Cell and Gene Therapies: FDA's Initiatives in Accelerating Product Development

27th Symposium on the Interface of Regulatory and Analytical Sciences for Biotechnology Health Products (WCBP 2023)

Parallel Session 2 - Cell and Gene Therapy – New Frontier and Our Best Hope to Cure

Washington DC, January 24, 2023

Ramjay Vatsan PhD CQA

DCGT/OTAT/FDA

Outline Of The Talk

Introduction
- Cell and Gene Therapy Product Types
- FDA approvals
- Increase in INDs
- BT/RMAT Designations

New Guidance for CGT products (a brief summary)
- CMC guidance (2020)
- Genome Editing guidance (2022)
- Chimeric Antigen Receptor T (CAR T) guidance (2022)

PDUFA VII Commitments
- CMC expectations have not changed
- Compressed Development Timelines
- FDA proposes to assist sponsors meet the CMC requirements by enabling additional meetings

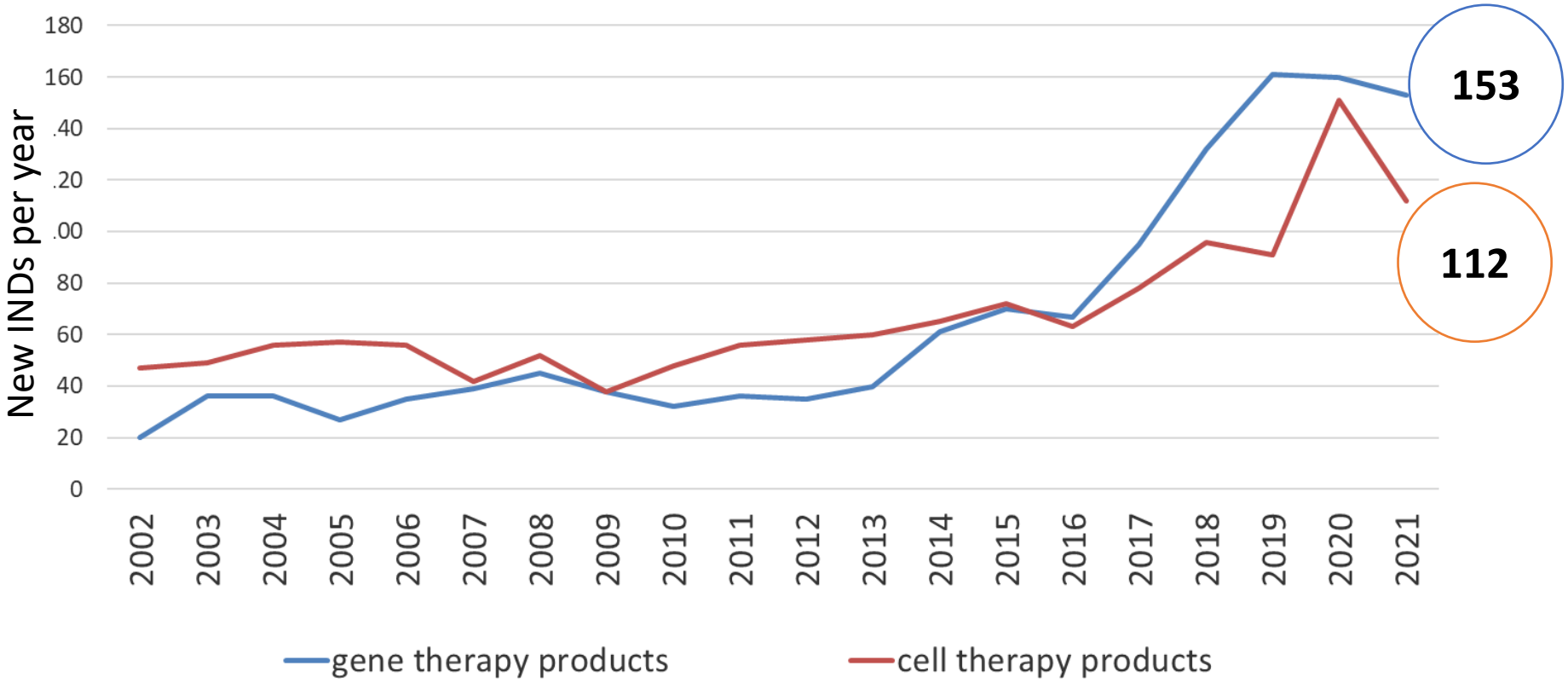
Summary
- Townhall meetings
- Office of Tissues and Advanced Therapies is expanding to meet the demand

Diversity of OTAT-Regulated Products

- **Gene therapies (GT)**
  - Ex vivo genetically modified cells
  - Non-viral vectors (e.g., plasmids)
  - Replication-deficient viral vectors (e.g., adenovirus, adeno-associated virus, lentivirus)
  - Replication-competent viral vectors (e.g., measles, adenovirus, vaccinia)
  - Human genome editing products
  - Microbial vectors (e.g., Listeria, Salmonella)
- **Stem cells/stem cell-derived**
  - Adult (e.g., hematopoietic, neural, cardiac, adipose, mesenchymal)
  - Perinatal (e.g., placental, umbilical cord blood)
  - Fetal (e.g., neural)
  - Embryonic
  - Induced pluripotent stem cells (iPSCs)
- **Products for xenotransplantation**

