

## Resources for Patient Groups or Advocates

Rare disease patient groups and advocates interested in the Bespoke Gene Therapy Consortium may find the following resources informative and helpful.

### Rare Disease Umbrella Associations

Rare disease umbrella associations provide information, resources, and services to help individuals living with a rare disease and their caregivers as they navigate the medical and research landscape. Through their websites and dedicated service teams, these organizations can assist with finding disease-specific information, including research studies and clinical trials. They also can help make connections to other families or patient groups that may provide further information and support services, as well as to clinical centers of excellence and individual experts. In addition, umbrella associations offer programs to assist patients or caregivers interested in starting a rare disease nonprofit or expanding the capabilities of existing organizations to help advance research for a disease or condition.

#### The National Organization for Rare Disorders (NORD)

- 🌐 [rarediseases.org](http://rarediseases.org)
- ✉ [orphan@rarediseases.org](mailto:orphan@rarediseases.org)
- 📞 (203)744-0100
- 📞 Patient Call Center: (800)999-6673

#### Genetic Alliance

- 🌐 [geneticalliance.org](http://geneticalliance.org)
- ✉ [info@geneticalliance.org](mailto:info@geneticalliance.org)
- 📞 (202)966-5557

#### Global Genes

- 🌐 [globalgenes.org](http://globalgenes.org)
- ✉ [careaboutrare@globalgenes.org](mailto:careaboutrare@globalgenes.org)
- 📞 (949)248-7273

### Other Resources for Disease-Specific Information

These resources can help patient advocates learn more about organizations, research, and care for specific diseases.

- **ClinicalTrials.gov** is a searchable database of open clinical trials. Advocates can search their disease of interest to find clinical trial opportunities.
- The **Genetic and Rare Diseases (GARD) Information Center** is a program of the National Center for Advancing Translational Sciences (NCATS) and is funded by two parts of the National Institutes of Health (NIH): NCATS and the National Human Genome Research Institute (NHGRI). GARD provides the public with access to current, reliable, and easy-to-understand information about rare or genetic diseases in English or Spanish.
- **NIH Reporter** is a searchable database of NIH-funded research. Advocates can search their disease and related biological terms to find researchers with relevant projects or expertise.

## Rare Disease Research Resources

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Rare disease research takes place in a complex ecosystem, and patient advocates can take on important roles.

- The [NCATS Toolkit for Patient-Focused Therapy Development](#) is a curated, organized collection of hundreds of online resources that can help patient groups understand and support the process of developing a therapy or cure for their disease.

## Learn More About Gene Therapy

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Gene therapy treats disease by replacing a malfunctioning gene causing the disease, with a working version of the gene using a delivery system often called a “vector.” There are many resources to help patient advocates learn more about gene therapy.

- The American Society for Gene and Cell Therapy (ASGCT) [Patient Education website](#) contains an excellent selection of educational resources.
- The Food and Drug Administration’s Center for Biologics and Research (FDA CBER) [Patient Engagement Program](#) has held multiple events to help patients understand opportunities to engage the FDA and advance the development of gene therapies, including recorded [workshops](#) and [webinars](#).
- [Healing Genes](#) is a jargon-free resource to learn about the science, benefits, limitations and possible risks of therapeutic gene medicine technologies provided by the ARM Foundation.
- NORD maintains [a collection of gene therapy resources](#), including fact sheets, videos, recorded presentations, podcast episodes and publications.
- Global Genes [Platforms of Hope Series](#), developed in partnership with the NIH, explains recent developments and advances in the gene therapy field, providing information of interest for diseases that are not suited to traditional AAV-based gene therapy.

For more information about the Bespoke Gene Therapy Consortium (BGTC), contact:

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