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Billion Dollar Orphans: Tension Between the Legal Intent and Social Purpose of the Orphan Drug Act

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BILLION DOLLAR ORPHANS: TENSION BETWEEN THE LEGAL INTENT AND SOCIAL PURPOSE OF THE ORPHAN DRUG ACT

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* J.D. Candidate, University of California, Davis School of Law, 2019. Loyola Marymount University, M.A. Urban Education Policy, 2015. Brown University, A.B. Public Policy; A.B. Economics, 2013. As our country continues to grapple with whether, and to what extent healthcare is a fundamental right, I eagerly offer this Comment as a small contribution to the dialogue, and to the shared goal of remedying diseases for which affordable cures remain elusive. Inspiration for this Comment is owed to my father, Dr. William F. Sheridan. Our spirited discussions on public policy continue to yield a healthy blend of frustration and resolve in how to improve the plights of others. Lastly, without the astute edits of Danielle Lauber, Professor Peter Lee, and the undying support of my family, these thoughts on the Orphan Drug Act would have never made it to publication. Final thanks are owed to the Editors of the *Texas A&M Law Review* for their careful effort in bringing this Comment to press.

I. INTRODUCTION

Pharmaceutical companies leverage the inelastic demand[1] of the pharmaceutical drug market by offering remedies, treatments, and cures to sick people to generate substantial profits.[2] However, rare diseases continue to frustrate scientists trying to find cures.[3] Frequently, prohibitive costs or confounding biological and technological barriers thwart the would-be development of remedies for serious diseases.[4] In 1983, a bipartisan U.S. Congress sought to take on the financial barriers that stall the development of drugs for diseases without any cost-effective pathway to development.[5] Recognizing that rare diseases frequently lack a sufficiently profitable market to motivate pharmaceutical companies to develop and research remedies, Congress enacted the Orphan Drug Act ("ODA" or the "Statute").[6] The ODA emerged with a variety of financial incentives for pharmaceutical companies, including tax breaks, periods of marketing exclusivity, and a fast-track approval process for the development of drugs targeted at orphan diseases.[7] Orphan diseases are those that affect fewer than 200,000 people in the United States or diseases for which the likelihood of recuperating development costs to make the drug profitable is small.[8]

1. *See, e.g.*, Marin Gemmill, The Price Inelasticity of Pharmaceutical Drugs: An Exploration of Demand in Different Settings, 49 (January 2008) (unpublished Ph.D. dissertation, London School of Economics and Political Science), https://etheses.lse.ac.uk/2944/1/U615895.pdf [https://perma.cc/8EEG-DT3K] ("[G]iven that the demand for brand-name drugs should be very inelastic when there are few therapeutic and no molecular substitutes and much higher when there are generic drugs available.").

2. Richard Anderson, *Pharmaceutical Industry Gets High on Fat Profits*, BBC NEWS (Nov. 6, 2014), http://www.bbc.com/news/business-28212223 [https://perma.cc/8H6S-PPWD].

3. Greg Breining, *Rare Diseases Difficult to Diagnose, Cures Hard to Come by*, ASS'N AM. MED. COLLEGES NEWS (Apr. 11, 2017), https://news.aamc.org/research/article/rare-diseases-difficult-diagnose-cures-hard-come/ [https://perma.cc/9BXD-KSSR].

4. *See id.*

5. *See* Orphan Drug Act of 1983, Pub. L. No. 97-414, § 1(b)(5)–(6), 96 Stat. 2049, 2049 (1983).

