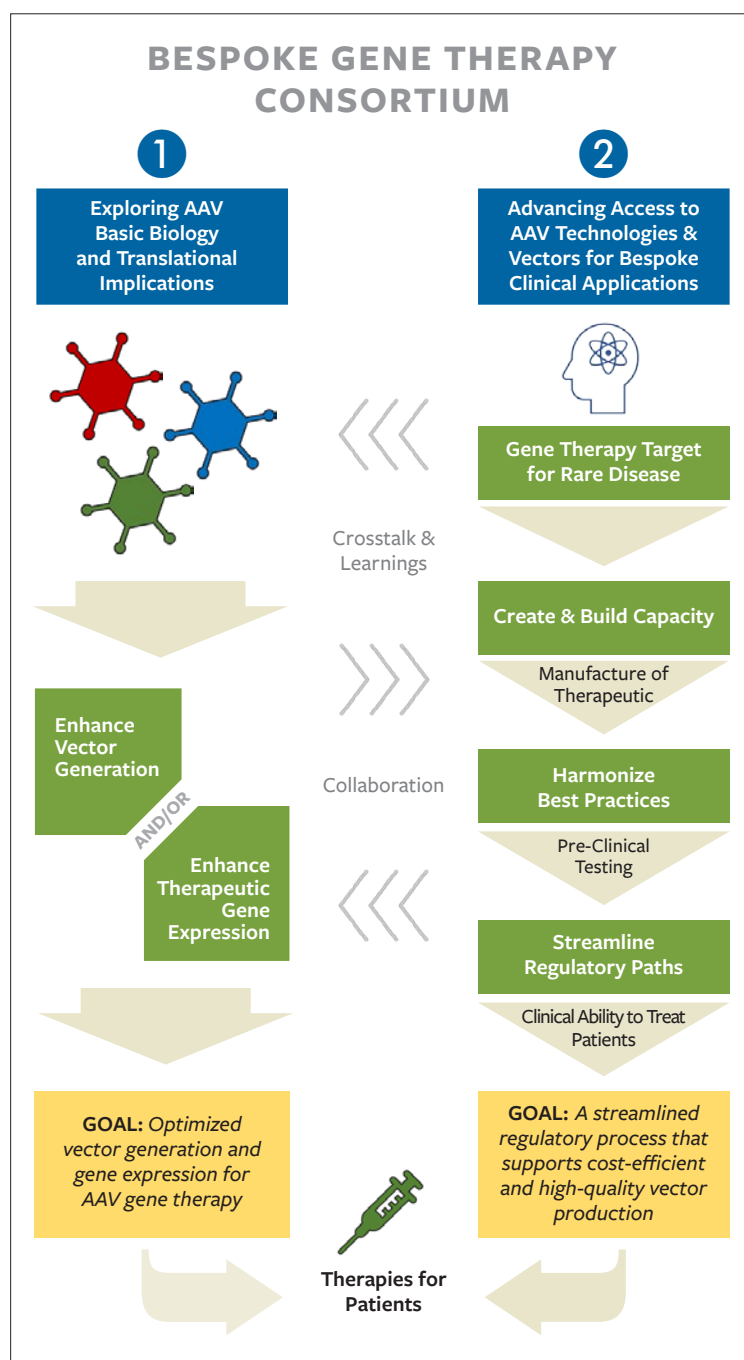


Addressing an Unmet Need

Over 30 million people in the United States live with the devastating effects of rare diseases. There are more than 10,000 rare diseases, and over 80% are caused by genetic defects. These patients frequently lack access to effective treatment, as knowledge about many rare diseases and funding for research often lags behind more common diseases.

For the rare genetic diseases caused by a single defective gene, one promising treatment to emerge is adeno-associated virus (AAV) gene therapy. This process replaces the defective gene with a functional one. Gene therapies have been successfully used to treat genetic diseases and have received U.S. Food and Drug Administration (FDA) approval for human use (e.g., retinal dystrophy). These gene therapies can be tailor-made, or “bespoke,” for an individual or a very small population. Still, the development process is complex, expensive, and hampered by a lack of common scientific, manufacturing, and regulatory standards.

