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Illinois Department of
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New Advisory Council Studies Medicaid Financing Models to Expand Equitable Access to Sickle Cell and High-Cost Drug Treatments

The Council will deliver recommendations by the end of 2024

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The newly formed Advisory Council on Financing and Access to Sickle Cell Disease Treatment and Other High-Cost Drugs and Treatment has begun its work to study options and make recommendations for developing payment models and financing structures for novel Sickle Cell Disease treatments, as well as other high-cost drugs and therapies that are not readily accessible to those who could benefit from them.

Earlier this year, Governor JB Pritzker signed Executive Order 2024-01 to form the Advisory Council. The Advisory Council was tasked with providing the Illinois Department of Healthcare and Family Services (HFS) input as they develop new financing arrangements to ensure equitable access to these innovative treatments, and ultimately improve health outcomes for Illinois residents.

The group's first meeting was on June 25, and they will continue to meet through 2024, until delivering their recommendations to the Governor and the Illinois General Assembly by the end of the year.

"The pipeline for new and potentially transformative cell and gene therapies is expanding quickly, and Medicaid will be a major payor," **HFS Director Elizabeth Whitehorn said.** "I am thrilled that the Advisory Council has begun its incredibly important work to develop solutions that will expand access to these new drugs and treatments, which often have costs that are insurmountable for most people who could benefit from them."

Under the leadership of HFS, the Advisory Council will complete a comprehensive review of innovative approaches to establish sustainable publicly funded payment models and financing structures for high-cost drugs and treatments. To ensure equitable access, the Advisory Council will also focus on value-and-outcome-based models and engage with subject matter experts, providers, insurers, drug manufacturers, and Medicaid customers living with conditions for which these drugs and treatments exist.

The Advisory Council will make recommendations on financing models that could be implemented in Illinois, or nationally, with the support of the federal Centers for Medicare and Medicaid Services (CMS) and will identify state and federal policies and legal and regulatory changes necessary to implement the recommendations.

At the federal level, the U.S. Department of Health and Human Services is launching a new Cell and Gene Therapy Access model, which will negotiate and administer outcomes-based agreements with drug manufacturers on behalf of participating states, with savings tied to patient outcomes. States can join this access model beginning in January 2025, and the initial focus is on treatment for Sickle Cell Disease. Other cell and gene therapies may be added to the model in the future.

People living with Sickle Cell Disease often encounter barriers to accessing treatment to improve their quality of life, including limitations in geographic access to care, high cost of treatment, limited providers, and disproportionate effects on communities of color. In December, the Food and Drug Administration (FDA) approved two gene therapies to treat Sickle Cell Disease. The cost of one-time gene therapy ranges from $2 million to $3 million, and the cost of managing the disease over a lifetime for the most severely-impacted individuals can range from $4 million to $6 million.

Sickle cell disease is a group of lifelong, inherited blood disorders that affect more than 5,000 Illinois residents. Sickle cell disease disproportionately affects people of color, particularly Black Americans. Sickle cell disease affects an estimated one in every 365 Black Americans and one in every 16,300 Latino Americans.

More than 40% of Illinoisans living with Sickle Cell Disease have healthcare coverage through the Medicaid program, and an estimated 49% of those customers have a severe form of the disease.

Despite the cause of sickle cell disease being known for nearly 70 years, racial discrimination within the healthcare system has resulted in a historic lack of resources dedicated to supporting research and treatment of the disease. As a result, a limited number of health care providers have expertise in comprehensively managing sickle cell disease, and individuals living with the disease often encounter geographic and cost barriers to accessing care, further exacerbating the existing inequities in the health care system that disproportionately affect people of color.

In addition to treatments for Sickle Cell Disease, the FDA has recently approved drugs to treat Duchenne muscular dystrophy, metachromatic leukodystrophy, and Hemophilia A and Hemophilia B. The FDA is scheduled to review additional drugs this year.