6. *Id.*

7. *Id.*

8. *See* 21 U.S.C. § 360bb(a)(2) (2012) (defining a "rare disease or condition" as one "affect[ing] less than 200,000 persons in the United States"); *see also* Scott Got-

Since the early 1980s, pharmaceutical companies have leveraged the benefits of the ODA to successfully develop more than 600 orphan drugs.[9] The ODA survived thirty-five years without major amendment, evidencing its general efficacy and success.[10] In 2016 alone, the Office of Orphan Products Development ("OOPD") received 568 new requests for designation—more than double the number of requests received in 2012.[11] This dramatic increase in petitions illustrates the ODA's continued success.[12] In response to the increasing requests for designation, the Food and Drug Administration ("FDA") recently issued guidance on how it will address the "backlog of existing designation requests."[13] This recent guidance seeks to improve the efficiency of the ODA and streamline the approval process for new orphan drug designations.[14]

Though the ODA has successfully stimulated the development of orphan drugs, other consequences of the ODA currently dominate the conversation about the Statute.[15] Recent trends showing dramatic increases in ODA petitions and approvals cause commentators to question whether pharmaceutical companies abuse the ODA.[16] Critics allege that ambiguous language within the Statute provides pharma-

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tlieb, *FDA Is Advancing the Goals of the Orphan Drug Act*, U.S. FOOD & DRUG ADMIN., (Sept. 12, 2017), https://www.fda.gov/NewsEvents/Newsroom/FDAVoices/ucm612012.htm [https://perma.cc/497D-EYGB] ("[A] rare disease [is] defined as a disease [that] generally affects fewer than 200,000 people in the United States . . . .").

9. *See* U.S. FOOD & DRUG ADMIN., FDA'S ORPHAN DRUG MODERNIZATION PLAN 2 (2017), https://www.fda.gov/downloads/ForIndustry/DevelopingProductsforRareDiseasesConditions/HowtoapplyforOrphanProductDesignation/UCM565068.pdf [https://perma.cc/K5NW-7EAW]; *see also* Sarah Jane Tribble, *Sen. Grassley Launches Inquiry Into Orphan Drug Law's Effect on Prices*, NPR (Feb. 10, 2017, 10:10 AM ET), https://www.npr.org/sections/health-shots/2017/02/10/514373480/sen-grassley-launches-inquiry-into-orphan-drug-laws-effect-on-prices [https://perma.cc/EAD2-W3AL].

10. *See generally* Kurt R. Karst, *Fitting New Scientific Advances Into an Old Regulatory Paradigm: Fusion Proteins and Orphan Drug "Sameness"*, FDA L. BLOG (July 25, 2017), http://www.fdalawblog.net/fda_law_blog_hyman_phelps/2017/07/fitting-new-scientific-advances-into-an-old-regulatory-paradigm-fusion-proteins-and-orphan-drug-same.html [https://perma.cc/8JHG-D4NF] (noting that "there are other, less visible measures of the success of the ODA, such as FDA's ability to keep up with and address scientific advances in an aging regulatory paradigm").

11. *See* U.S. FOOD & DRUG ADMIN., *supra* note 9.

12. *Id.*

13. *Id.*

14. *See id.*

15. *See generally* Shannon Gibson & Barbara von Tigerstrom, *Orphan Drug Incentives in the Pharmacogenomic Context: Policy Responses in the US and Canada*, 2 J.L. & BIOSCIENCES 236 (2015) (discussing how new genomic technologies changes the definition of an orphan-drug subset); Lydia Raw, Note, *Are We Adopting the Orphans, or Creating Them? Medical Ethics and Legal Jurisprudential Guidance for Proposed Changes to the Orphan Drug Act*, 9 WASH. U. JURIS. REV. 295, 296 (2017).