The Bespoke Gene Therapy Consortium (BGTC) aims to make gene therapy more accessible by creating a platform approach to deliver novel therapies for many different genetic disorders. The BGTC is one of the latest initiatives to emerge from the Accelerating Medicines Partnership® (AMP®) Program, a public-private collaboration among the NIH, the FDA, the pharmaceutical industry, non-profit organizations and patient organizations—coordinated by the FNIH—to speed drug development across different diseases. At a cost of over \$90 million spanning six years, the BGTC is the most significant effort of its kind to streamline the development of therapies for rare diseases where currently there is no commercial interest.



The Two Critical Pathways of BGTC Research

Advancing Understanding of AAV Biology for Gene Therapy

While AAV technology is a popular platform for delivering functional genes to cells, many aspects of the underlying biology could be better understood and optimized for

treating rare diseases. The partnership will support a series of research projects to improve transgene expression and AAV manufacturing.

Building the Tools to Standardize and Streamline Processes for Development of Bespoke Gene Therapies

Leveraging up to eight clinical trial test cases and the unparalleled combined expertise of Consortium partners, BGTC will develop a clinical development manual, or “playbook,” for developing gene

therapies for very rare diseases. This playbook will include templates and uniform analytical requirements to create a repeatable process for development of AAV-mediated gene therapies.

BGTC Goals

- Make adeno-associated virus technology more accessible to a broader range of diseases
 - Optimized AAV vector production protocols
 - Improvements in AAV target gene expression
- Streamline preclinical and product testing
 - Harmonized and validated sets of manufacturing and pre-clinical testing requirements
- Facilitate scientific and regulatory advances that will ultimately benefit the entire AAV gene therapy field
 - Standardized regulatory submission package templates
- Bring gene therapies to all affected populations sooner
 - Clinical development manual to help advance all future AAV gene therapies

Public-Sector Partners

- California Institute for Regenerative Medicine (CIRM)
- National Center for Advancing Translational Sciences (NCATS)
- National Institute of Neurological Disorders and Stroke (NINDS)
- National Institute of Mental Health (NIMH)
- National Human Genome Research Institute (NHGRI)
- National Heart, Lung and Blood Institute (NHLBI)
- Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD)
- National Eye Institute (NEI)
- National Institute on Deafness and Other Communication Disorders (NIDCD)
- National Institute of Dental and Craniofacial Research (NIDCR)
- National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS)
- The Brain Research Through Advancing Neurotechnologies® (BRAIN) Initiative, a trans-NIH initiative involving 10 NIH Institutes and Centers
- U.S. Food and Drug Administration (FDA)

Private-Sector Partners

- Alliance for Regenerative Medicine (ARM)
- American Society of Gene & Cell Therapy (ASGCT)
- Biogen Inc.
- CureDuchenne
- Danaher Corporation
- Elpida Therapeutics
- Foundation Fighting Blindness
- GENETHON
- Janssen Research & Development, LLC
- National Organization for Rare Disorders (NORD)
- Novartis Institutes for BioMedical Research
- Ovid Therapeutics Inc.
- Pfizer Inc.
- REGENXBIO Inc.
- Rett Syndrome Research Trust
- RTW Charitable Foundation
- Spark Therapeutics
- Takeda Pharmaceutical Company Limited
- The National Institute for Innovation in Manufacturing Biopharmaceuticals (NIIMBL)
- Thermo Fisher Scientific
- Ultragenyx Pharmaceutical

Private-Sector Partners provide financial support for this program.

ACCELERATING MEDICINES PARTNERSHIP and AMP are registered service marks of the U.S. Department of Health and Human Services.,

For more information contact:

👤 **Courtney Silverthorn, PhD**
Associate Vice President,
Translational Science
✉️ csilverthorn@fnihi.org

👤 **Brad Garrison, MBA, PMP**
Senior Project Manager,
Translational Science
✉️ bgarrison@fnihi.org

👤 **Juan Esparza-Trujillo, M.S., GLPCP, GMPCP**
Project Manager,
Translational Science
✉️ jesparza@fnihi.org

🌐 fnihi.org/BGTC

