Synthetic Control Arm®: The Smart External Control Arm Solution

Overview

Sponsors often experience unique and significant challenges with randomized trials:

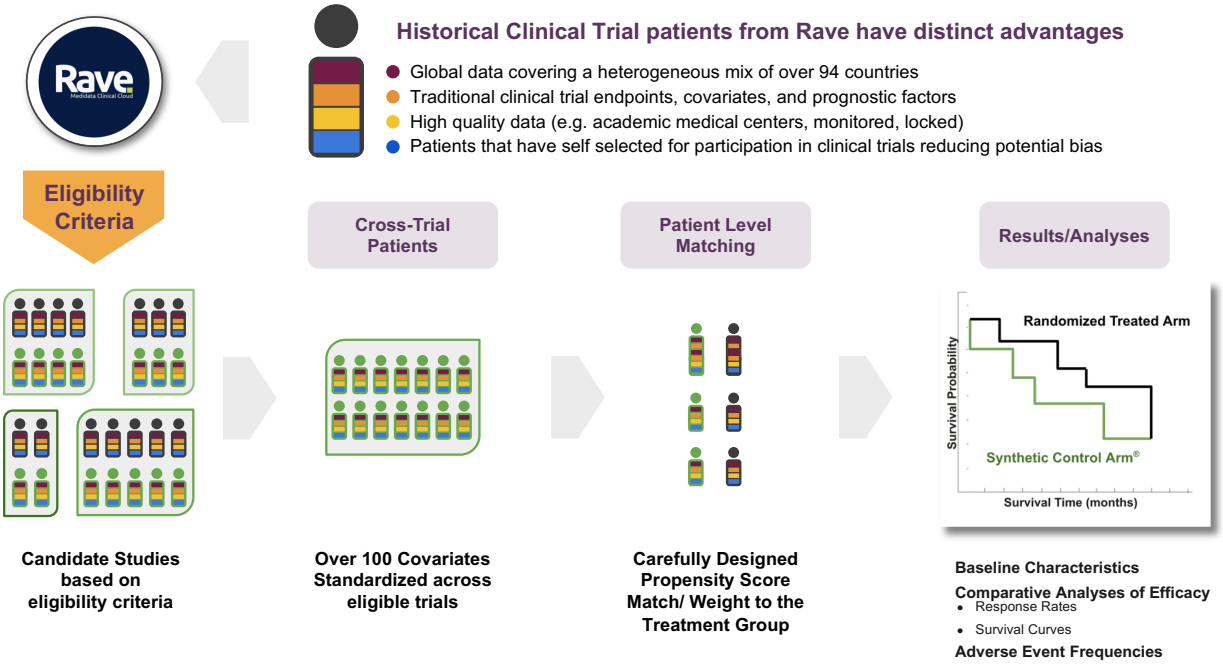
- Randomization is not always possible due to ethical reasons or rarity of a disease
- Noncompliant use of the investigational agent, or similiar agent in the same class, by control patients
- Slow recruitment due to unacceptability of control treatment to patients
- Deciding which are the most promising products to advance in pipeline

ENTER ACORN AI

Acorn AI by Medidata is uniquely positioned at the nexus of historical clinical trial and real world data; with a platform that integrates disparate datasets. Our Synthetic Control Arm® (SCA) solution can bring significant value to your company's trials and ultimately help to increase the probability of overall success of your key pipeline assets. SCA supports trial design choices to enhance or replace control arms for registrational trials or early phase studies. This improves decision making, accelerate enrollment and reduce clinical development costs.

HOW WE DO IT

Medidata has helped sponsors run clinical trials for over 20 years, accumulating a clinical trial data repository of 6 million+ patients and 22,000+ trials. This unique data asset enables distinct benefits over traditional real world data sources.



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BENEFITS

APPLICATION	DESCRIPTION
Go/No-Go Decisions	Enhance interpretation of single arm trial and increase efficiency in early phase RCT
Accelerated Approval	Enhance interpretation of phase 2 single arm trial
Augment or replace randomized control	When necessary and appropriate, produce regulatory-grade, matched historical controls to support regulatory submission and approval

Use Case: FDA Agreement of SCA Design in rGBM

Groundbreaking progress in the path to regulatory adoption

CONTEXT

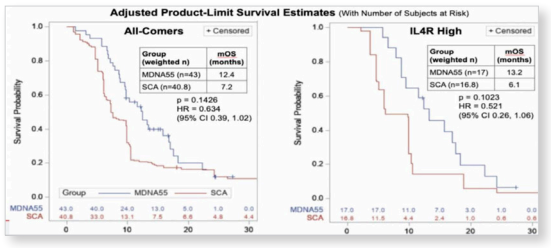
To demonstrate efficacy in proof-of-concept, single-arm Phase 2 trial, and use the results to design a Phase 3 SCA/external control based registrational trial.

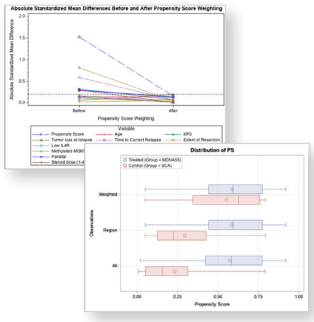
SOLUTION

Prepare the EoP2 briefing packet: design the external control/SCA based registrational trial, and prepare protocol profile; propose statistical analysis plan, accounting for the external control/SCA data.

RESULTS

- SCA demonstrated large efficacy effect size in all-comers subjects, as well as in subjects expressing high levels of IL4R.
- FDA endorsed use of hybrid (augmented) Synthetic Control Arm® in a Phase III study design in rGBM.





"We are extremely impressed with the Acorn AI team for providing a scientifically rigorous rationale for the design of an innovative registration trial incorporating an external control arm for the treatment of recurrent glioblastoma (rGBM) with MDNA55. Their expertise and collaborative effort with thought leaders was instrumental in demonstrating to the FDA the validity of a well designed external control in a registration trial. The FDA's acceptance of this unique design, will expedite completion of the Phase 3 trial in rGBM allowing earlier access of MDNA55 for a disease with poor prognosis and high unmet need."

Dr. Fahar Merchant,
President and CEO of Medicenna,
October 16, 2020

About AcornAI

Acorn AI™, by Medidata, a Dassault Systèmes company, combines data, technology, and deep expertise to help life sciences companies deliver actionable insights across the entire continuum of clinical development. Acorn AI's advanced analytics answers the most important questions in R&D and commercialization including accelerating breakthrough innovation, optimizing study execution and commercial success, and demonstrating the value of therapies. Built upon the Medidata platform comprising 20,000+ trials and more than six million patients, Acorn AI products feature the industry's largest structured, standardized clinical trial data repository connected with real world, translational, and other datasets. For more information, please visit:

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