult providers who have training in childhood-onset diseases and find challenges in making the transition to adult medical systems when making the transition from pediatric to adult medical system. Research identifying patient-centered needs related to the transition from adolescent to adult care, especially in multi-lingual, multi-cultural communities, is needed.

**Purpose:**
A Patient Centered Outcomes Research Institute (PCORI) Pipeline to Proposal award provided the means to build an interdisciplinary team of patients, parent advocates, students, health care providers, and researchers to examine adolescent health care transition and to identify relevant patient-centered outcomes for adolescent transition to adult care.

**Methods:**
We created a network of patient and family stakeholders who were invested in identifying the transitional issues facing our predominantly Latino spina bifida population, through structured focus meetings facilitated by researchers, a bilingual premedical student, and a nursing PhD student. Participants were asked to think about specific events in their life relevant to transition to adult care (e.g., risks associated with the transition; ability to communicate with clinical providers; experience making appointments; access to transportation; etc.). The participants described the fears, frustrations, uncertainties, and barriers that came to mind.

**Results:**
Adolescents, parents, and clinical stakeholders expressed a strong desire to be able to work with a trained individual to provide personalized care coordination, as well as
identifying barriers to transitions, i.e. social isolation, lack of transportation, physical restrictions, and financial limitations. Adolescents want to meet adult health care providers who are willing to be partners in the transition and suggest social media and other electronic programs should be incorporated in the transition. In addition to identifying future educational areas to address regarding transition, health care students were exposed to a shared partnership model in which patients and providers are using findings to guide research.

Conclusions:
Participants provided critical guidance to identify meaningful adolescent transition to care issues and to create structure for the next phase of a targeted program. Their input will determine patient-centered approaches to the transition process and will define which services they feel are essential to care continuity. The project also demonstrated effective methods to involve all stakeholders, including patients and caregivers, in health care education about participatory decision making and clinical practice.

Subsession 2: PCOR and CER cannot replace doctor-patient individualized care, yet
Speaker: Joseph Gulfo, Joseph V. Gulfo, MD, MBA, is Executive Director of the Lewis Center for health care Innovation and Technology at Fairleigh Dickinson University (FDU) where he is spearheading the Initiative for Patient-Centered Innovation and also serves as a Professor of Health Sciences.

Description:
Despite advances in genomics, epigenomics, and the promises of "big data" approaches, individualized medicine occurs best at the doctor-patient level. Clinical trials, no matter how large, gauge the "average patient" response. Even when specific biomarkers are employed, a normal distribution of response is seen. So, it is critical for physicians and patients to understand that "good medicine" has still not been reduced to science, rather, a good measure of titration via trial and error is still needed. This is the reason that we need as many therapies in the medical armamentarium, as possible.

Take, for example, eteplirsen (Exondys) for the treatment of muscular dystrophy. The drug was approved in September 2016 based on results from a small, nonrandomized study. Data were presented on 12 patients who took eteplirsen for four years and compared to matched control patients from Italy and Belgium. A randomized study was not possible to conduct due to ethical constraints – administering placebo to patients while requiring multiple muscle biopsies over an extended period offers no possible benefit. Results of the 6-minute walk test indicated that boys taking eteplirsen walked 162 meters further than the control group, and ten of the 12 boys on the
drug were still able to walk after four years, versus only three of 13 in the control group. FDA commented, "Know that if these results were from a well-designed and interpretable trial, there likely wouldn't be much to talk about," meaning, the data were quite compelling, but from the wrong kind of study in FDA’s view.

At the Advisory Committee meeting in April 2016, Dr. Bruce I. Ovbiagele, chairman of neurology at the Medical University of South Carolina, who voted against approval, said, "Based on all I heard, the drug definitely works, but the question was framed differently."

Panelists were instructed to consider only data from well-controlled studies, which flies in the face of FDA’s initiative to capture the voice of the patient in decision-making. “Well-controlled” implies randomized, multi-arm studies. But, randomized two-arm studies are not the only trials that are considered capable of providing substantial evidence; historical controlled trials are adequate according to the law. Further, competing standards were given to the Panel – one calls for a safety determination first, followed by an effectiveness determination, while the other calls for the effectiveness determination first, followed by safety. This is important for eteplirsen, which is very safe, but the effectiveness data from controlled clinical trials are not considered definitive by many. According to the first standard, as long as the drug is proven to be safe, any clinical benefit is meaningful and should result in approval.

