
Platform Technology Designation Program for Drug Development Guidance for Industry

DRAFT GUIDANCE

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**U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)**

May 2024

Platform Technology Designation Program for Drug Development Guidance for Industry

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**U.S. Department of Health and Human Services
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TABLE OF CONTENTS

I.	INTRODUCTION.....	1
II.	PLATFORM TECHNOLOGY DESIGNATION REQUEST	2
A.	Eligibility for the Platform Technology Designation Program.....	4
B.	Potential Benefits of a Platform Technology Designation	6
C.	Recommended Content for a Designation Request	7
D.	Meetings to Discuss a Planned Designation Request	8
E.	Submitting a Designation Request	9
F.	Timing of Designation Request Submissions by the Requester and Timeline for FDA Evaluation of Designation Requests.....	10
III.	REVOCAION OF A PLATFORM TECHNOLOGY DESIGNATION	10
IV.	POSTAPPROVAL CHANGES TO A DESIGNATED PLATFORM TECHNOLOGY	10
V.	GENERAL CONSIDERATIONS FOR ELIGIBILITY	11
VI.	GLOSSARY.....	14

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1 **Guidance for Industry: Platform Technology Designation Program**
2 **for Drug Development¹**
3

4
5 This draft guidance, when finalized, will represent the current thinking of the Food and Drug
6 Administration (FDA or Agency) on this topic. It does not establish any rights for any person and is not
7 binding on FDA or the public. You can use an alternative approach if it satisfies the requirements of the
8 applicable statutes and regulations. To discuss an alternative approach, contact the FDA staff responsible
9 for this guidance as listed on the title page.
10

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12
13 **I. INTRODUCTION**
14

15 This guidance provides details about the implementation of the platform technology designation
16 program established by section 506K of the Federal Food, Drug, and Cosmetic Act (FD&C
17 Act).² This guidance outlines eligibility factors for receiving a platform technology designation,
18 potential benefits of receiving a designation, how to leverage data from designated platform
19 technologies, how to discuss a planned designation request as part of a milestone meeting, the
20 recommended content of a designation request submission, and the review timelines for a
21 designation request. This program is intended to result in efficiencies in drug³ development,
22 manufacturing, and review processes for drug product applications that incorporate designated
23 platform technologies.
24

25 FDA acknowledges that the term “platform technology” has been used by both industry and
26 FDA to describe technologies in ways that differ from the definitions of **platform technology**⁴
27 and **designated platform technology** that are outlined in statute and this guidance. Some
28 technologies that industry and FDA have historically considered to be platform technologies
29 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.

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30 eligible for the designation program. Ineligibility for designation does not preclude a sponsor⁵
31 from leveraging **prior knowledge** across applications.⁶ FDA has allowed sponsors to leverage
32 prior knowledge from previously submitted applications when authorizing or approving drugs in
33 an application submitted by the same sponsor.

34
35 For the platform technology designation program, section 506K of the FD&C Act establishes
36 criteria outlining who can request designations and who, once that platform technology has been
37 designated, can leverage them. This guidance describes those categories and provides
38 recommendations for the types of platform technologies that may be eligible for consideration
39 for designation. This guidance also gives recommendations for what should be included in
40 submission requests to designate a platform technology, how to update a designated platform
41 technology and, when appropriate, the process for revoking a designation.

42
43 In general, FDA’s guidance documents do not establish legally enforceable responsibilities.
44 Instead, guidances describe the Agency’s current thinking on a topic and should be viewed only
45 as recommendations, unless specific regulatory or statutory requirements are cited. The use of
46 the word *should* in Agency guidances means that something is suggested or recommended, but
47 not required.

II. PLATFORM TECHNOLOGY DESIGNATION REQUEST

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49
50
51 If FDA has approved an Abbreviated New Drug Application (ANDA), New Drug Application
52 (NDA), or Biologics License Application (BLA) for a drug that incorporates or uses a platform
53 technology as defined in section 506K(h)(1) of the FD&C Act, a sponsor of a subsequent
54 Investigational New Drug (IND), NDA, or 351(a) BLA can request designation of that platform
55 technology to enable leveraging of the technology in new or future applications.⁷ FDA
56 recommends requesting the designation of a platform technology during the IND phase of drug
57 development for a planned subsequent NDA or 351(a) BLA, because by this stage of
58 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.

