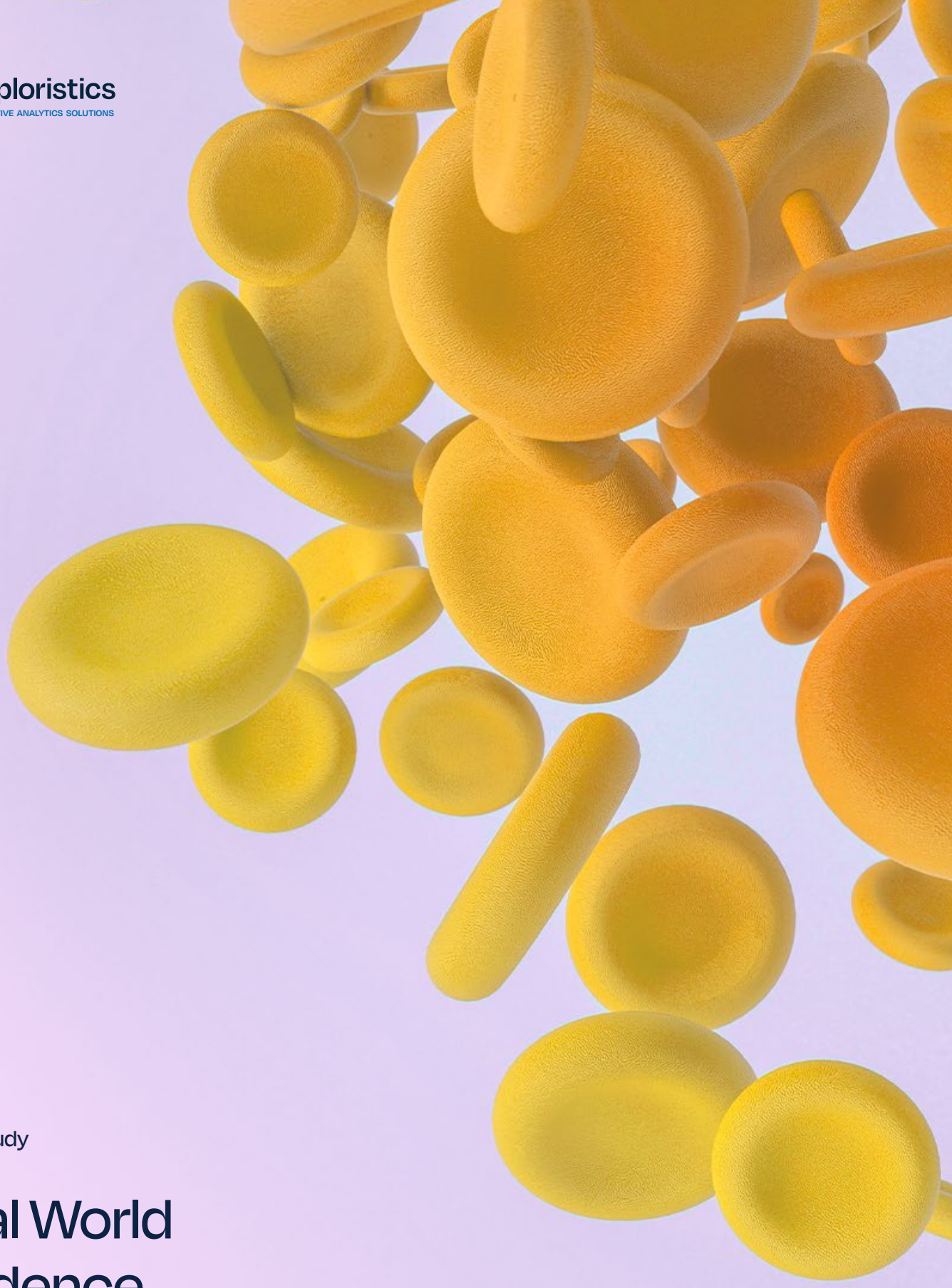
exploristics
INNOVATIVE ANALYTICS SOLUTIONS



Case Study

Real World Evidence

Simulating patient level outcome data to inform clinical trial design based on real-world, clinical trial and literature data.

KerusCloud®

KerusCloud®

Simulating patient level outcome data to inform clinical trial design based on RWE, clinical trial and literature data.

