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Research
for Impact





“ 守護小生命見成果 ”

Milestones in safeguarding tiny lives



香港母乳庫慶祝投入服務一周年。
The Hong Kong Breast Milk Bank celebrates its first anniversary.

院長的話

近來，香港兒童醫院有兩項守護小生命的關鍵服務，都有令人鼓舞的發展。

首先是我們營運的香港母乳庫，轉眼已投入服務一週年，努力將捐贈母乳送往各公立醫院的新生兒深切治療部，支援早產及危疾嬰兒。醫管局亦憑藉推廣母乳捐贈，奪得「中國公共關係行業最佳案例大賽」及「香港公共關係獎」金獎，肯定了團隊在開拓這全新觀念上的汗水。在這頭一年內，母乳庫共收集到超過2700升母乳，讓400多名脆弱的小生命受惠，成績遠超預期。這反映捐贈者、受惠家庭以至公眾對我們嚴謹程序的信任，確保每滴母乳都安全優質。

Recently, the Hong Kong Children's Hospital has seen encouraging developments in two vital services dedicated to saving young lives.

The Hong Kong Breast Milk Bank which we operate has just marked its first anniversary. We have been working tirelessly to supply donated breast milk to the neonatal intensive care units across public hospitals, supporting premature and critically ill babies. The Hospital Authority's efforts in promoting this new concept of breast milk donation have earned gold awards at both the China Golden Awards for Excellence in Public Relations, and the Hong Kong Public Relations Awards. In this first year, the bank collected over 2700 litres of breast milk, benefiting more than 400 fragile newborns, a result that far exceeded our expectations. This achievement reflects the trust that donors, recipient families and the public place in our rigorous processes to ensure every drop of milk is safe and of the highest quality.

另外，本院一直為醫管局的初生嬰兒篩查計劃提供檢測服務，及跟進異常個案。這項旨在及早診斷嚴重遺傳病或罕見病的服務持續擴大，目前已涵蓋超過30種疾病，並正以先導形式延伸至私家醫院，最新已有三間私院參與。篩查不僅能爭取黃金治療時間，保障孩子健康，長遠更能減輕公營醫療系統的壓力。

感謝醫療團隊的專業，及社會各界的信任。讓我們繼續攜手，為每一位小朋友的健康護航。

醫院行政總監
李子良醫生

HKCH also continues to provide testing and follow-up services for the Hospital Authority's newborn screening programme aimed at the early diagnosis of severe genetic or rare diseases. It has continued to expand, now covering over 30 conditions and being extended to private hospitals through a pilot scheme, with three private hospitals already onboard. By enabling early treatment, this programme not only protects children's health but also reduces the long-term pressure on the public healthcare system.

Thanks to our teams for their professionalism and to the community for their unwavering trust. Together, let's continue to safeguard the healthy future of every child.

Dr Lee Tsz-leung
Hospital Chief Executive

建構

兒科 研究 基地



香港兒童醫院匯聚兩所大學醫學院的科研實力，為下一代的健康尋求突破。
HKCH gathers the research strengths of two university medical faculties to drive life-changing discoveries for the next generation.

Building a hub for paediatric research excellence

「科研」從來不是香港兒童醫院的點綴，而是與臨床服務、教學培訓鼎足而立的**核心支柱**。我們深知醫學進步是對抗疾病的**關鍵**，因此不遺餘力推動研究，立志開拓創新的治療方法，培育新一代醫學科學家，在兒科及遺傳疾病領域不斷尋求突破，試圖在每一次發現中，為病童帶來轉機。

For the Hong Kong Children's Hospital, research is far from a mere embellishment; it stands as a core pillar alongside clinical service and professional training. We recognize that medical advancement is the key to conquering diseases. To this end, we spare no effort in driving research forward, dedicated to pioneering innovative treatments and nurturing the next generation of medical scientists. Through every breakthrough in childhood and genetic diseases, we strive to transform the lives of children battling complex illnesses.

作為教學醫院，我們與大學專家及科研團隊並肩協作。我們擁有得天獨厚的硬件與資源，並集中了最罕見複雜的病例。這些優勢賦予我們無限潛力，持續發展成為香港的科研基地。對身患頑疾的病童而言，香港兒童醫院是希望之光；對有志投身科研的專業人才，這裏是實踐抱負的追夢之所；對醫療業界，則是尋求合作的首選夥伴。

As a teaching hospital, we work hand-in-hand with university experts and research teams. Our state-of-the-art infrastructure and concentration of the most rare and complex cases also provide us with limitless potential as we advance toward the goal of becoming Hong Kong's definitive medical research hub. For children facing persistent health challenges, the Hong Kong Children's Hospital represents a beacon of hope. For professionals dedicated to scientific inquiry, we are a platform to realize their aspirations. For the wider medical industry, we are the destination of choice for impactful collaboration.



