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Duke-Margolis Center: Overview And High Priority Projects in Biomedical Innovation and Payment

Gregory Daniel, PhD, MPH

Deputy Director, Duke-Margolis Center for Health Policy

Clinical Professor, Fuqua School of Business

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Mission

Improve health and the value of health care by developing and implementing evidence-based policy solutions locally, nationally, and globally.

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Who We Are

- Duke University's Robert J. Margolis, M.D., Center for Health Policy was established in January 2016

- The Center brings together capabilities that generate and analyze evidence across the spectrum of policy to practice, supporting the triple aim of health care – improving the experience of care, the health of populations, and reducing the per capita cost

- Our team is based within the Fuqua School of Business, with offices in both Durham and Washington, D.C.

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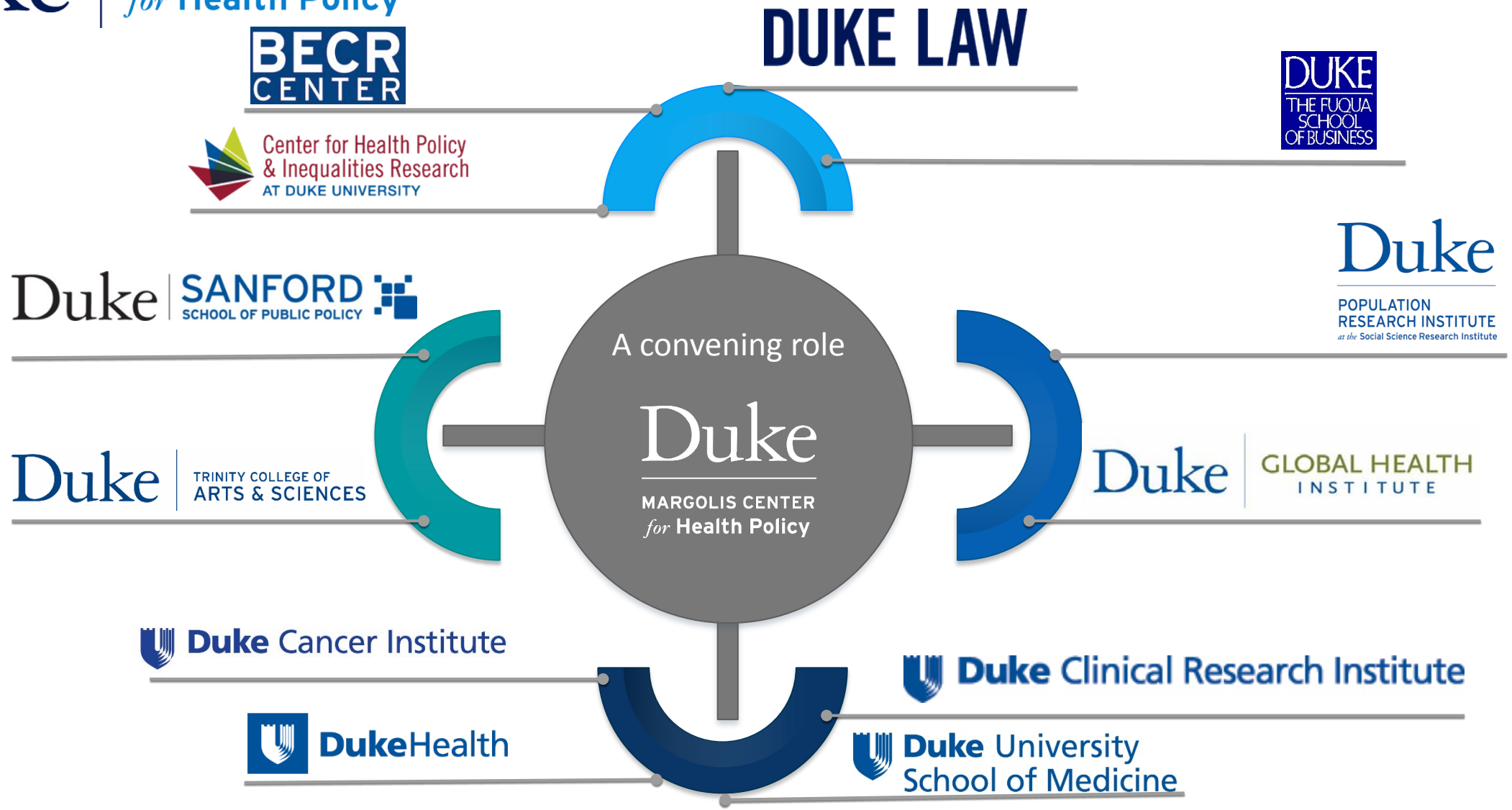

