Platform Technology Designation Program for Drug Development
Guidance for Industry

DRAFT GUIDANCE

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Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)

May 2024

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May 2024

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Guidance for Industry: Platform Technology Designation Program for Drug Development[1]

This draft guidance, when finalized, will represent the current thinking of the Food and Drug Administration (FDA or Agency) on this topic. It does not establish any rights for any person and is not binding on FDA or the public. You can use an alternative approach if it satisfies the requirements of the applicable statutes and regulations. To discuss an alternative approach, contact the FDA staff responsible for this guidance as listed on the title page.

I. INTRODUCTION

This guidance provides details about the implementation of the platform technology designation program established by section 506K of the Federal Food, Drug, and Cosmetic Act (FD&C Act).[2] This guidance outlines eligibility factors for receiving a platform technology designation, potential benefits of receiving a designation, how to leverage data from designated platform technologies, how to discuss a planned designation request as part of a milestone meeting, the recommended content of a designation request submission, and the review timelines for a designation request. This program is intended to result in efficiencies in drug[3] development, manufacturing, and review processes for drug product applications that incorporate designated platform technologies.

FDA acknowledges that the term "platform technology" has been used by both industry and FDA to describe technologies in ways that differ from the definitions of **platform technology**[4] and **designated platform technology** that are outlined in statute and this guidance. Some technologies that industry and FDA have historically considered to be platform technologies might not meet the statutory definition and statutory eligibility factors and, if not, would not be

[1] This guidance has been prepared by the Center for Drug Evaluation and Research (CDER) and the Center for Biologics Evaluation and Research (CBER) at the Food and Drug Administration, in consultation with the Office of Combination Products (OCP).

[2] Section 506K of the Federal Food, Drug, and Cosmetic Act (FD&C Act) (21 U.S.C. 356k) was added by the PREVENT Pandemics Act, which was enacted as part of the Consolidated Appropriations Act, 2023 (Public Law 117-328).

[3] For the purposes of this guidance the terms *drug*, *drug product*, and *product* refer to a drug as defined in section 201(g)(1) of the FD&C Act (21 U.S.C. 321(g)(1)). This includes biological products as defined in section 351(i) of the Public Health Service Act (PHS Act) (42 U.S.C. 262(i)). The term drug also applies to a drug or biological product constituent part (21 CFR 4.2) of a combination product being developed for review under section 505 of the FD&C Act (21 U.S.C. 355) or section 351 of the PHS Act.

[4] Bolded terms are defined in the Glossary section of this guidance.

eligible for the designation program. Ineligibility for designation does not preclude a sponsor[5] from leveraging **prior knowledge** across applications.[6] FDA has allowed sponsors to leverage prior knowledge from previously submitted applications when authorizing or approving drugs in an application submitted by the same sponsor.

For the platform technology designation program, section 506K of the FD&C Act establishes criteria outlining who can request designations and who, once that platform technology has been designated, can leverage them. This guidance describes those categories and provides recommendations for the types of platform technologies that may be eligible for consideration for designation. This guidance also gives recommendations for what should be included in submission requests to designate a platform technology, how to update a designated platform technology and, when appropriate, the process for revoking a designation.

In general, FDA's guidance documents do not establish legally enforceable responsibilities. Instead, guidances describe the Agency's current thinking on a topic and should be viewed only as recommendations, unless specific regulatory or statutory requirements are cited. The use of the word *should* in Agency guidances means that something is suggested or recommended, but not required.

II. PLATFORM TECHNOLOGY DESIGNATION REQUEST

If FDA has approved an Abbreviated New Drug Application (ANDA), New Drug Application (NDA), or Biologics License Application (BLA) for a drug that incorporates or uses a platform technology as defined in section 506K(h)(1) of the FD&C Act, a sponsor of a subsequent Investigational New Drug (IND), NDA, or 351(a) BLA can request designation of that platform technology to enable leveraging of the technology in new or future applications.[7] FDA recommends requesting the designation of a platform technology during the IND phase of drug development for a planned subsequent NDA or 351(a) BLA, because by this stage of development, the sponsor should have sufficient knowledge to outline for FDA how the

[5] For purposes of this guidance, unless otherwise stated, the term *sponsor* refers to the same business entity and/or applicant across the designation request and application submission process.

