Zongli Zheng – Co-founder and Chair, GenEditBio

Our goal is to develop genome editing therapies that are both highly potent and fundamentally safe.

26.05.2025

Tags: Hong Kong, GenEditBio, Cell & Gene

With global momentum building around CRISPR-based therapeutics, GenEditBio stands at the frontier of in vivo genome editing, developing next-generation delivery platforms designed for both precision and safety. In this conversation, Dr Zongli Zheng, Co-founder and Chair, shares how his scientific path from Boston to Hong Kong, shaped by pioneering work on technologies like GUIDE-seq and new delivery tools, laid the foundation for the company's unique approach.

What scientific work in your journey led to the creation of GenEditBio, and how did your experience with CRISPR shape the company's vision?

My work with Clustered Regularly Interspaced Short Palindromic Repeats (CRISPR) began nearly 15 years ago during my postdoctoral training at Massachusetts General Hospital and Harvard Medical School, supported by the Swedish Research Council. I was fortunate to work with a pioneering Boston-based laboratory focused on precision genome editing. Our efforts centred on enhancing enzyme specificity through protein engineering and developing techniques to accurately detect and quantify off-target effects, unintended edits to similar genomic sequences. These off-target events pose a major safety concern in therapeutic applications, and understanding how to minimise them has long been a central focus of my research.

When I returned to Hong Kong nearly a decade ago, I established my own academic laboratory to continue this work while expanding into in vivo genome editing. Unlike the more complex ex vivo

methods, which involve editing patient-derived cells outside the body and reinfusing them, in vivo approaches allow for the direct delivery of genome-editing agents to tissues that need editing. This method offers a simpler, more scalable, and potentially more accessible therapeutic solution. It also opens new possibilities for targeting diseases where ex vivo methods are impractical or limited.

This scientific direction ultimately led to the founding of GenEditBio, based at the Hong Kong Science and Technology Park (HKSTP). The company was established to translate years of academic innovation into real-world therapies, with a clear focus on safety, precision, efficacy and affordability.

How did returning to Hong Kong enable you to translate academic research into a commercial platform for genome editing?

After completing my postdoctoral fellowship in Boston, returning to Hong Kong felt like a natural next step in establishing my independent research career. Hong Kong offered an ideal environment to expand on my work in precision genome editing while also allowing me to reconnect with my roots. Upon settling in, I continued to refine genome-editing tools and explored in vivo delivery systems, supported by the research infrastructure of the Karolinska Institute from Sweden, which set up the Ming Wai Lau Centre of Reparative Medicine in Hong Kong. These efforts eventually developed into a formal collaboration between the City University of Hong Kong and the Karolinska Institute, where I also hold an academic appointment. This dual-laboratory model provided a robust scientific foundation for what would later become GenEditBio.

The company was founded three years ago as a spin-off from this research platform. I was introduced to who later became our CEO, Dr Tian Zhu, thanks to a mutual connection. Dr Zhu is an experienced entrepreneur with complementary expertise in pharmacology and business development that helped a company through a successful IPO Our partnership was born from a shared vision for the future of medicine, and together, we established GenEditBio with a clear mission: to develop safe, precise, efficacious, and affordable genome-editing technologies capable of addressing serious unmet medical needs.

What advantages does in vivo genome editing offer over traditional methods, and why is it central to your therapeutic approach?

In vivo genome editing holds transformative clinical potential due to its simplicity, versatility, and ability to address disease at its genetic root. Unlike ex vivo techniques, which require harvesting, modifying, and reinfusing cells, in vivo approaches deliver editing components directly into the patient's body, allowing targeted correction within the desired tissue. While this streamlines treatment, it also raises specific regulatory considerations. Most importantly, ensuring that edits are confined to somatic cells and do not affect the germline. Agencies such as the FDA remain particularly vigilant about these safety parameters.

From a scientific perspective, in vivo editing offers two major advantages. First, the platform is inherently programmable. By altering a short guide sequence, the same technology can be tailored to target different genes, enabling potential applications across thousands of rare genetic disorders with high unmet need. Just as significantly, it supports a "one-and-done" paradigm in which a single intervention corrects the underlying mutation at the DNA level, eliminating the need for ongoing treatment. For many chronic or life-limiting conditions, this capacity to resolve disease at its source represents a profound shift in how therapeutics are conceived and delivered.

Also, I would like to emphasise that confidence in the field was significantly bolstered by the FDA approval of the first CRISPR-based therapy in 2023, manufactured by Vertex Pharmaceuticals for the treatment of sickle cell disease. Notably, a key safety assay referenced during the FDA's evaluation was GUIDE-seq, a genome-wide off-target detection method that I co-developed while in Boston. Its incorporation into the regulatory framework underscored both the maturity of the field and the value of robust analytical tools in supporting clinical translation.

What are your lead therapeutic programmes, and how are you approaching early clinical development?

Our lead candidate, currently advancing toward clinical evaluation, targets an ocular disease and represents the most mature programme within a pipeline of five therapeutic assets. The initial trial will be conducted in China under the investigator-initiated trial mechanism – a regulatory pathway that enables early-stage safety and efficacy testing ahead of formal Investigational New Drug filings with global agencies such as the FDA. The study will enrol approximately ten patients in its first year. Ocular indications provide a practical and clinically relevant entry point for in vivo genome editing, given the accessibility of the tissue and the ability to directly observe therapeutic effects.