Research and Implementation Areas

- **Healthcare Delivery Reform**
  - Developing evidence to inform and evaluate healthcare financing models
  - Expanding and implementing better models of care and clinical leadership
  - Boosting efforts for international health reform
- **Biomedical Innovation Policy and Evidence Development**
  - Supporting innovative and efficient medical technology development and regulation
  - Leading value-based policy development by linking medical technologies to value
  - Advancing biomedical evidence development through use of Real World Evidence (RWE) and electronic health information
- **Education and Workforce Development**
  - Enriching undergraduate and graduate education
  - Encouraging collaborative research opportunities
  - Enhancing virtual, executive, and corporate education

Health Care Delivery Reform

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Developing and Delivering New Models of Care: Opportunities

- **Alternative Care Delivery Models for Cancer Survivors**
  - Encourage care collaboration between primary care physicians and oncologists to facilitate the treatment of patients, particularly pediatric patients, who are in remission
  - Patients no longer require primary care from their oncologists, but do require special considerations as they live with the after-effects of treatment
- **Expansion of Community Based Palliative Care Model to Cancer Patients**
  - This care delivery model features interdisciplinary collaboration and the integration of palliative care into the health care system, continuity of care across transitions, and longitudinal, individualized support for patients and families.
  - With this model, care coordination will ensure clinical follow-up of cancer patients as they transition across settings
- **The Payment Reform Evidence Hub**
  - Utilize the evidence hub to evaluate new oncology care and payment delivery models

Biomedical Innovation Policy and Evidence Development

Current Project Areas

Regulatory Policy for Biomedical Products

- Enhancing the Sentinel System as a national evidence development resource
- Promoting the safe use of medical products
- Expediting the development and review of promising therapies
- Advancing the science of drug discovery and development
- Facilitating the adoption of safe and efficient manufacturing practices
- Supporting regulatory uses of real-world evidence

Current Project Areas, cont.

Linking Biomedical Products to Value

- Developing economic incentives for antibacterial drug development
- Establishing value-based reimbursement for high-cost drugs (e.g. specialty drugs, chronic, and curative treatments)
- Developing a framework for incorporating medical products into alternative payment models (e.g. medical devices)

Biomedical Innovation Policy Evidence Hub

- Build a Duke "hub" by leveraging existing state-of-the-art infrastructure or by developing new tools as needed
  - Identify appropriate financial models to ensure sustainability
  - Design a governance model to lead infrastructure optimization
  - Identify and align on high priorities areas to be tested
- Expanded utilization of the Sentinel Initiative data infrastructure (e.g. HCV, PCSK-9 inhibitors)

Supporting Regulatory Uses of RWE

What is Driving the Demand for Real-World Evidence?

- The nation's growing electronic health information infrastructure has enabled routine and increasingly robust collection of digital data at the point of patient care
- Opportunities to leverage such data will only grow:
  - Drug discovery and development is longer and more costly, with growing public attention on resultant prices
  - Providers and payers are moving toward payment and reimbursement models focused on value over volume
  - Patients are more involved than ever before in their own care decisions and the push for more personalized treatments
- Learning from real-world patient experiences can support better informed health care decision-making by a range of stakeholders

Policymakers, FDA, and Industry Are Focused on Real World Evidence

- Congressional action
  - House: 21st Century Cures Act would require FDA to further explore the use of RWE in regulatory decision making and establish opportunities for Sentinel extensions
  - Senate: May eventually merge with House legislation, but no RWE focus at this time
- FDA Commissioner Califf recently identified RWE and the building of a national evidence development system as a “top programmatic priority”
- Prescription Drug User Fee Act VI (vote expected in 2017)
  - Draft commitment letter includes provisions similar to those in 21st Century Cures
  - Will require FDA to
    - host multiple RWE-related public events and workshops
    - Issue guidance on the enhanced use of RWE in regulatory decision making at the end of five years
    - Pilot potential applications to test feasibility and close data, methods gap
  - Also includes goals related to advancing model-informed drug development, innovative trial designs, and Sentinel expansions

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Duke-Margolis and FDA Are Engaged in Work to Get the Science and Policy Right

- Stream of activities focused on exploring development and use of RWE in the regulatory context
- Assembled a planning group of expert advisors to lay out key questions, forthcoming papers, and continued public meetings
- **Goal:** To strengthen the development of credible RWD, rigorous methods, and trusted applications of the evidence to improve product development and patient care

RWE Priorities Areas

Moving Beyond "One-Size-Fits-All"