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.

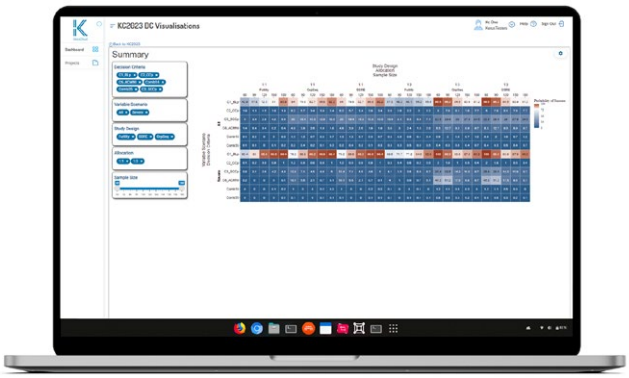
Diverse information and data types inform the simulations with sources including the scientific literature, disease registries, historical trials and real-world data. These data are captured in the platform as synthetic data sets, avoiding privacy constraints, and used to build virtual patient populations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

GSK were designing a phase III programme for a novel treatment for anaemia associated with chronic kidney disease in dialysis patients. Both the novel treatment and recombinant human erythropoietin (current standard of care) modulate the Haemoglobin (Hb) levels. However, there were some complexities to consider when designing the study:

- Low and/or very high levels of Hb are both associated with an increased risk of major adverse cardiac events (MACE) which was the primary outcome.
- Baseline risk factors (older age, diabetes, prior myocardial infarction (MI), heart failure (HF), stroke) are also known to increase risk of MACE.
- The complex relationship of varying risks over time in different risk groups at baseline may lead to uncertainty for accurate outcome prediction.



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The Approach

KerusCloud was used to simulate realistic patient level outcome data to inform clinical trial design under different scenarios.

- Input data was derived from multiple sources including real-world data, clinical trial data and the published literature.
- A range of study design and analysis options were evaluated for their ability to detect differences in treatment for time to MACE. Design options included studies that were enriched for high-risk patients.
- Additional analysis options included standard cox regression models, adjusted models and time variant models.
- Various metrics were derived from simulated studies including probability of success, study duration and sample size.

The Results

Simulations using KerusCloud identified that:

- Studies enriched with high-risk patients resulted in smaller sample size or duration.
- The analysis approach had minimal impact on study power when Hb levels were well controlled.

The Impact

- KerusCloud successfully utilised real-world data to inform simulations that helped optimize design and analysis options for a study where there were **multiple sources of uncertainty**.
- The simulations confirmed the validity of assumptions for the study under **different conditions** for the design and analysis options and treatment effects.
- Viable alternative study approaches were identified e.g., enriched study design which **reduced study duration and sample size by as much as 30%**.

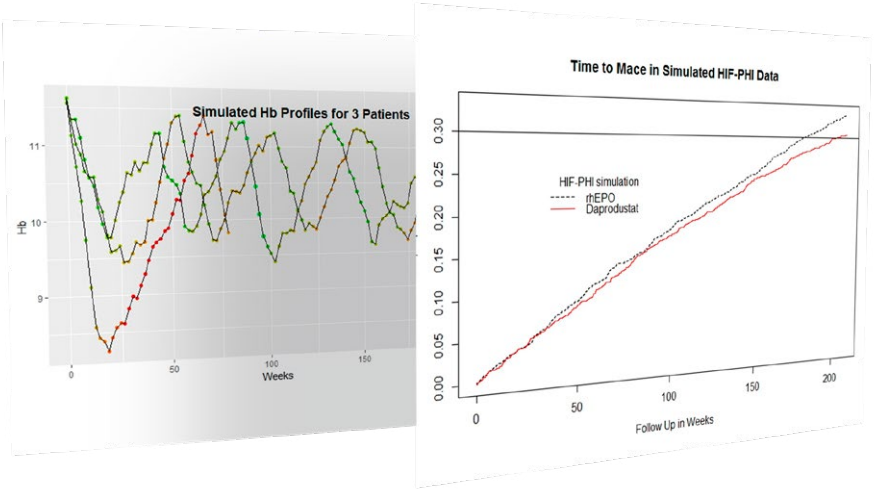

Figure 1. Typical simulated patient profiles

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.

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.

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**.

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