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

61
62 Designation of a platform technology does not give third parties additional rights to reference
63 information from an approved product application containing that platform technology if they do
64 not own or have full rights of reference to it. In addition, a BLA holder is generally expected to
65 have knowledge of and control over the manufacturing process for the biological product for
66 which it has a license.⁸ Any referencing of data or information by an application based on a
67 platform technology designation should be consistent with this general expectation. Any relevant
68 information regarding a full right of reference agreement should be submitted with the
69 administrative documents that are included in Module 1 of Electronic Common Technical
70 Document (eCTD) submissions.⁹

71
72 FDA will review a request for designation to determine if the technology meets eligibility
73 requirements, issuing a determination not later than 90 days of receipt.¹⁰ The Agency will
74 examine if the platform technology (as defined in section 506K(h)(1)) meets the eligibility
75 factors outlined in section 506K(b) of the FD&C Act, including whether incorporation or use of
76 the platform technology is reasonably likely to bring significant efficiencies to the application
77 review process. The requester should provide a detailed justification in this regard for FDA
78 review. FDA will determine if a platform technology designation request meets the eligibility
79 factors under section 506K(b) and will provide a written explanation to the requester regarding
80 the determination. For designated platform technologies, FDA may take actions to expedite
81 development and review of any subsequent application submitted under section 505(b) of the
82 FD&C Act or section 351(a) of the Public Health Service Act (PHS Act) for a drug that uses or
83 incorporates the platform technology under section 506K(e), as appropriate.¹¹

84
85 Once the sponsor has received a platform technology designation, information previously
86 submitted in support of such designation can be leveraged in subsequent NDAs, 351(a) BLAs, or
87 requests for emergency use authorization (EUAs)¹² from the same sponsor.¹³ Sponsors of NDAs
88 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.

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

95
96 A different sponsor may also be able to leverage platform technology data if they receive a full
97 right of reference to the leveraged data under a business arrangement with the originator of the
98 platform technology.¹⁵

99

100

101 **A. Eligibility for the Platform Technology Designation Program**

102

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

114

115 Under section 506K(b) of the FD&C Act, a platform technology incorporated within or used by a
116 drug or biological product is eligible for designation as a designated platform technology by
117 FDA if (1) it is incorporated in, or used by, an approved drug (i.e., FDA reviewed and approved
118 an application for a product incorporating or using the platform technology); (2) preliminary
119 evidence¹⁶ demonstrates that the platform technology has the potential to be incorporated in, or
120 used by, more than one drug without an adverse effect on quality, manufacturing, or safety; and
121 (3) data or information submitted by the applicable person indicates that incorporation or usage
122 of the platform technology has a reasonable likelihood to bring significant efficiencies to the
123 drug development or manufacturing process and to the review process.

124

125 For the purposes of this guidance, **preliminary evidence** as referred to in section 506K(b)(2)
126 means information from completed tests or studies comparing the platform technology used in
127 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.

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128 under investigation described in the designation request. To support a designation, this
129 information must sufficiently demonstrate the potential for the platform technology to be
130 incorporated in, or used by, the drug(s) under investigation without adversely affecting quality,
131 manufacturing, or safety.¹⁷ For example, if the sponsor wants to leverage stability testing, the
132 preliminary evidence should demonstrate the similarities in the molecule, the manufacturing
133 process¹⁸ such that leveraging stability data would be justified. There should be minimal
134 differences between the approved or licensed drug(s) using the platform technology and the
135 drug(s) under investigation as part of an IND application that proposes to use the same platform
136 technology. Such information could involve establishing that there are minimal differences in
137 aspects of structure, mechanism of action, biological effect, or manufacturing processes that
138 could affect quality or safety. Preliminary evidence should also consider what information that
139 the applicant proposes to leverage. Preliminary evidence could include but is not limited to
140 information on:

- 141
- 142 • Structurally similar drug substances, such as similarly sized nucleic acid sequences with
143 comparable backbone chemistry, subunit modifications, and targeting moieties
144
- 145 • Minimal differences in drug product formulation, qualitatively and quantitatively; and/or
146
- 147 • Nearly identical manufacturing processes for drug substance and/or drug product
148 manufacturing, and purification
149

150 As part of establishing preliminary evidence, the requester should include in their assessment all
151 of their products that use or incorporate the platform technology regardless of current
152 developmental or marketing status. The designation request should include summary data from
153 the assessments of all such products. The requester should include an adequate justification
154 explaining why the summary data are sufficient to show that certain product-specific tests,
155 analyses, or studies can be leveraged.
156

157 For purposes of the platform technology designation program, **significant efficiencies** to the
158 drug development or manufacturing process and to the review process means that a prior test,
159 study, or manufacturing process involving the approved or licensed drug described in section
160 506(K)(b)(1) of the FD&C Act could be leveraged in a subsequent application in such a way as
161 to allow the subsequent application incorporating such information to generally be developed
162 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.

