

# Li Chi Kong - Research Professor, Department of Paediatrics, The Chinese University of Hong Kong

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*After decades spent advancing paediatric oncology in Hong Kong, Professor Li Chi Kong has increased his focus toward the frontier of cell and gene therapies, spearheading efforts to establish a locally manufactured CAR-T platform. Against a backdrop of systemic barriers, regulatory gaps, and limited commercial incentives for paediatric drug development, his work reflects a broader effort to reshape what is possible in childhood leukaemia care. From early clinical trial involvement to the coordination with the locally set up GMP-compliant manufacturing infrastructure, Professor Li has played a pivotal role in translating global innovation into local capability. "Our goal is always a cure, not just prolonged management."*

**How has your clinical practice in paediatric oncology developed over the years, and how do you view cell and gene therapies as part of the clinical strategy to treat patients?**

Over the course of more than four decades as a paediatric oncologist in Hong Kong, my work has centred on acute leukaemia and stem cell transplantation. During this time, we have undertaken numerous clinical trials aimed at improving the prognosis for children diagnosed with leukaemia. While these efforts have led to meaningful advances, it has become increasingly apparent that the therapeutic ceiling of chemotherapy has been largely reached. Consequently, our focus has shifted towards more innovative and targeted approaches, particularly those in the realm of cell and gene

therapy which involve genetically engineered immune cells designed to recognise and destroy malignant cells with greater precision and significantly less collateral damage to healthy tissue. Among these, antibody-based therapies have shown promise by selectively binding to leukemic cells, enabling more effective cytotoxicity while sparing normal tissues. However, it is cellular therapy, specifically CAR-T therapy, that offers the most transformative potential.

This approach involves extracting a patient's lymphocytes, genetically modifying them to express tumour-specific receptors, and reinfusing them into the patient, where they can exert a sustained anti-leukaemic effect. Unlike conventional pharmacological agents, which are rapidly cleared from the body, CAR-T cells can persist for months or even years, providing prolonged surveillance and a durable therapeutic response. This shift towards cellular immunotherapy marks an important and promising evolution in our efforts to improve long-term outcomes for paediatric patients with leukaemia.

**In your view what have been the systemic challenges preventing wider access to novel oncology therapies for paediatric patients, which cell and gene therapies can potentially have an impact on?**

Within the global paediatric oncology community, there is a growing recognition that despite decades of meaningful progress in childhood cancer care, therapeutic advancement is now approaching a plateau. Achieving further improvement will depend on the availability of new agents, yet the introduction of such treatments is often impeded by structural and economic barriers.

Pharmaceutical companies, which bear the financial burden of conducting clinical trials, are typically guided by market potential when determining where to invest. Given that paediatric cancers account for less than one percent of all cancer cases, the commercial incentive to initiate dedicated paediatric trials remains extremely limited. As a result, even when promising agents already approved for adult cancers demonstrate potential efficacy in paediatric indications, these are rarely pursued through the formal regulatory pathway required for use in children.

The absence of specific paediatric trials leaves such treatments without approved indications in this population, despite their likely benefit. This disparity has created a deeply ingrained imbalance. While adult oncology continues to benefit from robust investment and rapid innovation, children with cancer remain underserved by the current drug development ecosystem, facing delayed or restricted access to next-generation therapies that could meaningfully alter their

outcomes.

**When did you first recognise the potential of CAR-T therapy, and how did this shape your efforts to bring it to Hong Kong?**

My interest in CAR-T therapy emerged in the early 2010s, when pioneering trials in the US began to show striking early responses in patients with haematological malignancies. While these preliminary results were undeniably encouraging, particularly for a clinician focused on paediatric oncology, our immediate concern was whether such outcomes could be sustained over time. In our field, success is not defined by short-term remission alone, but by long-term disease eradication. Our goal is always a cure, not prolonged management.

