Human Gene Therapy for Retinal Disorders (Draft Guidance for Industry)

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The Food and Drug Administration (FDA) released a draft guidance for industry, Human Gene Therapy for Retinal Disorders [4], in July 2018.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. These validation experiments include in vitro in vivo proof-of-concept studies, biodistribution studies to determine pharmacokinetics [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.

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