[6] Leveraging information, when scientifically justified and legally permissible, is available outside of the platform technology designation program. For example, a sponsor can already leverage their own data previously submitted in an IND, NDA, or BLA. Other applicants can leverage certain information in an approved NDA or BLA (e.g., based on a letter of authorization from the application holder), or rely on the Agency's prior findings of safety and/or effectiveness as part of an application submitted under section 505(b)(2) or 505(j) of the FD&C Act. See also the draft guidance for industry *Bridging for Drug-Device and Biologic-Device Combination Products* (December 2019). When final, this guidance will represent the FDA's current thinking on this topic. We update guidances periodically. For the most recent version of a guidance, check the FDA guidance web page at https://www.fda.gov/regulatory-information/search-fda-guidance-documents.

[7] See Section 506K(f) of the FD&C Act. Although data from an ANDA or 351(k) BLA can be referenced as part of satisfying the eligibility factors for a platform technology designation request under section 506K(b)(1) of the FD&C Act, the benefits of designation described in sections 506K(d)(2), (e), (f), and (g) are not available to ANDAs and 351(k) BLAs.

proposed platform technology would meet the eligibility factors as outlined in section 506K(b) of the FD&C Act. This should facilitate a more complete request and its timely review by FDA.

Designation of a platform technology does not give third parties additional rights to reference information from an approved product application containing that platform technology if they do not own or have full rights of reference to it. In addition, a BLA holder is generally expected to have knowledge of and control over the manufacturing process for the biological product for which it has a license.[8] Any referencing of data or information by an application based on a platform technology designation should be consistent with this general expectation. Any relevant information regarding a full right of reference agreement should be submitted with the administrative documents that are included in Module 1 of Electronic Common Technical Document (eCTD) submissions.[9]

FDA will review a request for designation to determine if the technology meets eligibility requirements, issuing a determination not later than 90 days of receipt.[10] The Agency will examine if the platform technology (as defined in section 506K(h)(1)) meets the eligibility factors outlined in section 506K(b) of the FD&C Act, including whether incorporation or use of the platform technology is reasonably likely to bring significant efficiencies to the application review process. The requester should provide a detailed justification in this regard for FDA review. FDA will determine if a platform technology designation request meets the eligibility factors under section 506K(b) and will provide a written explanation to the requester regarding the determination. For designated platform technologies, FDA may take actions to expedite development and review of any subsequent application submitted under section 505(b) of the FD&C Act or section 351(a) of the Public Health Service Act (PHS Act) for a drug that uses or incorporates the platform technology under section 506K(e), as appropriate.[11]

Once the sponsor has received a platform technology designation, information previously submitted in support of such designation can be leveraged in subsequent NDAs, 351(a) BLAs, or requests for emergency use authorization (EUAs)[12] from the same sponsor.[13] Sponsors of NDAs can leverage platform technology information from other applications submitted by the same

[8] See the final rule, "Biologics License Applications and Master Files" (89 FR 9743, February 12, 2024).

[9] See the guidance for industry *Providing Regulatory Submissions in Electronic Format — Certain Human Pharmaceutical Product Applications and Related Submissions Using the eCTD Specifications* (February 2020) and the "eCTD Backbone Files Specification for Module 1" at https://www.fda.gov/drugs/electronic-regulatory-submission-and-review/ectd-submission-standards-ectd-v322-and-regional-m1.

[10] See Section 506K(d)(1) of the FD&C Act.

[11] Expedited review in the context of this guidance does not refer to an expedited UFA review clock.

[12] See Section 564 of the FD&C Act for information on EUAs.

[13] Section 506K(f)(1) of the FD&C Act.

sponsor using the cross-reference mechanism.[14] However, BLA sponsors seeking to leverage data and information from a platform technology in a prior application should include the full information in their subsequent application. Whether leveraging platform technology information is appropriate in another application will ultimately depend on the particular request and what rationale the sponsor provides to show that the leveraging would enable the application to meet the relevant approval standard.