Another significant source of hesitation lay in the treatment's toxicity profile. At the time, severe cases of cytokine release syndrome and neurotoxicity were being reported with some frequency, largely due to limited understanding of the mechanisms behind these effects and a lack of established management protocols. Over time, however, the international medical community's growing experience with CAR-T therapy brought about substantial improvements in patient selection, pre-conditioning strategies, and the management of adverse events. Today, thanks to better risk stratification and therapeutic interventions, the proportion of patients requiring intensive care following CAR-T infusion has decreased from over 30 percent to below 10 percent.

Our team's involvement with CAR-T therapy began even before its commercial introduction in Hong Kong. Faced with limited local access, we arranged for patients to receive CAR-T treatment in mainland China, after which they returned to Hong Kong for ongoing care. Although the therapy itself was administered across the border, our team was responsible for managing all post-infusion follow-up, providing us with invaluable hands-on experience at an early stage. We later documented these cases in a peer-reviewed publication, and our clinical knowledge continued to expand with the arrival of commercially approved CAR-T products from regulatory agencies such as the US FDA and the EMA. Collectively, these experiences have laid a strong foundation for the establishment of a dedicated, locally administered CAR-T programme in Hong Kong.

**What factors prompted the decision to establish a locally manufactured CAR-T therapy, and how did regulatory and institutional support come together to enable it?**

The initiative to develop a locally manufactured CAR-T therapy in Hong Kong stemmed from two critical challenges associated with commercial products: accessibility and logistical complexity. At the time, the cost of a single commercial CAR-T treatment was approximately USD 400,000, placing it well beyond the financial means of many patients. In parallel, the logistical process was far from straightforward. Patient cells had to be harvested, frozen, and shipped to manufacturing sites overseas – typically in the US or, more recently, Singapore – before undergoing genetic modification. The final product was then frozen once again and returned to Hong Kong for reinfusion. This process, spanning four to six weeks, introduced delays that, in certain cases, had devastating consequences. Some patients, whose cells had been sent abroad for processing, experienced rapid disease progression and sadly passed away before the modified cells could be returned and administered.

These cases underscored the urgent need for a faster, more efficient system. The concept of local manufacturing emerged as a response, aiming not only to shorten turnaround time but also to eliminate the risks associated with repeated cryopreservation and international shipping, factors that could potentially compromise cell viability. The Chinese University of Hong Kong (CUHK) was instrumental in advancing this vision, supporting the establishment of a GMP facility at the Hong Kong Institute of Biotechnology (HKIB). At the time, cellular therapy was still a novel concept in the local regulatory landscape. However, the Department of Health had begun to lay the groundwork for a framework governing advanced therapy products, drawing inspiration from established European regulatory models.

Recognising the opportunity, our team engaged early with the Department of Health to study the draft regulations in detail, while CUHK invested in the necessary infrastructure, personnel, and compliance processes. This parallel momentum between institutional commitment and regulatory evolution created an environment conducive to innovation. What emerged was a unique alignment of vision across academia, government, and clinical leadership, setting the stage for the development of a domestically produced CAR-T platform in Hong Kong, with both clinical trial and future commercial potential.

**How did you lay the groundwork for Hong Kong's CAR-T production capabilities, and what impact did global partnerships and public funding have on that process?**

The development of a locally manufactured CAR-T therapy in Hong Kong required a concerted effort to build scientific expertise, regulatory readiness, and compliant infrastructure – all within a

landscape where cellular therapy was still emerging. At the time, Hong Kong lacked a sufficient pool of specialists with hands-on experience in advanced cell manufacturing, particularly in the rigorous standards required for GMP environments. Recognising this limitation, HKIB partnered with leading international institutions, most notably the Scottish National Blood Transfusion Service (SNBTS), to facilitate technology transfer and specialised training. Local scientists travelled abroad to observe established workflows, including the critical nuances of air handling, laboratory zoning, and personnel movement within GMP-certified facilities; elements that are vital to ensuring the sterility and reliability of cell-based products.