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

165

B. Potential Benefits of a Platform Technology Designation

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

174

- 175 • Engaging in early interactions with FDA to discuss the use of a platform technology,
176 including information relevant to establishing, as applicable, safety, purity, potency, or
177 quality.
- 178 • Receiving timely advice from and having additional engagement with FDA during the
179 development program, such as additional interactions and/or meetings on the use of the
180 platform technology. Depending on resources, FDA might prioritize interactions or
181 additional engagements regarding a designated platform technology for those products
182 where the Agency has determined that there is the most significant public health benefit
183 or impact.
- 184 • Leveraging data from a prior product that used the designated platform technology, such
185 as leveraging batch and stability data from a related product as prior knowledge that can
186 supplement product development studies (e.g., in-use stability studies to define
187 administration conditions and/or light exposure studies to inform the design of the
188 container closure system), or support shelf-life extrapolation and determination for
189 structurally alike products.
- 190 • Leveraging certain nonclinical safety data from prior products that used the designated
191 platform technology such that a product-specific assessment for specific, designated
192 endpoints might not be warranted.
- 193 • Considering previous inspectional findings by FDA for subsequent marketing
194 applications related to the manufacture of a drug that incorporates or uses the designated
195 platform technology.
- 196 •
- 197 •
- 198 •
- 199 •
- 200 •

201

202 Once a platform technology has received a platform technology designation, the development or
203 assessment of subsequent applications for drugs that use or incorporate the designated platform
204 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.

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

207

C. Recommended Content for a Designation Request

209

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

212

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

216

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

222

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

227

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

234

235 • Risk assessment to evaluate how differences between a prior product and the subsequent
236 proposed product²¹ could affect the use of the platform technology and the relevance of
237 prior information, and therefore how much prior information would be appropriate to be
238 leveraged in support of the subsequent proposed product.

239

240 • Information to justify why the use of the platform technology would bring significant
241 efficiencies to the drug development or manufacturing process and to the review process
242 for the application (e.g., allow testing or validation performed as part of developing one
243 of the products to reduce some testing or validation for the other products and thus
244 increase efficiency). The ability to reduce certain testing and validation for manufacturing
245 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.

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

248
249 The information above should be described with sufficient detail to support an evaluation of the
250 risks associated with leveraging information about the platform technology. The sponsor should
251 clearly explain what data or information from the designated platform technology they propose
252 to leverage. When specifying the data, studies, or other information from the designated platform
253 technology to be leveraged in the subsequent proposed product, the sponsor should include an
254 adequate justification explaining why this can be leveraged where otherwise the sponsor might
255 conduct specific tests for the subsequent proposed product.

256
257 The risk assessment should include identifying failure modes²² related to the product differences,
258 providing developmental data or prior knowledge that addresses potential failure modes, and
259 considering proposals to address residual risk at the initial filing of the application (e.g.,
260 additional specification tests, in-process controls, a higher number of in-process parameters, or
261 narrower ranges for critical process parameters).

262
263 No other structural elements in the subsequent proposed product should interfere with the ability
264 to leverage the development information on the prior product to support the subsequent proposed
265 product (i.e., the sponsor should show how the platform technology can be used in the same way
266 and to the same effect in the subsequent proposed product without other factors interfering).
267 There should also be no differences in manufacturing process parameters that would create
268 uncertainty when leveraging the manufacturing for the subsequent proposed product.

269
270 Although some minor differences in product design, operating conditions, and/or context of use
271 might exist between products, the experience with the platform technology in one or more other
272 products might allow for formulation and stability bracketing approaches to cover differences in
273 operating conditions or contexts. In the absence of cross-product experience, studies in relevant
274 models can be used to expand the operating conditions or contexts to which a platform
275 technology could be applied. If applicable, a comparison of raw material sources for products
276 manufactured across a platform should be provided.

277
278

D. Meetings to Discuss a Planned Designation Request

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280
281 Any meeting requests that include a discussion of a planned platform technology designation
282 request with FDA should be made in accordance with the electronic submission guidelines²³ 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>.

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

287
288 Sponsors can have a preliminary discussion with the Agency regarding a planned platform
289 technology designation request at any pre-submission meeting. In the meeting background
290 package, the sponsor should include a summary of the data to support their platform designation
291 request as outlined in [section II.C](#) of this guidance. At the meeting, the sponsor and the review
292 division should discuss (1) the data that will be used to support the request and (2) future
293 development and commercialization plans.