A different sponsor may also be able to leverage platform technology data if they receive a full right of reference to the leveraged data under a business arrangement with the originator of the platform technology.[15]

A. Eligibility for the Platform Technology Designation Program

To determine eligibility for designation as a designated platform technology, FDA will first determine whether the technology qualifies as a platform technology. Under section 506K(h)(1) of the FD&C Act, a platform technology is a well-understood and reproducible technology, which may include a nucleic acid sequence, molecular structure, mechanism of action, delivery method, vector, or a combination of any such technologies that FDA determines to be appropriate, where the sponsor demonstrates that the technology (1) is incorporated in or used by a drug or biological product and is essential to the structure or function of such drug or biological product; (2) can be adapted for, incorporated into, or used by, more than one drug or biological product sharing common structural elements; and (3) facilitates the manufacture or development of more than one drug or biological product through a standardized production or manufacturing process or processes.

Under section 506K(b) of the FD&C Act, a platform technology incorporated within or used by a drug or biological product is eligible for designation as a designated platform technology by FDA if (1) it is incorporated in, or used by, an approved drug (i.e., FDA reviewed and approved an application for a product incorporating or using the platform technology); (2) preliminary evidence[16] demonstrates that the platform technology has the potential to be incorporated in, or used by, more than one drug without an adverse effect on quality, manufacturing, or safety; and (3) data or information submitted by the applicable person indicates that incorporation or usage of the platform technology has a reasonable likelihood to bring significant efficiencies to the drug development or manufacturing process and to the review process.

For the purposes of this guidance, **preliminary evidence** as referred to in section 506K(b)(2) means information from completed tests or studies comparing the platform technology used in the approved or licensed drug(s) with the proposed use of the platform technology in the drug(s)

[14] See sub-section 1.4, Relationship to Other Documents, of the Electronic Common Technical Document(eCTD) v.4.0 TECHNICAL CONFORMANCE GUIDE at https://www.fda.gov/media/135573/download.

[15] See section 506K(f)(2) of the FD&C Act.

[16] The preliminary evidence would be submitted by the sponsor of the approved or licensed drug or by an applicant who has been granted a right of reference to data submitted in the application for such drug.

under investigation described in the designation request. To support a designation, this information must sufficiently demonstrate the potential for the platform technology to be incorporated in, or used by, the drug(s) under investigation without adversely affecting quality, manufacturing, or safety.[17] For example, if the sponsor wants to leverage stability testing, the preliminary evidence should demonstrate the similarities in the molecule, the manufacturing process[18] such that leveraging stability data would be justified. There should be minimal differences between the approved or licensed drug(s) using the platform technology and the drug(s) under investigation as part of an IND application that proposes to use the same platform technology. Such information could involve establishing that there are minimal differences in aspects of structure, mechanism of action, biological effect, or manufacturing processes that could affect quality or safety. Preliminary evidence should also consider what information that the applicant proposes to leverage. Preliminary evidence could include but is not limited to information on:

- Structurally similar drug substances, such as similarly sized nucleic acid sequences with comparable backbone chemistry, subunit modifications, and targeting moieties

- Minimal differences in drug product formulation, qualitatively and quantitatively; and/or

- Nearly identical manufacturing processes for drug substance and/or drug product manufacturing, and purification

As part of establishing preliminary evidence, the requester should include in their assessment all of their products that use or incorporate the platform technology regardless of current developmental or marketing status. The designation request should include summary data from the assessments of all such products The requester should include an adequate justification explaining why the summary data are sufficient to show that certain product-specific tests, analyses, or studies can be leveraged.

For purposes of the platform technology designation program, **significant efficiencies** to the drug development or manufacturing process and to the review process means that a prior test, study, or manufacturing process involving the approved or licensed drug described in section 506(K)(b)(1) of the FD&C Act could be leveraged in a subsequent application in such a way as to allow the subsequent application incorporating such information to generally be developed and reviewed in a more streamlined manner.[19] Summary evidence from completed studies

[17] Section 506K(b)(2) of the FD&C Act.