In terms of manufacturing, the team opted for a semi-automated process to enhance precision and compliance while reducing manual variability. This led to a collaboration with Miltenyi Biotec and the adoption of its CliniMACS Prodigy platform, a sophisticated, closed-system technology that streamlines cell processing in a manner consistent with international regulatory expectations.

Crucially, the entire manufacturing and infusion process is now contained within Hong Kong, eliminating the logistical complexity and delays associated with cross-border shipping.

This self-sufficiency, coupled with Hong Kong's open regulatory environment, which allows for the import of critical materials without the bureaucratic constraints seen in mainland China, has created a highly efficient and responsive ecosystem for cell therapy. Nevertheless, establishing and maintaining such a system comes at significant financial cost. Unlike conventional trials, CAR-T studies demand constant environmental control, single-use consumables, and highly skilled personnel, all of which place pressure on operational budgets. To overcome this, the team successfully secured funding from the Hong Kong government's Innovation and Technology Commission (ITC), an agency tasked with advancing research and innovation across academic and industry sectors. This grant has enabled the launch of a clinical trial that will treat up to 20 patients with locally produced CAR-T therapy, an important step toward building a sustainable and sovereign model for advanced therapeutics in Hong Kong.

**What guided your patient selection criteria and dosing strategy for the CAR-T clinical trial, and how have you approached minimising treatment-related risks?**

Patient selection for our CAR-T clinical trial was guided by both the realities of local clinical epidemiology and the ambition to design a study with meaningful translational impact. In Hong Kong, the annual incidence of paediatric acute lymphoblastic leukaemia is modest, with approximately 40 new cases per year, of which 80 to 90 percent can be successfully treated with

conventional chemotherapy. As a result, the number of children eligible for CAR-T remains extremely limited. To ensure sufficient enrolment and broader applicability, we extended the trial to include adult patients with relapsed or refractory lymphoma, who represent the predominant population in global CAR-T studies. Collaborating closely with adult haematologists such as Dr Kenny Lei and Dr Raymond Wong, we designed an inclusive protocol that permits earlier intervention than the current regulatory framework for commercial CAR-T therapies typically allows, where use is often restricted to patients who have relapsed twice or more.

Our rationale is grounded in clinical logic – earlier administration when disease burden is more manageable and the patient’s condition more stable is likely to yield improved outcomes and reduce the risk of complications. Of the five patients treated to date, two paediatric and three adult, both children received CAR-T after a first relapse, which would conventionally have led to stem cell transplantation. While transplantation remains a curative option, its long-term toxicity profile, particularly in children, is far from negligible. Growth impairment, endocrine dysfunction, and radiation-induced secondary malignancies are well-documented risks, and it is precisely these complications we hope to mitigate by offering CAR-T at an earlier point in the therapeutic journey.

At present, our trial protocol involves a single infusion, as cryopreservation has not yet been incorporated into our manufacturing process. Although multiple infusions may offer certain advantages, whether through dose fractionation to improve tolerability or re-dosing to clear residual disease, this remains a question for future investigation. Importantly, the earlier stage at which our patients are being treated has likely contributed to a favourable safety profile, none have required admission to intensive care, a marked contrast to the higher ICU admission rates historically associated with CAR-T therapy in more advanced cases. Our first infusion was administered in August last year, and the paediatric patient continues to do well more than seven months post-treatment.

### **How well does Hong Kong’s healthcare infrastructure support the long-term data tracking required for CAR-T therapy, particularly in paediatric cases?**

Long-term follow-up is a critical component of CAR-T therapy, particularly in paediatrics, where the full scope of therapeutic benefit and potential late toxicities may only become apparent over many years. In Hong Kong, the healthcare infrastructure is well equipped to meet these demands, thanks to its fully digitalised and centralised electronic medical record system which enables secure and efficient access to comprehensive longitudinal patient data. To ensure the consistency and quality

of information captured throughout the study, we have appointed a dedicated research assistant to extract, verify, and consolidate data from both the electronic system and direct clinical observations.