E. Submitting a Designation Request

294
295
296 Sponsors can request the designation of a platform technology at any time concurrent with or
297 after the submission of an IND. Any platform technology designation request should be made in
298 accordance with the electronic submission guidelines.²⁵ Although a sponsor can request
299 designation concurrent with the submission of an IND, the timing of the request for designation
300 should consider whether there are adequate product-specific data. FDA recommends the sponsor
301 submit far enough into their development cycle to permit a determination of suitability for
302 platform technology designation (e.g., of whether the platform technology has the potential to be
303 incorporated in, or used by, more than one drug without an adverse effect on quality,
304 manufacturing, or safety).²⁶ The sponsor’s submission should clearly indicate in the
305 administrative documents in Module 1 that it is a request for a platform technology designation.
306 The submission should also contain the following information in Module 1:

- 307
308
- 309 • If a platform technology designation request is submitted to the sponsor’s IND as an
310 amendment, identification of the submission as a **“REQUEST FOR PLATFORM**
311 **TECHNOLOGY DESIGNATION”** in bold, uppercase letters.
 - 312
313 • If the request is submitted with an original IND, identification of the submission as both
314 an **“INITIAL INVESTIGATIONAL NEW DRUG SUBMISSION”** and **“REQUEST**
315 **FOR PLATFORM TECHNOLOGY DESIGNATION”** in bold, uppercase letters.
 - 316
317 • The name of the sponsor’s contact person and the contact person’s address, email
318 address, telephone number, and fax number.
 - 319
320 • 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.

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- 321
- 322
- If available, for drugs subject to review and approval under section 505(b) of the FD&C Act, the proprietary name and active ingredient.
- 323
- 324
- For biological products, the proprietary name and proper name, if available.
- 325
- 326

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

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330 A sponsor with an approved NDA or BLA that incorporates or uses the platform technology can
331 submit a request for a platform technology designation concurrent with or at any time after the
332 submission of an IND application.²⁷ The timing of the request for designation should consider
333 whether there is adequate product-specific data available for the prior product and subsequent
334 product. Although a sponsor can request designation concurrent with the submission of an IND,
335 FDA recommends that the sponsor submit far enough into their development cycle of the product
336 to permit a determination of suitability for platform technology designation.²⁸ Any designation
337 requests that are submitted at the same time as a new IND or a subsequent IND amendment will
338 be evaluated separately from the safety assessment of the new IND or of any subsequent IND
339 amendments (e.g., a proposed new clinical protocol, a Chemistry, Manufacturing, and Controls
340 (CMC) or Pharmacology/Toxicology amendment).²⁹ FDA will determine whether the
341 designation meets the eligibility factors and if the platform technology will be designated within
342 90 calendar days from receipt of the platform technology designation request.³⁰ FDA will
343 provide a written explanation to the requester regarding the determination.³¹

344

III. REVOCATION OF A PLATFORM TECHNOLOGY DESIGNATION

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346

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

351

IV. POSTAPPROVAL CHANGES TO A DESIGNATED PLATFORM TECHNOLOGY

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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.

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

360
361 A sponsor of more than one approved application that uses a designated platform technology
362 may submit a single submission of grouped supplements for CMC postapproval changes³⁴ and a
363 single supplement per proposed change for nonquality-related changes to that platform
364 technology.³⁵ Supplements should include a rationale to support the conclusion that the updated
365 technology continues to meet the eligibility factors of the platform technology designation
366 program and, as applicable, appropriately cross-reference data and information submitted in
367 other applications. In advance of a planned change to a designated platform technology, an
368 original application or a prior approval supplement can include one or more comparability
369 protocols to provide for future changes to the platform technology. Such protocols should
370 include a risk assessment regarding how the changes to the platform technology would be made
371 for each applicable drug.³⁶ A new supplement should be submitted as appropriate for each
372 impacted application.

373

V. GENERAL CONSIDERATIONS FOR ELIGIBILITY

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375
376 Platform technologies that are appropriate for the designation program are those that meet the
377 definition of a platform technology and the eligibility factors for designation as described in
378 [section II.A](#) of this guidance document.³⁷ Included below are examples of potential platform
379 technologies, with examples of key elements of each technology:

380

381 • Lipid nanoparticle (LNP) platforms for mRNA vaccine or gene therapy products:³⁸

382

383 – Composition, including type, amount, and manufacture of the lipids

384

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.

Contains Nonbinding Recommendations

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- 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
- 396
- Monoclonal antibody³⁹ platform technologies:
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 - 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
- 405
- Platforms using a chemically defined targeting moiety in conjugation with a well characterized synthetic siRNA:
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 - 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
- 424
- Lipid nanoparticle platforms encapsulating different short, single stranded or double stranded oligonucleotides:
 - 425
 - 426
 - 427
 - 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.

