

Case Study

Identifying Best Strategy

Identifying the best overall strategy for study designs and drug development pathways.



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KerusCloud is a revolutionary simulation-guided study design tool that ensures clinical trials are designed effectively to collect the **right data**, in the **right patients**, in the **right way**. Its use supports evidence-based design decisions to extensively **de-risk real clinical studies**, reducing development time, costs and patient burden.

The Software

KerusCloud allows multiple study uncertainties to be explored simultaneously, in minutes, within a virtual environment. Study outcomes are visualised with an interactive heatmap where detailed results help identify the pros and cons of different design options. This allows the key drivers of study success to be pinpointed rapidly so that the best design and analysis approach can be selected, first time.

Diverse information and data types inform the simulations with sources including the scientific literature, disease registries, historical trials and real-world data. These data are captured in the platform as synthetic data sets, avoiding privacy constraints, and used to build virtual patient populations to answer 'what if' study scenarios questions.

KerusCloud's synthetic data driven simulations are uniquely informative. They best represent the complexity found in real studies by accurately mimicking the quirks found in real patient-level data, like missingness. Therefore, KerusCloud provides exceptional advanced analytical insights able to deliver the smarter studies needed to address today's complex clinical research challenges.

The Challenge

EU and US regulators are seeking to enable early access to novel treatments for patients with severe unmet need. In the EU, the Medicines Adaptive Pathways for Patients (MAPPs) shown in Figure 1 is an initiative looking at ways to enable early access including:

- + Flexible development and access pathways within the current regulatory framework that balance early patient access, public health and societal benefits.
- + Early authorization of a product focused on a well defined and targeted population with a clear safety and efficacy profile.

In collaboration with **NICE**, **MIT** and **NEWDIGS**, Exploristics examined how the MAPPs concept could be applied in development, evaluating the benefits and risks of a treatment, and the commercial impact of the development options on the treatment.

MAPP development pathway

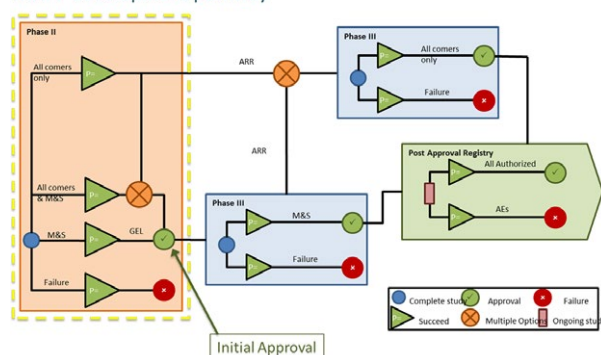


Figure 1. The MAPPs Development pathway

The Approach

Information on a marketed treatment for relapsing Multiple Sclerosis (MS) was used by KerusCloud to generate realistic patient level data in silico and design alternative MAPP development plan scenarios in a virtual environment to:

- + Identify a higher benefit moderate & severe (M&S) sub-population for early authorization and real-world evidence collection.
- + Continue to develop the All-comers indication with no launch delay.
- + The outputs from KerusCloud including probability of success and sample size were used as inputs for commercial forecasting.



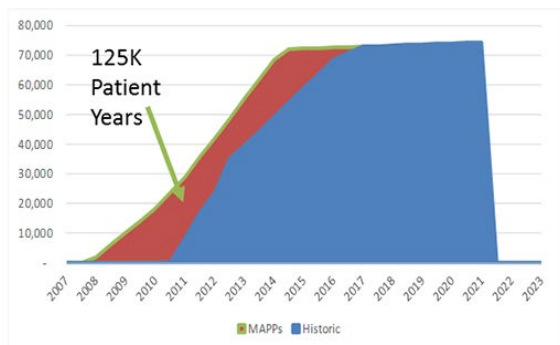
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The Results

The outcomes of simulated scenarios in KerusCloud broadly supported the MAPPs approach and demonstrated that:

- + The MAPPs design would provide **sufficient evidence for approval** in a larger Phase II trial.
- + The Phase II trial would need to randomize **450 patients rather than 240** in the original design.
- + Initial approval could be in **5 rather than 8 years**.
- + Financial modelling of the impact of MAPPs for Patients, Payers and Sponsors (Figure 2) indicated an increased expected Net Present Value (eNPV) of **\$600M** rather than **\$460M** for sponsor.

Patient Access Increases



The Impact

With KerusCloud it was possible to show that:

- + The MAPPs approach can provide access for patients up to **3 years earlier**.
- + MAPPs was **beneficial** for developer and payer economics.

Sponsor MPV & eNPV Increase



Figure 2. Financial modelling of the impact of MAPPs for Patients, Payers and Sponsors

Testimonial

“Adaptive Pathways integration of staged approvals with real world evidence poses many issues regarding patient safety, patient access, developer incentives and payer affordability. The Exploristics team worked seamlessly with a joint MIT and IMI ADAPT-SMART team to create a case study that quantified not just a single study but an entire adaptive clinical development plan of multiple trials for dual approvals. The work was done in only a few months, demonstrating improved patient safety and increased population benefit inducing further support for adaptive pathways policies.”

Mark Trusheim, NEWDIGS Strategic Director, **MIT Center for Biomedical Innovation**, USA

Why Exploristics?

Expertise In Early Development

The development of investigational drugs is a complex and expensive process with many risks. For over ten years our teams have been supporting and de-risking clinical development with their in-depth statistics and modelling expertise. Our study planning, statistical analysis and programming services add value to early stage development programmes by ensuring they deliver the robust evidence needed for incisive, informed decision-making.

With many of our development solutions built around our unique **KerusCloud** platform, we can provide exceptional, bespoke, end-to-end biostatistics support from strategic decision-making and protocol development to analysis, reporting and stakeholder engagement.

Let's talk!

If you'd like to discuss this case study further or learn more on how our **technology enabled services** can support your development project, please contact our VP of Sales & Marketing, Abbas Shivji, at abbas.shivji@exploristics.com or **book a call**.

Robust Evidence Packages

The unique offering of our comprehensive biostatistics services in combination with **KerusCloud** ensures that Exploristics can help to generate strong evidence packages to support regulatory engagement or investment, accelerating development timelines and increasing the value of pipelines.



Exploristics.
Your Essential Biostatistics Services Partner.