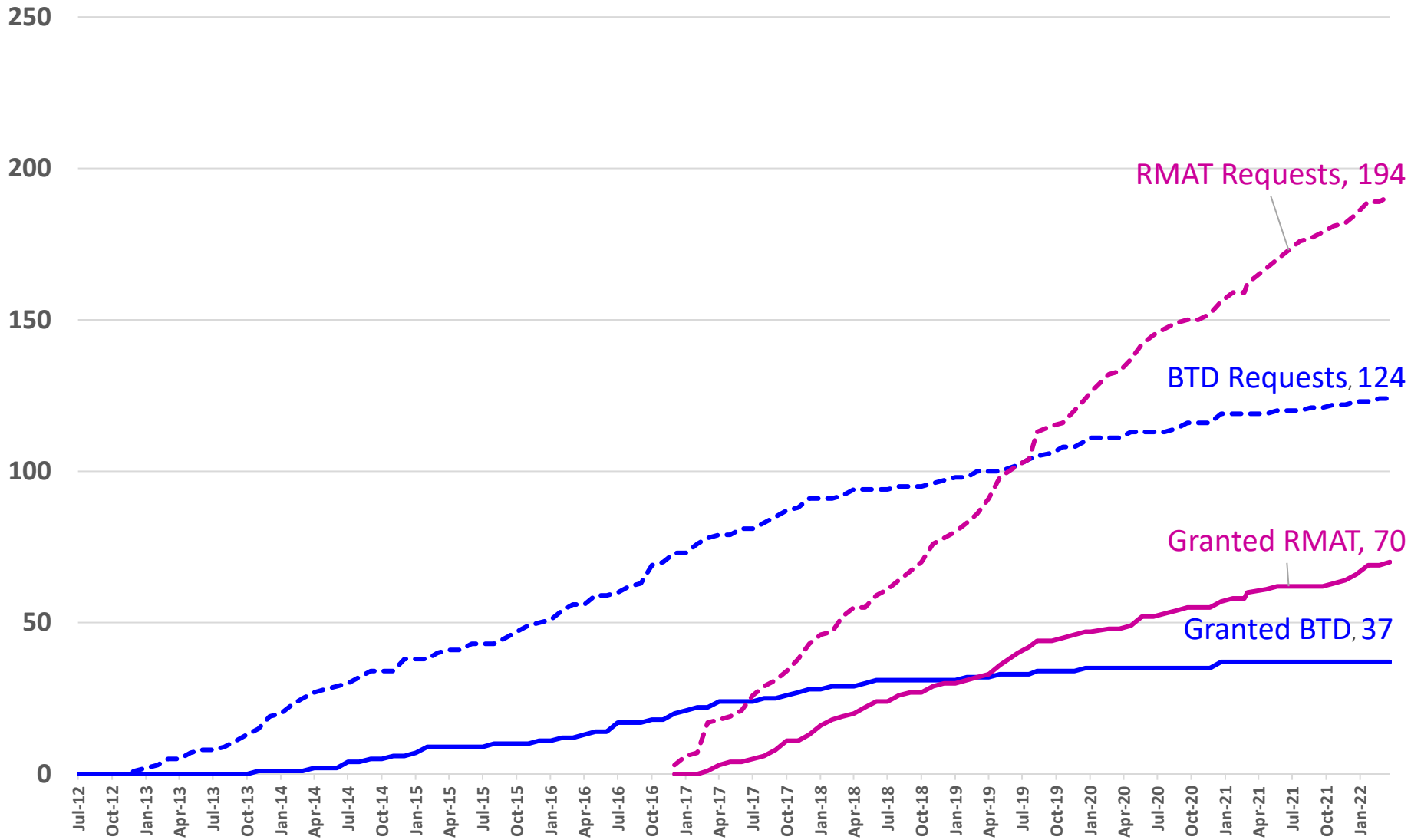
- **Functionally mature/differentiated cells** (e.g., retinal pigment epithelial cells, pancreatic islets, chondrocytes, keratinocytes)
- **Therapeutic vaccines and other antigen-specific active immunotherapies**
- **Blood- and Plasma-derived products**
  - Coagulation factors
  - Fibrin sealants
  - Fibrinogen
  - Thrombin
  - Plasminogen
  - Immune globulins
  - Anti-toxins
  - Venom antisera for scorpions, snakes, and spiders
- **Combination products**
  - Engineered tissues/organs
- **Devices**
- **Tissues**

Cell and Gene Therapies: Research* INDs 2002 – 2021



*Numbers do not include single patient or expanded access INDs

Breakthrough (BT) and RMAT Designation Requests



Gene Therapy: List of Approved Products

- **Kymriah (tisagenlecleucel)** – For treatment of
  - Patients up to 25 years of age with B-cell precursor acute lymphoblastic leukemia (ALL) that is refractory or in second or later relapse
  - Adult patients with relapsed or refractory (r/r) large B-cell lymphoma after two or more lines of systemic therapy including diffuse large B-cell lymphoma (DLBCL) not otherwise specified, high grade B-cell lymphoma and DLBCL arising from follicular lymphoma
  - Adult patients with relapsed or refractory follicular lymphoma (FL) after two or more lines of systemic therapy
    - Under accelerated approval
- **Yescarta (axicabtagene ciloleucel)** – Indicated for the treatment of
  - Adult patients with large B-cell lymphoma that is refractory to first-line chemoimmunotherapy or that relapses within 12 months of first-line chemoimmunotherapy
  - Adult patients with relapsed or refractory large B-cell lymphoma after two or more lines of systemic therapy including diffuse large B-cell lymphoma (DLBCL) not otherwise specified, primary mediastinal large B-cell lymphoma, high grade B-cell lymphoma, and DLBCL arising from follicular lymphoma.
  - Adult patients with relapse or refractory follicular lymphoma (FL) after two or more lines of systemic therapy
    - Under accelerated approval
- **Luxturna (voretigene neparvovec-rzyl)** - Indicated for the treatment of patients with confirmed biallelic RPE65 mutation-associated retinal dystrophy.
- **Zolgensma (onasemnogene abeparvovec-xioi)** – Indicated for the treatment of pediatric patients less than two years of age with spinal muscular atrophy (SMA) with bi-allelic mutations in the *survival motor neuron 1 (SMN1)* gene

Gene Therapy Approved Products

- **Tecartus (brexucabtagene autoleucel)** – Indicated for the treatment of
  - Adult patients with relapsed or refractory mantle cell lymphoma
    - Under accelerated approval
  - Adult patients with relapsed or refractory B-cell precursor acute lymphoblastic leukemia (ALL)

- **BREYANZI (lisocabtagene maraleucel)** – Indicated for the treatment of adult patients with large B-cell lymphoma, including diffuse large B cell lymphoma not otherwise specified, high-grade B-cell lymphoma, primary mediastinal large B-cell lymphoma, and follicular lymphoma grade 3B, who have
  - refractory disease to first-line chemoimmunotherapy or relapse within 12 months of first-line chemoimmunotherapy ; or
  - refractory disease to first-line chemoimmunotherapy or relapse after first-line chemoimmunotherapy and are not eligible for hematopoietic stem cell transplantation (HSCT) due to comorbidities or age; or
  - relapsed or refractory disease after two or more lines of systemic therapy.
- **ABECMA (idecabtagene vicleucel) –** Indicated for the treatment of relapsed or refractory multiple myeloma
- **CARVYKTI (ciltacabtagene autoleucel)** – Indicated for the treatment of relapsed or refractory multiple myeloma

Gene Therapy Approved Products

- **Zynteglo (betibeglogene autotemcel)** – Indicated for the treatment of adult and pediatric patients with ß-thalassemia who require regular red blood cell (RBC) transfusions

- **Skysona (elivaldogene autotemcel)** – Indicated to slow the progression of neurologic dysfunction in boys 4-17 years of age with early, active cerebral adrenoleukodystrophy (CALD)
  - Under Accelerated Approval
- **HEMGENIX (etranacogene dezaparvovec-drlb)** - indicated for the treatment of adults with Hemophilia B (congenital Factor IX deficiency) who: currently use Factor IX prophylaxis therapy, or have current or historical life-threatening hemorrhage, or have repeated, serious spontaneous bleeding episodes
- **ADSTILADRIN (nadofaragene firadenovec-vncg)** – indicated for the treatment of adult patients with high-risk Bacillus Calmette-Guérin (BCG)-unresponsive non-muscle invasive bladder cancer (NMIBC) with carcinoma in situ (CIS) with or without papillary tumors

