



**MAKING  
GENETIC THERAPIES  
AFFORDABLE AND  
ACCESSIBLE**

# EXECUTIVE SUMMARY

We have arrived at a new frontier in medicine. Cell and gene therapies hold the promise of targeted treatments - potentially even cures - for an array of devastating and life-altering diseases. While innovation in biomedicine continues, many novel therapies may never reach patients despite the existence of clinic-validated components. To accelerate affordability and access to genetic therapies in the United States and globally, we assembled a Task Force to delineate the challenges but also concretize opportunities and alternative avenues that can make high-cost therapies affordable, and move new therapies from bench to bedside.

This report is the conclusion of a yearlong deliberation by 30 individuals with expertise spanning from preclinical development of genetic therapies to healthcare economics, intellectual property rights, and biomanufacturing, with the goal of identifying concrete steps to make genetic therapies affordable and accessible. Genetic therapies hold the potential of transformational health outcomes, yet at prices surpassing \$3M, affordability and access are of significant concern to patients and payers alike. We evaluated alternative approaches to developing and deploying a genetic therapy that would reach more patients. We discussed how a non-traditional entity would be organized and financed and how it might price a genetic therapy. We also scoped manufacturing efficiencies and identified strategies for intellectual property (IP) and pricing.

The three decade history of cell and gene therapy shows that academic institutions are the primary originators of novel therapeutic strategies and typically accept government and philanthropic grants to conduct research, generating significant intellectual property. In turn this IP is licensed to for-profit organizations who further develop the product. This model belies a contradiction for academic institutions; while most have a public benefit mission, which supports making final products generated with university IP affordable and accessible, they generate valuable income from licensing intellectual property and are reasonably concerned about requirements that would deter licensees. We believe that changes to intellectual property licensing practices are one of the easiest/first changes that academic institutions can take to promote access. We propose that academic institutions should impose reasonable requirements in licenses that ensure access to life-saving therapies. Some recommendations include explicitly supporting academic technology transfer offices (TTOs) in activities to improve affordability and access, consideration of non-exclusive licenses particularly in low- and middle-income countries, and the development of access plans that identify how the product will reach patients without private insurance or facing other barriers to access.

With regard to organizational models that can operate in parallel to publicly traded, for-profit companies, Task Force members first evaluated existing, non-traditional, pharmaceutical entities. They determined a mixed organizational model comprising an academic institution, a nonprofit medical research organization (MRO), and a public benefit corporation (PBC) could be an ideal structure. The MRO would accept funding from grants and private philanthropy to conduct research, it could concentrate intellectual property, conduct clinical trials, and generate further income by selling priority review vouchers from FDA approvals. Subsequently, the MRO could license core

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technology to a PBC, which could price a drug based on the cost of goods and labor to manufacture, plus some surplus to ensure sustainability. For example, a PBC could manage manufacturing, distribution, and negotiations with payers. The PBC would also be charged with fundraising and expanding sources of revenue by working with socially-oriented VC firms and seeking early investment from payers or offering services.

Lastly, we would like to acknowledge that manufacturing a genetic therapy to stringent regulatory standards is a key driver of cost. While entities currently developing therapies need to comply with existing regulations, the FDA has shown an impetus to update regulatory requirements to make products more accessible. In particular, we expect that increased regulatory support for point-of-care manufacturing models would drive down prices and allow greater geographic access while not reducing the safety or efficacy of the treatments. We provide examples where other governments, who have supported point-of-care manufacturing models, have increased affordability.

In the year since we initiated this report several companies have decided to either delay or discontinue further development of genetic therapies in their pipeline, some for explicit business reasons. From our analysis, it seems that in addition to challenging manufacturing and delivery mechanisms, the need to generate enough capital to recoup investments is confounding. We present concrete actions that academic institutions and downstream stakeholders can take to address these issues, allowing more therapies to enter the market and thereby improve access through competition.

Each section in this report begins with an executive summary and recommendations for that section, then delves into background on the topic followed by a conclusion. We also include a section on actionable policy recommendations and provide illustrative examples of an implementation strategy at the end.

A challenge of this magnitude requires a wide range of stakeholders to implement innovative solutions while, for the sake of equity, not seeking maximum profit. We hope this report builds a robust foundation for these and similar solutions to take hold.