The 20-member Advisory Council is made up of a range of individuals with diverse backgrounds and expertise. The Advisory Council members are:

- **Elizabeth Whitehorn, Advisory Council Chair,** is the Director of HFS. Gov. JB Pritzker appointed Director Whitehorn to lead HFS in January 2024, following five years serving as First Assistant Deputy Governor for Health and Human Services. In this position, she played a leading role in the state's response to the COVID-19 pandemic, including testing and vaccine efforts and the nation-leading rental assistance and childcare restoration grant programs. Director Whitehorn has worked closely on the administration's healthcare initiatives, including legislation to authorize a state-based healthcare exchange, increased access to healthcare services, and expanded programs supporting reproductive healthcare, as well as efforts to strengthen the state's children's behavioral health and early childhood systems. Before joining the administration, Whitehorn served as a senior policy advisor on Governor Pritzker's campaign, where she focused on healthcare, economic development and criminal justice policy proposals.

- **Alexandra Carpenter**
  Carpenter's daughter was diagnosed with Spinal Muscular Atrophy (SMA) type 2, in November 2020. SMA is a genetic condition where the body is missing the SMN gene, which causes muscle weakness and atrophy and affects the child's ability to sit up, have head control, walk, as well as damaging the muscles involved with breathing and swallowing. Without treatment, this disease is typically fatal before an individual's 2nd birthday. In 2019, the FDA approved a new gene therapy treatment for SMA. This gene therapy, Zolgensma, has a purchase price of $2.1 million dollars for a 1x infusion. At the time of her daughter's birth, screening for SMA at birth was not occurring in Illinois, and she was treated after she already had permanent muscle damage at 17 months old. Early treatment can prevent permanent damage to muscles. Due to Carpenter's diligent and persistent pressure on the family's insurance company, her daughter received her gene therapy just 10 days after her diagnosis. As of January 2024, all 50 states screen every newborn at birth for SMA, which will allow treatment to begin prior to symptoms developing.

- **Ifeanyi "Beverly" Chukwudozie** is an experienced public health administrator and researcher with a dual master's degree in public health and business administration. With over 25 years of advocacy experience, Beverly is dedicated to advocating for patient rights, particularly those affected by Sickle Cell Disease (SCD). Her expertise in health equity research for chronic diseases gives her a deep understanding of patients' systemic challenges to care. Her unique perspective as an individual living with SCD enriches her advocacy and research efforts. She has shared her expertise on SCD at local, regional, and national levels. Beverly serves on multiple advisory boards for community

organizations and national grant-funded research programs, ensuring patient perspectives remain central to healthcare discussions, research, and policies.

- **Dr. James LaBelle** is an Associate Professor of Pediatrics at the University of Chicago and the Director of the Pediatric Stem Cell and Cellular Therapy Program at Comer Children's Hospital. In this role, he has advanced the clinical leadership and comprehension of the evolving science, oversight, application, economy, and meeting unmet needs for the care of children with malignant and non-malignant diseases. Dr. LaBelle also runs at translational science laboratory dedicated to making inroads on challenging issues that necessitate significant overlap between biomedical engineering, immunology, cancer biology, pharma, and clinical translation. His main focus has been manipulation of the immune system using small molecules targeting the BCL-2 family of apoptotic proteins and nanoparticle-based targeted peptide and small-molecule drug delivery into diseased cells.

- **Dr. Ruchika Goel** is a Professor of Pediatrics and Internal Medicine in Hematology Oncology, Simmons Cancer Institute at SIU School of Medicine and an Adjunct Professor in the Division of Transfusion Medicine, School of Medicine, Johns Hopkins University. She also serves as Senior Medical Director for Vitalant, one of the nation's largest nonprofit blood and biotherapies healthcare organizations, providing hospitals and patients across the United States with a safe blood supply. Vitalant has a network of 115 donation centers nationwide, and host approximately 60,000 blood drives annually, providing blood and special services to patients in roughly 900 hospitals.

- **George Kitchens** is co-CEO of Artia Solutions, which he founded in 2005. For almost two decades, Kitchens has provided consultation services to pharmaceutical manufacturers operating within the Medicaid environment. Prior to Artia, Kitchens was the Vice President of Business Development for Provider Synergies, a company managing rebate programs for Medicaid. He has also served as Pharmacy Bureau Chief at Florida Medicaid, and held numerous positions for the Eckerd Corporation, including Regional Pharmacy Operations Manager and Director of Managed Care Development. He is a member of the Academy of Managed Care Pharmacy, Florida Pharmacy Association and Rho Chi Honor Society.