醫院科研網頁
HKCH research website

從藍圖到實現：

醫院的科研發展之路

From blueprint to reality: the roadmap for research

早在香港兒童醫院籌建階段，「科研」這顆種子便已埋下。香港兒童醫院科研督導委員會聯席主席蘇志釗醫生從管治、基建到財政角度，暢談各項條件如何相互配合，成就醫院科研發展。

In the Hong Kong Children's Hospital, the seeds of research had been sown before the first brick was laid. Co-chairman of the HKCH Research Steering Committee Dr Jason So talked about how various factors, from robust governance and state-of-the-art infrastructure to strategic funding support, work together to power the hospital's vision toward research excellence.

蘇志釗醫生指：「本院專治複雜罕見病症，如遺傳病及癌症，當中存在對這些疾病的成因和治療效果等許多的未知。科研正是填補這些知識差距的不二法門。無論是基礎或轉化研究，最終目的都是給病人最好的治療，這是我們的使命。」

擁科研專屬基建與資源

本院在設計時特別闢出科研空間，如將教學及科研大樓的八至九樓總樓面面積超過3,700平方米的空間劃為實驗室，供進行基礎研究。蘇醫生說：「這對醫管局來說是史無前例的！」醫院並獲政府撥款，購置儀器和設備，供科研人員共用。這種前瞻性規劃令港大及中大的研究團隊在開院之初得以順利遷入，落地生根。另外，政府的醫療衛生研究基金亦在開院之初委託本院進行多個兒科研究項目，撥款總額達二千萬元。

良好管治作基石

要提升研究質量和獲取信任，需靠穩健的管治。本院成立了科研督導委員會，成員

包括醫院管理層、香港醫學專科學院及兩所大學醫學院代表，負責制訂科研策略及監督資源分配，其轄下幾個委員會則管理不同科研設施。另外，本院支援醫管局中央研究倫理委員會，審批與兒童相關的研究申請，確保符合道德與科學標準。由2019至2025年，經本院批核的申請共568宗。

由於科研規模較大，本院設有科研辦公室。「同事們負責各委員會的行政支援、研究倫理及撥款申請、儀器採購、實驗室日常運作等，令研究團隊工作更加暢順。」蘇醫生說。

把握機遇 發展轉化研究

蘇醫生表示：「有着這些獨特條件，任何人要來香港做兒科研究，兒童醫院理應是不二選擇。」他認為醫院下一步應與國內及其他國家建立更多聯繫，利用周邊龐大人口的優勢，並將研究的管理更加規範化，取得相關機構的認證，從而吸引各方投資和合作，令研究可轉化成新的藥物、檢測及醫療儀器等，造福病人。

科研實驗室配備多種先進儀器，供研究團隊共用，進行廣泛類型的研究。
Equipped with a suite of advanced equipment, the research laboratories provide a shared platform for different teams to conduct a diverse range of scientific studies.



蘇志釗醫生深信醫療知識一日千里，如不能趕上便會落後於人，因此醫院必須靠科研的支撐，才能成為真正的卓越醫療中心。

Dr Jason So believes that medical knowledge advances in a tremendous pace. Research is the backbone that ensures the HKCH can keep up and become a true centre of excellence.

科研實驗室主要設備 Core facilities of research laboratories

- 細胞與組織培養實驗室
Cell and tissue culture rooms
- 生物成像平台（如共軛焦顯微鏡、超高分辨率顯微鏡）
Bioimaging platform (e.g. confocal and super-resolution microscopy systems)
- 流式細胞分析與分選平台
Flow cytometry platform
- 多組學平台（如蛋白質體學分析儀、串聯質譜儀）
Multiomics platform (e.g. proteomic analyzer, tandem mass spectrometer)
- 生物樣本庫與低溫儲存室
Biobank and cryogenic storage
- 生物資訊學與運算平台
Bioinformatics and computing platform

香港兒童醫院設有科研督導委員會，強化科研管治。委員會聯席主席為（左起）蘇志釗醫生、李民瞻教授、張耀輝教授。

HKCH set up the Research Steering Committee to strengthen research governance. Its Co-chairmen are (from left) Dr Jason So, Prof. Albert Li and Prof. Cheung Yiu-fai.



Dr Jason So explained, "HKCH specializes in managing complex and rare diseases like genetic disorders and cancers, where many unknowns remain regarding causes and outcomes. Research is the only way to fill these knowledge gaps. Whether it is basic or translational research, our mission is clear: to provide patients with the best possible treatment."

Designated infrastructure and resources

During the design stage, the hospital specifically allocated zones for research, including more than 3,700 square metres on the 8th and 9th floor of the Training and Research Tower as basic research laboratories. Dr So noted, "This is unprecedented in the

Hospital Authority!" The hospital also received government funding to procure core research equipment to be shared by researchers. This forward-looking planning enabled research teams from the University of Hong Kong and the Chinese University of Hong Kong to smoothly move in in the early days. Moreover, the government's Health and Medical Research Fund invited HKCH to conduct commissioned paediatric research projects, with a total budget of \$20 million.

Good governance as cornerstone

To ensure research quality and trust, robust governance is essential. HKCH Research Steering Committee, comprising the hospital management and representatives from the Hong Kong Academy of Medicine and the two

medical faculties, oversees research strategy setup and resources allocation. There are other committees to manage different research branches. Additionally, HKCH supports the Hospital Authority Central Institutional Review Board in vetting research applications related to children to ensure they meet ethical and scientific standards. From 2019 to 2025, 568 applications were approved via the HKCH.

