CELL & GENE THERAPY INSIGHTS

NAVIGATING THE GLOBAL REGULATORY LANDSCAPE

SPOTLIGHT

SPECIAL REPORT

FDA Resources for Cell and Gene Therapy Product Development

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In the USA, the FDA Office of Cellular, Tissue, and Gene Therapies (OCTGT) is principally responsible for regulation of cell and gene therapy products. Based in FDA's Center for Biologics Evaluation and Research, OCTGT's perspective on regulating cell and gene therapy products reflects the growth and development of the field, as well as FDA experience reviewing these complex biologics. OCTGT guidance documents and the webinar series OCTGT Learn are invaluable resources for cell and gene therapy product development.

GUIDANCE DOCUMENTS

The FDA's regulatory expectations and advice regarding cell and gene therapy products are outlined in a growing set of guidance documents. Selected guidance documents are listed in Table 1, grouped topically into three main categories – preclinical, manufacturing, and clinical. Four of these guidance documents together represent the foundation of FDA's regulatory approach to cell therapy and gene therapy products, and should be read by anyone involved in developing these products. Considerations for the Design of Early-Phase Clinical Trials of Cellular and Gene Therapy Products (June, 2015) provides a synthesis of FDA's advice on issues to address in early development, with references to other key guidance documents on preclinical, clinical, and manufacturing aspects. Preclinical Assessment of Investigational Cellular and Gene Therapy Products (November, 2013) discusses design, conduct, and interpretation of preclinical pharmacology/toxicology studies. Cell therapy product manufacturing and testing is covered in Content and Review of Chemistry, Manufacturing, and Control (CMC) Information for Human Somatic Cell Therapy Investigational New Drug Applications (INDs) (April, 2008), and separately for gene therapy products in Content and Review of Chemistry, Manufacturing, and Control (CMC) Information for Human Gene Therapy Investigational New Drug Applications (INDs) (April, 2008).

TABLE 1.

US FDA guidance documents: cell and gene therapy products.
Preclinical Pharmacology/Toxicology
Preclinical Assessment of Investigational Cellular and Gene Therapy Products (November, 2013)
Manufacturing and Testing
Content and Review of Chemistry, Manufacturing, and Control (CMC) Information for Human Gene Therapy Investigational New Drug Applications (INDs) (April, 2008)
Content and Review of Chemistry, Manufacturing, and Control (CMC) Information for Human Somatic Cell Therapy Investigational New Drug Applications (INDs) (April, 2008)
Potency Tests for Cellular and Gene Therapy Products (January, 2011)
Testing for Replication Competent Retrovirus in Retroviral Vector Based Gene Therapy Products and During Follow-up of Patients in Clinical Trials Using Retroviral Vectors (November, 2006)
Determining the Need for and Content of Environmental Assessments for Gene Therapies, Vectored Vaccines, and Related Recombinant Viral or Microbial Products (March, 2015)
Assay Development for Immunogenicity Testing of Therapeutic Proteins (December, 2009)
Eligibility Determination for Donors of Human Cells, Tissues, and Cellular and Tissue-Based Products (August, 2007)
Minimal Manipulation of Human Cells, Tissues, and Cellular and Tissue-Based Products (December, 2014)
Clinical
Considerations for the Design of Early-Phase Clinical Trials of Cellular and Gene Therapy Products (June, 2015)
Gene Therapy Clinical Trials - Observing Subjects for Delayed Adverse Events (November, 2006)
Human Cells, Tissues, and Cellular and Tissue-Based Products (HCT/Ps) from Adipose Tissue (December, 2014)
Preparation of IDEs and INDs for Products Intended to Repair or Replace Knee Cartilage (December, 2011)
Clinical Considerations for Therapeutic Cancer Vaccines (October, 2011)
Cellular Therapy for Cardiac Disease (October, 2010)
Considerations for Allogeneic Pancreatic Islet Cell Products (September, 2009)

OCTGT LEARN

The FDA webinar series OCTGT Learn is an educational resource for cell and gene therapy product developers and investigators, discussing key regulatory topics, and providing insight into topics of particular concern to FDA OCTGT. These are points FDA OCTGT wants to make sure you understand. Selected OCTGT Learn webinar titles are shown in Table 2.

AFFILIATION

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TABLE 2.

Selected OCTGT Learn webinars.

Webinar	Topic
Target Product Profile	Role of the Target Product Profile in facilitating product development
Early-Phase Trials of Cellular and Gene Therapies	Clinical risks of cellular and gene therapy products, and considerations for design of early-phase trials
Fast Track (FT) for Products Regulated in OCTGT	OCTGT experience and advice regarding Fast Track requests and designation for cell and gene therapies, and other OCTGT-regulated products
Regulatory Obligations for Investigator-Sponsored Research	Regulatory requirements for an investigator who is also a sponsor of an IND or IDE
Pediatric Clinical Trials	Regulation and design of pediatric clinical trials
IND Safety Reporting	FDA safety reporting requirements for products under IND
Data Monitoring Committees	Data Monitoring Committees - establishment, operation, and responsibilities
Endpoint Assessment and Adjudication Committees	Circumstances in which an Endpoint Assessment and Adjudication Committee (EAAC) may be useful, charter and operation of an EAAC, and potential bias in endpoint assessment
Successful Development of Quality Cell and Gene Therapy Products	Guidance for successful development of quality cell and gene therapy products
Cellular Therapy Products	Information needed for cell therapy product INDs
The Chemistry, Manufacturing and Controls (CMC) Section of a Gene Therapy IND	Elements of the IND CMC section for gene therapy products
Preclinical Considerations for Products Regulated in OCTGT	Preclinical considerations for cell and gene therapy INDs
"361" Human Cells, Tissues, & Cellular and Tissue Based Products (HCT/Ps)	Definition of HCT/Ps and how they are regulated
IND Basics in OCTGT	IND submissions to OCTGT
Sponsor Meetings with OCTGT	Types of sponsor meetings with OCTGT