- The umbrella definition of RWE is imprecise and potentially confusing
- Need to better map data and evidentiary needs to specific contexts of intended use

Data Development

- Challenges with underlying real world data (RWD)
- Standardized outcomes or tailored endpoints may be needed for improved monitoring and data capture

Harnessing RWD to Support Trials

- Where can RWD support or enhance traditional clinical trials?
- Opportunities to use RWD to increase the efficiency of safety data collection and reporting

Randomization in the Clinical Setting

- Blended study designs (e.g., pragmatic clinical trials) could use randomization in real-world settings to generate more representative outcomes data
- Methods and study designs still need to mature

Increasing Credibility Of Observational Studies

- Best practices developed by groups like ISPOR will help generate useful data for payers/providers, but need to look at suitability for regulatory applications
- Demonstration projects needed

Value-Based Drug Payment and Utilization

Increasing the Value of Health Care Delivery

- As health care expenditures rise, more emphasis is on value and quality
- Providers and payers implementing alternative payment models (APMs)
  - Shift from volume and intensity to patient-level payments that enable more flexibility in how services are provided but create new financial accountability for providers
  - Provide support for care coordination and innovative care delivery
- Challenging for health care providers
  - Steep learning curves in shifting to new payment structures, requiring new patient care capabilities and capacities to bear financial risk
  - Increasingly complex drug and device pipeline with high prices and traditional FFS payments that aren't aligned with shift

Path to Value-Based Reimbursement for Drugs and Devices

- New payment arrangements are needed for drugs, aligned with the shift to risk sharing and better value
- Including drugs and devices can include a number of approaches:
  - Value-based contracting and risk sharing agreements (RSAs)
  - PMPM or bundles for devices and physician-administered drugs and devices
  - Broader uses of value-frameworks by providers and payers
- APMs could enable shared accountability with manufacturers, not just providers
  - Most APMs today do not include a direct role for manufacturers in accountability for results and overall costs (even though manufacturers have considerable resources, expertise relevant to effective use of drugs and the capacity to share risk)
  - Some emerging RSAs between manufacturers and payers, but typically not combined with APMs for providers

Duke-Margolis Efforts on Value-Based Drug Payment and Utilization

- Build a path to value by developing medical product payment models or other approaches that ensure:
  - Incentives for a vibrant and diverse medical product pipeline
  - Better alignment of incentives to improve outcomes and value
  - Moving away from traditional fee-for-service (FFS) models that promote volume
- Identify and support better approaches to defining value, and measuring it more efficiently in the post-market setting

Duke-Margolis Approach

Advancing the Science of Patient Input

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Advancing the Science of Patient Input

Today

- The emergence of ‘patient-focused’ or ‘patient-driven’ initiatives has highlighted the need for rigorous, systematic approaches to gathering patient input and measuring the effectiveness and ultimate impact of that engagement process.
- Over the last five years, FDA has strived to develop a more systematic approach to obtaining the patient perspective and has emphasized this again in its PDUFA VI Commitment Letter.

Tomorrow

- COAs measure a patient’s symptoms, overall mental state, or the effects of a disease or condition on how the patient functions, and can be used to determine treatment benefit.
- Over the next several years, FDA will develop a series of guidance documents aimed at defining evidentiary standards for these measures

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Duke-Margolis Engagement on Advancing the Science of Patient Input

- Convened two workshops that focused on enhancing the development and use of patient-reported outcomes (PROs) in drug development
  - Workshop conducted in July 2014: focused on the major issues related to the development and use of PRO instruments to support labeling claims
  - Workshop conducted in October 2014: sought specific input on the value and uses of a potentially acceptable endpoints (PAE) list, using case examples to highlight the benefits and challenges associated with developing such a list.
- One outcome of these workshops was the Clinical Outcomes Assessment compendium, which serves as key source of information on outcome assessment tools that the agency considers to be potentially acceptable

Upcoming Efforts: Patient-Focused Drug Development

- 2016: Workshop will explore conceptual and methodological approaches to determining whether a performance outcome assessment (PerfO) designed to assess cognitive function or physical function is fit-for-purpose.
- 2017: Series of workshops to gather input on evidentiary and methodological standards for regulatory uses of COAs with particular emphasis on:
  - Identifying challenges for industry collection and use of patient preference data
  - Exploring approaches to engage patient groups and the clinical community
  - Defining potential methods and research strategies to advance the incorporation of patient preferences in regulatory decision-making
  - Duke-Margolis: Workshop and paper(s) regarding methods for inclusion of patient generated data in Benefit/Risk (B/R) frameworks

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Thank You

Any Questions?