Brad Ringeisen – Executive Director, Innovative Genomics Institute (IGI)



Genome editing must be applied to more diseases, and it must become meaningfully more affordable

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Genome editing is moving from proof of concept to clinical reality, but questions around scale, cost, and responsibility now define its future. In this interview, Brad Ringeisen outlines how the Innovative Genomics Institute is shaping CRISPR as a platform for real-world impact, bridging philanthropy, academia, and industry while navigating regulation, trust, and global competition. The focus is not on isolated breakthroughs, but on what it takes to make genome editing durable, accessible, and transformative.

How would you describe the Innovative Genomics Institute today, both in terms of its mission and its role within the US genome-editing landscape?

I serve as Executive Director of the Innovative Genomics Institute (IGI), which is headquartered at the University of California, Berkeley and operates as a formal multi-campus partnership with the University of California, San Francisco and, since March 2025, UC Davis. Berkeley functions as our central hub, UCSF anchors our health and clinical collaborations, and UC Davis extends our work into agriculture and climate, giving IGI a structure that reflects both the scientific breadth of genome editing and its applications beyond human health.

My background is in physical chemistry. I earned my PhD from the University of Wisconsin-Madison and spent several years at the US Naval Research Laboratory, where I led the Bioenergy and

Biofabrication Section, working on bioprinting, tissue engineering, and early organ-on-a-chip systems. I later joined the Defense Advanced Research Projects Agency (DARPA) in 2016, first as Deputy Director and then as Director of the Biological Technologies Office (BTO), overseeing programmes across genome editing, epigenetics, neurotechnology, and biomanufacturing. That experience provided a broad view of emerging biotechnologies and deepened my exposure to CRISPR, including through DARPA funding that already supported IGI's early efforts to improve the safety and effectiveness of genome editing.

IGI was founded in 2015 by Jennifer Doudna, a UC Berkeley scientist, co-inventor of CRISPR-Cas9, and Nobel Prize laureate in Chemistry. When the Executive Director role opened in 2020, I stepped into the position and have been here for just over five years. Our mission is to translate advances in genome editing, cell therapy, and gene therapy into treatments and technologies that are affordable and accessible. We pursue that through a combination of company formation, nonprofit approaches, and global partnerships, with the consistent objective of ensuring that these innovations move beyond the laboratory and reach the patients and communities that need them most.

How does IGI move discoveries from basic research toward clinical readiness, and how do funding and partnerships support that process?

IGI operates primarily as a philanthropic organisation, with roughly 70 percent of our research supported by philanthropy and the remainder coming from a combination of government grants and industry-sponsored collaborations. Philanthropic funding plays a central role because it allows us to take early scientific risks, whether that involves developing new CRISPR modalities, creating disease models, or exploring therapeutic concepts that are still too nascent for conventional funding mechanisms. Alongside this, we work closely with public agencies, including the National Institutes of Health (NIH) through the Somatic Cell Genome Editing programme, on projects such as Huntington's disease and amyotrophic lateral sclerosis (ALS), in collaboration with teams at UC Berkeley, UCSF, and external partners.

From those early discoveries, we pursue several translation pathways. One is company formation. Since IGI was founded, our research has contributed to the launch of more than 30 start-ups, which together have raised approximately USD 5.2 billion in private capital. In that model, our role is to incubate and de-risk the science using philanthropic and public funding, and then step back once a company is positioned to scale independently. A second pathway is direct collaboration with

industry. A key example is the Danaher-IGI Beacon for CRISPR Cures, which brings together IGI's innovation with Danaher's manufacturing and industrial capabilities to build a repeatable pipeline for genome-editing therapies. Initial programmes focus on rare inborn errors of immunity, including Artemis-deficient severe combined immunodeficiency and familial hemophagocytic lymphohistiocytosis (HLH), where IGI scientists and UCSF clinicians are advancing CRISPR-based approaches toward clinical development.

We also pursue nonprofit models of clinical translation. With support from the Chan Zuckerberg Initiative, we launched the Center for Pediatric CRISPR Cures with UCSF and UC Berkeley, building on recent success in delivering a personalised CRISPR therapy to an infant with a rare metabolic disease. The Centre aims to develop treatments for children with severe genetic conditions that currently lack viable options, while establishing a repeatable process that can be extended to other ultra-rare disorders. Across all of these efforts, the objective is consistent: to generate robust safety and efficacy data for regulators, work with manufacturers to reduce production costs, and advance in vivo delivery approaches that shorten timelines and lower expense, so that genome-editing therapies can move beyond one-off breakthroughs and become accessible treatments rather than million-dollar interventions available to only a few patients.