In addition to institutional records, we maintain a secure, standalone research database designed to capture supplementary variables that may not be routinely included in the central system. This dual-track approach ensures the completeness of clinical datasets while adhering to the stringent requirements of our Research Ethics Committee, which mandates data retention for a minimum of 15 years. Such infrastructure is particularly well suited to the demands of CAR-T follow-up, allowing us to monitor patient outcomes over time and assess the emergence of any long-term complications in a rigorous and systematic manner.

### **What options are being explored to ensure continued access to locally produced CAR-T therapy beyond the current clinical trial?**

The future of our locally developed CAR-T therapy is an ongoing point of strategic focus. The current CD19-targeted product is being delivered within the framework of a clinical trial, which, by design, has a defined scope and endpoint. However, given the clear advantages observed – such as improved accessibility, significantly reduced turnaround times, and more manageable costs, we are actively exploring ways to ensure that the therapy can continue beyond this initial phase. The key challenge lies in sustainability, as clinical trial funding is limited to research objectives and does not support long-term provision of the product once the trial is complete.

To move forward, we are evaluating alternative pathways that could support local access in a regulated and financially viable manner. One such option is the hospital exemption model, already in place in several European countries. This framework allows for the use of non-commercial, hospital-produced advanced therapies under defined regulatory conditions. Should a similar pathway be introduced in Hong Kong, and paired with a reimbursement mechanism, it could provide a practical solution for continued patient access.

That said, the cost of maintaining GMP facilities and ensuring consistent product quality remains considerable, with estimates reaching several hundred thousand Hong Kong dollars per patient. Despite these challenges, we remain optimistic that with the right structural and policy support, this homegrown CAR-T platform can evolve into a sustainable and impactful therapeutic option for patients in Hong Kong.

**Where do you see opportunities for Hong Kong to engage in the development of next-generation cell and gene therapies, and what role can commercial partners play in scaling this work?**

With CD19-directed CAR-T therapies offering only moderate cure rates in clinical practice, attention is turning to newer, more potent alternatives. We are actively engaging with companies developing next-generation products and exploring opportunities to manufacture these therapies locally, potentially at HKIB. Early collaboration could allow us to conduct clinical trials that benefit patients while also supporting future product registration through Hong Kong's "1+" regulatory mechanism, which permits local approval based on prior authorisation by a recognised international body, provided that local data and expert input are also in place. From an operational standpoint, our team has acquired valuable hands-on experience with the current CAR-T platform over the past year, including validation runs using donor samples.

While transitioning to new products would require further training, I am confident in their capacity to adapt. Additional funding from commercial partners or institutional sources would allow us to expand the team and scale production. Moreover, Hong Kong's evolving innovation ecosystem and talent-friendly policies create a strong foundation for collaborative development in cell and gene therapy, making it a compelling environment for industry engagement.

**Why is Hong Kong well positioned to emerge as a regional hub for cell and gene therapy development from a clinical and translational perspective?**

Hong Kong offers a robust foundation for the development of cell and gene therapies, driven by its established healthcare infrastructure and experienced clinical research community. Physicians, nurses, and support staff are well-versed in managing complex clinical trials, providing a reliable platform for the introduction and evaluation of novel therapeutic technologies. Alongside this clinical strength, the city also possesses the technical capabilities to manufacture cell therapies locally, supported by dedicated facilities and a growing pool of trained personnel.

However, advancing locally developed innovations from the laboratory to the clinic will require greater investment. Our team, for instance, has designed CAR-T constructs that demonstrate superior activity in preclinical models, yet progressing these candidates into clinical use demands additional funding and support. Discussions are already underway with potential industry partners,

and commercial collaboration, possibly through a spin-off model, may offer a viable path forward.

With the right alignment of scientific expertise, infrastructure, and investment, Hong Kong is well positioned to become a regional leader in cell and gene therapy innovation.

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