Advisory Council

Advance Financing & Access to Sickle Cell Disease Treatment & Other High-Cost Drugs & Treatment in Illinois

June 25, 2024

HFS
Illinois Department of
Healthcare and Family Services

HFS
Illinois Department of Healthcare and Family Services

OUR VISION FOR THE FUTURE

We improve lives.

- We address social and structural determinants of health.
- We empower customers to maximize their health and well being.
- We provide consistent, responsive service to our colleagues and customers.
- We make equity the foundation of everything we do.

This is possible because:

We value our staff as our greatest asset.

We do this by:

Fully staffing a diverse workforce whose skills and experiences strengthen HFS.

Ensuring all staff and systems work together.

Maintaining a positive workplace where strong teams contribute, grow and stay.

Providing exceptional training programs that develop and support all employees.

We are always improving.

We do this by:

Having specific and measurable goals and using analytics to improve outcomes.

Using technology and interagency collaboration to maximize efficiency and impact.

Learning from successes and failures.

We inspire public confidence.

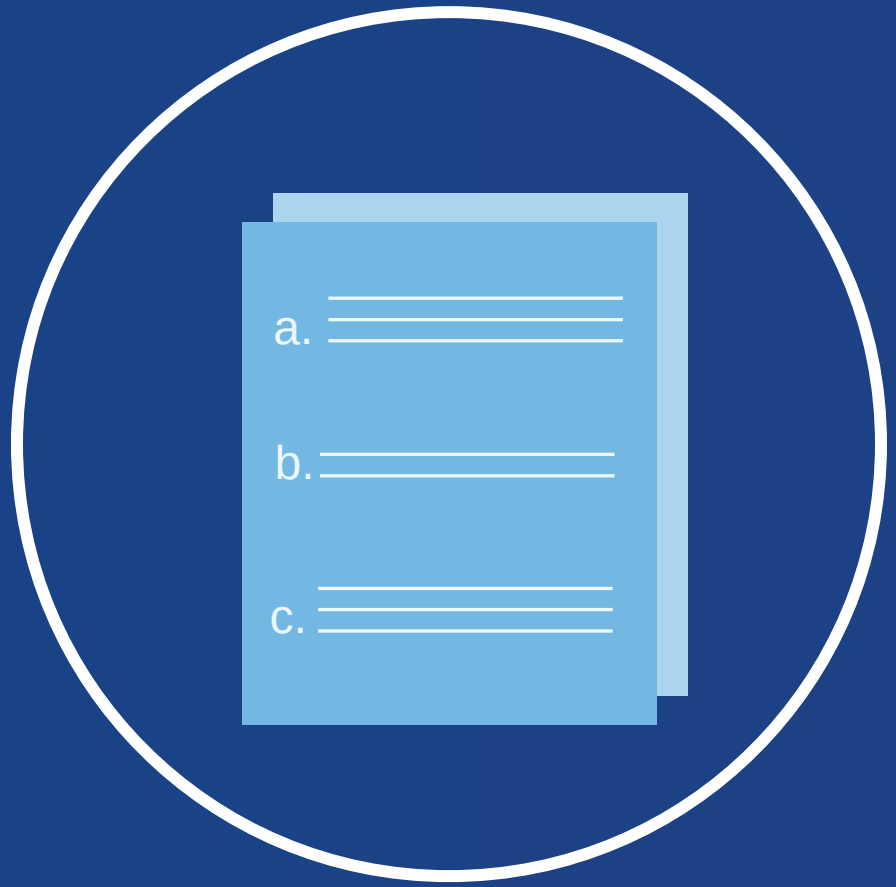
We do this by:

Using research and analytics to drive policy and shape legislative initiatives.

Clearly communicating the impacts of our work.

Being responsible stewards of public resources.

Staying focused on our goals.



Agenda

Presenter: Director Whitehorn

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Summary of Agenda

Presenter:
Lizzy Whitehorn, Director

I. Call to Order
II. House Keeping
III. Roll Call and Introduction of Council Members
IV. Introduction of HFS staff
V. Review and Approval of Bylaws
VI. Review of Advisory Council Meeting Framework
VII. Financing and Access Challenges Overview
VIII. Illinois Medicaid Overview
IX. Brainstorming Discussion
X. Next Steps
XI. Public Comments
XII. Additional Business
XIII. Announcements
Adjournment

II.