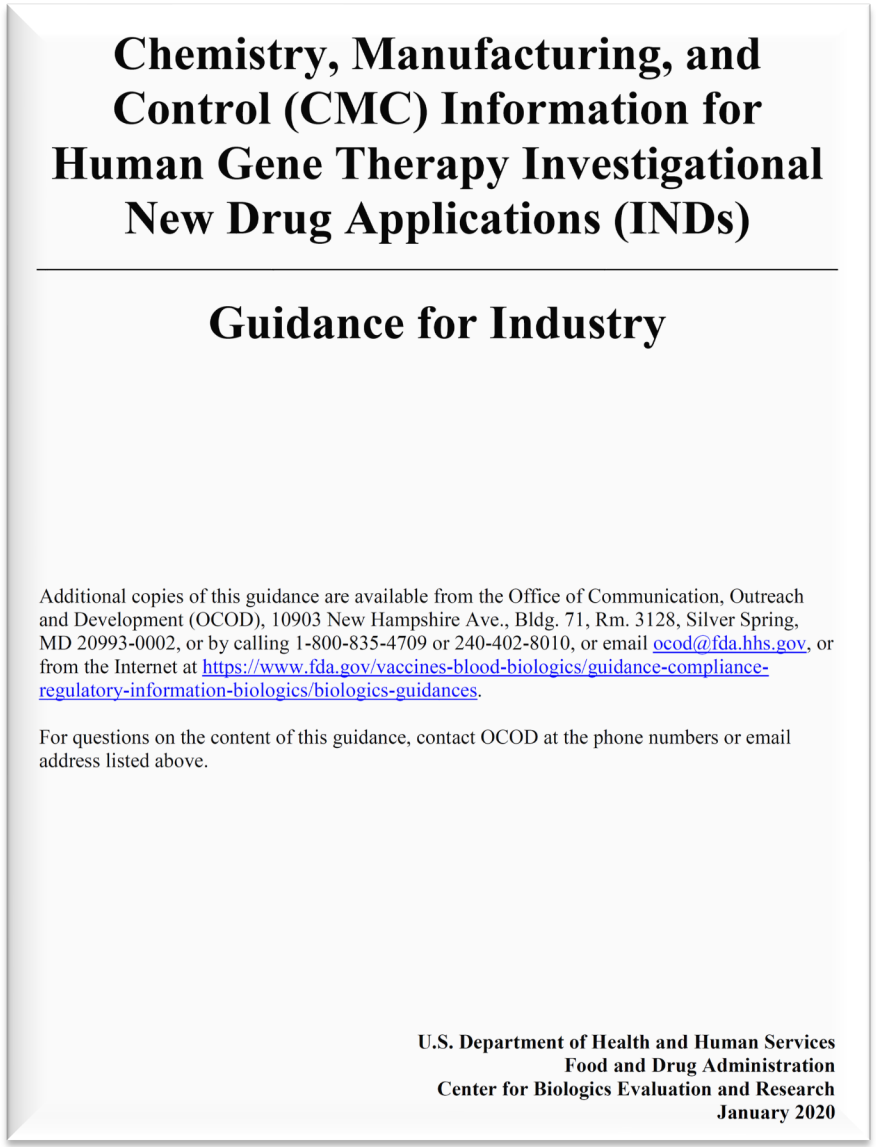
Cellular Therapy Approved Products

- **PROVENGE (sipuleucel-T)**: Autologous T-cell immunotherapy for treatment of asymptomatic or minimally symptomatic metastatic castrate resistant (hormone refractory) prostate cancer
- **HPC (hematopoietic progenitor cells), Cord Blood**: For use in unrelated donor hematopoietic progenitor cell transplantation procedures in conjunction with an appropriate preparative regimen for hematopoietic and immunologic reconstitution in patients with disorders affecting the hematopoietic system that are inherited, acquired, or result from myeloablative treatment.
- **LAVIV (Azficel-T):** Autologous fibroblasts for improvement of the appearance of moderate to severe nasolabial fold wrinkles in adults
- **GINTUIT (Allogeneic Cultured Keratinocytes and Fibroblasts in bovine collagen):** For topical (non-submerged) application to a surgically created vascular wound bed in the treatment of mucogingival conditions in adults
- **MACI (Autologous Cultured Chondrocytes on porcine collagen membrane)**: For repair of single or multiple symptomatic, full-thickness cartilage defects of the knee with or without bone involvement in adults
- **STRATAGRAFT allogeneic cultured keratinocytes and dermal fibroblasts in murine collagen-dsat**: Treatment of adults with thermal burns containing intact dermal elements for which surgical intervention is clinically indicated (deep partial-thickness burns)
- **RETHYMIC allogeneic processed thymus tissue–agdc**: For immune reconstitution in pediatric patients with congenital athymia

Recent Cellular & Gene Therapy Guidances

- Considerations for the Development of Chimeric Antigen Receptor (CAR) T Cell Products; Draft Guidance for Industry 3/2022
- Human Gene Therapy Products Incorporating Human Genome Editing; Draft Guidance for Industry 3/2022
- Interpreting Sameness of Gene Therapy Products Under the Orphan Drug Regulations; Guidance for Industry 9/2021
- Studying Multiple Versions of a Cellular or Gene Therapy Product in an Early-Phase Clinical Trial; Guidance for Industry 11/2022
- Manufacturing Considerations for Licensed and Investigational Cellular and Gene Therapy Products During COVID-19 Public Health Emergency; Guidance for Industry 1/2021
- Human Gene Therapy for Neurodegenerative Diseases; Guidance for Industry 10/2022
- Cellular & Gene Therapy Guidances | FDA

Gene Therapy CMC Guidance



Chemistry, Manufacturing, and Control (CMC) Information for Human Gene Therapy Investigational New Drug Applications (INDs)

Guidance for Industry

Additional copies of this guidance are available from the Office of Communication, Outreach and Development (OCOD), 10903 New Hampshire Ave., Bldg. 71, Rm. 3128, Silver Spring, MD 20993-0002, or by calling 1-800-835-4709 or 240-402-8010, or email ocod@fda.hhs.gov, or from the Internet at https://www.fda.gov/vaccines-blood-biologics/guidance-compliance-regulatory-information-biologics/biologics-guidances.

For questions on the content of this guidance, contact OCOD at the phone numbers or email address listed above.

