The Food and Drug Administration (FDA) released a draft guidance for industry, *Human Gene Therapy for Retinal Disorders* [4], in July 2018. This document provides recommendations for product development, preclinical testing, and clinical trial design for *gene therapy* [6] (GT) products designed to treat retinal disorders.

The FDA provides three general areas of recommendations:

1. Considerations for product development: This section sets out guidelines for evaluating the identity, purity, quality, dose, and safety of the GT. Prior to beginning clinical trials, the FDA recommends a potency assay in order to test for such outcomes. Many GTs are delivered by injections, and therefore other specifications for drug development include setting levels for dose, volume, and toxicity limits;
2. Considerations for preclinical studies: Once a therapy has been developed, the FDA provides recommendations to validate the *proof of concept*. [7] These validation experiments include *in vitro* and *in vivo* proof-of-concept studies, biodistribution studies to determine *pharmokinetics* [8], toxicology studies, and animal studies. Incorporating these different studies can determine the local and systematic toxicities, the dynamics of the drug’s clearance from different tissue sites, and the immune response to the therapies; and
3. Considerations for clinical trials: The FDA provides further recommendations to evaluate the effectiveness, feasibility, and safety of new GTs. Some clinical trial recommendations include carrying out natural studies to characterize the disorders, choosing adequate and ethical controls, conducting dose-ranging studies, and determining appropriate endpoints (e.g., visual acuity or rate of photoreceptor loss). Other recommendations include considering safety and patient experiences in order to develop a comprehensive risk/benefit analysis.

These recommendations aim to help with efficient trial design and accelerated FDA approval of GTs.

*Retinal disorders* [9] are conditions that can lead to vision loss or blindness; these disorders typically result from either genetic or age-related factors. For example, one group of retinal disorders, *diabetic retinopathy* [10], is *partially based in genetic factors* [11] and leads to vision loss. This is because genetic mutations in insulin-producing genes can lead to *diabetes* [12], which produces high blood sugar levels in the cell. These high levels of sugar can damage small blood vessels in the retina by exerting pressure on them. Since some retinal disorders have a genetic component, these disorders are targets for gene therapy. Thus, the FDA provides recommendations to help gene therapy stakeholders develop safe, efficient, and scientifically-backed therapies.

**SciPol Summary authored by**
Miara Humayun, MA Candidate[13]

**Related News**

- Gene Therapy in U.S. Is On Track for Approval as Early as Next Year[14]


  READ MORE [14]
The promise of gene therapy for Eli and Ella, but not Calliope Joy

Philly.com / The Philadelphia Inquirer – By the time 2-year-old Calliope Joy Carr was diagnosed with an incurable degenerative brain disease, two children with the same deadly ailment were being offered a tenuous lifeline.

Gene Therapy in a Box

MIT Technology Review – Gene therapy is moving quickly from experiment to medical reality. But scientists are worried that the technology is so complex that patients will not benefit as quickly as they should because of a shortage of trained technicians and suitable facilities.

Related Engagements

Write for SciPol.org

SciPol.org invites faculty, researchers, policymakers, and students to consider how they can communicate how scientific research is integrated into the policymaking process. One way is by contributing to SciPol.org as a volunteer author or editor of our original policy development briefs and other related content.

Organize A Citizens Group

Many philanthropic or community science organizations around the country have made it their mission to improve their local community through science and science advocacy. By joining or starting a local science community group, you can find a way to extend your impact beyond the bench.

Consult with NGOs

Non-governmental organizations (NGOs) and nonprofit organizations (NPOs) are public organizations whose primary function is to serve the public good over the pursuit of profits.

Source URL: https://scipol.duke.edu/track/83-fr-32302-human-gene-therapy-retinal-disorders-draft-guidance-industry-availability

Links