[18] In addition to the same manufacturing process—to ensure consistency and mitigate unanticipated minor differences that could result in differences in product performance and safety—the drug product manufacturing itself generally should also occur at the same manufacturing site. For a proposed manufacturing site change, FDA may ask for additional quality data, e.g., stability data, to bridge between different manufacturing sites.

[19] This interpretation for determining significant efficiencies would not affect the User Fee Agreement (UFA) goal date for an application because goal dates are determined by specific UFA commitments.

should be submitted to demonstrate that there is a reasonable likelihood that significant efficiencies exist.

B. Potential Benefits of a Platform Technology Designation

Information about a designated platform technology may be leveraged in a subsequent application when supported by sufficient preliminary evidence. The application should be from the sponsor that was originally granted the platform technology designation. Alternatively, it can be from a sponsor that has full rights of reference to that information.[20] Potential benefits to a sponsor that is granted a platform technology designation for a subsequent application may generally include one or more of the following, as deemed appropriate by FDA:

- Engaging in early interactions with FDA to discuss the use of a platform technology, including information relevant to establishing, as applicable, safety, purity, potency, or quality.

- Receiving timely advice from and having additional engagement with FDA during the development program, such as additional interactions and/or meetings on the use of the platform technology. Depending on resources, FDA might prioritize interactions or additional engagements regarding a designated platform technology for those products where the Agency has determined that there is the most significant public health benefit or impact.

- Leveraging data from a prior product that used the designated platform technology, such as leveraging batch and stability data from a related product as prior knowledge that can supplement product development studies (e.g., in-use stability studies to define administration conditions and/or light exposure studies to inform the design of the container closure system), or support shelf-life extrapolation and determination for structurally alike products.

- Leveraging certain nonclinical safety data from prior products that used the designated platform technology such that a product-specific assessment for specific, designated endpoints might not be warranted.

- Considering previous inspectional findings by FDA for subsequent marketing applications related to the manufacture of a drug that incorporates or uses the designated platform technology.

Once a platform technology has received a platform technology designation, the development or assessment of subsequent applications for drugs that use or incorporate the designated platform technology will not automatically be granted priority review based on using or incorporating a platform technology. The criteria for being granted a priority review is separate from this

[20] See section 506K(f)(2) of the FD&C Act.

program. A platform technology designation does not affect product eligibility for any expedited approval pathways if it is otherwise eligible.

C. Recommended Content for a Designation Request

A submission requesting that FDA grant a platform technology designation should include the following:

- Description of the platform technology and how it meets the statutory factors described in section II.A above. Specifically, the request should explain how the technology meets the definition under 506K(h)(1) and how it is eligible under 506K(b).

- Identification of an approved application (NDA, BLA, or ANDA) where the technology was incorporated, with applicable cross-references to other applications or submissions which the sponsor owns or has full right of reference to as part of a business agreement, and an appropriate eCTD link to the relevant and identified information (in INDs, NDAs, BLAs, or ANDAs).

- Identification of the shared structural element between drug products and how the shared structural element facilitates the use of the platform technology. Such a demonstration for a shared element could be based on a logical assertion that is supported using relevant prior knowledge and/or experimental studies.

- Justification and scientific support for the use of a platform technology across multiple drugs including how utilizing the technology in subsequent proposed products would not affect safety, quality, or manufacturing. The justification should include information to demonstrate, for example, how the technology can be incorporated in other drugs with no or only very minor differences in the relevant parts of the manufacturing process, in how the technology functions, and in relevant aspects of the safety and quality profile.

- Risk assessment to evaluate how differences between a prior product and the subsequent proposed product[21] could affect the use of the platform technology and the relevance of prior information, and therefore how much prior information would be appropriate to be leveraged in support of the subsequent proposed product.

