

# Analysis of the Committee's Evidence-based Review Process

## Summary of the Expert Advisory Panel Meeting

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Presented to the Advisory Committee on Heritable Disorders in Newborns and Children

March 22, 2019

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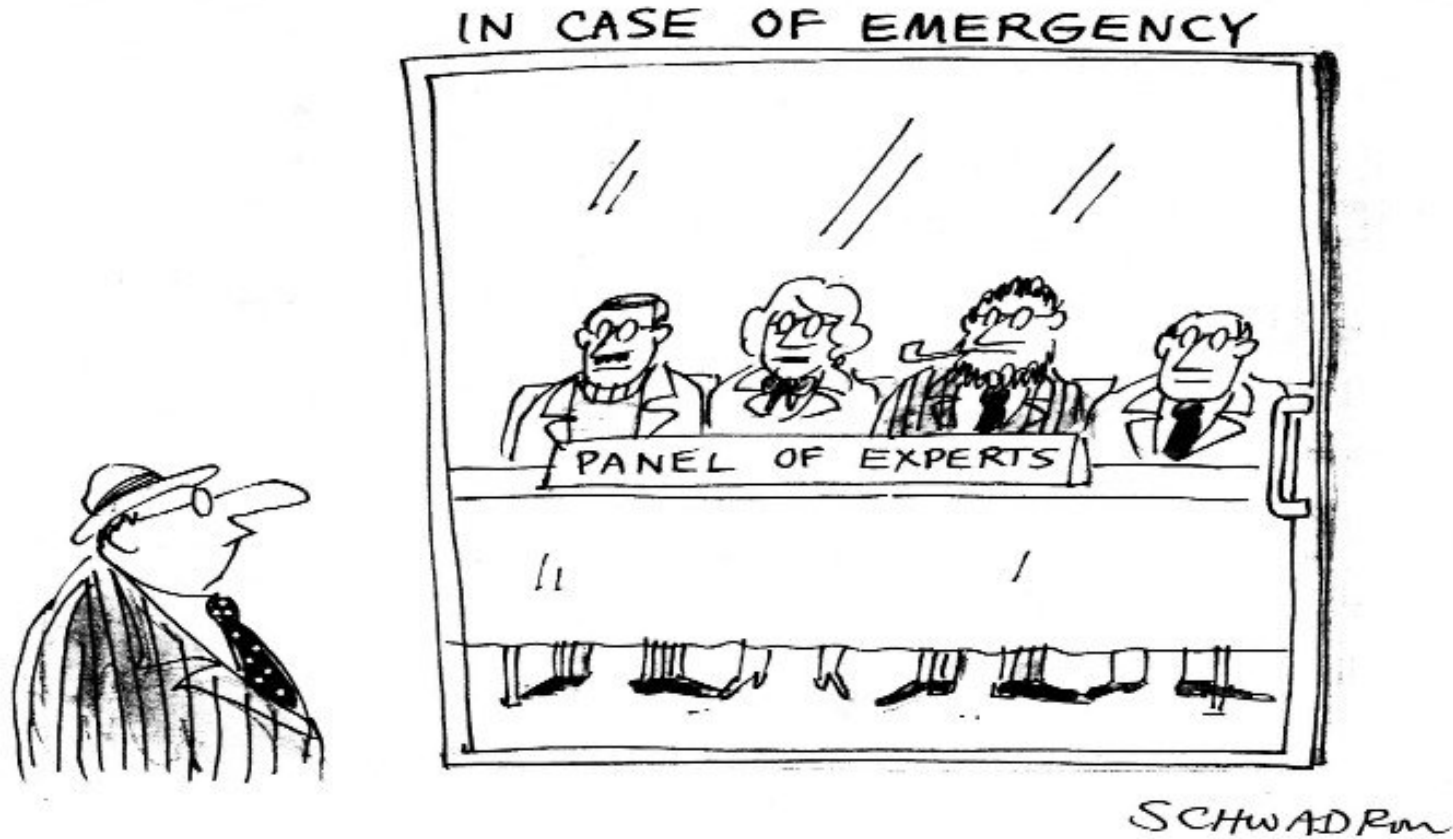
# Project Aims

- Gather information to learn how to improve the processes related to:
  - Nomination
  - Evidence-Review Process
  - Decision Making
  - Review of the RUSP