**Subsession 3: Rheumatoid arthritis patient characteristics and willingness to participate in research**

**Speaker: Seth Ginsberg,** is an international health advocate and thought leader based in New York City. Ginsberg was diagnosed in 1994 with Spondylarthritis, and in 1999, at age 18, he helped pioneer the world’s first online patient community for the millions of people worldwide suffering from arthritis, called CreakyJoints. CreakyJoints has grown into a trusted and recognized website and patient community, delivering robust patient education, community-based and web-based support, advocacy initiatives and patient-centered research. In 2007, Ginsberg and social entrepreneur Louis Tharp co-founded the Global Healthy Living Foundation, the 501(c)(3) non-profit parent organization of CreakyJoints and related advocacy campaigns. CreakyJoints Australia, also lead by Ginsberg, is the first international patient community under the GHLF umbrella and constitutes a growing and vibrant community of patients throughout Australia.

Since 2014, Ginsberg has served as a Principal Investigator of the PCORI-funded patient-powered research network ArthritisPower®. The web and smartphone app is an innovative registry engineered for and by people with rheumatoid arthritis and related autoimmune and inflammatory conditions. Research efforts are overseen by a patient governor
group as well as a distinguished research advisory board.

**Description:**

Background:  
CreakyJoints (CJ) is an international online arthritis patient community offering education, support, advocacy, and the opportunity to participate in research. In 2014, CJ partnered with the University of Alabama at Birmingham (UAB) to create ArthritisPower (AR-PoWER ARthritis Partnership with Comparative Effectiveness Researchers), a Patient-Powered Research Network. ArthritisPower is part of PCORnet and is supported by a multi-year investment by PCORI. ArthritisPower aims to conduct patient-centered comparative effectiveness research and has a patient governor group and research advisory board to ensure the relevance and transparency of research activities. ArthritisPower includes an online portal and innovative mobile application to engage patients and collect diagnosis, symptom, and medication data. ArthritisPower already launched studies and collaborates with other PCORnet networks and external organizations on research activities.

Recent Findings  
CJ members completed an online form preparatory to launch of ArthritisPower. The online survey captured demographic, diagnosis, treatment, and willingness to participate in research. Of 2,147 patients with RA, 970 registered CreakyJoints members completed the survey on their willingness to participate in research during 2014-2015. Most patients (68%) had never taken part in research, but nearly all (93%) would do so if the study was recommended by their doctor. Similarly, patients would be willing to participate if invited by a medical school/hospital (87%), non-profit organization (79%), or the government (73%). A majority (75%) would refuse to divulge their Social Security Number (SSN) to researchers and half would refuse to give the last 4 digits of their SSN, compared with the 20% and 34% who would refuse to share email address or medical records for research.

In another survey, members of CreakyJoints participated in one of six nominal groups held in June 2015. Each of the six groups generated a set of educational topics and then rank-ordered them within each focus group. Eight educational themes emerged that were related to 28 unique educational topics. Among the individual items rated as “Extremely Important” by a majority of patients were: How arthritis affects more than just your joints (86%), What are the signs that the medication is not working (84%), and Importance of knowing about how the disease will progress even if the news is bad (83%), among others.
Conclusions
These studies demonstrate that people living with arthritis are willing to participate in research and have concrete ideas about the questions that need to be answered in order for them to make informed health care decisions with their doctor. Considering patients as unique stakeholders is vital to maximize the relevance and patient-centeredness of future research endeavors.

**Subsession 4: Transforming health care and medical education through clinical Big Data analytics**

**Speaker:** William D. Paiva, PhD, is Executive Director of Oklahoma State University's Center for Health Systems Innovation (CHSI). The Center is focused on transforming rural and Native America health through implementation of innovative rural care delivery and information technology solutions developed with the largest clinical dataset representing clinical records for 63 million patients. In addition, the Center is leading the University in adding innovation to their medical education curriculum.