Such large scale of operations is supported by a dedicated research office. "The team handles everything from administrative support for various committees to ethics and grant applications, procurement and lab operations. This allows our research teams to work more smoothly," Dr So remarked.

Seizing the opportunity for translational research

Dr So said, "With these unique strengths, HKCH is second to none for those looking to conduct paediatric research in Hong Kong." He reckoned that the next steps are to strengthen ties with the Chinese Mainland and other countries, in particular to leverage the vast population of the vicinity, to further standardize research management, and to obtain relevant accreditations. This would attract more investment and collaboration, allowing the translation of research into tangible drugs, diagnostic tools and medical devices that benefit patients.



院方會靈活運用實驗室空間，除現時兩間大學，亦讓其他有興趣及能力進行兒童疾病研究的機構和團隊有機會使用。
The hospital will allocate its laboratory space flexibly so that apart from the current two universities, other institutions and teams with the interest and capability to advance paediatric medicine can also conduct research here.

數據中心的高效能運算集群能處理龐大的研究數據量，協助研究人員進行複雜的分析。
The high-performance computing clusters housed in the data centre can handle massive research data, assisting researchers to perform complex analyses.



匯聚醫學院力量

共創科研機遇

Synergy unlocks research opportunities

香港兒童醫院同時是香港中文大學及香港大學的教學醫院。兩大醫學院的進駐，帶來了專家和資源，亦促進科研人員的交流，達致「一加一大於二」的效果，令醫院的科研實力如虎添翼。

The Hong Kong Children's Hospital (HKCH) is the teaching hospital of the Chinese University of Hong Kong (CUHK) and the University of Hong Kong (HKU). The coexistence of research expertise and resources of the two medical faculties fosters powerful synergy that significantly bolsters the hospital's research capabilities.

分別執掌中大及港大兒科學系的李民瞻教授及張耀輝教授身兼本院的副醫院行政總監，及科研督導委員會聯席主席。兩位均認同，兒童醫院是一座橋樑，提供了空間讓兩間大學的人員共享寶貴科研資源。李教授表示：「醫院匯聚以往分散於各區的罕見複雜病例。不少從前因為樣本不足而難以做到的研究，在這裏得以開展。」張教授指：「當專家與數據集中在同一個平台，研究成果便

更容易轉化為臨床應用，縮短病童等候新治療的時間。」

優勢互補 發揮協同效應

目前，兩間大學合共有超過200名人員在兒童醫院內進行研究、臨床及教學工作。張教授認為，雙方專注的研究範疇不同，各有強項。他指：「跨校、跨專科團隊合作可以取長補短，進行研究時能

Prof. Albert Li and Prof. Cheung Yiu-fai, who head the paediatrics departments of CUHK and HKU respectively, also serve as HKCH's Deputy Hospital Chief Executives and co-chair the HKCH Research Steering Committee. They both view HKCH as a bridge where personnel from both universities can share valuable research resources. Prof. Li noted, "The hospital pools together rare and complex cases

that were previously scattered across different districts. Many studies that were difficult to conduct due to small sample sizes can now take root here." Prof. Cheung added, "With experts and data centralised on a single platform, research findings can be more readily translated into clinical applications, thereby shortening the wait for new treatments for patients."



香港中文大學醫學院兒科學系系主任李民瞻教授認為兒童醫院有頂尖的設施及儀器，鼓勵科研人員勇於創新，登上國際舞台，才能吸引更多資源及合作機會，壯大科研發展。

Prof. Albert Li calls on researchers to fully leverage the world-class facilities at the HKCH, and embrace innovation to make their mark on the international stage. Only then can they attract greater resources and partnerships needed to expand the research horizons.

從不同角度分析問題，達致互助共贏的效果。」

李教授舉例說：「上任後我不斷思考如何將自己專攻的睡眠及呼吸疾病研究帶進不同專科。例如腎科或內分泌科的病童，在一天中的不同時間洗腎或注射生長激素，效果會否有不同？」他期望以自己獨特的身份連繫更多專科，產生更大的協同效應。

Unleashing synergy through collaboration

Currently, there are over 200 university staff members working at the HKCH, engaged in research, clinical service and teaching. Prof. Cheung believes the two faculties have distinct research focuses and strengths. "Cross-university and multidisciplinary collaborations allow us to complement each other. Analysing research problems from different perspectives leads to mutually beneficial outcomes," he said.

Prof. Li shared, "Since taking up this role, I have been exploring how to integrate my own research interests in sleep and respiratory diseases into other specialties. For instance, would the timing of dialysis or growth hormone injection yield different outcomes for renal and endocrine patients?" He hopes to leverage his unique position to connect more specialties and generate greater synergy.

