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Decisions, decisions: Using research translation to inform health policy options amid uncertainty

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Sound public health policymaking depends on a solid evidence base to frame options and alternatives around critical decisions that can potentially affect millions of people. But when the available evidence is scarce, and the outcomes of various scenarios appear murky, how can decision-makers fairly evaluate policy options and their consequences?

Increasingly in the United States, the insights of decision analysis and economic evaluation are providing essential findings that directly contribute to health policy decision-making. These methods provide a systematic and transparent approach for synthesizing available evidence and providing projected estimates of health and economic outcomes, even when data are limited. These analyses consider factors such as potential lives saved, quality of life, and costs, providing a clear and quantifiable outlook on the likely outcomes of pursuing (or not pursuing) a particular course of action, applied across entire populations of people.

Over the last two decades, **Lisa Prosser** and her team have conducted these types of studies to inform major health policy development at the national level. This brief describes Prosser's more recent work in evaluating the effectiveness and value of new health interventions within two important areas of health services: immunization and newborn screening. Her research has a direct impact on the decisions that shape the health and healthcare of millions of Americans.

Answering complex questions around immunization

Shingles is a painful condition that one in three people will develop in their lifetime, typically as older adults.

In October 2017, the federal committee that establishes nationwide vaccine policy voted to recommend a newly licensed shingles vaccine for nearly all adults aged 50 years and older–a full decade earlier than the recommendation based on the existing vaccine.

The panel–the Centers for Disease Control and Prevention's Advisory Committee on Immunization Practices, or ACIP–also voted to endorse the new vaccine, Shingrix, over the existing one, Zostavax. In another rare preferential decision, it recommended that people who were already vaccinated with the existing vaccine get revaccinated with the new one.

Prosser's analyses of the new shingles vaccine directly informed each of these key policy decisions, by using cost-effectiveness simulation modeling to consider the value of the vaccines in terms of the health benefits they produce (defined in quality-adjusted years of life added) relative to their costs.

Her team's research demonstrated that the new vaccine would be decisively more effective than the existing vaccine, which helped the committee decide among its policy options.

Prosser led the studies as a member of the ACIP's Zoster Work Group, whose charge is to identify the key policy questions around shingles vaccine and consider options for recommendations by reviewing the body of evidence.

Cost-effectiveness analysis is a required part of the evidence considered for every vaccine recommendation in the U.S., but these types of studies are not used routinely in other areas of regulatory decision-making for health.

Prosser and her team have worked collaboratively with the CDC to conduct vaccine policy studies since 2001. Her economic analyses have also been used to expand recommendations for influenza vaccination, and to prioritize groups for influenza immunization in vaccine shortage years.

Evidence-Based Healthcare

> ## "I've been collaborating with CDC for almost two decades now,
> and it's exciting and gratifying to be a part of one of the few areas of regulatory decision-making in the country that uses the type of research we produce."

Informing healthcare decisions in the earliest days of life

Every year in the U.S., four million newborns routinely undergo a simple blood test to detect a variety of serious disorders that could affect their long-term health and survival. Newborn screening has proven to be one of the most successful public health programs in this country, providing the means for early diagnosis, intervention, and treatment that can prevent death and disability.

Since 2003, the federal Advisory Committee for Heritable Disorders in Newborns and Children, or ACHDNC, has established the standard set of conditions included in newborn screening, known as the recommended uniform screening panel (RUSP).

The committee recommends adding conditions to the panel if evidence demonstrates that the benefits of screening and the availability of early treatment would outweigh the harms. Most states adopt new panel recommendations into their screening programs within 1–2 years after the ACHDNC's endorsement.

However, decisions about expanding the conditions included on the screening panel are complicated. Because these disorders are often extremely rare, the data about them are too.

This is where decision modeling comes in. This approach provides critical information that allows the committee to consider the projected benefits and harms of screening the entire newborn population in the U.S. for a condition, compared to usual clinical identification (meaning the diagnosis and treatment that would be expected to occur without universal screening). Decision analysis leverages various forms of available evidence, even some that may

be considered "substandard" in other settings, which is especially useful for evaluating rare diseases and those with new or emerging treatments.

Since the committee incorporated decision modeling as a standard component of its evidence review process in 2011, Prosser's team has used this methodology to evaluate five candidate conditions brought before the ACHDNC, four of which have been added to the panel. Prosser serves as a member of the committee's condition review workgroup and developed the methodology used to estimate population level outcomes.

Before decision analysis was added to the review process, several conditions had been nominated for the screening panel but insufficient data prevented them from being fully considered.

A sustained impact on health policy development

Prosser's team continues to collaborate with CDC on studies related to pneumococcal and shingles vaccine policies. Her team's analyses also remain an integral part of the newborn screening condition review process: in February 2018, the ACHDNA added spinal muscular atrophy (SMA) to the screening panel, a decision based in part on the simulation modeling evidence that Prosser and colleagues presented, which projected population-level outcomes expected from universal screening for the condition.

The application of these analyses has been instrumental in the development of national policy recommendations across a range of crucial preventive health services in the U.S. Prosser's work continues to have a direct impact on key policy decisions that affect the health of people across the country.

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