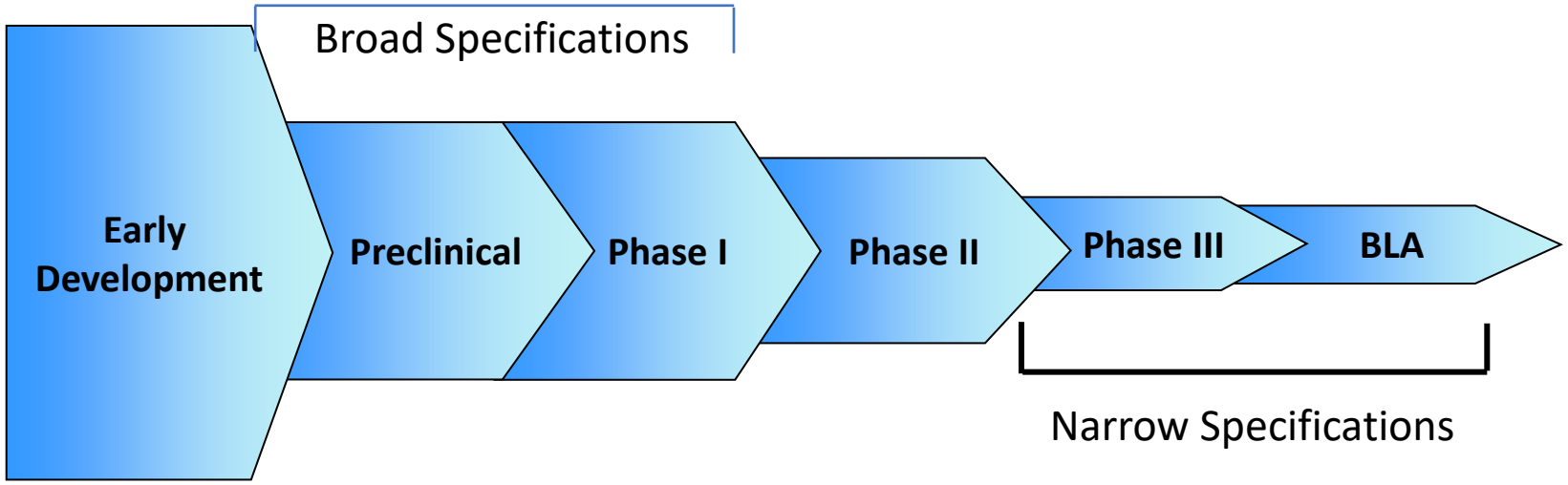
U.S. Department of Health and Human Services
Food and Drug Administration
Center for Biologics Evaluation and Research
January 2020

- **Updated recommendations based on FDA and ICH guidance documents and changes to regulations since 2008**
- **Updated the list of terms and definitions**
  - e.g., human gene therapy, human gene therapy product, genome editing
- **Provided recommendations for submitting CMC information into eCTD**
  - Module 1: recommendations for administrative information
  - Module 2: summary information detailed in Module 3
  - Module 3: detailed instructions for CMC information to be submitted to support an IND
- **Appendices:**
  - Facility/equipment, quality unit, COAs, adventitious agents safety data

CMC Guidance

- **Viral vectors for genetically modifying cells**
  - Critical manufacturing component, recorded in DS section of Module 3 to capture all necessary information
  - Manufactured under GMPs, process and method validation for licensure.
    - Manufacturer may be inspected during BLA review
- **Key updates to product and method development**
  - Cell bank selection, impurity testing, and residual DNA testing
    - Quality controls and verification for CMO
  - Qualification of dose determining assays
  - Plasmids for further manufacture
  - Replication competent virus testing (see RCR guidance)

CMC Expectations: Progressive Refinement of Product Specifications



- Early product characterization is a **multi-parameter** analysis
- Number of parameters progressively get pruned from phase I to phase III
- Major manufacturing changes during a pivotal trial should be avoided
- All changes need to be scientifically valid and justified

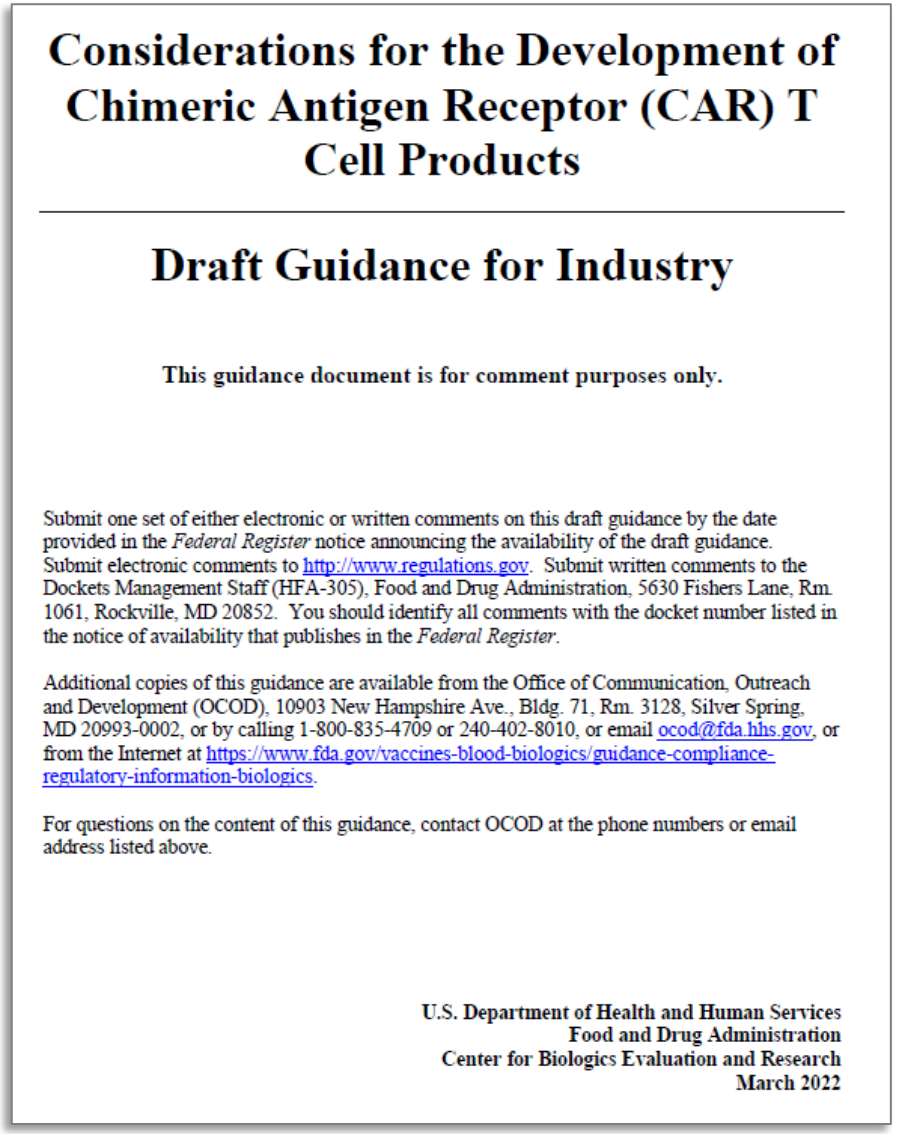
Frequently Asked Questions

Common topics for discussion:

- Vector Copy Number (VCN)
- Comparability
- Potency

FDA has tried to incorporate these information in various guidance documents.

DRAFT CAR T cell Guidance



Considerations for the Development of Chimeric Antigen Receptor (CAR) T Cell Products

Draft Guidance for Industry

This guidance document is for comment purposes only.

Submit one set of either electronic or written comments on this draft guidance by the date provided in the *Federal Register* notice announcing the availability of the draft guidance. Submit electronic comments to http://www.regulations.gov. Submit written comments to the Dockets Management Staff (HFA-305), Food and Drug Administration, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852. You should identify all comments with the docket number listed in the notice of availability that publishes in the *Federal Register*.