港大中大最近聯合完成了一項研究，針對患有睡眠窒息症的兒童，在切除扁桃體及腺樣體後的心血管健康狀況。這正是兩校以各自的專長為基礎，在本院攜手合作進行研究的實例。

臨床研究中心提升對業界吸引力

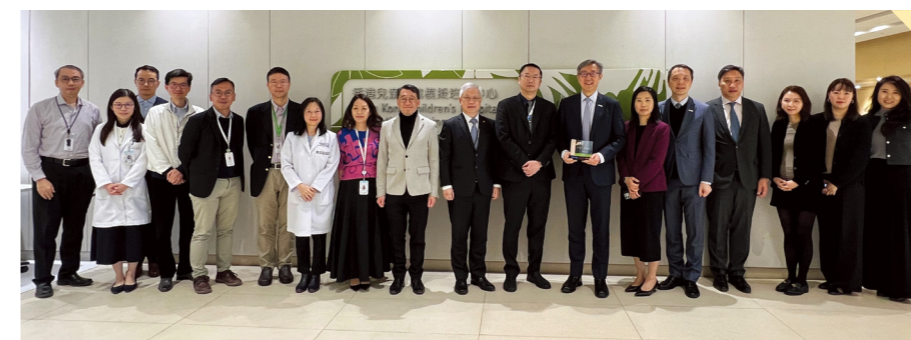
本院設有臨床研究中心，設施包括病床、程序室、訪談室、檢測儀器和臨床研究藥劑部等，為科研活動提供全方位支援。除研究人員發起的醫學研究外，亦可與製藥及生物科技公司合作，進行由業界資助的臨床試驗。

張耀輝教授表示：「中心投入運作初期主要進行一些無創／微創性活動，如超聲波、抽血等。現在已陸續有病人參與一些藥物的臨床試驗，例如基因治療和癌症藥物。」放眼未來，他期望成立專責團隊去統籌各項臨床試驗，循序漸進爭取更多藥物試驗來港。

A recent joint study by HKU and CUHK on the cardiovascular outcomes of children with obstructive sleep apnoea following adenotonsillectomy exemplifies how both universities build on their respective strengths to collaborate in the HKCH.

Clinical Trial Centre boosts industry appeal

HKCH's Clinical Trial Centre provides comprehensive support for research activities. Its facilities include research beds, procedure rooms, interview



引進重點企業辦公室與投資推廣署去年到訪本院，探討合作機會，希望引入更多臨床試驗及創新療法，以提升兒科服務水平，並鞏固香港作為國際醫療創新樞紐的地位。

Last year, the Office for Attracting Strategic Enterprises and Invest Hong Kong visited the HKCH to explore research collaboration opportunities, hoping to introduce more clinical trials and innovative therapies to enhance paediatric care and solidify Hong Kong's status as an international health and medical innovation hub.

香港大學臨床醫學學院兒童及青少年科學系系主任張耀輝教授認為，港大和中大各有歷史文化和強項，科研究人員透過交流合作擦出火花，達致相得益彰的協同效應。

Prof. Cheung Yiu-fai believes that HKU and CUHK possess distinct historical and cultural identities and strengths. Through mutual exchange and collaboration, researchers from both institutions can spark new ideas, achieving a win-win outcome.



位於B座8樓的臨床研究中心設備完善，為各項臨床及藥物試驗提供全面支援。

The Clinical Trial Centre located on 8/F in Tower B is well equipped to provide all-rounded support for various clinical and drug trials.



醫院設有專門管理研究用藥物的設施。其中獲得認證的無菌潔淨室配備負壓隔離器，可配製化療及危險藥物。

There are designated facilities in the HKCH for managing investigational medicinal products. For example, this certified cleanroom is equipped with negative pressure isolators for preparing chemotherapy or hazardous drugs.

rooms, examination equipment, and a dedicated research pharmacy. In addition to investigator-initiated studies, it also facilitates collaboration with pharmaceutical and biotech companies to conduct industry-sponsored clinical trials.

Prof. Cheung stated, "In its early stages of operation, the centre mainly focused

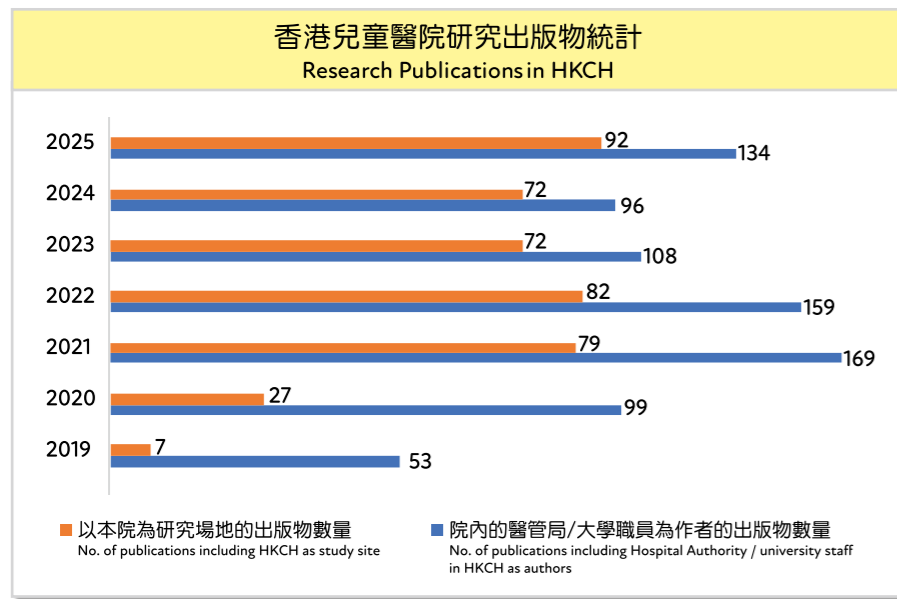
on non-invasive or minimally invasive activities, such as ultrasound and blood tests. Now more patients are participating in clinical trials for medications, such as gene therapy and cancer drugs." Looking ahead, he hopes to establish a dedicated team to coordinate various clinical trials, with the ultimate goal of attracting more drug trials to Hong Kong.

