

PRESS RELEASE

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NUS artificial intelligence platform demonstrates promising results in effectively treating a patient with a rare cancer

In a first-of-its-kind clinical trial, a team at the NUS Yong Loo Lin School of Medicine leveraged an artificial intelligence-derived platform to guide treatment for a patient with a rare blood cancer

Singapore, 4 September 2024—Rare diseases affect fewer than 1 in 2,000 people individually. However, with over 7,000 different types identified, their global impact is significant. In the Asia-Pacific region, approximately 258 million people¹ have a rare disease, the highest number globally, with over 45 million² in Southeast Asia alone. This vast number highlights significant challenges in treatment, as the diversity of this patient pool contributes to significant healthcare disparities and increased challenges in clinical trial recruitment. Additionally, within the small patient pool, each patient is different from each other, and individual patients' conditions change over time. This emphasises the critical need for accessible and personalised treatments for this population of patients, while highlighting the profound challenges faced in developing treatments for patients with rare diseases.

To address the need for effective treatment for rare diseases without using large population data, researchers from the **Institute for Digital Medicine (WisDM) at the Yong Loo Lin School of Medicine, National University of Singapore (NUS Medicine)** leveraged small amounts of data from a single patient with a rare disease to guide his treatment, with promising results. Co-led by **Professor Dean Ho, Director of WisDM at NUS Medicine**, the team conducted a clinical trial for the patient who was diagnosed with Waldenström macroglobulinemia—a rare blood disorder affecting approximately three in 1,000,000 people annually—by leveraging an artificial intelligence (AI) derived platform, CURATE.AI. Unlike traditional AI models that depend on large datasets, CURATE.AI utilises small data to dynamically adjust treatment dosages based on individual patient responses. Since the trial began in October 2021, substantial improvement in this patient's red blood cell levels was observed, and the patient was able to avoid blood transfusions. Importantly, the patient did not suffer serious side effects from the treatment and hospital admissions were minimised.

¹ <https://www.asiapathways-adbi.org/2020/07/rare-diseases-asia-pacific-must-be-tackled-too/>

² <https://ojrd.biomedcentral.com/articles/10.1186/s13023-016-0460-9>

In the trial, the research team worked with clinicians from the **National University Cancer Institute, Singapore (NCIS)** to identify drug doses for the patient, based on guidance from the CURATE.AI platform. The drug doses were selected based on the patient's own responses prospectively, making the treatment strategy a first of its kind. Compared to the overall dose under the standard of care regimen, the trial's recommended drug doses were lower and well-tolerated by the patient, demonstrating durable control of the disease. As a result, the patient was able to save approximately USD 8,000 (around SGD 10,500) on drug costs over the first two years of the treatment.

The trial with treatment recommended by CURATE.AI is ongoing and is now open for recruitment of new, suitable patients. The results collected from the first two years of the trial are published in the journal *NPJ Digital Medicine*, a Nature Portfolio journal.

Prof Ho said, "No two patients are alike, and even the same patient can change over time as well. It is essential for treatment to evolve alongside the patient. Our study highlights the effectiveness of using small data to treat extremely rare diseases—addressing the gaps where traditional big data methods fall short, and where large-scale trials are not feasible due to the limited patient population. CURATE.AI's approach, which tailors treatment using small datasets, offers a practical solution for the urgent and challenging need of developing personalised strategies for rare diseases." Prof Ho is also Head of the Department of Biomedical Engineering at the NUS College of Design and Engineering, and Director of the NUS N.1 Institute for Health.

Dr Sanjay de Mel, Senior Consultant in the Division of Haematology, Department of Haematology-Oncology, NCIS, and the clinical lead for the trial, added, "Achieving a good treatment response while minimising side effects is crucial when treating patients with Waldenström macroglobulinemia. Patients may differ significantly in how their bodies handle the treatment and the types of side effects they experience. A personalised approach to medication dosing is therefore required to address this inter-individual variability."

About CURATE.AI

Through the AI-derived technology platform, CURATE.AI, which provides actionable N-of-1 (i.e. single patient) therapy for the entire duration of patient care, trials are customised based on individual profiles, to develop drug therapies and interventions that achieve better outcomes for patients. Dynamically adjusting drug doses, CURATE.AI sustains the optimisation of treatment as patient responses are recorded.

Traditionally, AI uses big data from a large number of patients to train and test an algorithm. The algorithm is then validated again using data from a different patient population. This can lead to important advances in AI. However, in the absence of prospective implementation, or real-world use of the algorithm to guide patient care, this can lead to prediction without implementation, resulting in the algorithm remaining theoretical.

The approach of CURATE.AI proves to be very different—it is one of the few in the world where small data is used to guide actual treatment prospectively. As part of the WisDM team's clinical trials, clinicians are often approving and reducing doses that are given to patients based on this small data platform. As CURATE.AI works with single patient data, it overcomes

the need for large patient populations—which often result in non-personalised patient trial designs and may not be practical in treating rare diseases where there is no large pool of data.

In a previous pilot clinical study conducted in collaboration with a US-based hospital, a patient with advanced prostate cancer was recommended a 50% reduction in dose of an investigational inhibitor drug for increased efficacy. The patient subsequently resumed an active lifestyle as the lower dose also proved to be more tolerable. Another patient in Singapore with advanced cancer who was prescribed a reduced dose of nab-paclitaxel saw his lung tumour shrink while also maintaining a stoppage in progression of the cancer. This has also allowed the patient to continue treatment for a much longer duration compared to most patients who are being given this drug.

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About National University of Singapore (NUS)

The National University of Singapore (NUS) is Singapore's flagship university, which offers a global approach to education, research and entrepreneurship, with a focus on Asian perspectives and expertise. We have 16 colleges, faculties and schools across three campuses in Singapore, with more than 40,000 students from 100 countries enriching our vibrant and diverse campus community. We have also established more than 20 NUS Overseas Colleges entrepreneurial hubs around the world.

Our multidisciplinary and real-world approach to education, research and entrepreneurship enables us to work closely with industry, governments and academia to address crucial and complex issues relevant to Asia and the world. Researchers in our faculties, research centres of excellence, corporate labs and more than 30 university-level research institutes focus on themes that include energy; environmental and urban sustainability; treatment and prevention of diseases; active ageing; advanced materials; risk management and resilience of financial systems; Asian studies; and Smart Nation capabilities such as artificial intelligence, data science, operations research and cybersecurity.

For more information on NUS, please visit <http://www.nus.edu.sg/>

About the NUS Yong Loo Lin School of Medicine (NUS Medicine)

The NUS Yong Loo Lin School of Medicine is Singapore's first and largest medical school. Our enduring mission centres on nurturing highly competent, values-driven and inspired healthcare professionals to transform the practice of medicine and improve health around the world.

Through a dynamic and future-oriented five-year curriculum that is inter-disciplinary and inter-professional in nature, our students undergo a holistic learning experience that exposes them to multiple facets of healthcare and prepares them to become visionary leaders and compassionate doctors and nurses of tomorrow. Since the School's founding in 1905, more than 12,000 graduates have passed through our doors.

In our pursuit of health for all, our strategic research programmes focus on innovative, cutting-edge biomedical research with collaborators around the world to deliver high impact solutions to benefit human lives.

The School is the oldest institution of higher learning in the National University of Singapore and a founding institutional member of the National University Health System. It is one of the leading medical schools in Asia and ranks among the best in the world (Times Higher Education World University Rankings 2024 by subject and the Quacquarelli Symonds (QS) World University Rankings by subject 2023).

For more information about NUS Medicine, please visit <https://medicine.nus.edu.sg/>