NEWDIGS

LEAPS

Learning Ecosystems Accelerator for Patient-centered, Sustainable innovation

PROJECT DESCRIPTION

January 2019

The Dynamic Dossier in the Cloud

THE CHALLENGE/OPPORTUNITY

Emerging science brings tremendous hope for long awaited treatments – even cures – from biomedical innovation. But, in terms of the growing administrative and operational burden associated with the ensuring the safety, efficacy, and quality of growing number of therapeutic products, we are becoming victims of our own success!

In 1997, the FDA made long-term investments to modernize its information technology through the Prescription Drug User Fee Act (PDUFA) II. Fast-forward to the present, the FDA has made substantial progress in improving the drug review process from paper-based submissions to electronic submission with data standards. FDA submissions that totaled 50,000 in 2006 grew to over 4 million in 2017.

As the volume of products in the R&D pipeline and on-market have grown substantially, so too have costs, complexity, and need for evolutionary advancements in innovation processes. For example, the emergence of precision medicines trials with smaller study populations is driving greater emphasis on post market studies to reduce uncertainties related to the heterogeneity of effect in real world use. Cell and gene therapies are associated with greater uncertainties in safety, efficacy, and quality, and require new, tailored approaches to innovation. Patient-generated data has exciting potential for improving product development, access, and use, but must be structured for meaningful integration into analysis and decision-making. And, finally, the submission process itself involves of series of steps that introduce operational inefficiencies for both industry sponsors and the FDA, as well as data-related time lags that undermine patient-centered decision quality. The need to modernize the current process of electronic submissions from industry sponsor to the FDA is clear.

As these challenges are playing out at the interface between biopharmaceutical companies and the FDA, many of these same organizations are now embarking internally on the digital transformation of their data storage into cloud-based environments. It is important that these transformational roadmaps are informed by the regulatory process innovations that are evolving in parallel, now, and in the future.

THE SOLUTION: DYNAMIC DOSSIER IN THE CLOUD

The Dynamic Dossier in the Cloud Project provides a valuable opportunity for prospectively coordinating inter-dependent process, technology, and policy innovations across stakeholder siloes (regulatory and developer), within a neutral, safe haven environment. In addition, the collaborative design approach involves front-end consideration of potential future transactional transformations with other stakeholders (e.g., payers, providers, and patients).

"A large global biopharma like Sanofi with 5,000 products on the market may be required to make as many as 50,000 regulatory submissions per year. The current practice for submission and approval is woefully inadequate. An AI/machine learning-based design space' approach as envisioned by Dynamic Dossier in the Cloud, would be more far more effective and efficient."

- Hilary Malone, Chief Regulatory Officer and Head, Global Regulatory Affairs, Sanofi

CONCEPT

Dynamic Dossier in the Cloud represents a vision for a radical new platform built on the principles of data sharing and collaboration while protecting proprietary information and patient privacy. It envisions a world where regulators can access emerging data on a therapeutic across the product lifecycle (i.e., safety, efficacy, and/or quality) in real time, within a shared dynamic, cloud-based environment. It will enhance the ability of sponsors and regulators to use and integrate new sources of data like Real World Data, leverage the full potential of new technologies like blockchain and machine learning, and improve decision making with transparency and accountability.

The integrated data tracking across pre- and post-marketing, and the use of advanced technologies process innovations, such as:

- *Cloud-based Environment:* greater efficiencies and accuracy from common, shared data
- *Blockchain:* inter-operability; secure access; auditing and time stamp trails
- *Integrated application of AI and Machine Learning:* Management of "smart contracts" with pre-defined parameters of acceptable outcomes; deviations trigger alerts

APPROACH

The Dynamic Dossier in the Cloud will be advanced within the safe haven, pre-competitive collaboration environment of NEWDIGS, the the MIT Center for Biomedical Innovation serving as the neutral intermediary. Design activities will leverage proven NEWDIGS tools/methods for multi-stakeholder collaborative system innovation and MIT expertise in data science, digital health technologies, and systems engineering, while the MIT-Harvard Center for Regulatory Science provides expertise in biomedical informatics, initial funding, and an avenue for FDA interaction.

Dynamic Dossier in the Cloud is the first "Incubator" project of LEAPS, and as such, will benefit from the expertise of members of the LEAPS Strategic Advisory Network, as well as Senior Advisors to the Harvard-MIT Center for Regulatory Science.

TIMELINE/DELIVERABLES

First year deliverables to be completed by February 2020 will focus on stakeholder-specific functional requirements and a roadmap to prototyping and pilots.

About the MIT NEWDIGS Initiative:
MIT NEW Drug Development ParadIGmS (NEWDIGS) is an international "think and do tank" dedicated to delivering more value faster to patients, in ways that work for all stakeholders. NEWDIGS designs, evaluates, and initiates advancements that are too complex and cross-cutting to be addressed by a single organization or market sector. Its members include global leaders from patient advocacy, payer organizations, biopharmaceutical companies, regulatory agencies, clinical care, academic research, and investment firms. For more information, visit http://newdigs.mit.edu.

About LEAPS
The LEAPS Project (Learning Ecosystems Accelerator for Patient-centered, Sustainable innovation) is advancing the mission of the MIT NEWDIGS consortium – to deliver more value from biomedical innovation faster to patients, in ways that work for all stakeholders – through a new collaborative systems approach to the planning, generation, and use of evidence across R&D and healthcare delivery. A model system for Rheumatoid Arthritis will be piloted in Massachusetts (2020 launch), and will inform related efforts in other diseases and geographies. Success in LEAPS targets better patient outcomes while also reducing waste and inefficiency across the system.

About the LEAPS Incubator
The LEAPS Incubator is a safe haven design "space" for early stage, potentially transformative digital health innovations that are likely to deliver greater value to the system if initial prototyping involves multi-stakeholder input. Selection criteria for inclusion in the incubator requires the interest of 3 or more stakeholders and the resources of 3 or more sponsors.

Join LEAPS!

Success in the LEAPS Project requires hands-on participation by diverse partners. To learn more about how to participate, please contact us.

More information on NEWDIGS projects can be found at: https://newdigs.mit.edu

CONTACT

Eric A. Small
NEWDIGS
MIT Center for Biomedical Innovation
phone: 617-324-4841
email: easmall@mit.edu

MIT CBI
Center for Biomedical Innovation

NEWDIGS