高水準研究帶來

High-caliber research brings new hope

香港兒童醫院是本港兒科醫學發展的重鎮。憑藉這個優勢，醫院各團隊積極進行多項創新及具影響力的研究。下文將介紹其中三個項目，見證科研如何為病人尋找曙光。

HKCH spearheads the development of paediatric medicine in Hong Kong. Various hospital teams are leveraging on this role to pioneer innovative and impactful research. The following highlights three of these projects, demonstrating how research brings new hope for our patients.



搭建平台 試驗港產 CAR-T

本院於2021年引入 CAR-T 細胞治療，醫治兒童急性淋巴白血病。然而，有關產品於海外生產，價格高昂且等候時間較長。本院現正聯同兩所大學的研究人員，分別進行兩項本地製造的 CD19 CAR-T 產品的臨床試驗。

負責其中一項試驗的兒童及青少年科顧問醫生卓家良表示，團隊會研究病童接受治療後的成效及副作用，分析產品的質量。「本地生產能節省時間及成本，如果研究得出正面結果，病人可能會受惠。」

本院設有造血幹細胞移植及細胞治療中心，配備血液成份分離機、超低溫儲存設備、隔離病室等配套，加上全港患有相關病症的病童均集中在本院，為 CAR-T 研究提供有利條件。

卓醫生指需要用到 CAR-T 治療的，都是復發或難治的個案，因此每年人數不多。「將病人集中在兒童醫院，有助醫護人員累積經驗，並更有效善用資源。」他認為，目前香港在相關治療正在跟上國際步伐，期望將來會有更多針對不同病種的 CAR-T 產品在香港面世。

A platform to test HK-made CAR-T products

HKCH introduced CAR-T cell therapy in 2021 to treat children with acute lymphoblastic leukaemia. However, relying on overseas manufacturing results in high costs and extended lead times. HKCH is now collaborating with investigators from two universities to conduct two clinical trials for locally made CD19 CAR-T cell products.

Dr Daniel Cheuk, Consultant (Paediatrics and Adolescent Medicine), is leading one of

the trials which studies the efficacy and side effects of the product and evaluates its quality. "Local production is a game-changer," said Dr Cheuk. "It can significantly cut costs and waiting times, possibly benefitting patients if the research yields positive results."

The hospital's haematopoietic stem cell transplantation and cellular therapy centre provides the conclusive conditions for the research, boasting advanced apheresis, isolation, and cryopreservation facilities, as well as concentration of all related patient cases in Hong Kong.

"Because CAR-T therapy targets refractory and relapsed cases, patient numbers are naturally limited," explained Dr Cheuk. "Centralising these cases allows us to accumulate experience and manage resources effectively." He reckoned that Hong Kong is keeping pace with international standard, and hopes that more CAR-T products for different conditions will be available in Hong Kong soon.

甚麼是 CAR-T 細胞治療?

What is CAR-T cell therapy?

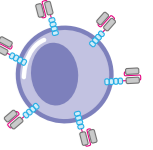
CAR-T 細胞治療是一種免疫療法，透過基因改造病人的免疫 T 細胞，使其能夠識別並攻擊癌細胞。

CAR-T cell therapy is an immunotherapy that genetically modifies a patient's T cells to specifically identify and attack cancer cells.

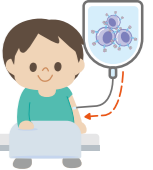
1 從病人的血液中提取 T 細胞
T cells are extracted from the patient's blood



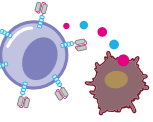
2 在實驗室中將 T 細胞的基因修改，並大量繁殖
T cells are genetically modified in the laboratory and cultured to multiply



3 將 CAR-T 細胞重新輸入病人體內
The CAR-T cells are infused back into the patient's body



4 CAR-T 細胞尋找癌細胞，將之消滅
CAR-T cells seek and attack cancer cells



貢獻香港基因組研究 打破診斷迷思

不少罕見病患者耗費大量時間和精力，仍未能尋獲正確診斷。本院作為香港基因組計劃的夥伴中心之一，正招募患者進行全基因測序，協助探索病因。

醫學遺傳科部門主管陸浩明醫生表示，以往的基因組資料主要來自西方，缺乏亞洲及南中國人的數據。收集本地基因組數據不僅能為病人的臨床診斷和治療提供新方向，還可應用於醫學研究。他提到：「香港兒童醫院

