ASGCT and FDA Liaison Meeting

February 23, 2024
1:30-3:30 PM ET

U.S. FOOD & DRUG ADMINISTRATION

OTP Overview and Key Initiatives

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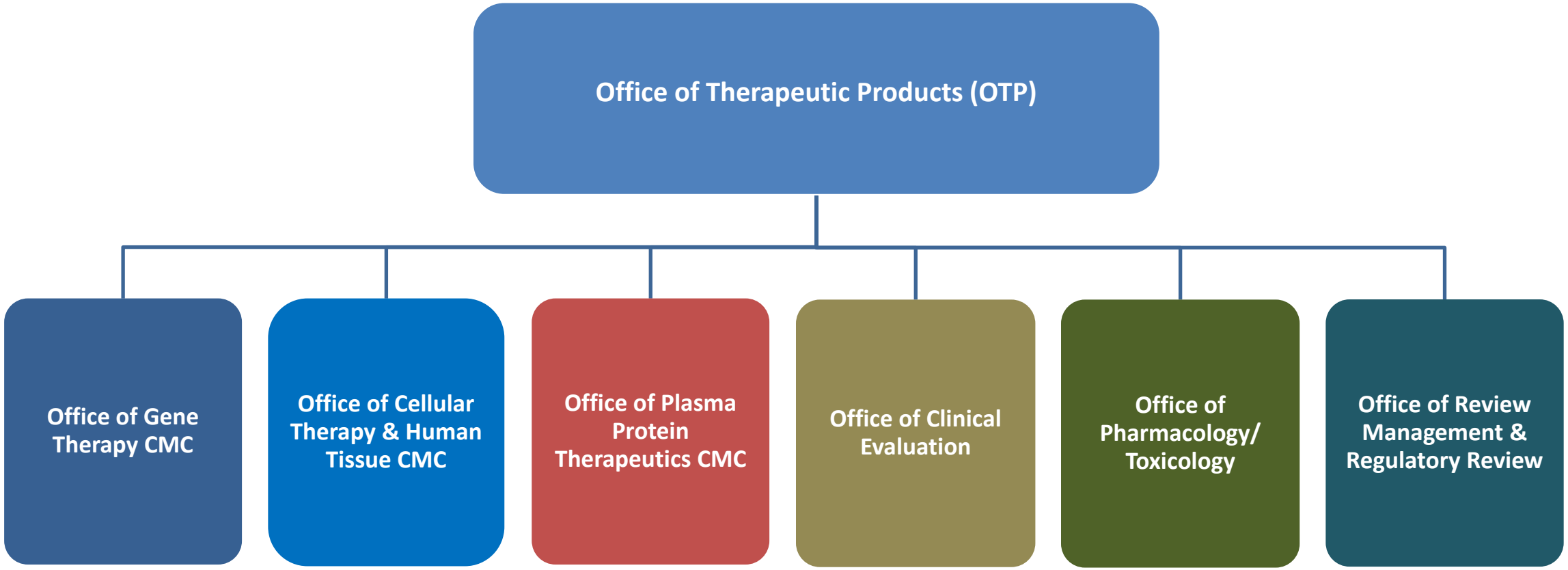
OTP Organizational Chart



Actions to Advance the Field

- Work to more clearly define the use of accelerated approval for gene therapy
- Exploring concurrent submission and product review with other regulatory authorities
- Communication pilots

Support for clinical Trials Advancing Rare disease Therapeutics (START) Pilot

- **Goal**: Further accelerate the pace of development for products that are intended to address an unmet medical need as a treatment for a rare disease
- Products eligible (CBER)
  - Existing OTP-regulated IND for a cellular or gene therapy
  - Product is being developed toward a marketing application
  - Product is intended to address an unmet medical need as a treatment for a rare disease or serious condition
  - Likely to lead to significant disability or death within the first decade of life

START Pilot

- Selection: select up to three eligible products in the initial iteration
- More products may be selected upon evaluation of the pilot
- Enhanced communications if selected for the pilot:
  - An initial meeting to review features of the pilot program
  - Additional ad hoc interactions via email or tcon on a scheduled and/or as needed basis as agreed upon by the sponsor and FDA

START Pilot Timelines

- Federal Register Notice: September 29, 2023
  - Additional details noted
- Request to participate: January 2 – March 1, 2024
- FDA acknowledgment of the receipt of the request: within 14 days of receipt of request to participate in pilot
- FDA notification to sponsors for acceptance: by May 30, 2024

RDEA

- Seek to advance rare disease drug development programs by providing a mechanism for sponsors to collaborate with FDA throughout the efficacy endpoint development process.