Additional copies of this guidance are available from the Office of Communication, Outreach and Development (OCOD), 10903 New Hampshire Ave., Bldg. 71, Rm. 3128, Silver Spring, MD 20993-0002, or by calling 1-800-835-4709 or 240-402-8010, or email ocod@fda.hhs.gov, or from the Internet at https://www.fda.gov/vaccines-blood-biologics/guidance-compliance-regulatory-information-biologics.

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U.S. Department of Health and Human Services
Food and Drug Administration
Center for Biologics Evaluation and Research
March 2022

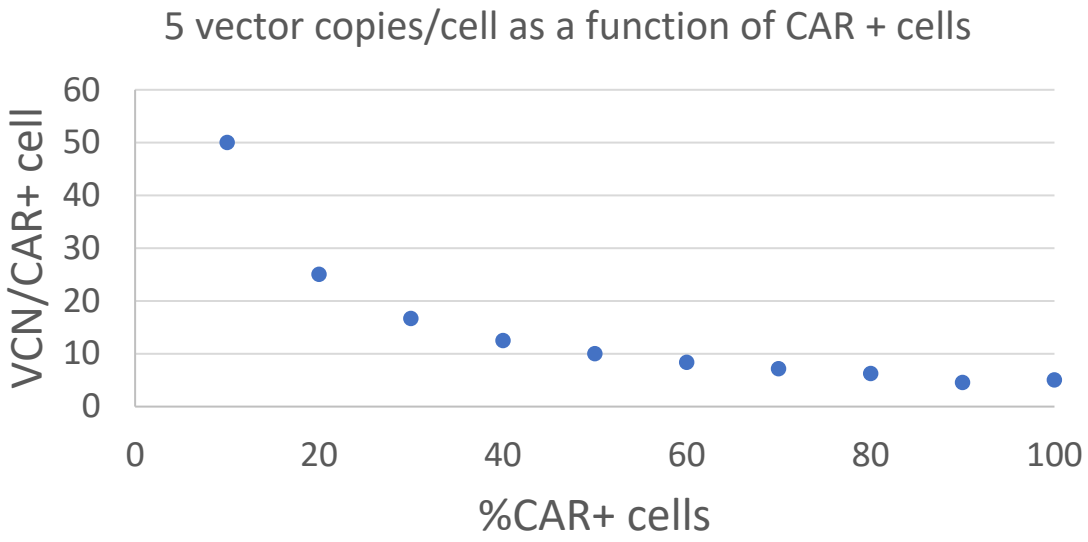
- CMC, preclinical, and clinical
- Recommendations specific to autologous or allogeneic CAR T cell products
- Applicable to other genetically modified lymphocyte products, such as CAR Natural Killer (NK) cells or T cell receptor (TCR)-modified T cells
- Considerations beyond those recommended in this guidance would depend on the specific product and manufacturing process

DRAFT CAR T cell CMC Recommendations

- General information is cross-referenced to the GT CMC guidance
- Considerations for control of the transgene vector and cellular starting material
- Drug Product Analytical testing
  - Flow cytometry
  - VCN/CAR+ cell
  - Identity testing should distinguish the DP from other products in the same facility
  - Potency assay

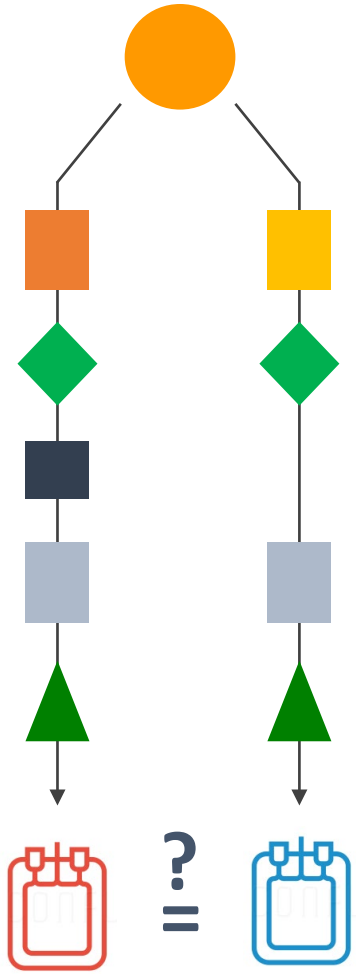
DRAFT Recommendations for Vector Copy Number (VCN)

- If the vector system directs transgene integration, report the average number of integrations per CAR-positive cell.
  - Determining VCN as a function of total cells, includes non-transduced cells in the denominator and lowers the reported vector integration rate.
  - We recommend that the transduction process be optimized to control VCN while meeting target transduction frequency.

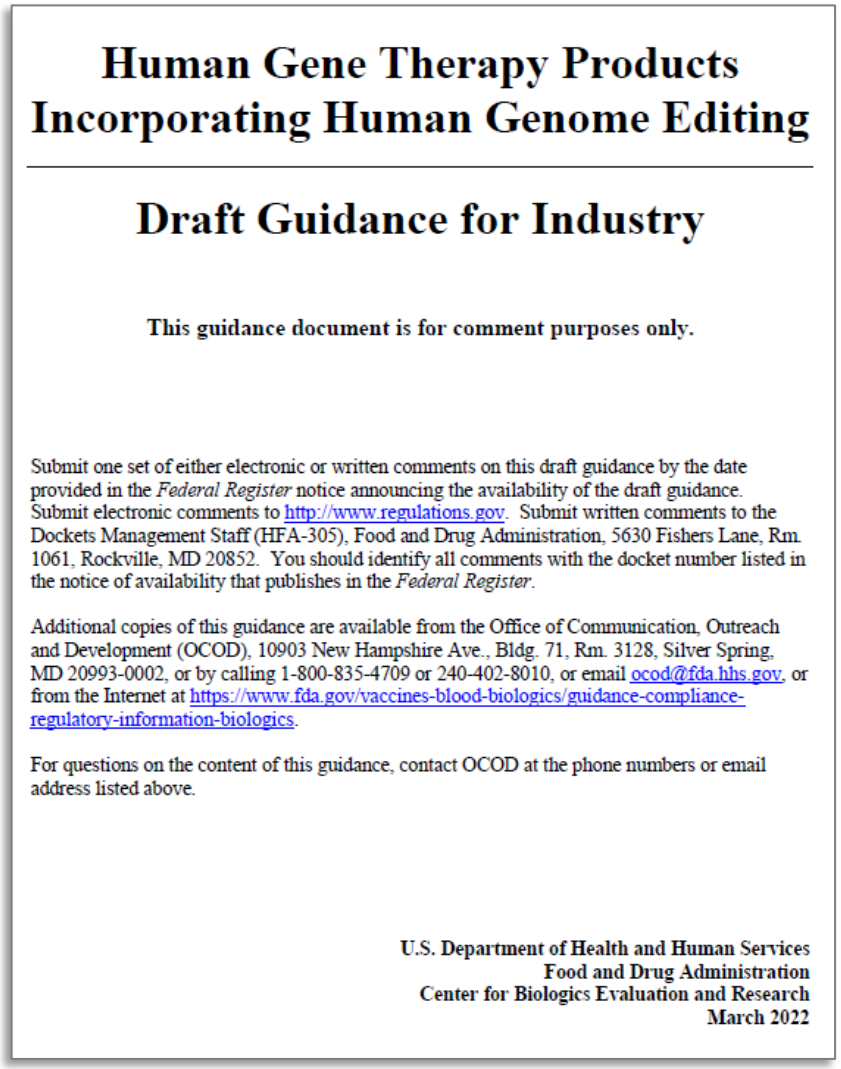


DRAFT Recommendations for Managing Manufacturing Changes and Assessing Comparability During the CAR T Cell Product Life Cycle

