NSCEB
National Security Commission on Emerging Biotechnology

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Bringing Rare Disease Treatments to Patients

Addressing America's most difficult diseases through an all-of-government strategy to advance biotechnology.

Emerging biotechnologies enable doctors to not just treat the symptoms of a rare disease but cure the disease itself. With over 10,000 identified rare diseases, the vast majority of which lack approved treatments, rare disease patients and families acutely experience the hurdles to discovery and delivery of transformative cures.[1] For example, patient populations are small and difficult to operationalize for traditional trials and there is little existing data on how rare diseases progress. Those impacted by rare disease look to the federal government to fund, accelerate, and regulate emerging biotechnologies to enable cutting-edge personalized medicines and beat the odds for themselves and their families in a race against time.

Developing novel rare disease treatments in the United States is critical to improving U.S. health outcomes, as well as ensuring U.S. national security. The current system supporting the development of these treatments is slow, expensive, and not well suited for the scale-up of today's innovative therapeutics. Meanwhile, China now accounts for a significant share of global biopharmaceutical innovation fueled by accelerated clinical trial pathways and active recruitment of Chinese patients.[2] Unless the U.S. government adopts the necessary policies to catalyze its own domestic biotechnology industry, China's competitive edge in biopharmaceuticals may soon expand into an insurmountable lead and point of geopolitical leverage.

Following the release of its April 2025 report, the National Security Commission on Emerging Biotechnology (NSCEB) did a deep dive with rare disease stakeholders on how its recommendations and policy options could accelerate cures for every American with a rare disease.[3] The following document outlines the roadblocks that rare disease researchers and treatment developers face and what policymakers can do to better enable cutting-edge treatments for over 30 million affected Americans.[4]

Common and Unique Challenges to Developing Innovative Therapeutics for Rare Diseases

The U.S. biotechnology ecosystem leads the world in developing cures for rare disease, but without a unified national strategy to fund, develop, and regulate new therapies for patients, that lead is eroding. The U.S. government must address real hurdles to therapeutic development for rare disease to unlock innovations in the sector and heal Americans. These include:

Investment in research and development (R&D).

Many future cures develop well upstream of an innovative product. Sustained R&D into molecular tools that engineer biology unlock new therapies, while research into new approach methodologies can hasten and cheapen pre-clinical trials. In addition, more research is needed for new statistical models that would be more appropriate for small patient populations as they currently lack sufficient evidence to ensure safety and accuracy, making it difficult for regulators to approve modern, scientifically appropriate standards and methodologies for rare disease.

Regulation of novel biotechnologies.

The variability in small patient populations also makes clinical trials difficult to regulate. In trials with large eligible populations, researchers can select patients with similar disease progressions to study the effects of a new product. The limited patient pool for rare diseases, and the associated costs of clinical trials often not adequately covered by insurance, results in a smaller number of qualifying participants. This challenge requires innovative statistical models, novel trial designs, or individualized standards per patient to account for variance in disease progressions, all of which take time and FDA regulatory flexibility.

Data collection and availability.

To develop a new treatment, researchers must understand the natural course of a disease. This is difficult with rare diseases, where patient populations are small and variations between patients are statistically larger. The Food and Drug Administration (FDA) has not standardized its approach to dealing with this variability, leaving developers uncertain and driving patients to self-organize, self-fund, and collect these data on their own.

Manufacturing and scale-up of new therapeutics.

Effective treatments are often personalized to the patient, requiring individualized and expensive manufacturing conditions that necessitate FDA review. Regulatory offices are often understaffed and unfamiliar with the manufacturing protocols for the new therapeutic, adding to the cost and lab-to-patient delay of a treatment. Even if the FDA has seen a similar therapeutic manufactured before, there is currently no clear standard for using the previous data and analysis to speed the review of manufacturing a new iteration of that treatment.

Other countries are successfully addressing or avoiding these roadblocks for rare disease treatment, driving more innovators to develop their treatments in countries like China and Australia.[5] U.S. patients with a rare disease are currently losing out on both potential treatments during clinical trials and the downstream therapeutics.

Next Steps for Policymakers

Policymakers can take concrete steps to overcome the abovementioned challenges and ensure U.S. patients get the innovative treatments they need. The following recommendations and policy options are primarily derived from the Commission's April 2025 Report, *Charting the Future of Biotechnology*, and January 2026 Discussion Paper, *The Future of Biotechnology Regulation: Modernizing Medical Biotechnology Regulation*.[6]

April 2025 Report Recommendations

Recommendation 1.1a

Establish a National Biotechnology Coordination Office (NBCO).

