FDA Product Inquiry Avenues

INTERACT- **IN**itial **T**argeted **E**ngagement for **R**egulatory **A**dvice on **C**BER produc**T**s

"Through an INTERACT meeting, sponsors can obtain initial, nonbinding advice from FDA regarding chemistry, manufacturing and controls, pharmacology/toxicology, and/or clinical aspects of the development program. This informal meeting can: Assist sponsors conducting early product characterization and preclinical proof-of-concept studies; initiate discussion for new delivery devices; inform sponsors about overall early-phase clinical trial design elements; and identify critical issues or deficiencies for sponsors to address in the development of innovative products."

TRIP- **T**RG (Tissue Reference Group) **R**apid **I**nquiry **P**rogram **(ends 10/31/20)**

"In November 2017, FDA announced its comprehensive regenerative medicine framework. At that time, FDA also announced that until November 2020, the agency generally intends to exercise enforcement discretion for certain HCT/Ps, with respect to FDA's investigational new drug application (IND) and premarket approval requirements, when the use of the product does not raise reported safety concerns or potential significant safety concerns. However, since the framework was announced, FDA has seen only modest progress by regulated industry in coming into compliance. Therefore, FDA instituted the TRIP of the TRG, for regulated industry to rapidly obtain a preliminary assessment from FDA as to how their HCT/Ps are appropriately regulated. As resources permit, FDA intends to provide preliminary, informal, non-binding responses within one week after receiving an inquiry that contains sufficient detail for evaluation."

The program has now been extended twice by FDA.

CATT- **C**BER **A**dvanced **T**echnologies **T**eam

"The CATT was established to promote dialogue, education, and input among CBER staff and between CBER and prospective innovators/developers of advanced manufacturing technologies. As part of these efforts, CBER is providing an interactive mechanism for prospective innovators/developers of advanced manufacturing and testing technologies to discuss with CBER staff issues related to the implementation of these technologies in the development of CBER-regulated products."

Pre-IND- **Pre-I**nvestigational **N**ew **D**rug Application Consultation Program

"Pre-IND advice may be requested for issues related to data needed to support the rationale for testing a drug in humans; the design of nonclinical pharmacology, toxicology, and drug activity studies, including design and potential uses of any proposed treatment studies in animal models; data

requirements for an Investigational New Drug (IND) application; initial drug development plans, and regulatory requirements for demonstrating safety and efficacy."

FDA Designations for Expedited Review

RMAT- Regenerative Medicine Advanced Therapy Designation

"As described in Section 3033 of the 21st Century Cures Act, a drug is eligible for regenerative medicine advanced therapy (RMAT) designation if: (a) The drug is a regenerative medicine therapy, which is defined as a cell therapy, therapeutic tissue engineering product, human cell and tissue product, or any combination product using such therapies or products, except for those regulated solely under Section 361 of the Public Health Service Act and part 1271 of Title 21, Code of Federal Regulations; (b)The drug is intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition; and (c) Preliminary clinical evidence indicates that the drug has the potential to address unmet medical needs for such disease or condition."

The RMAT designation allows for accelerated approval pathways and opportunities for sponsors of such products to interact with CBER review staff as described in this FDA guidance (Feb. 2019).

Fast Track

"Fast track is a process designed to facilitate the development, and expedite the review of drugs to treat serious conditions and fill an unmet medical need. The purpose is to get important new drugs to the patient earlier. Fast Track addresses a broad range of serious conditions."

Breakthrough Therapy

"Breakthrough Therapy designation is a process designed to expedite the development and review of drugs that are intended to treat a serious condition and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over available therapy on a clinically significant endpoint(s)."

Accelerated Approval

"Mindful of the fact that it may take an extended period of time to measure a drug's intended clinical benefit, in 1992 FDA instituted the Accelerated Approval regulations. These regulations allowed drugs for serious conditions that filled an unmet medical need to be approved based on a surrogate endpoint. Using a surrogate endpoint enabled the FDA to approve these drugs faster."

Priority Review

“In 1992, under the Prescription Drug User Act (PDUFA), FDA agreed to specific goals for improving the drug review time and created a two-tiered system of review times – Standard Review and Priority Review. A Priority Review designation means FDA’s goal is to take action on an application within 6 months (compared to 10 months under standard review).”

RPD-**R**are **P**ediatric **D**isease Designation Program

“Under Section 529 to the Federal Food, Drug, and Cosmetic Act (FD&C Act), FDA will award priority review vouchers to sponsors of rare pediatric disease product applications that meet certain criteria. Under this program, a sponsor who receives an approval for a drug or biologic for a "rare pediatric disease" may qualify for a voucher that can be redeemed to receive a priority review of a subsequent marketing application for a different product.”