House Keeping

Presenter: Melishia Bansa

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Comments or questions during the meeting

House Keeping

- Please note, this meeting is being recorded.
- To ensure accurate records, please type your name and organization into the chat.
- If possible, members are asked to attend meetings with their camera's turned on, however, if you call in & need materials, please email Melishia.Bansa@Illinois.gov as soon as safely possible.
- Please be sure to mute your audio except when speaking.
- Please note that HFS staff may mute participants to minimize any type of disruptive noise or feedback.

Meeting Basics

House Keeping

- If you are an Advisory Council member and wish to make a comment or ask a question during the meeting, please use the WebEx feature to raise your hand, contact the host/co-host, or unmute yourself during QA sections facilitated by chair.
- Please state your full name when asking a question or passing a motion.
- If you are a member of the general public and wish to make a comment, please register to make a public comment prior to the meeting. Instructions to make public comments have been provided for you in the public meeting posting located on the HFS Boards and Commissions website.
- If you have a question during the meeting, please utilize the Webex chat feature to send your question directly to the host or co-host.

Meeting Basics

House Keeping

- HFS is committed to hosting meetings that are accessible and ADA compliant. Closed captioning will be provided. Please email Melishia.Bansa@Illinois.gov in advance to report any requests or accommodations you may require or use the chat to alert me of challenges you may have encountered during the meeting.
- Patience, please – many meeting attendees may be new to these advisory council proceedings.
- After today's meeting, meeting minutes will be circulated to Committee members in advance of the next meeting. Once approved, they will be posted to the website along with today's presentation deck.

III.

Roll Call and Introduction of Council Members

Presenter: Melishia Bansa

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Advisory Council Representation

Lizzy Whitehorn
Illinois Department of Healthcare and Family Services (state Medicaid agency)

Joan Ehrhardt
Illinois Department of Public Health

Adam Flores
Illinois Department of Insurance

Theodore (Ted) Tapas
Governor's Office of Management and Budget

Beverly Chukwudozie
Persons with lived experience as a person with sickle cell disease

Alexandra Carpenter
Persons with lived experience as a person with a condition requiring access to new, innovative drugs or treatment

Dr. James LaBelle
Providers treating sickle cell disease patients

TaLana Hughes
Sickle cell disease advocacy organization

Nathan Schaefer
Advocacy organization for a condition(s) requiring access to new, innovative drugs and treatment

Steve Sproat,
Brian Smolich
Health insurance industry

George Kitchens,
Susan Stuard
Experts in prescription drug rebate negotiations and outcomes-based agreements

Melissa Creary,
Rachel Sachs
Experts with published research in financing new, innovative drugs and treatments within public health insurance programs

Dr. Anirban Basu,
Rena Conti
Experts with health economist or actuarial backgrounds

Dr. Radhika Peddinti,
Mark Trusheim
Members who may or may not meet the qualification requirements for the other appointees.

Dr. Ruchika Goel
Providers treating patients with another condition requiring access to new, innovative drugs or treatment

IV.

Introduction of Staff

Presenter: Melishia Bansa

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V.

Review and Approve Bylaws

Presenter: Melishia Bansa

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Bylaws: Review and Vote

- Bylaws have been disseminated electronically to each member
- Reading of the Bylaws
- Discussion
- Vote

VI.