This programme leverages a proprietary delivery vehicle: a non-replicative viral vector engineered to transport a ribonucleoprotein editing complex directly to the target tissue. Unlike mRNA-based approaches, which require intracellular translation before therapeutic action can occur, our protein-based system is active immediately upon delivery. This eliminates translational delays, reduces molecular instability, and removes the need for chemically modified guide RNAs, which can pose manufacturing and regulatory challenges. By correcting the genetic defect responsible for pathogenic protein accumulation, we aim to achieve a durable, one-time intervention capable of restoring and preserving vision.

What long-term strategy is guiding GenEditBio's evolution from a technology platform to a therapeutic developer?

GenEditBio is intentionally evolving as both a technology platform company and a therapeutic developer, reflecting a model successfully pursued by several pioneering biotech firms in the US. With a proprietary genome editing system at its core and a growing portfolio of therapeutic programmes, the company is well-positioned to pursue multiple strategic avenues. While no singular exit strategy has yet been defined, we are carefully weighing options that include out-licensing select assets as well as advancing internal candidates through early and mid-stage clinical development. Our discussions with leadership and investors have centred on demonstrating the platform's translational potential, and to that end, we are particularly focused on progressing our lead ocular programme into Phase I/II trials. This asset, which combines high potency with no observable off-target effects in preclinical animal studies, has the potential to serve as a benchmark for in vivo genome editing, a space that remains largely clinically unvalidated despite increasing momentum globally. In this context, GenEditBio aims not only to develop novel disease-modifying therapies but also to help define the standards for safety and precision that will shape the future of the field.

What factors make Hong Kong a viable base for biotech growth, particularly in the cell and gene therapy space?

Hong Kong has increasingly positioned itself as a strategic base for biomedical innovation, particularly in the rapidly advancing field of cell and gene therapy. In recent years, the government has introduced a robust framework of support for early-stage companies, combining targeted funding mechanisms with structured incubation programmes. Initiatives such as the MedTech Co-

Create Programme, along with co-investment schemes involving such as the HKSTP, have provided meaningful momentum for companies like GenEditBio. This coordinated public backing has not only enabled early-stage development but has also fostered a strong culture of translational research.

While funding environments elsewhere can often be fragmented or difficult to navigate, Hong Kong's integrated and forward-looking approach has created an ecosystem where early-stage biotech startups can thrive. As cell and gene therapies become increasingly central to the future of healthcare, the city's deliberate investment in this domain positions it as a credible and competitive innovation hub in the region.

How have you structured operational synergies between Hong Kong and mainland China to support end-to-end development?

GenEditBio's operational model is designed to leverage the complementary strengths of both Hong Kong and mainland China. Our upstream research and early development activities are anchored in Hong Kong, where we have established a dedicated scientific team supported by strong institutional infrastructure. For downstream processes such as manufacturing scale-up and later-stage development, we collaborate with Contract Research Organisations (CROs) and Contract Development and Manufacturing Organisations (CDMOs) in mainland China, which offer greater depth and capacity at scale. This collaborative approach enables us to move efficiently through the value chain while maintaining scientific rigour and operational flexibility.

Encouragingly, the Hong Kong government is taking active steps to build a more integrated life sciences ecosystem that spans discovery through to clinical translation. Recent efforts to streamline and enhance regulatory frameworks, such as the introduction of the "1+" approval mechanism and establishment of a drug approval authority based on "primary evaluation" in the long run, signal a clear intention to strengthen the city's role as a fully-fledged biopharmaceutical innovation centre.

When preparing for your Series A, how are you navigating today's funding climate and positioning GenEditBio for investor confidence?

Following successful funding rounds in 2022 and 2024, GenEditBio currently operates with a nearly three-year financial runway. Nevertheless, to accelerate the development of both our technology platforms and therapeutic pipelines, we are preparing to initiate a Series A round in early 2026.

While the broader genome editing sector has experienced an adjustment, driven by an initial wave of excitement and subsequent recalibration around key technical challenges such as delivery and manufacturing, we view this shift as an opportunity to underscore the strength and maturity of our approach. Expectations for the field were understandably high following its early breakthroughs, but investors are now focusing more closely on scalability, safety, and market access.

These are precisely the areas in which we believe GenEditBio is well-positioned to lead, particularly given our in-house chemistry, manufacturing, and controls capabilities and proprietary editing and protein delivery vehicle platforms. Although several existing investors are expected to reinvest, we are actively seeking new sophisticated investors from outside our immediate network, including the US and other global markets. Attracting strong international partners not only brings capital but also serves as external validation of our scientific and business strategies.

Despite current market volatility, we remain optimistic about the long-term outlook and confident in our ability to deliver transformative CRISPR-based therapies.

What final message would you like to share about GenEditBio's mission and technology?

At the heart of GenEditBio's mission is the belief that genome editing therapies can be both highly potent and fundamentally safe. Our proprietary protein delivery vehicle embodies this principle. By combining the precision and immediacy of protein-format editors with the proven delivery advantages of viral vectors, we have created a protein-based platform that avoids many of the limitations associated with nucleic acid-based systems.

Crucially, we are actively building in-house manufacturing capabilities to ensure full control over production quality and scalability, elements that are central to long-term clinical success. Grounded in a scientific background deeply focused on safety, our approach represents a viable and differentiated path forward for the next generation of CRISPR-based therapies. We remain committed to advancing this platform with the goal of delivering safe, precise, efficacious, and affordable one-time treatments for patients with high unmet medical needs.

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