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# NIH, FDA ANNOUNCE GENE THERAPIES CONSORTIUM TO FOCUS ON RARE DISEASES

November 02, 2021

The National Institutes of Health (NIH) and Food and Drug Administration will partner with 10 pharmaceutical companies and five non-profit organizations to accelerate the development of gene therapies through the newly launched **Bespoke Gene Therapy Consortium (BGTC)**. The consortium aims to optimize and streamline the gene therapy development process to help fill the unmet medical needs of people with rare diseases. NIH and private partners will contribute approximately \$76 million throughout the course of the next five years to support BGTC-funded projects.

BGTC researchers will examine the biological and mechanistic steps involved in adeno-associated virus (AAV) vector production, vector delivery of genes into human cells and how therapeutic genes are activated in target cells. These results will provide information for improving the efficiency of vector manufacturing and enhancing the overall therapeutic benefit of AAV gene therapy. The program will also develop a standard set of analytic tests to apply to the manufacture of viral vectors made by consortium researchers. Such tests could be broadly applicable to different manufacturing methods and make the process of developing gene therapies for very rare conditions much more efficient. Additionally, the BGTC will explore methods to streamline regulatory requirements and processes for the FDA approval of safe and effective gene therapies.

## Related News

### FDA Approves First Drug to Prevent aGVHD

December 17, 2021

The Food and Drug Administration approved abatacept (Orencia, Bristol Myers Squibb) yesterday to prevent acute graft versus host disease (aGVHD) in combination with certain immunosuppressants.

### White House Hosts Inaugural Maternal Health Day of Action

December 15, 2021

Vice President Kamala Harris issued a nationwide call to action to combat maternal morbidity and mortality Dec. 7 as part of the Biden Administration's inaugural White House Day of Action on maternal health.

### CMS Halts Vaccine Requirement for Health Care Workers Following Injunctions

December 8, 2021

The Centers for Medicare and Medicaid Services (CMS) has suspended enforcement of its recent COVID-19 vaccine mandate for health care workers while there are court-ordered injunctions in place prohibiting its enforcement.



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OUR SOCIAL MEDIA



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