- Information to justify why the use of the platform technology would bring significant efficiencies to the drug development or manufacturing process and to the review process for the application (e.g., allow testing or validation performed as part of developing one of the products to reduce some testing or validation for the other products and thus increase efficiency). The ability to reduce certain testing and validation for manufacturing and/or analytical methods will depend on the drug class. Whether the reduction of certain

[21] For the purposes of this guidance, a *subsequent proposed product* is a proposed drug product that is the subject of a marketing application and/or a candidate product that is the subject of an IND application.

testing or validation constitutes a significant efficiency would depend in part on the nature of the testing or validation.

The information above should be described with sufficient detail to support an evaluation of the risks associated with leveraging information about the platform technology. The sponsor should clearly explain what data or information from the designated platform technology they propose to leverage. When specifying the data, studies, or other information from the designated platform technology to be leveraged in the subsequent proposed product, the sponsor should include an adequate justification explaining why this can be leveraged where otherwise the sponsor might conduct specific tests for the subsequent proposed product.

The risk assessment should include identifying failure modes[22] related to the product differences, providing developmental data or prior knowledge that addresses potential failure modes, and considering proposals to address residual risk at the initial filing of the application (e.g., additional specification tests, in-process controls, a higher number of in-process parameters, or narrower ranges for critical process parameters).

No other structural elements in the subsequent proposed product should interfere with the ability to leverage the development information on the prior product to support the subsequent proposed product (i.e., the sponsor should show how the platform technology can be used in the same way and to the same effect in the subsequent proposed product without other factors interfering). There should also be no differences in manufacturing process parameters that would create uncertainty when leveraging the manufacturing for the subsequent proposed product.

Although some minor differences in product design, operating conditions, and/or context of use might exist between products, the experience with the platform technology in one or more other products might allow for formulation and stability bracketing approaches to cover differences in operating conditions or contexts. In the absence of cross-product experience, studies in relevant models can be used to expand the operating conditions or contexts to which a platform technology could be applied. If applicable, a comparison of raw material sources for products manufactured across a platform should be provided.

D. Meetings to Discuss a Planned Designation Request

Any meeting requests that include a discussion of a planned platform technology designation request with FDA should be made in accordance with the electronic submission guidelines[23] and

[22] See the ICH guidance for industry *Q9(R1) Quality Risk Management* (May 2023) and the draft guidance for industry *Benefit-Risk Considerations for Product Quality Assessments* (May 2022). When final, this guidance will represent the FDA's current thinking on this topic.

[23] See the "Submit Using eCTD" webpage at https://www.fda.gov/drugs/electronic-regulatory-submission-and-review/submit-using-ectd.

the Prescription Drug User Fee Act (PDUFA) meeting guidance,[24] and submitted as an amendment to the drug product's IND. The amendment should be clearly identified as a "**REQUEST FOR TYPE XX** (e.g., B, C, etc.) **MEETING**" and "**REQUEST FOR PLATFORM TECHNOLOGY DESIGNATION DISCUSSION**" in bold, uppercase letters.

Sponsors can have a preliminary discussion with the Agency regarding a planned platform technology designation request at any pre-submission meeting. In the meeting background package, the sponsor should include a summary of the data to support their platform designation request as outlined in section II.C of this guidance. At the meeting, the sponsor and the review division should discuss (1) the data that will be used to support the request and (2) future development and commercialization plans.

E. Submitting a Designation Request

Sponsors can request the designation of a platform technology at any time concurrent with or after the submission of an IND. Any platform technology designation request should be made in accordance with the electronic submission guidelines.[25] Although a sponsor can request designation concurrent with the submission of an IND, the timing of the request for designation should consider whether there are adequate product-specific data. FDA recommends the sponsor submit far enough into their development cycle to permit a determination of suitability for platform technology designation (e.g., of whether the platform technology has the potential to be incorporated in, or used by, more than one drug without an adverse effect on quality, manufacturing, or safety).[26] The sponsor's submission should clearly indicate in the administrative documents in Module 1 that it is a request for a platform technology designation. The submission should also contain the following information in Module 1:

- If a platform technology designation request is submitted to the sponsor's IND as an amendment, identification of the submission as a "**REQUEST FOR PLATFORM TECHNOLOGY DESIGNATION**" in bold, uppercase letters.