From your perspective, where does the gene-editing field currently stand in terms of maturity and readiness for broader clinical adoption?

The field has reached a meaningful inflection point. In 2023, the FDA approved Casgevy for sickle cell disease, demonstrating that a CRISPR-based therapy can meet regulatory standards for safety and efficacy. That progress accelerated further in 2025 with the first personalised in vivo CRISPR gene-editing treatment delivered to an infant at the Children's Hospital of Philadelphia (CHOP). In that case, a child born with CPS1 deficiency, a rare metabolic liver disorder, received a base-editing therapy that was designed, manufactured, and administered in roughly six months. It showed that, under the right conditions, it is now possible to move from diagnosis to treatment on timelines that would have been unthinkable only a few years ago, particularly when regulators and developers can build on delivery technologies, such as lipid nanoparticles, that are already well understood.

At the same time, that success also highlights the constraints that remain. Even for a single patient, extensive preclinical work in animal models was still required, which is expensive and difficult to scale. The core challenge now is how to accelerate this process safely while reducing cost. One promising direction is to think less in terms of isolated, one-off programmes and more in

terms of platforms. If multiple diseases share the same delivery system and editing machinery, and the primary variable is the guide RNA, there may be opportunities to reduce regulatory and development burdens once safety and delivery are well characterised. Those conversations are actively underway with regulators, including whether clinical trial designs could evolve to cover groups of related diseases rather than starting from scratch for each new indication.

The longer-term ambition is for genome editing to mature into a true platform technology. From the outset, Jennifer Doudna articulated the goal of CRISPR becoming a standard of care, potentially replacing interventions such as liver or bone marrow transplantation where appropriate. Achieving that vision will depend on continued accumulation of safety and efficacy data, clear demonstration of patient benefit, and sustained progress in lowering complexity and cost. The promise is real, but it remains early, and translating cautious optimism into broad clinical impact will require disciplined execution, regulatory evolution, and continued clinical experience across the field.

How would you assess the current level of federal investment, regulatory engagement, and policy support for genome-editing technologies in the United States?

There is still some uncertainty around long-term funding, particularly as the NIH continues to define its priorities, but there are clear signs of constructive engagement. One encouraging example was the CPS1 case at the CHOP, which NIH, within the US Department of Health and Human Services (HHS), publicly highlighted as an NIH-supported effort with relevance beyond a single patient. From a regulatory standpoint, the fact that a personalised in vivo gene-editing therapy was able to move forward at all indicates that the FDA is actively working with the field, even as regulatory standards and frameworks continue to evolve.

We are also seeing momentum from ARPA-H, which has adopted a high-risk, high-reward model influenced by DARPA. IGI is involved in an ARPA-H-funded programme led by Cytiva, part of Danaher, focused on engineering CAR-T cells directly in the body using non-viral lipid nanoparticle delivery combined with CRISPR genome editing. That collaboration brings together Danaher operating companies and academic partners, including UCSF, with the aim of improving scalability and reducing manufacturing complexity, particularly for solid tumours. While this is not a uniform surge in funding across the field, it does signal that parts of the US government see genome editing as a potential platform technology and are beginning to align resources with that longer-term ambition.

How can the field build trust around genome editing, given ethical considerations, cost pressures, and uneven progress across programmes?

Trust is built through transparency and consistency, and there is no alternative to that. Sickle cell disease illustrates this clearly. The approval of Casgevy was a major milestone, but it immediately exposed challenges around access, cost, and the complexity of centre-based treatment. At the same time, it demonstrated something essential: a CRISPR-based therapy can be safe, effective, and meet regulatory standards. The task now is to build on that foundation by developing approaches that are easier to deliver, more scalable, and ultimately more accessible, which is where much of the current clinical innovation is focused.

At IGI, transparency is a core principle. As an academic innovation centre, we publish our work, share data openly, and make tools and approaches available so others can build on them. From the beginning, Jennifer Doudna embedded a Public Impact programme into IGI, recognising that genome editing operates within society, not just within the laboratory. That team works alongside researchers to address ethics, governance, regulatory strategy, and affordability early in development, while engaging patients, clinicians, manufacturers, payers, and policymakers to ensure that approval can translate into real-world access.