# Approach and Project Timeline

Task	Target Dates
Convene Steering Committee	Throughout
Expert Advisory Panel Meeting	February 5-6, 2019
Report Outline	April 1, 2019
Refine Recommendations <ul style="list-style-type: none"><li>• Discussions with Advisory Committee and stakeholders</li><li>• Analysis of other evidence-based bodies</li></ul>	
Advisory Committee Presentations	April 23-24, 2019 August 1-2, 2019 November 7-8, 2019
Report Drafts	July 1, 2019 December 16, 2019
Final Report	March 1, 2020

# Expert Advisory Panel (EAP)



# EAP Composition

HRSA

Federal Agencies

Advisory Committee

Evidence Review  
Group

State Screening  
Programs

Other Physicians,  
Advocates,  
Evidence-Based  
Medicine Experts,  
etc.

# EAP Meeting Participants

## State Screening Programs

- Stanton Berberich, PhD
- Michele Caggana, ScD, FACMG
- John Thompson, PhD, MPH, MPA

## HRSA

- Alaina Harris, MSW, MPH\*
- Catharine Riley, PhD, MPH\*
- Debi Sarkar, MPH\*
- Joan Scott, MS, CGC\*
- Michael Warren, MD, MPH

## Advisory Committee

- Mei Baker, MD
- Joseph A. Bocchini Jr., MD\*
- Jeffrey P. Brosco, MD, PhD\*
- Cynthia Powell, MD, MS

## Evidence Review Group

- Scott D. Grosse, PhD
- Alex R. Kemper, MD, MPH, MS\*#
- K.K. Lam, PhD\*
- Ashley Lennox, PhD\*
- Jelili Ojodu, MPH
- Lisa A. Prosser, PhD, MS

\*steering committee member

#meeting moderator

## Federal Agencies

- Carla Cuthbert, PhD (CDC)
- Kellie Kelm, PhD (FDA)
- Kamila Mistry, PhD, MPH\* (AHRQ)
- Melissa Parisi, MD, PhD (NIH)

## Others

- Donald B. Bailey, Jr., PhD, Med
- Nedrow Calonge, MD, MPH\*
- Pranesh Chakraborty, MD
- Frederick Chen, MD, MPH David Grossman, MD, MPH\*\*
- John Lantos, MD
- Jana Monaco
- Holger Schünemann, MD, PhD, MSc Guy Van Vliet, MD
- Debra Waldron, MD, MPH
- Michael Watson, PhD, MS
- John Thompson, PhD, MPH, MPA

# EAP Agenda and Discussion Topics

- Introduction to Committee Procedures
- Grading of Recommendations Assessment, Development, and Evaluation (GRADE)– Holger Schünemann
- Assessing Published Evidence
- Assessing Unpublished Evidence
- Assessing Public Health System Impact
- Determining Value from Different Perspectives
- Revisiting the Decision Matrix
- Reconsideration of Conditions on the RUSP
- Revisiting the Nomination Process
- Developing an Research & Development Agenda

# EAP Suggestions





# Nomination

- Strengthen the nomination before the evidence review
  - Increase transparency of nomination process
  - Ask nominators to identify critical outcomes
- Defining the intended target of screening
  - Clarifying primary target, secondary targets, and incidental findings
- Consider a “scoping” review before full evidence review
  - Assess availability of evidence and critical gaps
  - Determine what this type of review would involve

# Evidence Review

- *A priori* specification of critical outcomes
  - Predefine list of core outcomes for all conditions
  - Consider critical outcomes from different perspectives
- Systematic incorporation of expert-derived evidence
  - Collect unpublished information using a formal written document to assist with completeness, transparency, and potentially assessing risk of bias and quality
- Long-Term Follow-up
  - Describe LTFU plans and framework for assessing the future quality of LTFU

# Evidence Review

- Assess values from different perspectives
  - Family Perspectives
  - Public Perspectives

# Decision Matrix

- Include values and preferences
- Consider
  - Resource implication for states
  - Panels instead of condition-by-condition approach
  - Provisional or conditional recommendation

# Updating or Reevaluating RUSP Conditions

- Post-RUSP surveillance system
  - Collect new data regarding epidemiology, net benefit, costs, long-term follow-up effectiveness
- Routinely re-assess conditions on the RUSP

# Next Steps

- Refine suggestions and related methods in partnership with the Advisory Committee
- Develop a manual of procedures