Synthetic Control Arm®: The Smart External Control Arm

While randomized controlled trials (RCTs) are the gold standard for understanding the safety and efficacy of an experimental therapy, there are scenarios where it is not appropriate or feasible to maintain a concurrent control arm. In these situations it can be difficult to generate the evidence necessary to understand or contextualize your trial findings — which can hinder decision-making, cause costly delays and potentially put your trial at risk.

An external control arm (ECA) — a control arm built using data from patients outside of the current trial — is a powerful tool that enables researchers to assess the efficacy of their novel treatment to generate scientifically rigorous evidence that can inform internal decision making, regulatory discussions and support market access.

Medidata AI Synthetic Control Arm (SCA®) is an ECA built from our one-of-a-kind database of patient-level, cross-industry, historical clinical trial data from over 33,000 clinical trials and 10 million patients — complete with endpoints and covariates as they were captured and validated in their original trial.

Benefits

Medidata's SCA can bolster evidence and increase scientific certainty across clinical and commercial development.

Provides an apples-to-apples comparison with your current trial

Historical clinical trial data offers the best comparator data because it includes patients with similar demographics to those in current trials, and measures endpoints at the same frequency

Propensity score models further enhance the comparability of your investigational arm and our historical clinical trial patients, leading to more precise estimates of comparative treatment effects than benchmarks in literature, or even cohorts with similar patient inclusion/exclusion criteria

Accelerates trials

Increase early scientific certainty and confidence to continue clinical development or fail fast, minimizing wasted time and resources

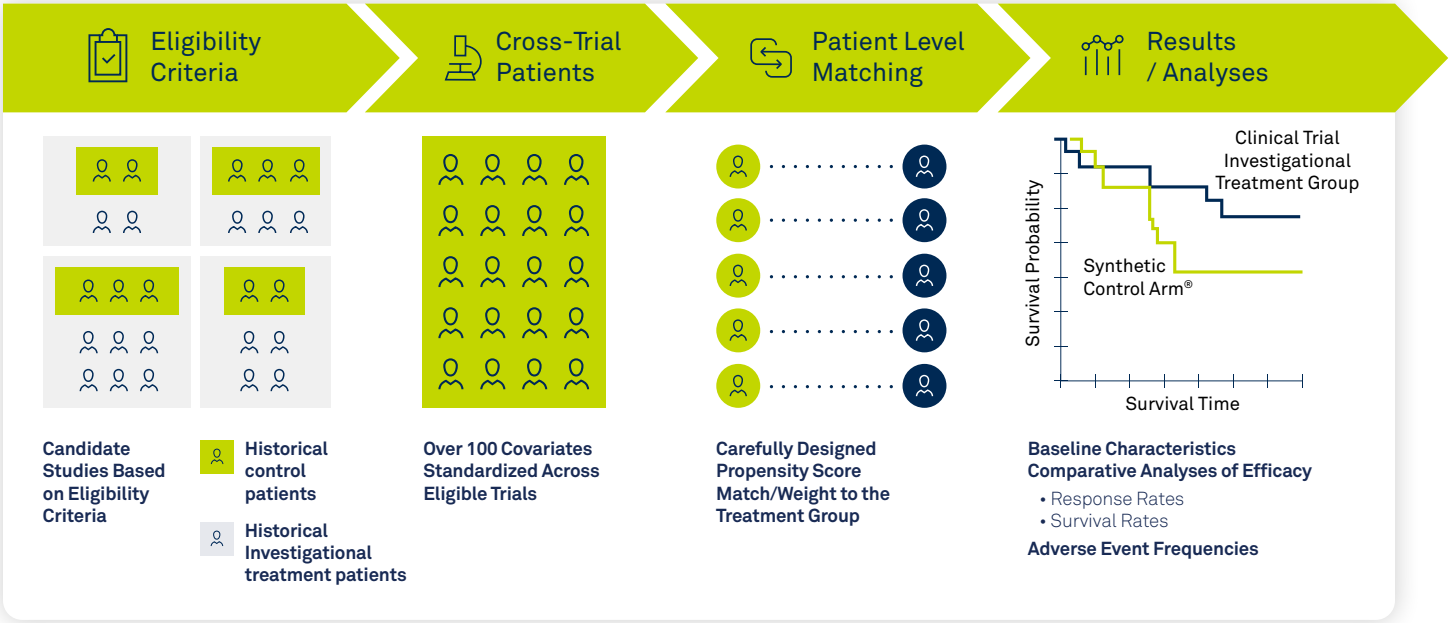
Reduce the number of patients required to enroll in a control arm and improve retention, bringing a product to market faster

Patient-Centric

Offer a more patient-centric solution that maximizes the proportion of patients receiving a potentially promising therapy in diseases with high unmet need and/or inadequate standard of care

How it works

To build an SCA, Medidata researchers mine a database of past clinical trials run on Medidata Rave Electronic Data Capture (EDC) platform to find control patients who match the patients in the target trial on key eligibility criteria. Patient data across eligible trials are standardized to ensure consistency, and target trial patients are then matched to the historical trial patients through propensity score models on prognostic variables such as age, gender, and performance measures to maximize comparability. Comparative analyses are then conducted between the SCA and target trial.



Applications

Contextualize early phase, single arm trials to:

- Support internal decision making and design for subsequent trials
- Publish early results to build investor and medical community awareness
- Support FDA breakthrough therapy designation

Accelerate the completion of non-pivotal randomized controlled trials

Support regulatory approval in pivotal trials in diseases with high unmet medical need and inadequate standards of care

Help understand the effect of crossover or supplement attrition when new effective therapies enter the market or become available during your clinical trial

Compare your investigational arm against comparator(s) or in populations of interest to payors which were not included in the clinical trial

View Customer Examples

- **Imunon: Estimate treatment effect to inform early stage decision making**
- **Arcus: Demonstrate comparative efficacy for evidence for investors**
- **Medicenna: Design Phase 3 Study Using Hybrid SCA**
- **Kite: Utilize an SCA to show efficacy in additional populations**