**Description:**
Health care is undergoing significant transformation, and digital health data is at the center of this change. According to the Centers for Disease Control, nearly 80 percent of the nation’s health care institutions have converted to an electronic medical record (EMR) system from the old paper-based system. New technologies like smartphone applications are also creating new stockpiles of digital data. Genetic data is growing as well; scientists can sequence a person’s entire DNA within 24 hours and for less than $1,000. Collectively, the amount of digital health data is expected to grow from 500,000 to 25 million terabytes over the next five years.

Why do we care that our health information is now in a digital format? How does it benefit all of us?

People who work in health care—and every industry for that matter—are smart, well trained, and do their best to stay up-to-date with the latest research, methodologies and trends. However, it is not rational to assume individuals have the depth of knowledge or data access to deal with every situation they encounter. Furthermore, the health care field is already understaffed, and this issue will only get worse as the looming mass retirement of baby boomers from the health care workforce creates an unprecedented supply-and-demand crisis.
Digitized health data has the potential to help mitigate this troubling situation. Predictive medicine uses computing power and statistical methods to analyze EMR and other health-related data to predict clinical outcomes for individual patients. Beyond health outcome forecasting, predictive medicine also can uncover surprising and often unanticipated clinical associations.

Oklahoma State University’s Center for Health Systems Innovation (CHSI), through its Institute for Predictive Medicine (IPM), is a leader in the exploding field of predictive medicine thanks to the unprecedented donation by Cerner Corporation of its HIPAA-compliant clinical health database, one of the largest available in the United States. Specifically, this dataset represents clinical information from over 63 million patients and includes admission, discharge, clinical events, pharmacy, and laboratory data spanning more than 16 years.

Over 20 full-time CHSI employees and nearly two dozen graduate students are working to execute the CHSI mission to transform rural and Native American health through data analytics. Further, CHSI has a number of ongoing partnerships with academia, health systems and corporations to extract value from digitized health data.

One example of CHSI’s numerous predictive medicine projects is an effort to help physicians determine whether the performance of particular cardiovascular drugs varies by gender or race, or both. Conversely, this study will help indicate which drugs perform poorly or even cause complications in these populations. Other CHSI studies are designed to give physicians insight into whether patients with a particular disease are likely to develop or already have an associated disease, which will aid in co-managing these conditions and lead to better health care. Another project is designed to help hospitals use data on patient demographic characteristics, comorbidities, discharge setting, and other medical information contained in comprehensive EMR systems to determine if patients are at high risk for being readmitted for disease-associated complications. If patients are considered high risk, they can get the care and support necessary to prevent frequent cycling through the health care system.

Predictive medicine can also lead to the creation and implementation of tools for managing larger patient loads, which can aid health care providers in dealing with supply-and-demand problems. For instance, CHSI has developed a clinical decision support system that can detect diabetic retinopathy with a high degree of accuracy using lab and comorbidity data available through primary care visits. This algorithm addresses the very real challenge of low patient compliance, particularly among rural and underserved populations, with annual ophthalmic eye exams, which are the gold standard for retinopathy detection and preventing vision impairment or total vision loss. CHSI is extending this work to other common diabetes-related microvascular complications with
the goal of developing a comprehensive suite of tools that can help increase prevention and management of these complications among the nation’s growing diabetic population.

The transformation of health care delivery is not a distant vision, but rather a current reality thanks to predictive medicine tools and insights. To quote the famed historian, explorer and philosopher Winwood Reade, “While the individual man is an insoluble puzzle, in the aggregate he becomes a mathematical certainty.” These words epitomize the power and potential of digitized health data to truly revolutionize health care as we know it. OSU’s Center for Health Systems Innovation (http://chsi.okstate.edu), a joint venture between the OSU Spears School of Business and the OSU Center for Health Sciences, will be at the center of this evolution by bringing critical, game-changing ideas and processes to health care for the present and the future.