- Promote innovation and evolving science by sharing learnings on novel endpoint development through FDA presentations, guidance documents, public workshops, and a public-facing website.

- Develop FDA staff capacity to enable and facilitate the development and use of novel endpoints to evaluate the efficacy of rare disease therapies.

RDEA

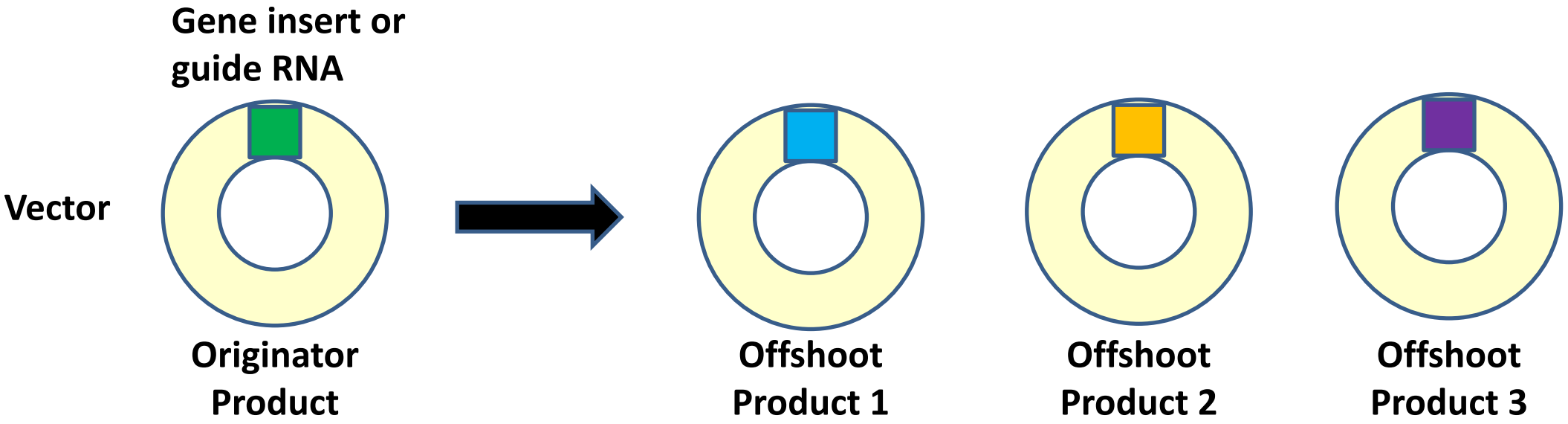
- Admitted sponsors may request up to 4 meetings with FDA to discuss development of their proposed novel endpoint.

- FDA's advice provided during and between RDEA meetings does not constitute a regulatory decision and is considered non-binding.

- Completing the 4 RDEA meetings does not guarantee approval for a regulatory submission that includes efficacy endpoints discussed during RDEA meetings.

- After completion of four RDEA meetings, the sponsor can request additional input from FDA, as needed, through other formal meeting mechanisms, such as Type B, Type C, Type C Surrogate Endpoint, or Type D meetings.

Platform Technologies



Premise

- In appropriate situations, non-clinical data and manufacturing information from one product may be able to be leveraged to another

Additional Tools For Development in the Pipeline- Platform Designation

- Acceleration of gene therapy product development for rare diseases
- Example: Same AAV backbone, different rare disease
- Same sponsor needed
- Goal: Minimize redundancy and expedite development

International Regulatory Collaboration in Gene Therapies for Rare Diseases: CoGenT (Collaboration on Gene Therapies) FDA/CBER Pilot Program Proposal

Participating in this pilot will lead to:

- Increased dialogue among the regulatory authorities, as well as the sponsor,
- Opportunities to optimize product development avoiding unnecessary duplication of effort
- The potential for regulatory convergence among the participants.

- Regulatory Authorities that are Founding or Standing Regulatory Members of the ICH are eligible to participate

- Eligible candidate products should be

  1) a human gene therapy, including genetically modified cells, that lead to a sustained effect on cells or tissues, and

  2) is a product intended to address an unmet medical need as a treatment for a rare disease or serious condition, at any stage of regulatory development.

  Each regulatory authority may at any time offer one or more candidate products for consideration in the pilot. If the product is at early stages of development, the collaboration may be limited to certain aspects of a regulatory submission(s).

Summary

- FDA is committed to working to support the development of novel cell and gene therapies and look forward to forging collaborations and partnerships.