Meeting Framework

Presenter: Director Whitehorn

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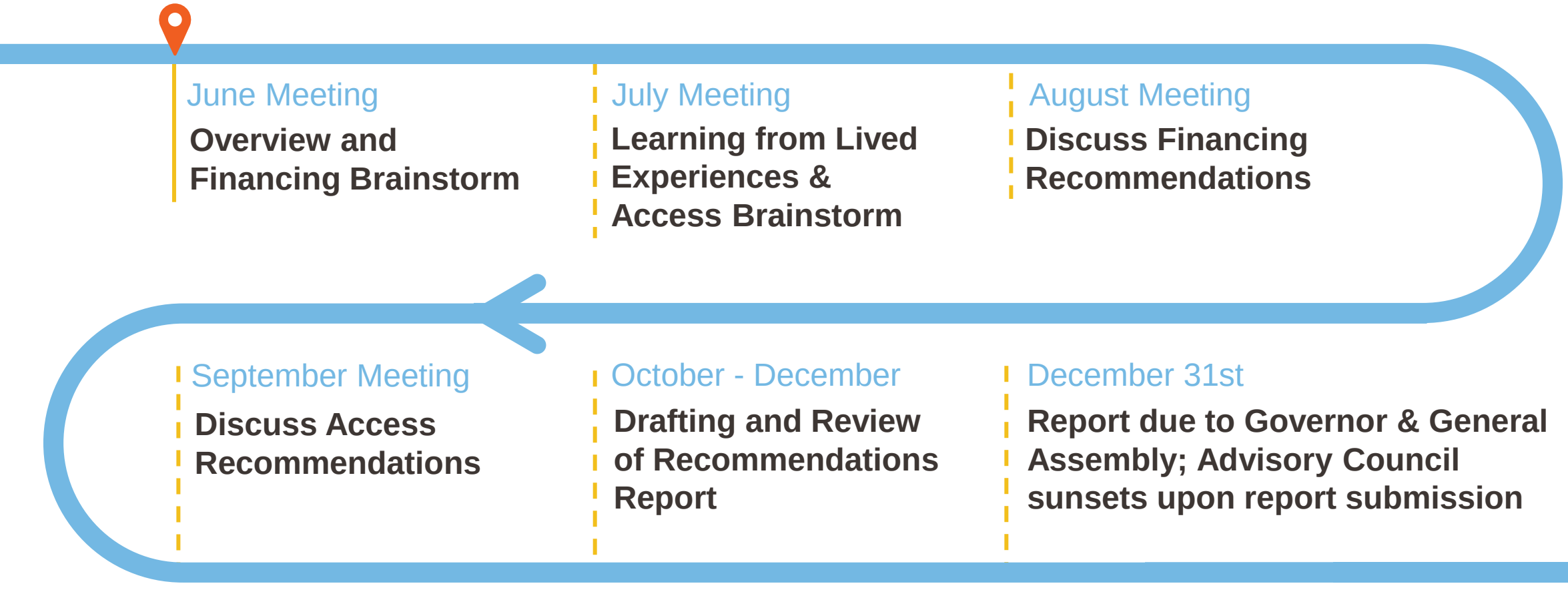
Advisory Council Meeting Framework



VII.

Access & Financing Challenges

Presenter: Director Whitehorn

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Sickle Cell Disease (SCD) and Other High-Cost Drugs

Access & Financing Challenges

Over 40% of Illinoisans with SCD receive care through Medicaid.

49% of Medicaid customers with SCD are estimated to have a severe form of the disease.

- People living with SCD 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 impacts on communities of color.
- December 8, 2023, the FDA approved two gene therapies for the treatment of Sickle Cell Disease (SCD)
- The cost of one-time gene therapy is $2-3 million.
- The cost of managing sickle cell disease over a lifetime is $1.2-$2.1 million.
- For the most severely impacted people, the lifetime costs are as high as $4 to $6 million.

Access & Financing Challenges for High-Cost Drugs

- Pipeline for cell and gene therapies is expanding rapidly, with new therapies projected to be approved each year.
- Major focus of cell and gene therapy is pediatrics; Medicaid will be a major payor
- These therapies are very expensive, creating concerns about:
  - Equitable access, especially for historically underserved populations
  - Health insurance restrictions
  - Potential budgeting challenges for public health insurance programs