- **Susan Stuard** is the Director for State Technical Assistance at the Center for Evidence-based Policy where she provides leadership, support and insight to the Center's state technical assistance projects, helping states to evaluate policy changes by facilitating meetings and stakeholder input processes, developing and

analyzing policy frameworks, translating evidence into policy outcomes, and helping states create strategies and decision-tools to enable change. Previously, she led THINC, the not-for-profit convening organization that established research-based criteria to enhance health care quality and value in the Hudson Valley and served as the director for technology policy development at the New York-Presbyterian Hospital. Stuard serves on the Board of Directors for the National Committee for Quality Assurance (NCQA).

- **Nathan Schaefer** is Senior Vice President of Public Policy and Access for the National Bleeding Disorders Foundation (NBDF), formerly the National Hemophilia Foundation. Schaefer is a senior Nonprofit Executive with 20 years of progressive leadership experience overseeing political advocacy organizations in supporting bleeding disorders, LGBTQ and HIV-affected communities in Chicago, New York and Washington D.C. The NBDF is dedicated to finding cures for inheritable blood and bleeding disorders and to addressing and preventing the complications of these disorders through research, education and advocacy.

- **Steve Sproat** is Pharmacy Director for Aetna Better Health of Illinois, overseeing the implementation and expansion of the pharmacy benefit for a large Medicaid Managed Care plan. Prior to that, Sproat served as Regional Director – Specialty Pharmacy at Prime Therapeutics, Inc., and in multiple roles for Caremark, Inc., including as Vice President – Product Management.

- **Brian Smolich** is Vice President of Quality and Managed Care Operations for Health Alliance Medical Plans. He leads a team of Managed Care Pharmacists and Pharmacy Operations Coordinators who provide expertise to help patients better understand their medications, maximize their care and troubleshoot prescription-related issues. While at Health Alliance, he has held a number of operational roles within the Pharmacy Department, including Prescription Benefit Manager oversight, Prior Authorization Policy Development and Review and engaging providers and members on quality initiatives. Prior to 2021, he was the Director of Pharmacy. He started his career in Pharmacy working for Walgreens as a retail pharmacist in the Champaign, Illinois market.

- **Melissa Creary** is an Assistant Professor in the Department of Health Management and Policy in the School of Public Health. She is also Senior Advisor of Public Health, Policy and Equity at the American Thrombosis and Hemostasis Network (ATHN) and Associate Director of Anti-Racism for Michigan Social Health Interventions to Eliminate Disparities (MSHIELD) at Michigan Medicine. She received her PhD in Interdisciplinary Studies focusing on Health, History and Culture, at the Graduate Institute for the Liberal Arts and Masters in Public

Health at Emory University. Over a nine-year career at the Centers for Disease Control and Prevention in the Division of Blood Disorders, she helped create and lead the first national program and data collection system for Sickle Cell Disease at the agency.

- **Rachel Sachs** is a Professor of Law at Washington University in St. Louis. Her research analyzes problems of innovation and access to new health care technologies. Professor Sachs recently served in the Biden-Harris Administration as a Senior Advisor at the Department of Health and Human Services Office of the General Counsel, Centers for Medicare and Medicaid Services Division. Prior to joining Washington University in St. Louis, Professor Sachs was an Academic Fellow at the Petrie-Flom Center for Health Law Policy, Biotechnology, and Bioethics and a Lecturer in Law at Harvard Law School. She also clerked for the Honorable Richard A. Posner of the United States Court of Appeals for the Seventh Circuit.

- **Dr. Anirban Basu** is a professor of Health Economics and the Stergachis Family Endowed Director of The CHOICE Institute at the University of Washington, with joint appointments in the Departments of Health Systems & Population Health and Economics. He is a Research Associate at the US National Bureau of Economic Research and an elected Fellow at the American Statistical Association. His research focuses on understanding the economic value of health care, generating causal evidence, and, on the potential for discrimination with machine learning and artificial intelligence algorithms. From 2018-204, he co-led the NHLBI's Cure Sickle Cell Consortium on Economic Impact and Analysis. He served on the 2nd Panel on Cost-effectiveness Analysis in Health and Medicine and serves on the Editorial Advisory Board *for Value in Health* Journal.