- If the request is submitted with an original IND, identification of the submission as both an "**INITIAL INVESTIGATIONAL NEW DRUG SUBMISSION**" and "**REQUEST FOR PLATFORM TECHNOLOGY DESIGNATION**" in bold, uppercase letters.

- The name of the sponsor's contact person and the contact person's address, email address, telephone number, and fax number.

- The IND application number.

[24] See the draft guidance for industry *Formal Meetings Between the FDA and Sponsors or Applicants of PDUFA Products* (September 2023). When final, this guidance will represent the FDA's current thinking on this topic.

[25] See the Submit Using eCTD webpage at https://www.fda.gov/drugs/electronic-regulatory-submission-and-review/submit-using-ectd.

[26] In most cases, this would likely be after a safe-to-proceed decision has been made for the IND.

- If available, for drugs subject to review and approval under section 505(b) of the FD&C Act, the proprietary name and active ingredient.

- For biological products, the proprietary name and proper name, if available.

F. Timing of Designation Request Submissions by the Requester and Timeline for FDA Evaluation of Designation Requests

A sponsor with an approved NDA or BLA that incorporates or uses the platform technology can submit a request for a platform technology designation concurrent with or at any time after the submission of an IND application.[27] The timing of the request for designation should consider whether there is adequate product-specific data available for the prior product and subsequent product. Although a sponsor can request designation concurrent with the submission of an IND, FDA recommends that the sponsor submit far enough into their development cycle of the product to permit a determination of suitability for platform technology designation.[28] Any designation requests that are submitted at the same time as a new IND or a subsequent IND amendment will be evaluated separately from the safety assessment of the new IND or of any subsequent IND amendments (e.g., a proposed new clinical protocol, a Chemistry, Manufacturing, and Controls (CMC) or Pharmacology/Toxicology amendment).[29] FDA will determine whether the designation meets the eligibility factors and if the platform technology will be designated within 90 calendar days from receipt of the platform technology designation request.[30] FDA will provide a written explanation to the requester regarding the determination.[31]

III. REVOCATION OF A PLATFORM TECHNOLOGY DESIGNATION

At any time after a platform technology designation is granted, FDA may revoke the designation if the Agency determines that the sponsor's designated platform technology no longer meets the eligibility factors for the platform technology designation program. FDA will communicate this revocation in writing with the rationale for the revocation.[32]

IV. POSTAPPROVAL CHANGES TO A DESIGNATED PLATFORM TECHNOLOGY

[27] This can be done under either section 505(i) of the FD&C Act or section 351(a)(3) of the PHS Act for a subsequent drug product.

[28] In most cases, this would likely be after a safe to proceed decision has been made for the IND.

[29] If the IND is placed on a full clinical hold, a simultaneously submitted designation request will be deemed inadequate for review.

[30] See section 506K(d)(1) of the FD&C Act.

[31] See section 506K(d)(3) of the FD&C Act.

[32] Section 506K(d)(4) of the FD&C Act.

A sponsor can submit changes to an approved application that incorporates the designated platform technology via a postapproval supplement to the application. The supplement should be submitted in accordance with 21 CFR 314.70 or 601.12 and as described by appropriate postapproval change guidances,[33] which outline reporting categories for postapproval changes to an approved NDA and BLA, respectively.

A sponsor of more than one approved application that uses a designated platform technology may submit a single submission of grouped supplements for CMC postapproval changes[34] and a single supplement per proposed change for nonquality-related changes to that platform technology.[35] Supplements should include a rationale to support the conclusion that the updated technology continues to meet the eligibility factors of the platform technology designation program and, as applicable, appropriately cross-reference data and information submitted in other applications. In advance of a planned change to a designated platform technology, an original application or a prior approval supplement can include one or more comparability protocols to provide for future changes to the platform technology. Such protocols should include a risk assessment regarding how the changes to the platform technology would be made for each applicable drug.[36] A new supplement should be submitted as appropriate for each impacted application.