**Title:** Walking the Walk: Initiatives towards co-creation and collaboration with patients and families in medical student education  
**Track:** Everyone Included  
**Speaker(s):** Genevieve Allen; Kori Jones, M.Ed; Rajesh Mangrulkar, MD; Greg Merritt, PhD.  
**Description:**

For more than 100 years, American medical education emphasized the acquisition and application of biomedical scientific knowledge, with a minor but growing focus on communication skills and empathy. While the model has evolved to become more balanced over the past 2 decades, there has been a systematic absence of the patient/family voice in the co-creation of this new model of medical education. Over the past decade, the University of Michigan Medical School (UMMS) has partnered with the Health System’s Patient and Family Centered Care (PFCC) to co-create educational sessions for students. However, as the medical school has embarked on its curricular transformation to a new model where students are immersed in more experiential and exploratory environments from day 1, it has significantly expanded its collaboration with this unit, as both co-creators and co-implementers of curricular experiences, assessment activities, admissions work, and on competency committees.

Our proposed Medicine X | ED panel presentation will walk session participants through “networked intelligence” perspectives of Everyone Included™ stakeholders. Panel participants will present innovative educational approaches and lessons learned surrounding the effective engagement of patients/families in UMMS’s newly transformed medical education system including:

1) Executive Planning: Serving as members on the Curriculum Policy Committee and Medical Student Competency Committee.
2) Curriculum Design: Conducting focus groups with learners and patients/families to understand where gaps exist in medical education, “what matters” to people receiving care, how we may partner to reduce harm and improve outcomes as well as the mindset, and learning preferences of medical students.

3) Curriculum Implementation: Selecting and educating patients/families to serve as didactic educators providing the foundation for patient and family centered philosophy, effectively share stories, facilitate small group reflection and discussion, and mentor students through open dialogue about illness, health care team experiences, and health care system navigation.

4) Admissions: Contributing to interview evaluation criteria, participating in simulation scenarios, and evaluating candidate performance on structured interviews.

5) Learning Support: Monitoring student progress and designing a model where patients/families may serve as coaches for students not meeting certain competencies.

Additionally, the panelists will discuss the extension of this model into interprofessional education and expansion to residents, fellows, and attending physicians to ensure that what is being learned in the classroom is being supported and reinforced in patient care areas. Finally, while it is too early to determine how these initiatives influence behavior and patient care over time, a preliminary evaluation of medical student perceptions and acceptance of engaging patients/families in education processes will be shared.

**Title:** Informed consent vs. talk therapy: Who decides when a transgender person can medically transition?

**Track:** Gaps in Medical Education

**Speaker(s): Charlie Blotner**, Precision Medicine Advocate, Cure Forward Corp. Charlie is a patient advocate who is dedicated to helping people be better informed about their health, research, policy, and the future implications of their disease. Charlie is also a social worker in training, graduating from with a BS from Arizona State University this spring and pursuing their MSW at the University of Washington starting in the fall. They are a Cure Forward precision medicine team member, Stanford Medicine X Student Advisor, and co-founder and co-moderator of #BTSM (Brain Tumor Social Media) Chats.

**Description:**

The National Institutes of Health recently announced that they have designated lesbian, gay, bisexual, and transgender (LGBT) people as a health disparity population. The rationale for this designation has to do with the mounting evidence that the LGBT population has less access to health care and experiences higher of burdens of diseases
such as depression, cancer, and HIV/AIDS. A study by Stanford University School of Medicine itself found that when surveying 176 medical schools, the median reported time dedicated to teaching LGBT-related content over the course of the entire curriculum was 5 hours. A third of those responses reported that their schools spent zero hours on LGBT health-related content during clinical training. While the extent of the causes of health disparities for the LGBT population are not yet fully understood, we have the power to educate current and future providers on how to better improve the lives of their LGBT patients.

I intend to discuss the gatekeeper model versus the informed consent model in the context of the World Professional Association for Transgender Health (WPATH) standards for transgender (trans) individuals seeking to start hormone replacement therapy (HRT) and or gender reassignment surgeries. The benefits and dangers that these models pose are crucial for providers to be aware of when treating their trans patients. Trans patients have been expected to undergo extensive talk therapy in order to access medical interventions and start HRT in recent years. However, with a lack of finances and access to health care to begin with, this step and pillar of the gatekeeper model and WPATH as a whole is extremely dangerous for an already vulnerable population in delaying care. While medical attention to trans people is starting to increase (see: Lancelet 2016 Transgender Health series), we still can and need to do better.

