Title: A Framework for Delivering a Globally Accessible Gene Therapy
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The convergence of innovative biotechnologies is propelling the field of gene therapy with consequential implications for treating and curing genetic and infectious diseases globally. Precise genetic alteration of specific cells opens the door to preventing, treating, or even curing a range of currently intractable diseases. Although dozens of gene therapies for a broad range of disease areas are expected to receive regulatory approval worldwide over the next decade, patients are unlikely to receive equitable access to these medical breakthroughs. In particular, the sophisticated infrastructure required to deliver gene therapies pose critical challenges for low and middle-income countries (LMICs) seeking to integrate gene therapies into resource-constrained health systems. Without critical foresight and targeted investments across LMICs, gene therapies will perpetuate global health inequity. Using gene therapies for sickle cell disease and human immunodeficiency virus (HIV) in sub-Saharan Africa as use cases, this project examines the necessary infrastructure required for effectively and sustainably delivering gene therapies in low-resource settings. This project approaches gene therapy infrastructure from five areas: engagement and education; facilities and manufacturing; information systems, workforce, and regulation. Although assessed individually, these domains are interdependent, highlighting the need to invest and co-develop across all areas simultaneously and synergistically.