- **Rena Conti** is an associate professor of Markets, Public Policy & Law at the Questrom School of Business at Boston University, and co-Director of the Technology Policy and Research Initiative. She was on faculty at The University of Chicago between 2006 and 2018. Professor Conti holds a Ph.D from the Harvard University Graduate School of Arts and Sciences in health policy and economics, and is an internationally recognized expert in the fields of health policy, economics and the biopharmaceutical industry. She served as Special Government Advisor to federal CMS in 2022 to 2023 and has held additional advisory roles at the FDA.

- **TaLana Hughes** is the Executive Director of the Sickle Cell Disease Association of Illinois (SCDAI) where she started as an intern while completing her last year at Northern Illinois University, where she obtained her Bachelor's Degree in

Public Health. Upon graduating, Hughes became the Case Manager and Sickle Cell Educator for the Newborn Screening Program, educating and counseling parents of infants who have tested positive for Sickle Cell Disease or Sickle Cell Trait. She has worked at SCDAI for more than 20 years, and obtained her Master's of Public Health degree from Walden University, becoming the SCDAI executive director in 2010. Hughes is the mother of a daughter with Sickle Cell Disease and a daughter and son with Sickle Cell Trait.

- **Dr. Radhika Peddinti** is an Associate Professor of Pediatrics/Pediatric Hematology/Oncology at the University of Chicago. Dr. Peddinti's focus is on an evidence-based approach to clinical care of children with Sickle Cell Disease at La Rabida and Comer Children's Hospital, one of the largest pediatric SCD centers in Illinois. Dr. Peddinti works closely with the Adult Sickle Cell Disease Program on care transition and standardizing care, with an emphasis on pain management in the emergency and inpatient settings.

- **Mark Trusheim** is Strategic Director, NEWDIGS at Tufts Medical Center where he also co-leads the Financing and reimbursement of Cures in the US (FoCUS) Project. Mark's research focuses on the economics of biomedical innovation, especially precision financing for patient access, precision medicine, adaptive pathways, platform trials, biosimilars, and digital health advances. Mark held appointments for 18 years at MIT Sloan in Applied Economics and has served as a Special Government Employee for the FDA's Office of the Commissioner. He is also President of Co-Bio Consulting, LLC. His career has spanned policy as the President of the Massachusetts Biotechnology Council, diagnostics as founder of Cantata Labs, genomics as President of Cereon Genomics, eHealth as Vice President of Monsanto Health Solutions, managed care marketing at Searle Pharmaceuticals, and big data at Kenan Systems.

- **Joan Ehrhardt**, MS, CGC joined the Illinois Department of Public Health (IDPH) as the Newborn Screening Genetic Counselor in the fall of 2020. Joan has prior clinical experience in reproductive and pediatric genetics. In her current role, she helps manage the IDPH Newborn Screening (NBS) Programs, and the NBS and Genetics Grant Programs. These include newborn screening for sickle cell disease and support of improved access to care for individuals through the Sickle Cell Follow Up Grant Program and development and implementation of the Sickle Cell Disease Prevention Care and Treatment Grant Program.

- **Theodore Tapas** is a Budget Analyst II at the Governor's Office of Management and Budget, working with state Health and Human Services agencies for the past three years. He is a 2020 graduate of Mount Mercy University, with a double

major in Finance and Human Resources and a minor in Economics. He currently lives in Springfield, Illinois, and with an accomplished background with servant leadership, Theodore enjoys finding ways to give back to the community by empowering others.

- **Adam Flores** is the Senior Insurance Advisor at the Illinois Department of Insurance (DOI) where he helps lead DOI's policy analysis, development, and implementation. Adam has worked in a variety of legislative and policy roles for the State of Illinois and City of Chicago. He is an alumnus of Washington University in St. Louis and The University of Chicago Harris School of Public Policy.

The Advisory Council will hold its next meeting in July.