是全港唯一設有醫學遺傳科專科部門的公立醫院。經轉介到基因組計劃的個案年齡層廣泛，病例亦相對複雜，對研究而言非常珍貴。」另外，如果能夠取得患者整個家庭的資料，會有助得出更完整的分析。陸醫生指出：「在兒童醫院裡，我們較容易接觸到患者的父母，安排他們接受檢驗，這對提高診斷率有莫大幫助。」

去年，包括香港兒童醫院在內的人員在醫學期刊《刺針》發表基因組計劃先導階段的研究成果，發現約三分一的參加者接受全基因測序後，能得到全新的診斷結果。陸醫生說：「每做一次測試，病人就多一個機會找到答案。」

Contributing to HK Genome Project to break diagnostic odyssey

For many living with rare diseases, the search for a diagnosis is a long and frustrating journey. As a partnering centre of the Hong Kong Genome Project (HKGP), HKCH is turning tide by recruiting patients for whole genome sequencing to uncover the causes of diseases.

Chief of Service (Clinical Genetics) Dr Luk Ho-ming highlighted that most available genomic data is from the West, leaving Asian and Southern Chinese populations underrepresented. Collecting local genomic data not only provides new insights to clinical diagnosis and treatments, but can also be used in medical research. He mentioned, "Among all public hospitals, only HKCH has a Department of

Clinical Genetics. The cases we refer to the HKGP encompass a wide age range, and they tend to be more complex. These are precious research materials." Also, obtaining genomic data from the entire family is preferred for a more thorough analysis. He explained, "It is easier for us to reach patients' parents here and arrange cascade testing for them, which is a big advantage for raising diagnostic yield."

Last year, HKCH staff co-authored a paper in *The Lancet*, revealing that one-third of participants in the HKGP pilot phase received a new diagnosis after undergoing whole genome sequencing. "With each test, patients have one more opportunity to get an answer," Dr. Luk expressed.



陸浩明醫生期望，本地基因數據庫不僅能幫助病人找出病因，還可為科學家研發新藥物及治療方法，奠定重要基礎。

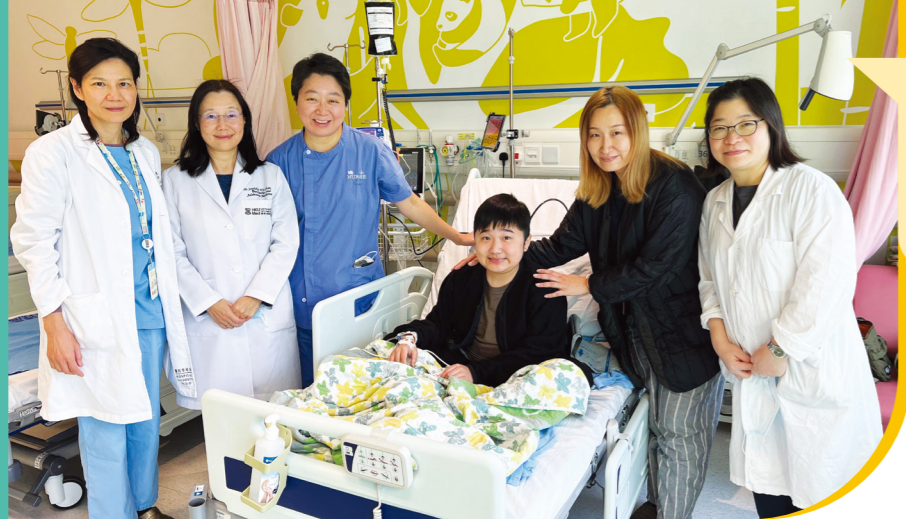
Dr Luk Ho-ming hopes that the local genome database will not only help patients identify the cause of their conditions, but also lay a foundation for scientists to develop new drugs and treatments.

香港兒童醫院參與香港基因組計劃情況

HKCH Participation in HKGP

| | |
|---|--------|
| 轉介家庭數目* No. of families referred* | 8,513 |
| 轉介病人人次* No. of patients referred* | 20,392 |
| 已取得報告病人的診斷率* Diagnostic yield among patients who have received report* | ~30% |

*截至2025年12月 As at Dec 2025
*截至2026年1月 As at Jan 2026



杜興氏肌肉萎縮症患者容孝一(右三)參與基因治療臨床研究。陳凱珊醫生(左二)表示，醫院上下全面配合研究，冀病童受惠。

Yung Hok-yat (third from right), a DMD patient, joined the gene therapy trial. Dr Sophelia Chan (second from left) said the hospital fully supports the research to benefit children.

