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Publisher's Spotlight: The Winners Of The 2024 Citeline Awards

by **Mary Jo Laffler**

Congratulations to the winners of the 2024 Citeline Awards, held 8 May in Boston.

The winners of the Citeline Awards reflect the growing investment in technology solutions and artificial intelligence and their application across a range of clinical research functions. The awards, announced 8 May in a celebration at the Westin Boston Seaport Hotel, cover 12 categories, spanning a spectrum of R&D activities from successes in early and late-stage research, to advances in technological tools, to innovations in trial design and execution.

Moderna and ProofPilot won the Best Patient-Facing Technology award for their Participant Experience Platform, which modernizes clinical trial participation with a seamless, digital-centric approach. The sponsors note that the platform, which is highly rated by users, offers participants control over their experience and is “where convenience meets confidentiality.” The judges were impressed with how the interactive tool supports data generation, collection and patient experience with trial conduct.

The judges also selected the Moderna/ProofPilot platform as the Clinical Partnership Of The Year, focusing again on how the platform improves the clinical trial process by offering simplification and incentives for completion. They noted that the tools for ongoing communication encourage high degree of compliance and reduce site bureaucracy. “This partnership enabled patients to be empowered to not only participate in important research but also to be co-creators of the research,” the panel said. “Kudos to Moderna and ProofPilot for appreciating the need to better understand patient experiences in a clinical trial to improve participation.”

Artificial intelligence factored into ICON's win for Excellence In Use Of Real-World Data/Evidence for its Cassandra AI tool for commercialization forecasting, powered with RWE/RWD. “Cassandra allows ICON to confidently predict the future imposition of post-

marketing requirements, allowing it to work with its customers to either plan for a post-authorization safety study or redesign the clinical trial protocol so that a post-marketing requirement is not required by the regulatory authorities,” according to the company. The judges appreciated the “innovative use of AI/ML in helping sponsors predict need for PM studies to inform clinical development planning.” With rising drug development costs, “leveraging AI to provide guidance on the probability of requiring a post-marketing surveillance program will be tremendous for development planning,” the judges said.

Artificial intelligence also is a component of the winner of the Best Sponsor-Facing Technology. Medidata's Clinical Data Studio is an AI-enhanced platform that orchestrates data integration, standardization, reconciliation, and quality management across multiple sources. The judges were impressed with the integrated work space as a sophisticated and user-friendly data management system that orchestrates data integration, standardization, reconciliation, and quality management across many sources, regardless of which system the data was originally collected.

Medidata was also recognized for Best Use Of AI In Clinical Trials for its AI Simulants – a patented synthetic data solution, generated from cross-sponsor historical clinical trial data, that the sponsor says has helped with designing and executing faster, safer trials that lower patient burden, reduce costs and deliver promising and effective therapeutics to patients. The judges noted that AI Simulants were able to shorten clinical trial timelines up to two years (saving \$10m-\$15m) and determine optimal regimens and study design by accessing global historical data from 30,000 clinical trials and 9 million subjects. Medidata reported Simulants has been used by numerous clinical development teams “to significantly elevate the probability of technical and regulatory success for their treatment development program.”

Merck's Diversity & Inclusion in Clinical Trials (DICT) team was recognized as Champion Of Diversity And Inclusion In Clinical Research for their work across Merck's portfolio. The judges were impressed with the metrics shown on how the DICT team has improved the diversity in Merck's late-stage clinical trials and the variety of partnerships the team established across different industry stakeholders. Taking a collaborative approach and working with external associations has helped promote awareness, education, participation, and access to studies and healthcare.

The Clinical Research Team Of The Year went to Agios Pharmaceuticals' team for the 017 ENERGIZE trial of mitapivat for thalassemia. The team exceeded enrollment targets and completed the pivotal Phase III study ahead of schedule. “Proactively engaging with patient advocacy groups, they ensured protocols reflected patient challenges, culminating in historic results: meeting primary and secondary endpoints and demonstrating mitapivat's potential to transform thalassemia treatment,” the firm said, adding that their cross-functional collaboration has set a new standard in rare disease research and laid the groundwork for further

breakthroughs in thalassemia care. The judges highlighted the initiatives implemented to collaborate with the sites and patient advocacy groups, resulting in exceeding enrollment targets for the rare disease trial.

