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FDA Platform Designation Draft Released

Incentivizes New Tech: 10 Per Year, Final Rules Likely More Robust

Relevant Companies

mRNA, GENE THERAPY, MONOCLONAL ANTIBODY, OTHER NEW TECHNOLOGIES

Our Take & Next Up

FDA released long-awaited draft guidance on the platform technology designation (here). This 18-page document outlines eligibility factors, potential benefits, the recommended content of a designation submission, and how to update a designated platform technology. As a reminder, we previewed (here) that the proposal may be vague in nature with more details upon finalization in 2H 2024. The final guidance will likely include more specific technologies and potential winners. We view this as an industry friendly document. Initially, the FDA expects 10 designation requests per year (here), making it a more limited program compared to expectations. A 60-day comment period will ensue upon publication of the draft rule. A finalized rule is likely to materialize this Fall.

Key Points

As a reminder, the platform technology designation program was established by the end of the year Consolidated Appropriations Act, passed in December 2022. The law required the FDA to establish a platform technology designation program that allows drug developers to build on and reference their prior knowledge for platform technologies.

In the guidance, the FDA defines their limits of what can be a designated platform technology. According to the agency, a platform technology is a “well understood and reproducible technology” where it: (1) is essential to the structure or function of a drug; (2) can be adapted for or used by more than one drug sharing common structural elements; and (3) facilitates the manufacturing or development of more than one drug through a standardized process. This platform tech can qualify for the designation if

- it is incorporated in, or utilized by, an approved drug
- preliminary evidence demonstrates that the platform technology has the potential to be incorporated in more than one drug without an adverse effect on quality, manufacturing, or safety

- data submitted indicates that incorporation or utilization of the platform technology has a reasonable likelihood to bring significant efficiencies to the drug development or manufacturing process and to the review process.

Eligible technologies that are named include mRNA, gene therapies, and monoclonal antibody therapies. Eligible platforms in the draft guidance include:

- lipid nanoparticle (LNP) platforms for mRNA vaccine or gene therapy products or oligonucleotides
- monoclonal antibody platform technologies
- platforms using a chemically defined targeting moiety with a well characterized synthetic siRNA.

The designation request must include all products that use or incorporate the platform technology (regardless of current developmental or marketing status). Summary data of all such products must be included with a justification explaining why the summary data show that certain product-specific tests, analyses, or studies can be leveraged.

The FDA recommends submitting a designation request during the Investigational New Drug (IND) phase of drug development with 10 designation requests expected per year. Sponsors can request the designation at any time concurrent with or after the submission of an IND. Potential qualifying technologies include nucleic acid sequence, molecular structure, mechanism of action, delivery method, vector, or a combination. FDA will determine if the platform technology will be designated within 90 calendar days of receiving the request.

The agency is focused on shared structural elements, justification for use, and the tech's potential for significant efficiencies to drug development or manufacturing or review process. Recommended content for a designation request include description of the platform tech, how it meets statutory factors, identification of an approved products and of the shared structural elements, justification of the use of a platform technology across multiple drugs without impact on safety quality or manufacturing, risk assessment of the differences between an approved product and proposed products, and why the use would bring significant efficiencies to the drug development or manufacturing process and to the review process.

A platform technology designation does not affect eligibility for any other expedited pathways (e.g., Accelerated Approval). FDA notes several benefits of the program like access to early interactions with the FDA, additional interactions with the FDA, and the potential to leverage non-clinical and other data (ex. batch and stability) submitted for a prior product.

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