#### The Decision Matrix

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

December 1, 2020

Alex R. Kemper, MD, MPH, MS

K.K. Lam, PhD





### Objectives

Review the development and use of the Decision Matrix









Decision-making process for conditions nominated to the Recommended Uniform Screening Panel: statement of the US Department of Health and Human Services Secretary's Advisory Committee on Heritable Disorders in Newborns and Children

Alex R. Kemper, MD, MPH¹, Nancy S. Green, MD², Ned Calonge, MD, MPH³, Wendy K.K. Lam, PhD¹, Anne M. Comeau, PhD⁴, Aaron J. Goldenberg, PhD, MPH⁵, Jelili Ojodu, MPH⁶, Lisa A. Prosser, PhD², Susan Tanksley, PhD³ and Joseph A. Bocchini Jr, MD³





## **Decision Matrix**

NET BENEFIT/ CERTAINTY		7	READINESS				ec a escularen		
			Roady Developmental Unprepared		FEASIBILITY				
SIGNIFICANT Benefit	Certainty	HSH	A1 Screening for the condition has a high certainty of significant net benefits, screening has high or moderate feasibility. Most public health departments are ready to screen.	A2 Screening for the condition has a high certainty of significant net benefits and screening has high or moderate feasibility. Public health departments have only developmental readiness.	A3 Screening for the condition has a high certainty of significant net benefits and screening has high or moderate feasibility. Public health departments are un prepared for screening.	Feasibility	HIGH or MODERATE		
			A4  There is high certainty that screening would have a significant benefit; however, most health departments have low feasibility of implementing population screening.			MOT			
		MOD	B 1-4 There is moderate certainty that screening would have a significant benefit.			I I	-		
Small to ZERO Benefit	HIGH		нісн	нісн	There is high or moderate certain a small to zero net benefit.	C 1-4 nty that adoption of screening for the t	argeted condition would have		16
NEG Benefit	Certainty	ноп/ном	D 1-4  There is high or moderate certainty that adoption of screening for the targeted condition would have a negative net benefit.				13		
1		TOW	L 1-4  There is low certainty regarding the potential net benefit from screening.				1		





## **Key Points**

There will always be uncertainty

NET BENEFIT/ CERTAINTY		7	READINESS				FEASIBILITY	
			Ready Developmental Unprepared		FEASIBILITY			
SIGNIFICANT Benefit	Certainty	HIGH	A1 Screening for the condition has a high certainty of significant net benefits, screening has high or moderate feasibility, Most public health departments are ready to screen.	AZ Screening for the condition has a high certainty of significant net benefits and screening has high or moderate feasibility. Public health departments have only developmental readiness.	Screening for the condition has a high certainty of significant net benefits and screening has high or moderate feasibility. Public health departments are unprepared for screening.	Feasibility	HIGH or MODERATE	
				A4 ning would have a significant benefit; t of implementing population screening			том	
		MOD	B 1-4  There is moderate certainty that screening would have a significant benefit.				T de la	
to ZERO Benefit		мор/нісн	C 1-4  There is high or moderate certainty that adoption of screening for the targeted condition would have a small to zero net benefit.				æ	
NEG Benefit	Certainty	/dow	D 1-4 There is high or moderate certainty that adoption of screening for the targeted condition would have a negative net benefit.					
1		TOW	L1-4 There is low certainty regarding the potential net benefit from screening.					





#### **Key Points**

• "...a B rating of the evidence indicates moderate certainty that screening would lead to a significant net benefit. The term moderate...indicates that the Advisory Committee believes that further research could change the magnitude or direction of findings...such that the assessment of net benefit would be

There is high or moderate certainty that screening would have a significant benefit.

| Column | Colum



small to zero or even negative."



#### Recommendations based on the Matrix

- A1 or A2 recommended to the RUSP
- A3 or A4 at the discretion of the Advisory Committee
- B, C, D, or L not recommended to the RUSP; the Advisory Committee will provide guidance regarding research needs

NET BEHEFIT/ CERTAINTY			READINESS					
			ficady Developmental Unprepared		FEASIBILITY			
SIGNIFICANT Benefit	Certainty	HIGH	A1 Screening for the condition has a high certainty of significant net benefits, screening has high or moderate feasibility. Most public health departments are ready to screen.	AZ Screening for the condition has a high certainty of significant net benefits and screening has high or moderate feasibility. Public health departments have only developmental readiness.	A3 Screening for the condition has a high certainty of significant net benefits and screening has high or moderate feasibility. Public health departments are unprepared for screening.	Feasibility	HIGH or MODERATE	
SIGNIFIC	ce		AA  There is high certainty that screening would have a significant benefit; however, most health departments have low feasibility of implementing population screening.			том		
	ľ	MOD	There is moderate certainty that	B 1-4 screening would have a significant ben	efit.			
Small to ZERO Benefit		мор/нісн	C 1-4  There is high or moderate certainty that adoption of screening for the targeted condition would have a small to zero net benefit.					
NEG	Certainty	/dow	D 1-4  There is high or moderate certainty that adoption of screening for the targeted condition would have a negative net benefit.			18		
1		MOT	L1-4 There is low certainty regarding the potential set benefit from screening.			ì	100	





# Decision Matrix and Subsequent Recommendation

Date of Vote	Condition	Matrix	Recommendation (Y:N:Recused)		
Feb 2018*	SMA	B2	Recommend to RUSP (8:5:2)		
Aug 2015*	X-ALD	A2	Recommend to RUSP (12:1:1)		
Feb 2015	MPS I	В3	Recommend to RUSP (11:3:0)		
May 2013*	Pompe	NA	Recommend to RUSP (11:2:0)		
Sept 2010*	CCHD	NA	Recommend to RUSP with Qualifications (13:1:0)		
Jan 2010*	SCID	NA	Recommend to RUSP (*count not recorded)		
*2 <sup>nd</sup> nomination and/or AC vote					





#### Refining the Decision Matrix

- Refining processes is normal and expected
- Next a discussion, led by Dr. Powell