- Changes to the CAR T cell design, manufacturing process, or manufacturing facility during product development
- Conduct a risk assessment to evaluate the potential impact of the intended change on product quality and safety
- Proposed change(s), the accompanying risk assessment, and the proposed change management strategy may be submitted as an amendment to the IND
- Comparability Study Design:
  - recommend that CAR T cell comparability be assessed by side-by-side testing using the same cellular starting material
  - appropriate statistical methods and predefined acceptance criteria
  - meeting current lot release criteria is typically insufficient to establish comparability



DRAFT Genome Editing (GE) Guidance



Human Gene Therapy Products Incorporating Human Genome Editing

Draft Guidance for Industry

This guidance document is for comment purposes only.

Submit one set of either electronic or written comments on this draft guidance by the date provided in the *Federal Register* notice announcing the availability of the draft guidance. Submit electronic comments to http://www.regulations.gov. Submit written comments to the Dockets Management Staff (HFA-305), Food and Drug Administration, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852. You should identify all comments with the docket number listed in the notice of availability that publishes in the *Federal Register*.

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U.S. Department of Health and Human Services
Food and Drug Administration
Center for Biologics Evaluation and Research
March 2022

- CMC, preclinical, and clinical
- Human GE is a process by which DNA sequences are added, deleted, altered or replaced at specified location(s) in the genome of human somatic cells, ex vivo or in vivo, using nuclease-dependent or nuclease-independent GE technologies
- Recommendations for how to assess the safety and quality of these products and address the potential risks of these products
- Specific risks associated with GE approaches include off-target editing, unintended consequences of on- and off-target editing, and the unknown long term effects of on- and off-target editing

DRAFT Genome Editing Guidance CMC Recommendations

- General information is cross-referenced to the GT CMC guidance
- Utilize design platforms that are most applicable to the genomic target and the type of intended genomic modification
- Optimize the GE components to reduce the potential for off-target genome modification
- Detailed descriptions of how each GE component is manufactured, purified and tested
  - Sterility, identity, purity and functionality of each component, as applicable, additional testing such as that for process residuals
  - Ex vivo-modified cells:
    - Determination of GE efficiency (e.g., the degree of cleavage at the on-target site)
    - Specificity (e.g., the degree of cleavage at off-target sites)

Guidance for Industry

Potency Tests for Cellular and Gene Therapy Products

Additional copies of this guidance are available from the Office of Communication, Outreach and Development (OCOD), (HFM-40), 1401 Rockville Pike, Suite 200N, Rockville, MD 20852- 1448, or by calling 1-800-835-4709 or 301-827-1800, or email ocod@fda.hhs.gov, or from the Internet at http://www.fda.gov/BiologicsBloodVaccines/GuidanceComplianceRegulatoryInformation/Guida nces/default.htm.

For questions on the content of this guidance, contact OCOD at the phone numbers listed above.

U.S. Department of Health and Human Services
Food and Drug Administration Center for Biologics Evaluation and Research
January 2011

Control of Product Variability : Evaluation of Potency

Potency Assays

21 CFR 610.10:

…shall consist of **either in vitro or in vivo tests**, or both, ….

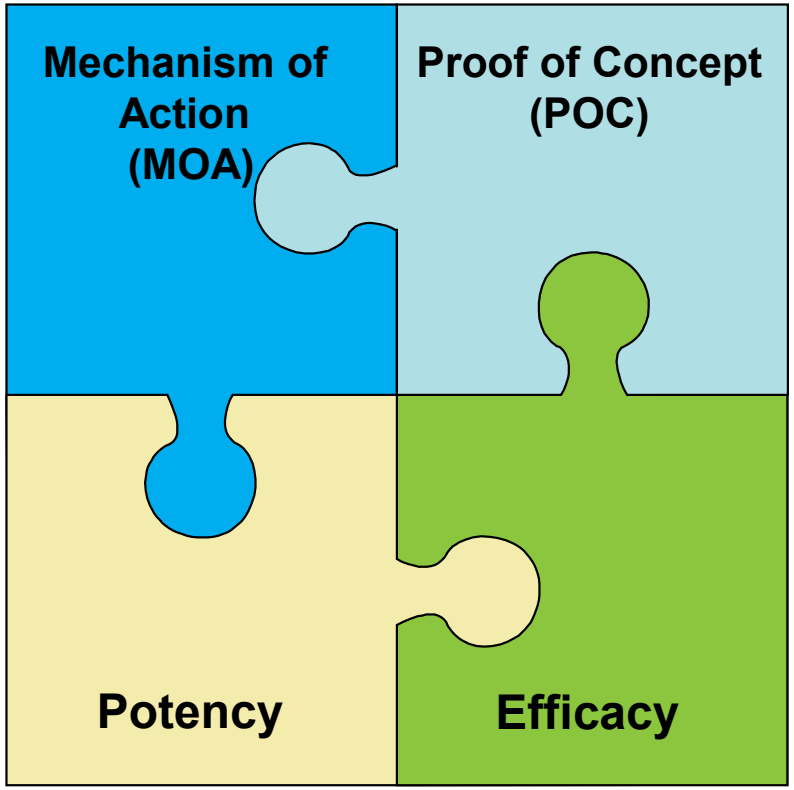
21 CFR 600.3(s):

…potency is interpreted to mean the **specific ability or capacity of the product,** as indicated by appropriate laboratory tests or by adequately controlled clinical data obtained through the administration of the product in the manner intended, **to effect a given result**.