基因治療研究 為病童踏出重要一步

本院正參與兩項跨國臨床試驗，研究基因治療對杜興氏肌肉萎縮症的成效及安全性。兩項研究分別涉及不同年齡層的病童，目前已有六人成功參加。

基因治療是利用無傳染性的 AAV 病毒作為載體，將經改造的基因帶入病童的肌肉細胞，從而製造正常功能的蛋白質，彌補先天不足，改善受影響肌肉細胞的功能。負責此項目的香港大學兒童及青少年科臨床副教授兼本院榮譽顧問醫生陳凱珊表示，為了進行研究，醫院在軟硬件上都作出全面配合。她舉例：「為確保研究藥物

不受污染，藥劑部特別闢出專用空間去調配藥物，並訂立處理基因治療藥物守則。」另外，所有相關的醫護人員都接受過訓練。「這是一個跨專科項目，腦神經科、心臟科、放射科、物理治療部、藥劑部和護士一同參與治理病人，及幫助他們順利完成臨床試驗和治療後跟進。」

陳醫生憶述，當得悉研究要展開時，便第一時間邀請全港的兒童腦神經科轉介合適病人。「我感受到醫護同事、管理層，甚至政府都上下一心推動研究，為病童找到最有效的治療。」

Gene therapy research - a big leap forward

HKCH is participating in two international clinical trials to study the efficacy and safety of gene therapy for Duchenne Muscular Dystrophy (DMD) across different age groups. Six participants have already successfully participated in the trials.

Gene therapy works by using a non-infective AAV viral vector to deliver modified genes directly into a child's muscle cells, enabling the body to produce normal functional proteins to compensate for the deficiencies and improve the function of affected muscle cells. Dr Sophelia Chan, Clinical Associate Professor of the University of Hong Kong and Department of Paediatrics and Adolescent Medicine and Honorary Consultant of the HKCH, emphasized the rigorous preparations behind the study. "To ensure that the medication is not contaminated, the Pharmacy Department

designated a room for its preparation, and established a new protocol for handling it." Additionally, all relevant staff have undergone training. "This is a true multidisciplinary effort among neurology, cardiology, radiology, physiotherapy, pharmacy, and nursing teams to support patients throughout their clinical trial journey and after the investigatory drug treatment."

When she learned that the trial was set to begin, Dr Chan immediately invited paediatric neurologists across Hong Kong to refer eligible patients. "I felt a united effort from our colleagues, hospital management, and the government to make this research possible to find the best therapy," she said.

藥劑部人員在專用房間調配基因治療臨床試驗的藥物。

In a designated room, a pharmacy staff prepares gene therapy products under clinical trial.

參加者心聲：終於見希望

16歲的容孝一是這項研究的參加者之一。他媽媽容太坦言，一早得知海外有相關技術：「但不覺得關香港事。」直到兒子被轉介至香港兒童醫院，她才看見轉機。「我感受到研究團隊與其他醫生有一個緊密網絡，能識別出適合參加研究的病童。」

孝一及容太感激研究團隊的悉心照顧。「他們耐心解答所有的疑問，甚至有電話可以直接查詢。」他們希望研究完成後跟病友分享經驗，互相支持。

Participant: a glimmer of hope at last

16-year-old Yung Hok-yat is a participant of the trial. His mother admitted that while she had long been aware of the technology overseas, she never imagined it would be available locally. Everything changed when her son was referred to the HKCH. "I could feel the close-knit network between the research team and other doctors," Mrs Yung noted. "They could identify children who are the right fit for the trial."

Both Hok-yat and his mother expressed deep gratitude for the research team's dedicated care. "They patiently answer all our questions. We even have a direct line to call whenever we need help." The family hopes to share their journey with other patients once the study is complete, offering a hand of support.



理想土壤 孕育 科研人才

A fertile ground for nurturing research talent

香港兒童醫院的科研風氣盛行，為有志投身者研究的同事提供優良環境，培養興趣和能力。

The Hong Kong Children's Hospital fosters a vibrant research culture, providing a nurturing environment for staff to pursue research and enhance their capability.

由醫院走進大學

陳宇軒是本院首批聘用的醫生，專治腎病兒童。他年多前轉換跑道，加入香港中文大學，將重心放到研究上。「當接觸越來越多病童，便發覺現有知識不足以理解其病因。看見他們承受治療副作用和失學，我便想返回原點，透過研究找到病因和可行治療，包括引入臨床研究藥物，給他們新選擇。」

他說，醫院在孕育他的科研夢上扮演重要角色，包括資助他到英國深造，不單遇上了科研啟蒙老師，更為他後來領導的跨國性研究鋪路。「我發現英國和香港就一種生物製劑的用法大不相同，觸發我研究怎樣用它去治療兒童腎病綜合症。最終全世界有超過30個兒童腎病中心加入研究網絡，而國際臨床用藥建議亦因我們的研究結果有所修訂。」

醫研雙軌發展拓新路

醫衛局聯同醫管局和兩間醫學院去年推出雙軌發展途徑，讓醫生可同時於公立醫院及大學任職，彈性分配科研與臨床工作時間。

本院有四位醫生參與計劃，卓家良醫生是其中一份子。他認為新聘任安排能作為橋樑，讓他在繁忙的臨床工作中抽時間做研究，亦可用教職員身份直接使用大學的資源。他舉例，大學有多元化的數據分析工具及軟件，和技術人員支援。他亦能與大學研究人員頻繁地切磋砥礪。「兩個角色相輔相成，不時為我的研究及臨床工作帶來新啟發。」

From bedside to bench

Dr Eugene Chan was among the first batch of doctors recruited by the HKCH, and has been specialized in kidney

diseases. About a year ago, he joined the Chinese University of Hong Kong to focus on research. Explaining his transition, Dr Chan said, "As I encountered more and more young patients, I realized that our current knowledge wasn't enough to fully understand why they fell ill. Seeing them endure treatment side effects and miss out on school made me want to go back to the basics. Through research, I wish to uncover the causes and find viable treatments like clinical trial drugs to offer them new options."

