Evidence of Impact in Biomedical Research

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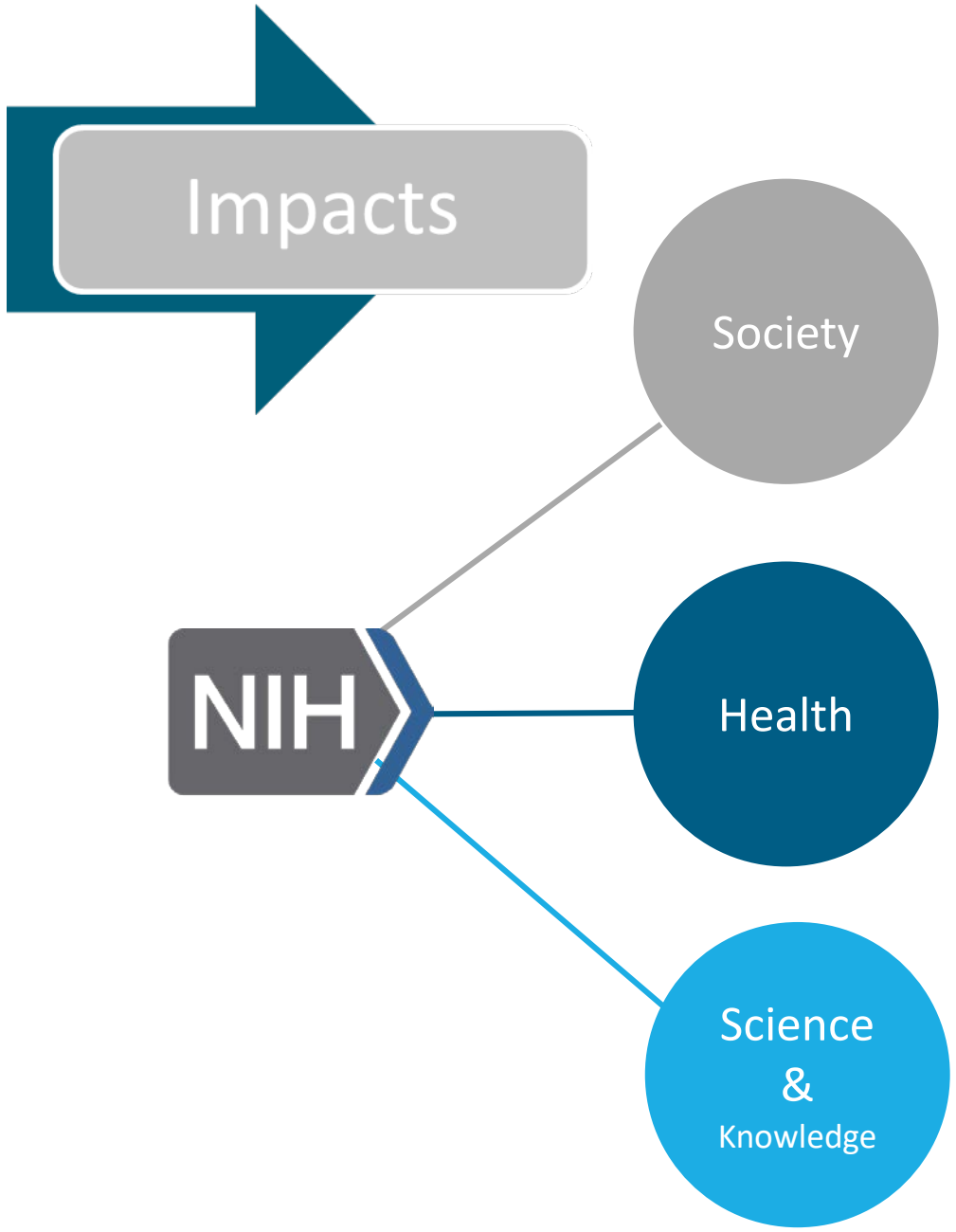
Some Major Challenges to Assessing Impact

- Easier to measure generation of knowledge (e.g. publications), but far more difficult to connect this knowledge to long-range impacts on public health
- NIH produces scientific evidence to improve public health, but is not responsible for its implementation
- NIH is not the only funder of biomedical research
- Timelines may be incredibly long, and value may change with time
  - The time lag that occurs between discovery and implementation;
  - One finding may have implications for numerous outcomes;
- Limitations of existing data.



- Cost savings from improved interventions and health outcomes
- Industry/commercial activity from medical products and technologies
- New businesses/start-ups created

- Number of people treated
- Lives saved
- Quality of life improvements
- Influence on follow-on FDA approvals, practice guidelines, health policy and services, etc.

- Growth/emergence of new fields
- “Spillovers” to other lines of research
- Increased methodological and technological capabilities
- Award-winning work (Nobels, Laskers, *Science’s* top discoveries)

RESEARCH-TO-PRACTICE MILESTONES FOR THE Hib VACCINE

For more information on the supporting evidence and research sponsors for these milestones, see the Web appendix.

FOUNDATIONAL RESEARCH

1920s

By deactivating disease-causing components of bacteria, vaccines were developed to prevent **tetanus and diphtheria.**[14] These advances would later help enhance the effectiveness of Hib vaccines.