MOA, POC, Potency, and Efficacy are Related, yet Distinct

The proposed MOA can guide the type of testing needed to establish potency, pre-clinical proof of concept, and efficacy

Potency should measure some relevant biological property consistent with the proposed MOA and guided by POC studies



Pre-clinical testing should be guided by the MOA and product potency, and the results should support the MOA and potency criteria

For a product to be efficacious it must be potent (though not necessarily in the way you are measuring it)

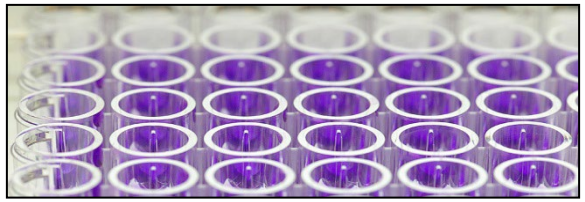
Clinical data from phase II studies can sometimes help guide potency specifications

Establishing a Potency Assays

- Should be guided by the underlying proposed mechanism of action and *in vitro* and *in vivo* pre-clinical proof of concept data
- Recommend evaluating multiple measures of product potency until you are confident you have an assay that is suitable for your needs
  - In some cases you may wish to choose one assay for product release while continuing to collect data on other assays
  - In some cases a single measurement may not be fully informative and a matrix approach may be needed
- Potency assays should be chosen based on successful qualification of the test method using your product

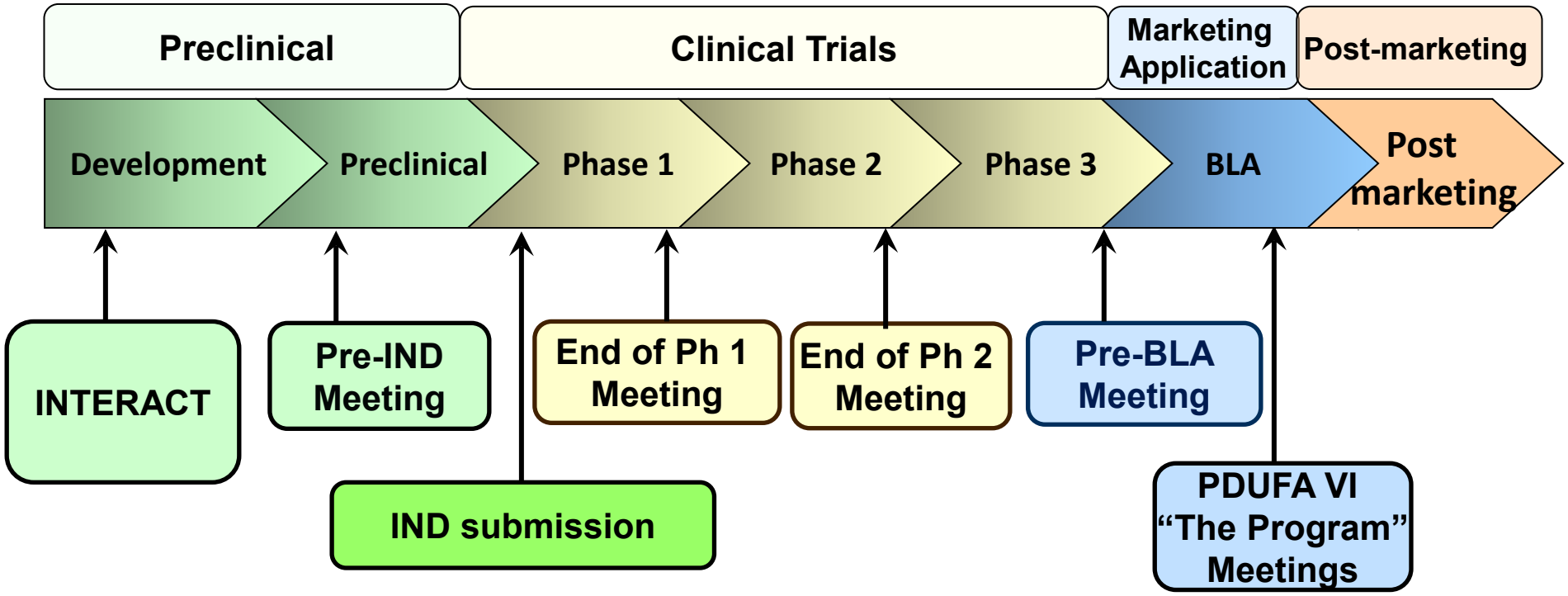
Proposed Mechanism of Action and Product Potency

- A single product could have multiple mechanisms of action in the patient:
  - Cell replacement
  - Stimulate endogenous cells
  - Protect host cells
  - Secrete trophic, growth factors, cytokines, etc.



- Although it is not a regulatory requirement to fully define the mechanism of action, having an understanding of how the product is likely to work could be very helpful in designing proof-of-concept preclinical studies and establishing lot release specifications.

Opportunities for Interaction During Product Development



PDUFA VII : CMC Development Readiness Pilot (CDRP)

65596 Federal Register / Vol. 87, No. 209 / Monday, October 31, 2022 / Notices

Estimated Total Annual Burden Hours: 744.

Authority: Section 105(b)(5) of the Child Abuse Prevention and Treatment Act of 1978 (42 U.S.C 5106(b)(5)), as amended by the CAPTA Reauthorization Act of 2010 (Pub. L. 111–320).

Mary B. Jones,

ACF/OPRE Certifying Officer.

[FR Doc. 2022–23673 Filed 10–28–22; 8:45 am]

BILLING CODE 4184–29–P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA–2022–N–2396]

Chemistry, Manufacturing, and Controls Development and Readiness Pilot Program; Program Announcement

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice.

SUMMARY: The Food and Drug Administration (FDA or Agency) is announcing the opportunity for a limited number of applicants to participate in a Chemistry, Manufacturing, and Controls (CMC) Development and Readiness Pilot (CDRP) program, to facilitate the expedited CMC development of products under an investigational new drug (IND) application, where warranted, based upon the anticipated clinical benefit of earlier patient access to the products. FDA is implementing this pilot program to facilitate CMC readiness for selected Center for Biologics Evaluation and Research (CBER)- and Center for Drug Evaluation and Research (CDER)-regulated products with accelerated clinical development timelines. To accelerate CMC development and facilitate CMC readiness, the pilot features increased communication between FDA and sponsors and explores the use of science- and risk-based regulatory approaches, such as those described in FDA guidance, as applicable. This notice outlines the eligibility criteria and process for submitting a request to participate in the pilot.

DATES: Starting April 1, 2023, FDA will accept requests to participate in the CDRP program. See the "Participation" section of this document for eligibility criteria, instructions on how to submit a request to participate, and selection criteria and process.

FOR FURTHER INFORMATION CONTACT: Tanya Clayton, Center for Drug Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 75, Rm. 4506, Silver Spring, MD 20903–0002, 301–796–0871; or Stephen Ripley, Center for Biologics Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 71, Rm. 7301, Silver Spring, MD 20993–0002, 240–402–7911.

For general questions about the CDRP Program for CBER: industry.biologics@fda.hhs.gov.

For general questions about the CDRP Program for CDER: cder-opq-opro-crad-inquiries@fda.hhs.gov.

SUPPLEMENTARY INFORMATION:

I. Background

Development programs for CBER- and CDER-regulated drugs and biologics intended to diagnose, treat, or prevent a serious disease or condition where there is an unmet medical need may have accelerated clinical development timelines. Yet marketing applications for products in expedited development programs still need to meet FDA's approval standards, including manufacturing facility compliance with current good manufacturing practice (CGMP). Products with accelerated clinical development activities may face challenges in expediting CMC development activities to align with the accelerated clinical timelines. Successfully expediting CMC readiness may require additional interactions with FDA during product development and, if applicable, warrant the use of science- and risk-based regulatory approaches allowing streamlining of CMC development activities, so that clinical benefits of earlier patient access to these products can be realized.