Access & Financing Challenges for High-Cost Drugs

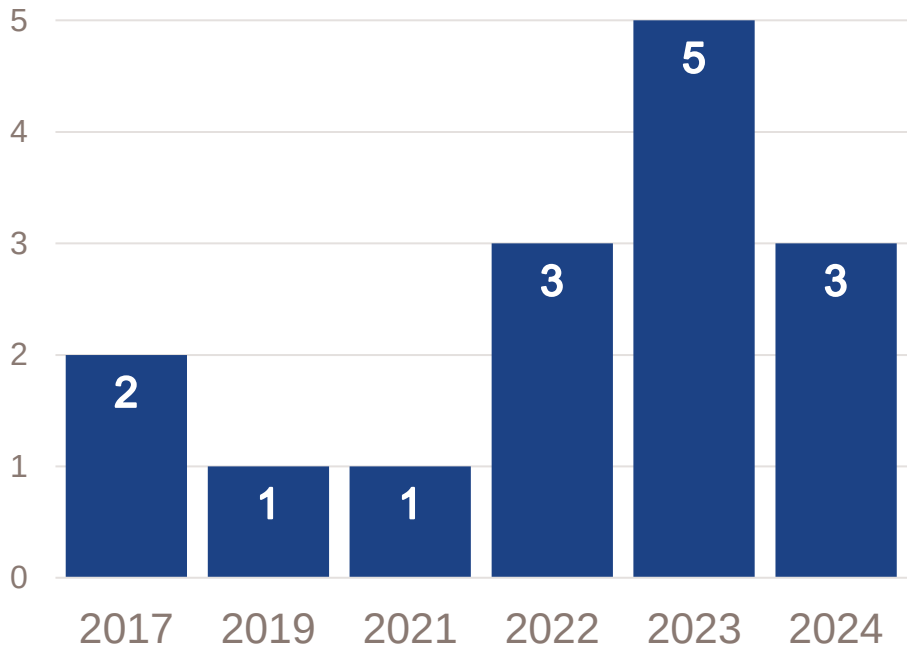
- The FDA accelerated approval pathway bring unique challenges for payors:
  - Approval based on surrogate endpoints rather than outcomes
  - Additional post-marketing studies required to prove efficacy
  - Possibility of immediate clinical benefits that are not durable long-term.
  - Given high cost, question of how to receive rebates back if results are not ultimately durable
  - Person may have different health insurance over time, complicating tying long-term outcomes back to the original payor.

Access & Financing Challenges for High-Cost Drugs

- Medicaid prescription spending trends are increasingly driven by high-cost drugs.
- From 2018 to 2021, the average cost of a new drug increased almost 50%, reflecting new cell and gene therapies and other high-cost specialty drugs.
- The federal Medicaid Drug Rebate Program provides federal rebates to states based on the manufacturer's lowest or best price, but essentially requires states to cover all FDA-approved drugs to access the rebates.
- New cell and gene therapies and other high-cost specialty drugs create uncertainty in state budgeting.
  - Uncertainty around how many individuals might seek treatment each year.
  - The cost for one patient can have a significant impact on the Medicaid budget.

High-Cost Drug Trends for One-Time Treatments

Count of New High-Cost Drugs by Approval Year



- The numbers reference one-time drugs with an estimated cost > $500K
- Estimated annual treatment costs range from $457K to $4.25M for each drug
  - Estimated member counts for one time gene therapies range from 0-4 to 5-10 depending on the therapy indication

High-Cost Drug Trends for One-Time Treatments

Recent drug approvals in 2023-24 (or expected in 2024) treat:

FDA Approved:

- Sickle cell disease, transfusion-dependent β-thalassemia (TDT) (Casgevy)
- Duchenne muscular dystrophy, Ambulatory patients, ages 4-5 years (Elevidys)
- Metachromatic leukodystrophy (MLD) (Lenmeldy)
- Sickle cell disease (Lyfgenia)
- Hemophilia A (Roctavian)
- Hemophilia B (Beqvez)

Scheduled for FDA Review in 2024:

- Aromatic l-amino acid decarboxylase deficiency (Upstaza)
- Severe leukocyte adhesion deficiency (Kresladi)
- Duchenne muscular dystrophy, all ages and ambulatory status (Elevidys)

Access & Financing Challenges

The U.S. Department of Health and Human Services and the Centers for Medicare & Medicaid Services (CMS) Innovation Center is launching a new Cell and Gene Therapy (CGT) Access Model.

CMS will negotiate and administer outcomes-based agreements with drug manufacturers on behalf of participating states, including savings tied to a patient's clinical outcomes.

States can join the CGT Access Model as early as January 1, 2025.

Currently focused on sickle cell disease treatment.

- Other cell and gene therapies may be added to the model in the future.
- Other high cost drugs that are not cell and gene therapies are not included.