He believes the HKCH plays a pivotal role in nurturing his research ambitions. It funded his training in the UK where he met his research mentor, which also paved the way for the international study he later led. "I noticed that a biologic was used differently in the UK. This triggered me to investigate how to use it to treat children with nephrotic syndrome. We eventually built a research network of over 30 paediatric nephrology centres. Our findings led to updates in international guidelines on medication usage."

Dual-track career pathway

The Health Bureau, alongside the Hospital Authority and two medical faculties, launched the dual-track development pathway last year. It allows doctors to hold concurrent positions in public hospitals and universities, giving them the flexibility to balance clinical work with research.

Dr Daniel Cheuk is one of the four HKCH doctors who have been appointed under the scheme. He views the new arrangement as a bridge, carving out space for research within a demanding clinical schedule.



陳宇軒醫生先後獲院方頒發優秀科研員工獎及最佳論文獎。他加入大學的一年內，已參與了三項臨床研究。

Dr Eugene Chan has received the HKCH Outstanding Staff Research Award and Best Research Paper Award. Since joining the university, he has already participated in three clinical trials.



參加雙軌發展途徑的卓家良醫生寄望其雙重身份的優勢，為自己的研究及臨床工作帶來新衝擊，貢獻創新醫療發展。

Dr Daniel Cheuk hopes the synergy between his two roles will drive meaningful breakthroughs and allow him to contribute even more to the future of paediatric medicine.

Beyond just time, it provides him with direct access to university resources like data analysis tools, software and technical manpower, and fosters frequent exchange with academic researchers. "The two roles complement each other, often sparking new ideas that elevate both my research and clinical practice," he said.

玩目挑選指南 好玩助發展

Choosing the right toys

玩具不僅帶來歡樂，更能促進兒童的體能、認知和社交發展。不過，過早引入遠超出能力的玩具會造成挫敗感，挑選符合發展需要及具合適挑戰性的玩具，最有利成長。

Toys not only bring joy, but also promote children's physical, cognitive and social development. However, introducing toys far beyond their abilities may cause frustration. Parents are recommended to choose toys of optimal level of challenge.



職業治療部 Occupational Therapy Division

發展重點 Development focus

0-1 歲
year

感官探索、肢體動作發展、建立物件因果關係
sensory exploration, body movement development, establish object causal relationships

1-3 歲
years

動作協調、手部精細動作、配對概念、寫前準備、模仿
motor coordination, hand manipulation, matching concepts, pre-writing, pretend play

3-5 歲
years

動作協調、精細動作、工具運用、創造力、角色扮演、社交技巧、解難能力、從輸贏中建立挫折承受能力
movement coordination, hand manipulation, tool use, creativity, role-playing, social skills, problem-solving, build up frustration tolerance

6 歲或以上
years or above

動作協調、反應速度、邏輯思維、策略規劃、社交技巧、挫折承受能力、創造力、特定興趣發展
motor coordination, response speed, logical thinking, strategic planning, social skills, frustration tolerance, creativity, develop specific interests

注意安全：避免有小部件（三歲以下）、毛刺、鋒利邊緣或過長繩索的玩具。
Safety precautions: avoid toys with small parts (for children under three), splinters, sharp edges or long cords.

推薦玩具 Recommended toys

布書、對比色圖卡、牙膠、按壓發聲玩具、搖鈴
cloth books, contrasting colour picture cards, teethers, press-to-sound toys, rattles



手推車、球類、積木、穿線玩具、分類玩具、蠟筆、煮飯仔
push carts, balls, building blocks, threading toys, sorting toys, crayons, cooking set



球類、平衡木、拼圖、拼砌式玩具、平衡疊高玩具、手工包、扮醫生玩具、簡單桌遊
balls, balance beam, puzzles, construction sets, balancing stacking toys, craft sets, medical play set, simple board games



球類、跳繩、模型組裝、策略性桌遊、手工藝套裝、科學實驗套裝
balls, jumping rope, model assembly, strategic board games, craft sets, scientific experiment sets



關於玩具的迷思 Myths about toys

用玩具代替陪伴
Use toys to replace companionship

玩具只是媒介，高品質的親子互動才是促進兒童健康成長的關鍵
Toys are just the medium; quality parent-child interaction is key to healthy child development

過度干預和指導
Over-intervention and guidance

給予孩子自主探索和從試錯中學習的機會
Let children explore freely, and learn through trial and error

不斷買新玩具
Keep buying new toys

將部分玩具收起，每一至兩周更換，能让孩子保持新鮮感，更專注探索
Put away some toys and rotate every one to two weeks to keep things fresh, and help children focus on exploring

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