THE DIFFERENCE IS IN THE DATA®

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Case Study

Enabling Early Approval

Enabling early approval with a smarter approach to generating robust clinical evidence

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 visualized 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.

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The Challenge

A small biotechnology company with limited resources was developing a new antibacterial treatment for *C. difficile* (CDI). CDI is the most common single organism causing healthcare associated infections. In vulnerable patients, CDI infections have high mortality rates, ~30% for severe CDI and ~40% in elderly patients. The Sponsor was seeking early access for patients via the breakthrough therapy initiative in the US and medicines adaptive pathways (MAPPs) in the EU.

- A previous study assessment indicated that the development program for the new antibacterial agent would need ~1000 patients. However, this development plan was impractical and could not be executed.
- How could evidence be generated to support rapid marketing authorization?

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The Approach

KerusCloud® was used to evaluate alternative development plan options that would be feasible given the Sponsor's constraints.

To do this:

- Information was collated from a variety of sources, including data on multiple correlated endpoints comprising clinical and pharmacodynamic measurements.
- The data sourced was processed and then converted into synthetic data sets within KerusCloud® to build virtual patient populations (Figure 1) that could inform study simulations.
- KerusCloud® simulated thousands of studies with outcomes explored using an interactive heatmap (Figure 2) so that the impact of different study design parameters and what if scenarios could be assessed rapidly *in silico*.

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The Results

An alternative development plan was identified with the best design and endpoints for generating an evidence package for rapid approval.

This showed that an initial evidence package could be generated using 180 rather than 1,000 patients.

Design options and simulated evidence were presented to the FDA and EMA, so Regulators could give scientific advice on how best to proceed.

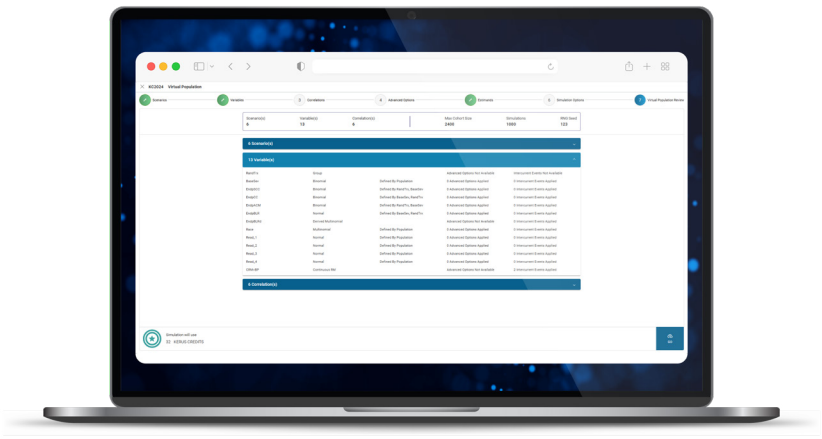

Figure 1. Construction of a virtual population in KerusCloud®

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The Impact

KerusCloud® provided an alternative development plan to accelerate the delivery of a new antibacterial treatment option to patients that:

- **Provided evidence that Regulators agreed would likely be sufficient for approval.**
- **Reduced the time to market by 3-5 years.**
- **Saved the sponsor USD22.5M in development costs.**

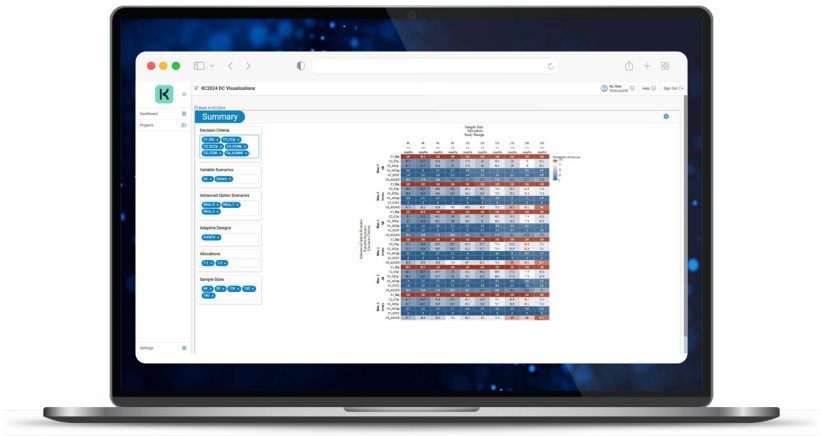

Figure 2. A typical results heatmap in KerusCloud®

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"The KerusCloud® simulation tool is very powerful! The simulations for our pivotal trials showed us a suitable and straight forward path to reach marketing approval with a smaller number of patients and quicker compared to our original plans. Discussions with statistic experts from CROs, investigators and key opinion leaders confirmed the approach."

CEO, Small Biotech, Germany

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Why MMS?

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 programs 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.

Robust Evidence Packages

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

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 visit our website: mmsholdings.com

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