Clinical Trial Result Of The Year went to Karuna Therapeutics, a Bristol Myers Squibb company, for its pivotal Phase III trial of KarXT (xanomeline-trospium) in schizophrenia, which showed impressive efficacy and differentiation from standard-of-care generic antipsychotics. KarXT is expected to be a first-in-class M1/M4-preferring muscarinic receptor agonist designed to preferentially stimulate muscarinic receptors in the central nervous system. Analysts deemed its efficacy comparable to prior antipsychotics but with a differentiated side effect profile. The judging panel also took note of the “significantly improved side effect profile vs. existing therapies” and felt “it offers a major benefit ... for the affected patient population that has only seen incremental improvement but no disruptive, meaningful innovation in therapeutic offerings - as this compound promises to be - over three decades.”

OcuTerra Therapeutics’ DR:EAM clinical trial evaluating nesvategrast eye drops for the treatment of diabetic retinopathy won the award for Excellence In Patient Recruitment & Engagement. The sponsor noted investigators described the patient enrollment process as one of the easiest in decades. The judges felt the trial used creative initiatives that fostered connection with the patient community, which translated into positive and meaningful impact on trial enrollment. “OcuTerra met with patient-led focus groups to develop a diabetic retinopathy study protocol,” the judges noted, “implemented a multi-prong approach to enhance recruitment/enrollment, and implemented a patient-friendly electronic compliance system using Aardex’s MEMS smart cap system, allowing patients to track each dose in a smartphone app by simply touching their eye drop bottles to their phones.”

The award for Excellence In Rare Disease Drug Development went to SpringWorks Therapeutics’ Ogsiveo (nirogacestat), the first and only treatment approved for adults with progressing desmoid tumors, which was previously only treatable by surgery. The judges lauded the “quite meaningful improvements” including in both function and quality of life for patients with long-standing severe illness. As nirogacestat was once a breast cancer drug divested from Pfizer, the judges appreciated SpringWorks’ successful repositioning of the drug for the rare solid tumors. “SpringWorks strategy not only derisked clinical development for a disfiguring non-malignant tumor, but shows rationale for expansion to other solid tumors and potentially myeloma,” the judges added.

Most Innovative Start-Up Company went to Mural Health and their technology solutions to facilitate participation in clinical research. The judges were impressed by Mural Health’s participant management platform, which “improved patient retention by helping participants get to their appointment, communicate with their site and gives them a voice to provide feedback. It reduced site burden by automating workflows for reimbursing participants,

coordinating travel and 1099s.” The “one-stop solution” makes patients’ clinical trial experience easier and less burdensome. “This innovative approach helps trial participants and sponsors and should help expand access to trials, thereby increasing cohort diversity,” the judges concluded.

Most Successful Early Phase Research (Preclinical & Phase I) went to Flare Therapeutics and its preclinical, translational and clinical research on the lead molecule from its proprietary technology platform, bringing FX-909 from de novo discovery to the clinic in under 2.5 years. The judges highlighted two “creative and novel elements” – the precision oncology tool known as GPS, “designed to accelerate reliable and predictive identification of cancer patients expected to benefit from this modality [which] helped recruitment and execution of the current trial and can be expected to continue facilitating the next clinical development milestones and ultimately launch-if approved. The sponsor also developed a normal skin biopsy assay, designed to reduce /avoid the need for invasive biopsies during a dose escalation study, further aiding successful and accelerated completion of the dose-escalation study.”

Citeline Awards 2024 Winners

- Best Sponsor-Facing Technology Initiative – Medidata: The Medidata Clinical Data Studio Team
- Best Patient-Facing Technology Initiative – Moderna and ProofPilot
- Most Innovative Start-Up Company – Mural Health
- Champion Of Diversity & Inclusion In Clinical Research (sponsored by ICON) – Merck, Diversity & Inclusion in Clinical Trials (DICT) Team
- Most Successful Early Phase Research (Preclinical & Phase I) – Flare Therapeutics
- Clinical Research Team Of The Year – Agios Pharmaceuticals, Agios 017 ENERGIZE Team
- Excellence in Rare Disease Drug Development (sponsored by TrialWire) – SpringWorks Therapeutics
- Best Use of Artificial Intelligence in Clinical Trials – Medidata
- Clinical Trial Result Of The Year (sponsored by Bioforum) – Karuna Therapeutics, A Bristol Myers Squibb Company
- Excellence In Patient Recruitment & Engagement – OcuTerra Therapeutics

- Excellence In Use Of Real-World Data/Evidence – ICON
- Clinical Partnership Of The Year – Moderna and ProofPilot