Access & Financing Challenges

Opportunity to develop an innovative and sustainable solution to make new drugs affordable and available to Illinoisans.

Value solutions centered around everyone having access to quality and affordable healthcare.

VIII.

Current State in Illinois Medicaid

Presenter: Kelly Cunningham

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Current State: Illinois Medicaid Overview

- The state's Drugs and Therapeutics (D&T) Advisory Board advises HFS on Illinois Medicaid's prior approval and Preferred Drug List (PDL) policies.
  - Prior to a drug going before the D&T Board, coverage decisions are made on a case-by-case basis using FDA label and/or the study inclusion protocol.
- Illinois negotiates state supplemental rebates for Medicaid drugs; these are in addition to the federal rebates
  - Illinois does not participate in a multi-state supplemental rebate negotiating consortium
- Have not negotiated an outcomes-based agreement with a drug manufacturer yet
  - CMS recently approved an Illinois Medicaid State Plan Amendment (SPA) allowing the state to negotiate outcomes-based agreements

Current State: Illinois Medicaid Overview

Work Underway

Carving high-cost drugs out of hospital DRG payments

- Allows high-cost drugs to be billed separately
- Required for CGT Access Model participation
- Enables the leveraging of rebates from drug manufacturers

Medicaid managed care high-cost drug pool effective 2025

- Risk pool spreads the cost of high-cost drugs more equitably across the MCOs
- Does not change the reimbursement model or financing structure for high-cost drugs

Note: Risk pools definition of high-cost drugs does not need to align with Advisory Council's

IX.

Brainstorming Discussion

Presenter: Director Whitehorn

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Contracting Strategy Definitions

- **Supplemental Rebates:** Voluntary rebates negotiated between the State and a drug manufacturer over and above the federal statutory rebate. Often supplemental rebates are used to leverage Medicaid Preferred Drug List (PDL) status.
- **Outcomes-Based Agreements:** Performance-based risk-sharing agreements in which agreed upon outcomes data are used to link a drug's price to its real-world efficacy.
- **Annuity Model Outcomes-Based Agreements:** Designed to reimburse the drug over time in installments as set milestones are reached, usually over years.
- **Subscription Model Outcomes-Based Agreements:** Defined by a negotiated fixed amount paid to the manufacturer in exchange for access to the specific drug for a specific patient population over a predetermined period of time.

Brainstorming

options for financing new cell & gene therapies and other high-cost drugs and treatment

Potential topics for consideration:

- When to use or combine different contracting strategies:
  - Supplemental rebates, outcomes-based agreements, annuity or subscription models, etc.
  - High-cost one-time vs. maintenance drugs
- How to overcome implementation challenges:
  - Outcomes-based payment when health insurer changes over time
  - State budgeting and cash flow issues
- When/if to participate in federal models
- Any other innovative ideas

X.

Next Steps

Presenter: Director Whitehorn

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For Future Meetings:

- Research or examples from other states to share?
  - HFS can compile submissions and share with Advisory Council.
- Any data we should pursue?
- Additional information about Illinois needed?

Advisory Council: Looking Ahead

July Meeting

- Focused on lived experiences, access challenges, and brainstorming innovative access solutions.

August Meeting

- Will discuss financing recommendations.
- Can send HFS ideas in advance for inclusion in meeting three slide deck.

September Meeting

- Will discuss access recommendations.
- Can send HFS ideas in advance for inclusion in meeting four slide deck.

XI.

Public Comments

Presenter: Melishia Bansa

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Public Comments

Name	Title	Org	Comment
CHAD Duncan, PharmD	Field HEOR Associate Director	Health Economics and Outcomes Research	

XII.

Additional Business

Presenter: Director Whitehorn

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Announcements

Presenter: Melishia Bansa

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Advisory Council Operational Reminders

- Advisory Council Meeting Resources: Boards and Commissions | HFS (illinois.gov)
  - Executive Order
  - Membership
  - Meeting Notice: Public Meeting Postings & Agendas
  - Meeting Schedule
  - Presentation Decks
  - Bylaws
- Bios & Headshots
- Mandatory Trainings

XIII.

Adjournment

Presenter: Director Whitehorn

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