

Assessing the Impact of Policy Levers in Addressing Unmet Medical Needs

Amber Jessup, PhD

Senior Economist

Office of the Assistant Secretary for Planning
and Evaluation

US Department of Health and Human Services

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- The views expressed are those of the presenter and not necessarily those of the Office of the Assistant Secretary for Planning and Evaluation, the Department of Health and Human Services.



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Medical Product Development

- Interest in approaches to foster innovation and reduce barriers to medical product development, while containing rising health care costs
- Number of studies
 - Antibacterial development
 - Costs of clinical trials
 - Modeling drug developers decision making process
 - Assessing the potential impact of policy levers in addressing unmet medical needs



Assessing the Potential Impact of Policy Levers in Addressing Unmet Medical Needs

- Work with Lewin Group, Inc
- Unmet need
 - Lack of suitable or practical options to prevent, treat, or manage a specific condition
 - Many of these conditions are rare, but in total millions of individuals have clinically significant unmet medical needs



Methodology

- Framework to identify areas of unmet needs
- Developed an algorithm to identify areas of pressing unmet medical need
 - the magnitude of the unmet need, i.e., the degree of need for effective pharmaceutical treatment for condition, and
 - the condition's burden on society, patients, and caregivers and its contribution to poor health.
- Limited to conditions suitable for drug therapy
- Excluded conditions already a significant focus of federal policy



Methodology (2)

- Identified list of barriers to development and potential policy levers
- Review of literature specific to conditions and to drug development
- Expert interviews
- Four expert panels with experts from government, academia, patient advocacy, and industry



Five Conditions

- Acute ischemic stroke
- Diabetic neovascular diseases (diabetic retinopathy and neovascular glaucoma)
- Huntington's disease
- Muscular dystrophy
- Pancreatic cancer



Barrier

	Acute ischemic stroke	Diabetic neovascular diseases	Huntington's disease	Muscular dystrophy	Pancreatic cancer
<i>Scientific</i>					
1. Lack of understanding of disease history and pathophysiology	X	X	X	X	X
2. Need for clinically meaningful biomarkers	X	X			X
3. Treatment complexity due to heterogeneity of disease	X			X	X
4. Need for clinically meaningful endpoints		X		X	
5. High clinical trial costs due to slow disease progression		X	X		
6. Lack of necessary tools and models	X				X
7. Lack of disease registries			X	X	
<i>Economic</i>					
1. Low disease incidence/market potential	X		X		X
2. Limited clinical research funding		X		X	X
3. Underutilization of current treatments	X				
4. Lack of understanding of long-term disease costs			X		
<i>Regulatory</i>					
1. Limited regulator guidance on clinical trial endpoints		X	X	X	X



Levers to Stimulate Development

- Prioritize funding for technical assistance and identification of disease biomarkers
- Support the development of disease registries for rare conditions that are unmet medical needs
- Develop and support consortia that link experts and organizations pursuing basic scientific research



Levers to Stimulate Development (2)

- Investigate use of prizes for overcoming specific research gaps
- Extensions of market exclusivity in exchange for R&D in areas of unmet need



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