1930s

Hib (*seen below right, in photo*) was found to be the primary cause of bacterial meningitis in children. The bacteria could be distinguished under the microscope by their **unique outer coat.**[15] Later, scientists determined this coat was made up of chains of specific **sugar molecules.**[16]

Because sugar chains typically do not stimulate immune responses as well as proteins, researchers began to look for ways to **link the chains to bacterial proteins** in order to enhance their immunogenicity.[17]

EARLY HIB VACCINE DEVELOPMENT

1968

The **first attempts at developing a Hib vaccine** focused on the sugar chains – researchers worked on isolating, purifying, and preparing these chains for clinical use.[18] NIH

1970s

NIH-funded clinical trials found that these early **Hib vaccines worked well for children** over age 2, but were not as effective in infants.[19] NIH

1980

FDA scientists (who later moved to NIH) linked Hib sugar chains to immune-stimulating proteins (e.g., from diphtheria), producing a **"conjugate vaccine."**[23] NIH

1983

Scientists leading these clinical trials **founded a company,** called Praxis Biologics (now incorporated into Wyeth), to develop and produce vaccines for children, including Hib vaccines.[20]

1984

NIH and FDA scientists found that this conjugate vaccine triggered immune responses in appropriate **animal models.**[24] NIH

1985

The Food and Drug Administration (FDA) **approved the first Hib vaccines** (including from Praxis Biologics) for use in children two years of age,[21] while researchers explored ways to make the vaccines more effective in infants.[22]

1977: A young girl receives a vaccination, administered by a public health nursing practitioner

PROTECTING INFANTS WITH A NEXT-GENERATION CONJUGATE VACCINE

1985-1989

NIH-funded clinical trials showed that the conjugate vaccine was **safe and effective in infants.**[25] NIH

1987-1993

FDA approved the first **4 conjugate vaccines for use in infants.**[25]

1993

The conjugate vaccine developed by NIH-funded scientists was licensed, manufactured, and **commercially distributed.**[27]

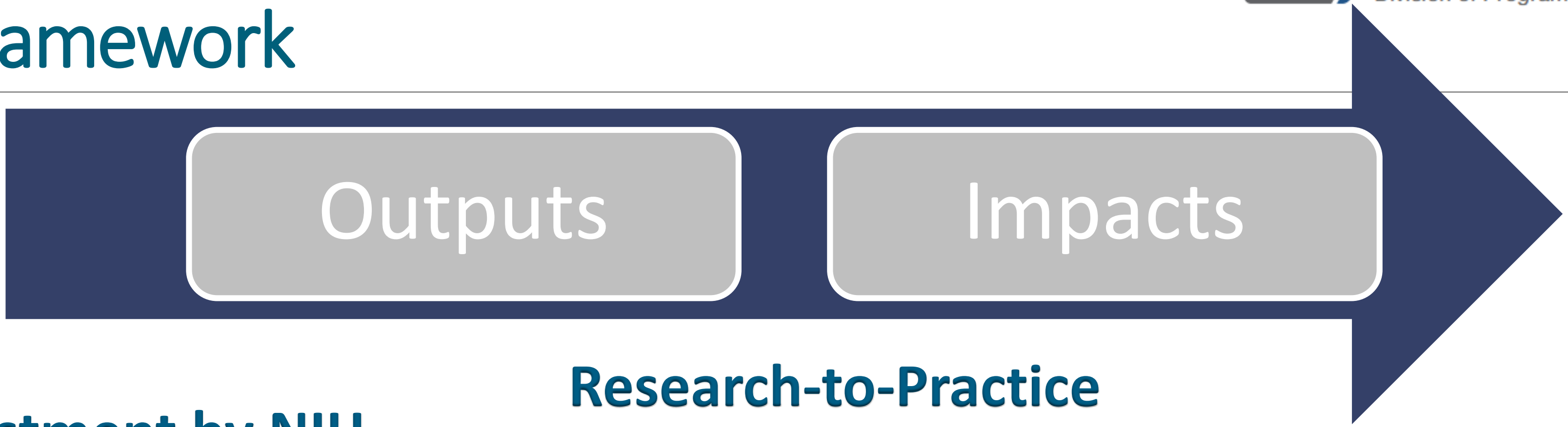
CDC included the Hib conjugate vaccine in its **recommended vaccine schedule.**[28]

1995

For their "groundbreaking work and …leadership in the development and commercialization of the [Hib] vaccine," the **Albert and Mary Lasker Foundation** awarded their prestigious Clinical Medical Research Award (often referred to as the "American Nobels") to four NIH-supported scientists.[29] **NIH's National Institute of Allergy and Infectious Diseases** provided grant funding to Drs. Porter Anderson and David Smith. Drs. Rachel Schneerson and John Robbins performed the majority of their research within the intramural laboratories of the ***Eunice Kennedy Shriver*** **National Institute on Child Health and Human Development.**

NIH *Indicates NIH-funded milestones*

Impact Framework



Investment by NIH & Others

- Identification of public health need and scientific opportunity
- Research initiatives
- **Funding acknowledgments**
- Funding amount (when feasible)

Research-to-Practice Milestones → Timeline

- Publications (basic to applied)
- Patents (role of USPTO)
- Private industry development
- Regulatory activities (e.g., FDA approvals)
- Uptake into programs and services of other HHS agencies
- Adoption into practice (e.g., inclusion in clinical practice guidelines)
- Evidence of “hand-off” to health and medical practice

Organized by Stream of Impact

- Health
- Knowledge
- Society

HHS Data Needs

- Access to comprehensive, structured data with improved ability to search and export:
  - FDA approved drugs, biologics, and devices
  - AHRQ clinical guidelines and evidence reports
  - CMS healthcare utilization data (payments, number of prescriptions)
  - CDC (and other agency-generated) health statistics