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- 428
429 – Demonstration that, within a narrow range of double stranded or single stranded
430 oligonucleotide length, there is no effect on product quality arising from sequence
431 differences of the oligonucleotides⁴⁰
432
433 – Manufacturing process parameters, in-process controls, and equipment critical to
434 the formation of the lipid nanoparticles
435

436 For a technology to be designated, it must not only meet the platform technology definition in
437 the statute, but it must also meet the eligibility factors for designation under 506(K)(b). It is
438 possible, therefore, for a technology to meet the definition of a platform technology under
439 506K(h) but *not* be designated by FDA as a designated platform technology. For example, a
440 technology that meets the definition of a platform technology might be inappropriate for the
441 designation program because current review processes already reflect the use of the well-
442 understood technology or there is a public standard. Therefore, FDA would not consider such
443 technologies to meet the criterion of bringing significant efficiencies to the drug development,
444 manufacturing, and review processes for the purposes of the designated platform technology
445 program. Examples of technologies that could be inappropriate for the designation program
446 because the technologies do not meet the definition, criteria, or both, include the following:
447

- 448 • Approaches to viral clearance for certain unit operations.
449
450 • Manufacturing unit operations that are sensitive to inputs (e.g., the general use of roller
451 compaction that might be sensitive to material properties).
452
453 • Technologies that rely on established manufacturing unit operations (e.g., blending,
454 compressing, or film coating operations).⁴¹
455
456 • Established formulation technologies that have been traditionally used for immediate
457 release and extended release solid oral dosage forms (e.g., matrix, osmotic pump),
458 established formulation technologies for oral and parenteral dosage forms, and other
459 established drug delivery systems.
460
461 • Near-infrared technologies for monitoring in-process material attributes.
462

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.

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- 463 • Analytical methods leveraging prior knowledge as described in the draft ICH guidance
464 for industry *Q14 Analytical Procedure Development* (August 2022).⁴²
465
- 466 • Device delivery technologies (e.g., syringe, autoinjector).⁴³
467

468 VI. GLOSSARY

469
470 **Designated Platform Technology:** In accordance with section 506K(b), (d), and the definition
471 of “designated platform technology” in section 506K(h)(2), a platform technology that meets the
472 following eligibility factors for granting the designation: (1) it is incorporated in, or used by, a
473 drug approved under section 505 of the FD&C Act, or a biological product licensed under
474 section 351 of the PHS Act; (2) preliminary evidence demonstrates that the platform technology
475 has the potential to be incorporated in, or used by, more than one drug without an adverse effect
476 on quality, manufacturing, or safety; and (3) data or information indicates that incorporation or
477 use of the platform technology has a reasonable likelihood to bring significant efficiencies to the
478 drug development or manufacturing process and to the review process.
479

480 **Platform Technology:** As defined in section 506K(h)(1) of the FD&C Act, a well-understood
481 and reproducible technology, which can include a nucleic acid sequence, molecular structure,
482 mechanism of action, delivery method, vector, or a combination of any such technologies that
483 the Secretary determines to be appropriate, that the sponsor demonstrates (1) is incorporated in
484 or used by a drug and is essential to the structure or function of such drug; (2) can be adapted for,
485 incorporated into, or used by, more than one drug sharing common structural elements; and (3)
486 facilitates the manufacture or development of more than one drug through a standardized
487 production or manufacturing process or processes.
488

489 **Preliminary Evidence:** Preliminary evidence, as used in section 506K(b)(1) of the FD&C Act,
490 refers to information from completed tests or studies that compare a platform technology that
491 was already used in an approved or licensed drug to the proposed use of that same drug in a
492 subsequent IND application. Preliminary evidence would include data and findings from tests or
493 studies that evaluate proposed use of the platform technology in an already approved drug
494 product, evaluate the proposed use of the same platform technology in a new drug product, or
495 draw comparisons between the use of a platform technology across scenarios.
496

497 **Prior Knowledge:** Prior knowledge, as used in this guidance, refers to the expertise and
498 understanding a manufacturer has built up over time, including knowledge gained from
499 developing and manufacturing similar compounds, products, and processes; it also includes
500 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).

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501
502 **Significant Efficiencies:** In the context of the platform technology designation program,
503 “significant efficiencies,” as used in section 506K(b)(3) of the FD&C Act, means leveraging a
504 previous test, study, or manufacturing process with an already approved or licensed drug in a
505 subsequent application in such a way as to help streamline drug development or manufacturing
506 and review.
507