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AI-Powered Pre-Competitive Disease and Care Modeling: Building a Reference Disease-Centric Framework for Value-Based Decision Making

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RWD141

INTRODUCTION

- Randomized Clinical Trials (RCTs) generate limited evidence to payer: poorly generalizable in real-world (RW) and limited to short term horizons
- Yet the use of RW data has become instrumental in drugs development and evaluation processes, notably empowered by RW data sources more accessible and interoperable
- Proper integrative methods to combine RCT and RW data have been used and increasingly accepted by health agencies
- Disease and care models have been developed in ad hoc pilots or research projects, calling for a need to systematize and platformize such disease-centric integration to build reference tools

OBJECTIVES

The purpose of this work is to propose a regulatory-grade, data-integrative, real-world-value-based and disease-centric approach to accelerate and de-risk drugs development and launch.

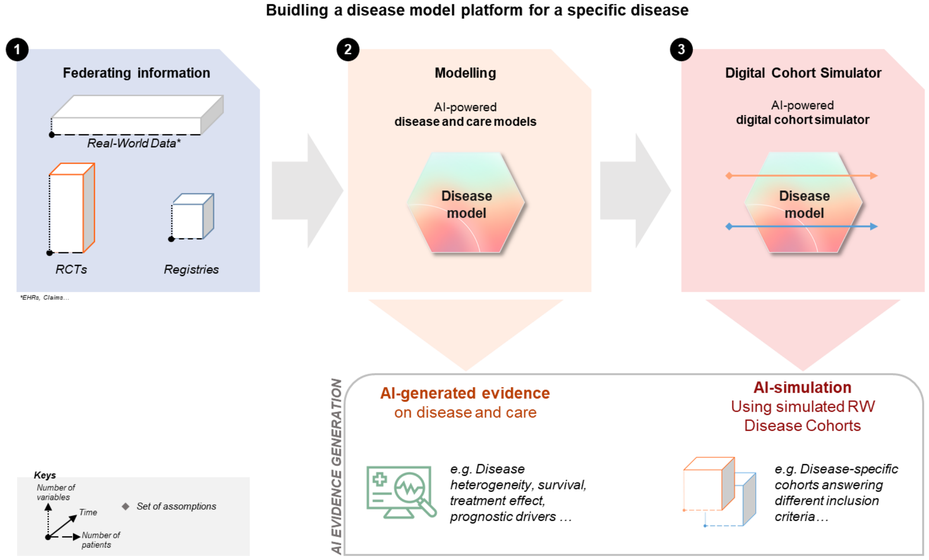
The aim is to develop an operational and technical framework for disease and care modeling and a simulation tool to accelerate patient access to personalized treatment and de-risk drug development

METHODS

- Leveraging from decades of experience in disease progression in RW and care modeling and from increasingly accessible RW data platforms and networks, we propose a patented framework, a methodology and a suite of published algorithms to transform evidence generation.
- A generic process is proposed to simulate RW disease progression jointly with the patient journey in the real-life practice:

❶ **Federating information**: combine multiple data sources using *e.g.* federated learning algorithms or Bayesian integrative modeling

Figure 1: General concept



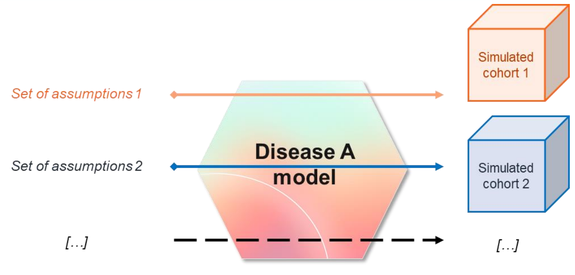
❷ **Use of Artificial Intelligence (AI) on federated information to create a disease-specific model.** Using a mix of interpretable machine learning (ML) algorithms and advanced statistical models, to infer on the variability structure of:

- Disease progression
- Treatment sequences/care pathways
- Patients' phenotyping, and responders profiling

❸ **Based on the modelling of the disease** and using sets of hypotheses, a **digital cohort simulator** tool will make it possible to create an environment capable of generating simulated cohorts (Fig. 2).

The simulated cohorts will serve several use cases (Fig. 3), including the use of integrative statistical models to propagate uncertainty, approximate effectiveness to efficiency, and enable short- and long-term extrapolation.

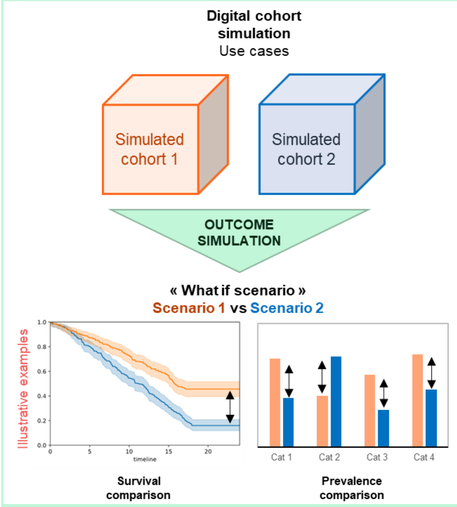
Figure 2: Example of use of digital cohort simulator



The disease model is used under the influence of different sets of assumptions to generate several simulated cohorts taking into account these input variables.

This proposed framework (Fig. 1) therefore allows the development of modules capable of drastically accelerating the generation of hypothesis on a given disease using AI-oriented methods

Figure 3: Illustrative results from digital cohort simulator



"What if" scenario comparison like optimal treatment positioning, bridge efficacy to effectiveness, enable short-to-long term extrapolations…

A Consortium-based approach

- A global public-private consortium is built for each disease to define model development priorities and endorse acceptability and scope of validity.
- First consortia being built in lung cancer, asthma, multiple sclerosis, and in rare disease.
- IP managed differently for pre-competitive vs. proprietary versions involving recent RCT data

RESULTS

- At this stage, the pilot showed success passing the technical, regulatory, legal, logistical and cultural feasibility barriers. ML methods combined with Bayesian approaches can augment drastically probability of success in of trials e.g. for rare neuro-degenerative disorders.
- Preliminary evaluations lead to a 5-10 fold acceleration of time to evidence compared to the sequential traditional approach.

DISCUSSION

- The proposed approach comes in line and synergies with multiple converging initiatives from e.g. the Critical Path Institute[1] and More Europa[2] project with HTAs and regulatory bodies.
- The success is pending to:
  - ✓ Access to robust, patient-level RWD that could be maintained over time
  - ✓ Validation processes transparently reported
  - ✓ A sustainable economic model, differentiating pre-competitive version vs proprietary ones
  - ✓ A significant level of endorsements by Health Authorities

Adaptability to new geographies given SoC data and local RW population characteristics distributions should allow development of reference models to be used e.g. in local submissions.

Such tools will pave the way for precision care when used at point of care by physicians.

CONCLUSIONS

- **Building reference patients simulators in RW will transform evidence generation**
- **This is made possible by federating multiple RCT and RW data sources via a mix of advanced ML and Bayesian modeling**
- **Governance (incl with data owners), validation and maintenance will be critical for success**

REFERENCES

1. Critical path institute ; https://c-path.org/
2. MORE Europa: More Effectively Using Registries to suppOrt PAtient-centered Regulatory and HTA decision-making; https://cordis.europa.eu/project/id/101095479/fr

DISCLOSURE

The authors of this poster are consultants at Quinten Health, located in Paris, France, a pioneering company in disease and care modeling.

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