

NEWDIGS: New Drug Development Parad**IGmS**



Applying MIT systems expertise to transform healthcare innovation





The NEWDIGS "Scenario Design" Methodology for Case-Based Collaborative Learning

Scenario Design Methodology

Asset Nominations and Fit Assessment



Design Session Preparation



Scenario Design Session



Consolidation & Summary



Numerous proprietary assets reviewed since 2011:

- 14 assets nominated by 9 companies
- 13 assets evaluated in scenario design sessions

Publications focused on:

- Overview of AL (multi-stakeholder co-author team)
- Modeling of stakeholder metrics in AL
- · A (hypothetical) case study of AL
- Legal foundations of AL
- In-depth description of scenario design methodology
- Alignment of regulator/payer perspectives in AL
- AL in TB drug/regimen development
- Global update/accelerated regulator & payer pathways
- From adaptive licensing to adaptive pathways

EMA Pilot Project on AL:

"In March 2014, the EMA began inviting companies to participate in a pilot project on adaptive licensing, and published a framework to guide discussions on individual pilot studies.

... The pilot project <u>builds on earlier work with the Massachusetts Institute of Technology's</u>
<u>Center for Biomedical Innovation (CBI)</u> to investigate the feasibility of this approach." *

^{*}www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000601.jsp&mid=WC0b01ac05807d58ce#non



Scenario Designs V2.0: Enabling the Implementation of MAPPs

Scenario Designs V2.0 (2014 ---)

Janus Program

- Process + open access integrated tool set
- Quantitative modeling/simulation
- Scalable



Strategic Coordination

Data Program

 Evaluating & advancing "readiness" of data systems, study designs, & analytic methods to support life span approaches to reducing uncertainty (safety, efficacy, & effectiveness)

Scenario Designs V1.0





Janus Program: Integrates Major Viewpoints Across Life Span to Explore Meaningful Impact for all Stakeholders

- Will I get better?
- Will I get access to new drugs faster?
- What are the risks?
- What are net health benefits?Social benefits?

Patient / Public Health

- Time & cost to market
- Patient access
- Evidence requirements
- Financial returns and risk

Payer

Open
Access
Integrated
Tool Set

Sponsor

- How will it affect aggregate& pharmacy cost?
- What clinical utility evidence exists?
- What are risks of over use?

Regulator

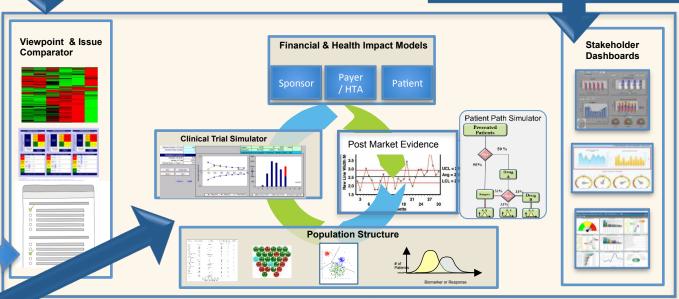
- Evidence requirements for Risk & Benefit
- Prioritization and staging of evidence generation across the product lifecycle and among regulators
- Developing an adequate safety database

Center for Biomedical Innovation

Janus Program: Quantified, Connected Stories.... Supported by Intuitive Visualization Tools.... Designed to Foster Creative Consensus

Visually Compare Stakeholder Perspectives & Risk Assessment

Multiple outputs: Evidence & risk, financial, patient, health as viewed by each stakeholder

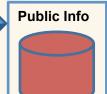


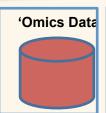
Stakeholders



Connect

Evidence To Actions





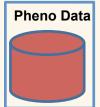




Parameters











NEWDIGS Data Program Focuses on Convergent Change Drivers & Their Implications for the Disruptive Advancement of Health Data Access, Use, & Value

Advancement of "precision medicine"

From

To

Blockbuster



Small populations, tailored treatments

Accelerating access to new & better treatments

Binary pre/post market generation



Lifecycle-based approaches to reducing uncertainty about safety, efficacy & effectiveness

Requirements from payers/HTAs for evidence of value

RCT only



Integrated use of RCTs & real world data to bridge efficacy-effectiveness gap

Maturation of collaborative innovation models & culture

Data silos

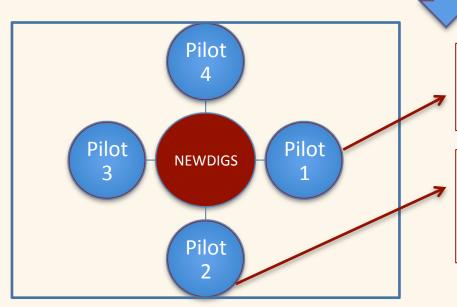


Data sharing to generate actionable knowledge

Sample (Hypothetical) Activity in NEWDIGS Data Program

Issue Identified: As innovation increasingly targets small sub-populations of patients, there will be an unprecedented need for international cooperation in the design & use of data systems in order to identify adequate numbers of patients to safely accelerate access & optimize value.

Scenario Design Session focused on uncertainty reduction/management plan for Initial Authorization phase of a targeted therapeutic involving multiple jurisdictions



Pilot 1: Evaluation of feasibility & methods for scaling of US Sentinel System for **safety** surveillance

Pilot 2: Evaluation of feasibility & methods for scaling of US Sentinel System for assessment of effectiveness