The final pillar is rigorous oversight. Every programme operates within established safeguards, including Institutional Review Board (IRB) review, careful risk-benefit assessment, and informed consent, particularly for first-in-human or highly experimental interventions. You move forward only when the potential benefit clearly justifies the risk and when patients or families fully understand what is being proposed. Taken together, transparency, early ethical engagement, and disciplined adherence to these frameworks are what allow innovation to advance responsibly and, over time, build durable trust with regulators, patients, and the public.

How does the United States compare globally in genome editing as investment and innovation accelerate in other regions?

From where I sit in the Bay Area, the United States still stands as the strongest innovator in biotechnology, including genome editing, but the gap with other regions is clearly narrowing. Over the past decade, China has moved quickly, supported by substantial public and private investment, while Europe has also become more assertive, with companies such as Novo Nordisk and BioNTech and increasingly dynamic innovation hubs in cities like London and Paris, particularly at the intersection of artificial intelligence and biotech. In that sense, the global landscape is becoming

more competitive overall, with multiple regions advancing in parallel rather than a single clear leader pulling away.

What continues to differentiate the United States is a deeply embedded willingness to take technological risk. Science is difficult and often unforgiving, but there remains a strong belief here in committing fully to ambitious ideas and pushing through setbacks based on the conviction that they can ultimately change lives. That mindset is reinforced by the entrepreneurial ecosystem, especially at institutions like UC Berkeley, which consistently ranks among the leaders in venture-backed start-ups. On a daily basis, we see students, postdocs, and graduate researchers who are focused not only on discovery, but on translating their work into companies that can scale and deliver real-world impact.

At the same time, this is a critical moment for sustaining US leadership. Continued strength depends on stable public and private funding, policies that support entrepreneurship rather than constrain it, and a predictable environment for biomanufacturing and innovation. Uncertainty, whether on the government or investment side, quickly slows momentum and translates into less activity in the lab. Talent is another growing pressure point. The United States has long benefited from attracting and retaining top global researchers, but increasing opportunities abroad and greater friction around long-term retention risk weakening that advantage. How funding stability and workforce competitiveness are addressed will play a decisive role in whether the US can maintain its leading position in genome editing in the years ahead.

Looking ahead to the next three to five years, what are IGI's top scientific and strategic priorities, and what needs to happen for genome editing to scale sustainably?

At its core, the path forward is defined by two requirements. Genome editing must be applied to more diseases, and it must become meaningfully more affordable. If those conditions are met, the technology can scale and deliver lasting impact. That is where IGI plays a distinct role as an innovation engine within UC Berkeley. Depending on the measure, IGI accounts for roughly 15 percent of the university's annual invention disclosures and related intellectual property filings, reflecting a sustained focus on developing new tools, applying them to the hardest problems in biology and medicine, and moving the strongest ideas forward through translation and company formation.

The unmet medical need remains substantial. More than 7,000 rare diseases have been identified, yet only a small fraction are currently treatable, making affordability and scalability central

challenges. Oncology is another priority. While cell therapies and immunotherapies have reshaped outcomes in certain blood cancers, solid tumours remain largely resistant to these approaches. We are seeing early evidence that CRISPR and genome-editing technologies, whether through improved cell therapies or entirely new mechanisms, could begin to make inroads in solid tumours. At the same time, neurological and neurodegenerative diseases represent what many consider the final frontier for genome editing, with active efforts across conditions such as Huntington's disease, frontotemporal dementia, amyotrophic lateral sclerosis, and potentially Alzheimer's and Parkinson's disease.

Progress across these areas will depend on advances in in vivo and systemic delivery, including the ability to cross the blood-brain barrier, target bone marrow and haematopoietic stem cells, or reach specific immune cell populations, alongside manufacturing approaches that can bring costs down. That same logic applies to the private sector. Therapies that cost millions of dollars and reach only a handful of patients each year are difficult to sustain, clinically or commercially. Expanding access means treating more patients, across more diseases, at lower cost. As a nonprofit academic centre, IGI's contribution is to generate high-quality data that helps de-risk these technologies, providing a stronger foundation for start-ups and investors and supporting the broader transition of genome editing from experimental promise to scalable clinical reality.

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