As described in the FDA Prescription Drug User Fee Act (PDUFA) VII Commitment Letter for fiscal years (FYs) 2023 through 2027 (Ref. 1), FDA is implementing the CDRP program to facilitate CMC readiness for selected CBER- and CDER-regulated products with accelerated clinical development timelines. To accelerate CMC development and facilitate CMC readiness, the pilot features increased communication between FDA and sponsors and explores the use of science- and risk-based regulatory approaches, such as those described in the FDA guidance for industry entitled "Expedited Programs for Serious Conditions—Drugs and Biologics" (May 2014) (Ref. 2), as applicable.

Starting in FY 2023, FDA (CBER and CDER) will conduct a CDRP to facilitate the CMC development of selected products under INDs which have expedited clinical development timeframes, based upon the anticipated clinical benefits of earlier patient access to the products. This includes products with Breakthrough Therapy (BT), Fast Track (FT), and Regenerative Medicine Advance Therapy (RMAT) designations. For sponsors participating in the pilot, FDA will provide product-specific CMC advice during product development, to include two additional CMC-focused Type B meetings, as well as a limited number of additional CMC-focused discussions, based on readiness and defined CMC milestones. The increased communication between FDA review staff and sponsors is intended to ensure a mutual understanding of approaches to completing CMC activities, including what information should be provided at the appropriate timepoint (*i.e.*, at the time of new drug application (NDA) or biologics license application (BLA) submission, prior to the end of the review cycle, or post-approval), to ensure CMC readiness for a marketing application.

II. Participation

Starting April 1, 2023, FDA will accept requests to participate in the CDRP program and select no more than nine proposals, with approximately two thirds being CBER-regulated products and one third CDER-regulated products. Taking into consideration lessons from the prior year, FDA will publish in the **Federal Register** a notice to announce pilot programs for each of the 3 following fiscal years. Sponsors who are interested in participating in the pilot program should submit a request to participate in the pilot as an amendment to their IND. The cover letter should state "Request to participate in the CMC Development and Readiness Pilot."

To promote innovation and understanding in this area, lessons learned through the pilot may be presented by FDA (*e.g.*, in a public workshop) as case studies, including when the product studied in the pilot has not yet been approved by FDA. FDA intends to conduct a public workshop and issue a strategy document focused on CMC aspects of expedited development incorporating lessons from the CDRP. Participation in the pilot program is voluntary and at the discretion of the sponsor. To be eligible for the pilot, the sponsor's written request should include the following statement:

"We, <sponsor's name>, acknowledge that certain information relevant to the CDRP may be publicly disclosed."

- Starting April 1, 2023, FDA will accept requests to participate in the CDRP program and select no more than nine proposals per year, with approximately two thirds being CBER-regulated products and one third CDER-regulated products.

To participate in the pilot program:

- Participant must have an active commercial CBER IND with an accelerated clinical development timeline (BT/RMAT designation)
- Submit a CMC Development Plan
  - Current state of CMC development
  - Projected timeline for product development
  - Plans for ensuring product availability for commercial launch (scale-up plans).
  - Overall plan for process validation
  - The plan should highlight these areas (exemplified in the bulleted list above, and any additional CMC challenges that may require FDA input), to facilitate FDA engagement

CMC Development Readiness Pilot

- **Two dedicated CMC meetings** (Type B meetings, in addition to existing meetings)
  - Product specific guidance

- **Follow-up discussions** (To address questions arising from the meeting or meeting minutes)

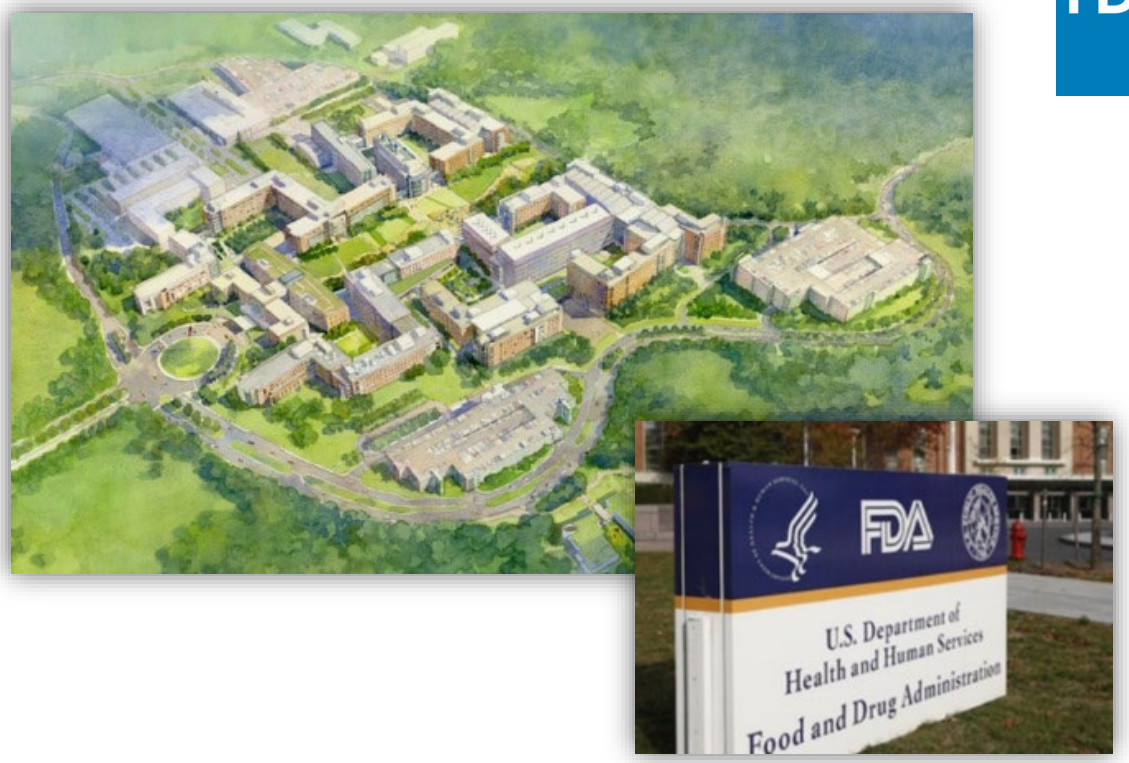
- To **promote innovation** and understanding in this area, lessons learned through the pilot may be presented by FDA (*e.g.,* in a public workshop) as case studies.

- FDA intends to **issue a strategy** document focused on CMC aspects of expedited development incorporating lessons from the CDRP.

Summary

- FDA has been very active in publishing guidance on topics related to CGT product development
- FDA's new PDUFA commitment for additional meetings with sponsors and public workshops will assist in CGT product development
- Some CGT products may have the potential to cure diseases and conditions that have been incurable
- With the approval of the initial few CGT products, there is high expectation of future application of CGT
- To address the present and the anticipated future increase in work-load related to the development of CGT products, OTAT/CBER/FDA is in the process of expanding its capacity

Contact Information



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