V. GENERAL CONSIDERATIONS FOR ELIGIBILITY

Platform technologies that are appropriate for the designation program are those that meet the definition of a platform technology and the eligibility factors for designation as described in section II.A of this guidance document.[37] Included below are examples of potential platform technologies, with examples of key elements of each technology:

- Lipid nanoparticle (LNP) platforms for mRNA vaccine or gene therapy products:[38]
  - Composition, including type, amount, and manufacture of the lipids

[33] See the guidance for industry *Changes to an Approved NDA or ANDA* (April 2004) and the guidance for industry *Chemistry, Manufacturing, and Controls Changes to an Approved Application: Certain Biological Products* (June 2021).

[34] See, e.g., MAPP 5015.6 Rev. 1, *Review of Grouped Product Quality Supplements* (December 2022).

[35] Section 506K(g) of the FD&C Act.

[36] See FDA guidance for industry *Comparability Protocols for Postapproval Changes to the Chemistry, Manufacturing, and Controls Information in an NDA, ANDA, or BLA* (October 2022).

[37] Platform technology designation is separate from a request for designation (classification as a drug, device, biological product, or combination product; and center assignment for review and regulation) submitted to Office of Combination Products. See 21 CFR 3.7.

[38] Although this example includes mRNA vaccine or gene therapy products, this is not intended to suggest that other cell or gene therapy products are not appropriate for the designation program.

- Manufacturing process unit operations (e.g., transcribing RNA, synthesizing lipid moieties, and formation of the lipid nanoparticles) that are not sensitive to inputs (e.g., template sequences), and yield consistent outputs across multiple products, and where sequence differences of the mRNA have no effect on product quality

- Manufacturing process parameters, in-process controls, and equipment critical to manufacture of the mRNA LNP vaccine or gene therapy

- Process-related impurity clearance across a defined downstream purification process

- Monoclonal antibody[39] platform technologies:

  - Approaches for cell substrate and expression construct engineering that can be used with multiple products with the same upstream manufacturing process developed for the specific cell substrate and expression construct backbone

  - Process-related impurity clearance evaluated across a defined downstream purification process that can be used for multiple products with little modification

- Platforms using a chemically defined targeting moiety in conjugation with a well characterized synthetic siRNA:

  - Identification of the targeting moiety, including its synthesis, incorporation into the final drug substance, and quality control

  - Modification of synthetic siRNA sequence has no biological effect on the product quality or safety arising from the differences such that some Pharmacology/Toxicology and CMC data is potentially appropriate to be leveraged

  - Safety of the targeting moiety is not altered when used with multiple different siRNA moieties such that some Pharmacology/Toxicology data is potentially appropriate to be leveraged

  - Use of a unique method of manufacturing, purification approach, or purification strategies that simplify downstream characterization of the drug product and that can be used for multiple products with little modification

- Lipid nanoparticle platforms encapsulating different short, single stranded or double stranded oligonucleotides:

  - Composition, including type and amount of the lipids

[39] These might apply to other classes of therapeutic proteins amenable to platform approaches such as, but not limited to, multispecific antibodies and Fc-fusion proteins.

- Demonstration that, within a narrow range of double stranded or single stranded oligonucleotide length, there is no effect on product quality arising from sequence differences of the oligonucleotides[40]

- Manufacturing process parameters, in-process controls, and equipment critical to the formation of the lipid nanoparticles

For a technology to be designated, it must not only meet the platform technology definition in the statute, but it must also meet the eligibility factors for designation under 506(K)(b). It is possible, therefore, for a technology to meet the definition of a platform technology under 506K(h) but *not* be designated by FDA as a designated platform technology. For example, a technology that meets the definition of a platform technology might be inappropriate for the designation program because current review processes already reflect the use of the well-understood technology or there is a public standard. Therefore, FDA would not consider such technologies to meet the criterion of bringing significant efficiencies to the drug development, manufacturing, and review processes for the purposes of the designated platform technology program. Examples of technologies that could be inappropriate for the designation program because the technologies do not meet the definition, criteria, or both, include the following:

- Approaches to viral clearance for certain unit operations.