The NBCO would promote federal coordination and streamline the regulatory structures currently inhibiting biotechnology innovation, ensuring a whole-of-government strategy to advance innovations essential for treating and curing rare disease.

Recommendation 2.3b

Establish a biopharmaceutical manufacturing center of excellence.

This center would advance biopharmaceutical manufacturing methods to improve reliability and efficiency, engage regulators to ensure alignment with regulatory requirements, and provide workforce training. Each of these functions would help standardize and improve the manufacturing of medicines essential for the treatment of rare disease.

January 2026 Medical Biotechnology Regulation Discussion Paper Policy Options

Policy Option 1

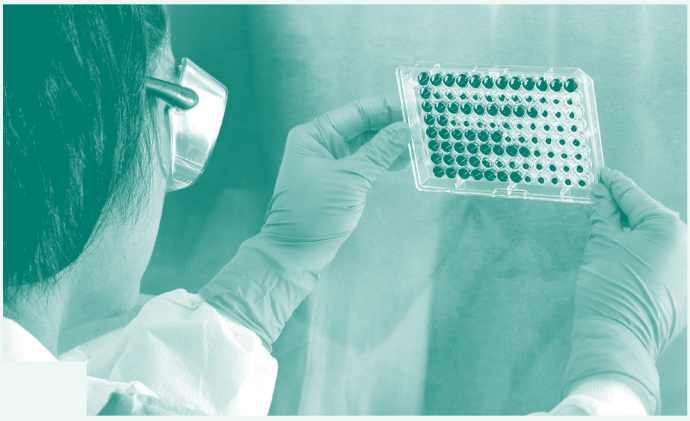
Finalize and expand the platform technology designation.

Biotechnology enables developers to rapidly build multiple therapies from the same well-characterized platform, including gene therapies common in the treatment of rare disease. Congress should instruct the FDA to finalize its draft platform technology guidance and establish a cross-center platform technology designation with uniform criteria, explicit carryover of validated data, and shared standards.

Policy Option 5

Validate modern testing methods.

New approach methodologies (NAMs) can generate safety and efficacy data faster and at lower cost than traditional animal studies. Lower development costs can make therapies for rare disease more economically viable, helping bring treatments to patients overlooked due to the sample population size already reducing the return on investment for developers. Congress should direct the FDA and the National Institutes of Health (NIH) to establish clear, science-based validation pathways for NAMs and other predictive tools to support innovation while maintaining a high bar for safety.



Policy Option 7

Allow trial designs for small populations.

Promising therapies often stall not because they are unsafe or ineffective, but because the required trial structure is mathematically or logistically impossible when only a small number of patients exist. Congress should clarify that developers can meet the requirement for "substantial evidence" through other scientifically valid trial designs when large trials are not feasible.

Policy Option 10

Remove barriers to insurance cost sharing.

Congress should consider new payment models and evaluate existing cost sharing laws to better serve patients. This would help lower costs for early-stage trials, improve predictability for developers, and support continued innovation in medical biotechnology. Reducing clinical trial costs would particularly help small patient populations by ensuring more patients could participate in clinical trials.

New Policy Option

Develop and publish resources for rare disease advocates.

Developers of innovative treatments for rare disease often struggle to fulfill data collection requirements for small patient populations, due to the cost and logistical burden. Patient advocacy organizations (PAGs) attempt to bridge this gap by collecting initial natural history studies and biomarker data for rare diseases, as this data provides the baseline for identifying disease improvement. The NIH and FDA should support this initial collection by providing formal training and financial support for PAGs wishing to navigate the regulatory and grant process.

Policy Options 11-13

Hire, train, and retain regulators.

Persistent staffing shortages and knowledge gaps limit the FDA's ability to review emerging technologies. Review teams often lack the necessary expertise in rare disease, cell and gene therapy, and data science. The FDA should target workforce gaps with existing tools, tie career progression to continuing education, and strengthen the FDA's internal policy capacity.

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Staff at the National Security Commission on Emerging Biotechnology authored this paper with input from the expert Commissioners. The content and recommendations of this white paper do not necessarily represent positions officially adopted by the Commission.