Take-a-ways from my presentation would include:

- Increased knowledge of the current WPATH standards of care and problematic models of starting hormone replacement therapy and having gender affirming surgeries
- Empowerment to taking changing the standard of care
- An understanding of hormone replacement therapy and general overview of gender affirming surgeries - Acquisition of transgender health care resources
- Empathy surrounding medical transition

**Title:** Meet Me Halfway: A patient-centered paradigm to address SGM-related health needs  
**Track:** Gaps in Medical Education  
**Speaker:** Subhashree Nayak, MS-II, University of Maryland School of Medicine

**Description:**  
Sexual and gender minority (SGM) populations experience significant health disparities including higher rates of chronic disease, anxiety/depression, and substance abuse disorders than heterosexual, cisgendered counterparts, and as a result, yield poorer physical and mental health outcomes. This is exacerbated by the SGM community’s low
rates of engagement with health care systems. One contributing factor is the lack of knowledge and clinical skills necessary to define and address the needs of SGM patients among providers, a phenomenon that contributes to the alienation and disengagement of SGM patients from health care systems.

Ill-defined and non-standardized SGM health curriculum in medical schools contributes to gaps in provider knowledge. Implementing current understanding of health disparities experienced by SGM communities and clinical skills tailored to define and address medical needs of SGM persons in medical education curriculum has emerged as a priority for medical schools in recent years, yet remains incremental in progress and challenging to develop. SGM-related medical education curriculum often focuses on broad public health concerns, however this often minimizes holistic, personalized, and patient-centered clinical intervention. Thus, we have developed a paradigm to more systematically address SGM-related health issues that is centered around the patient’s journey of sexual and/or gender identity development.

In this presentation, we will define three stages of sexual and/or gender identity development in SGM persons, highlight potential challenges that SGM patients encounter within each stage, and propose appropriate interventions that can be made on the part of the provider in order to best address the needs of the patient. This curricular paradigm is designed to familiarize medical students and providers with common elements of SGM patient narratives, foster empathy and rapport between providers and SGM patients, and increase provider awareness of SGM needs in a patient-centered fashion. A personalized medicine approach has the potential to better engage SGM patients and increase their retention within health care systems and ultimately improve health outcomes. This curricular paradigm seeks to address and contribute to the reduction of health disparities impacting SGM persons and communities and is consistent with the major public health goals outlined by the US Department of Health and Human Services: Healthy People 2020 Initiative.

**Title:** The power of storytelling to increase engagement and promote empathy in education  
**Track:** Engagement Learning Design  
**Speaker:** Kristen Terlizzi, Co-Founder. National Accreta Foundation. Kristen Terlizzi is a patient speaker, maternal health advocate and co-founder of the National Accreta Foundation. She is a survivor of Placenta Percreta, a life threatening pregnancy complication requiring attentive multidisciplinary care. Kristen has spoken at events for medical students, hospital leadership, perinatal quality collaboratives, patient experience conferences and blood banks. Kristen’s story is published in The Wall Street Journal and her medical case report is published
in Obstetrics & Gynecology, the official journal publication of the American Congress of Obstetricians and Gynecologists.

**Description:**

Medical school education across the country has a goal to become more patient-centered. The best way to accomplish this is to embed more patient stories into medical school education. Patients and families share a common theme that physicians need to “treat the person” and not the “disease.” Medical schools can help instill this mindset in their students by having patients elucidate the emotional, social, and spiritual ramifications to any illness. No one can better relay this connection than our patients and their caregivers.

Patient speakers bring a new voice to presenting clinical information while simultaneously providing students with unique insight into the patient perspective. A skilled speaker can take her audience on a journey where the learnings are shared and experience is empathized, thus sharing a universal message fit for any audience. Patient storytelling allows the stakes to be real for both the speaker and listener, leading to a much more memorable and poignant lesson than one from a textbook.

Kristen Terlizzi is a patient speaker at Stanford Health Care. Kristen will share her story and how patient storytelling is utilized in medical school education and hospital settings with physicians. We will explore the role of patients as the educator with the power of storytelling from the patients’ and caregivers’ perspectives.

We are in a revolution of patient engagement in health care. Patients are becoming educated experts in medical conditions and successful care plans often become a partnership between patient and physician. Employing patient storytelling in medical education promotes student engagement and prepares them for this evolving culture.