- Manufacturing unit operations that are sensitive to inputs (e.g., the general use of roller compaction that might be sensitive to material properties).

- Technologies that rely on established manufacturing unit operations (e.g., blending, compressing, or film coating operations).[41]

- Established formulation technologies that have been traditionally used for immediate release and extended release solid oral dosage forms (e.g., matrix, osmotic pump), established formulation technologies for oral and parenteral dosage forms, and other established drug delivery systems.

- Near-infrared technologies for monitoring in-process material attributes.

[40] Product specific stability data should be provided to demonstrate that the sequence changes modifications to the sugar backbone, phosphorothioate incorporation, or nucleobase modifications of the single stranded or double stranded oligonucleotide will not impact product quality.

[41] This prior knowledge can already be leveraged in formulation and manufacturing process development. Demonstration of the prior knowledge can also be used in applications to demonstrate unit operation robustness.

- Analytical methods leveraging prior knowledge as described in the draft ICH guidance for industry *Q14 Analytical Procedure Development* (August 2022).[42]

- Device delivery technologies (e.g., syringe, autoinjector).[43]

VI. GLOSSARY

Designated Platform Technology: In accordance with section 506K(b), (d), and the definition of "designated platform technology" in section 506K(h)(2), a platform technology that meets the following eligibility factors for granting the designation: (1) it is incorporated in, or used by, a drug approved under section 505 of the FD&C Act, or a biological product licensed under section 351 of the PHS Act; (2) preliminary evidence demonstrates that the platform technology has the potential to be incorporated in, or used by, more than one drug without an adverse effect on quality, manufacturing, or safety; and (3) data or information indicates that incorporation or use of the platform technology has a reasonable likelihood to bring significant efficiencies to the drug development or manufacturing process and to the review process.

Platform Technology: As defined in section 506K(h)(1) of the FD&C Act, a well-understood and reproducible technology, which can include a nucleic acid sequence, molecular structure, mechanism of action, delivery method, vector, or a combination of any such technologies that the Secretary determines to be appropriate, that the sponsor demonstrates (1) is incorporated in or used by a drug and is essential to the structure or function of such drug; (2) can be adapted for, incorporated into, or used by, more than one drug sharing common structural elements; and (3) facilitates the manufacture or development of more than one drug through a standardized production or manufacturing process or processes.

Preliminary Evidence: Preliminary evidence, as used in section 506K(b)(1) of the FD&C Act, refers to information from completed tests or studies that compare a platform technology that was already used in an approved or licensed drug to the proposed use of that same drug in a subsequent IND application. Preliminary evidence would include data and findings from tests or studies that evaluate proposed use of the platform technology in an already approved drug product, evaluate the proposed use of the same platform technology in a new drug product, or draw comparisons between the use of a platform technology across scenarios.

Prior Knowledge: Prior knowledge, as used in this guidance, refers to the expertise and understanding a manufacturer has built up over time, including knowledge gained from developing and manufacturing similar compounds, products, and processes; it also includes knowledge of established and accepted scientific principles.

[42] When final, this guidance will represent the FDA's current thinking on this topic.

[43] For purposes of this guidance, generally, such device delivery technologies are not essential to the structure (e.g., chemical or molecular formula) or function (e.g., molecular mechanism of action or the drug or biological product's characteristics or chemical or biological interaction with the body) of the drug or biological product. In addition, generally such device delivery technologies are not expected to facilitate the manufacture or development of a drug because generally drug manufacture is complete before the drug interacts with the delivery device. Also, the devices are not expected to bring significant efficiencies to the review process because of the existing leveraging options for delivery devices that are already incorporated in the review process (see FN 6).

Significant Efficiencies: In the context of the platform technology designation program, "significant efficiencies," as used in section 506K(b)(3) of the FD&C Act, means leveraging a previous test, study, or manufacturing process with an already approved or licensed drug in a subsequent application in such a way as to help streamline drug development or manufacturing and review.