16. *See* Karst, *supra* note 10 ("The success of the ODA is most apparent from the increasing number of orphan drug approvals and orphan drug designations each year[;] . . . [i]n fact, there are so many orphan drug designation requests these days that the FDA had to create an Orphan Drug Modernization Plan. . . .").

ceutical giants with latitude to misuse the incentives of the ODA for profit.[17] Many critics raise concerns that prohibitively expensive orphan drug prices are partially attributable to the way Congress drafted the ODA.[18] Ohio Senator Sherrod Brown commented in December 2015 that the ODA has never been purposed to "pad the profit margins of big pharma."[19] Senator Brown was concerned that loopholes in the Statute enable pharmaceutical companies to abuse the market exclusivity and tax-benefit provisions of the ODA.[20] Other critics regard the ODA as a waste of resources that diverts funds from more common diseases' cost-effective treatments to "ultra-orphan" disease treatment.[21]

National Public Radio's Kaiser Health News ("KHN") published a series of reports that echo these criticisms and highlight the extent to which the ODA drives up drug prices.[22] Some critics voicing skepticism that the ODA achieves its aims are original sponsors of the Statute.[23] Moreover, the inaccessibility of the medication due to the exorbitant pricing of the drugs concerns congressional leaders.[24] Other commentators similarly allege that the ODA contributes to the high price of orphan drugs, straining the ability of insurance companies to make the drugs available on the market.[25]

Affording drugs proves impossible for some patients suffering from orphan diseases.[26] In 2016, a thirty-day treatment of at least ten different orphan drugs would cost a patient more than $40,000.[27] Crystiva, for example, is an orphan drug that treats X-linked hypophosphatemia and costs $160,000 per year for kids and $200,000 for

17. *See* John T. Aquino, *Do Biopharmas Abuse the Orphan Drug Act? Debate Resurfaces*, BLOOMBERG (Jan. 30, 2017), https://www.bna.com/biopharmas-abuse-orphan-n57982083049/ [https://perma.cc/ZU8M-VGDA] (suggesting that KHN's criticisms aren't entirely new and that commentators have noticed approval of drugs that were never intended to support orphan populations).

18. *See* Sarah Jane Tribble & Sydney Lupkin, *High Prices for Orphan Drugs Strain Families and Insurers*, NPR (Jan. 17, 2017, 1:36 PM ET), https://www.npr.org/sections/health-shots/2017/01/17/509507035/high-prices-for-orphan-drugs-strain-families-and-insurers [https://perma.cc/42GJ-N9QF] (quoting U.S. Rep. Henry Wasman, D-Calif. as saying "[w]hat was intended for a good purpose can be used for a purpose that's harmful to patients who can't afford drugs").

19. *See* Dina Gusovsky, *How a Blockbuster Drug Can Become a Subsidized 'Orphan'*, NBR (Dec. 2, 2015), http://nbr.com/2015/12/02/how-a-blockbuster-drug-can-become-a-subsidized-orphan/ [https://perma.cc/2QFS-3EVM].

20. *See id*.

21. Jonathan Wilcox, *Orrin Hatch, It's Time to Defend the Orphan Drug Act*, HILL (May 29, 2017, 10:00 AM EDT), http://thehill.com/blogs/pundits-blog/healthcare/335372-orrin-hatch-its-time-to-defend-the-orphan-drug-act [https://perma.cc/Z3Q4-MDEL].

22. *See* Tribble & Lupkin, *supra* note 18. *See generally* Raw, *supra* note 15, at 307.

23. *See* Tribble & Lupkin, *supra* note 18.

24. *Id.*

25. *Id.*

26. *Id.*

27. *Id.*

adults.[28] In other cases, drugs may cost patients suffering from orphan diseases $28,000 for a thirty-day supply, or more than $336,000 annually.[29] While the astronomical orphan drug prices financially immobilize patients, these drug prices yield handsome revenues for pharmaceutical companies.[30] Though the high price of orphan drugs does not directly offend the Statute,[31] critics fear that the lucrative periods of exclusivity that breathe life into orphan remedies might perpetuate the extreme cost of these treatments.[32]

This Comment examines the extent to which Congress empowered the FDA to address the increase in petitions and the general accessibility of orphan drug remedies. Specifically, this Comment seeks to understand why the FDA's interpretation of the *purpose*[33] of the ODA seems to conflict with the statutory *intent*[34] as interpreted by federal courts.[35] This Comment considers a statute's ultimate goal or social purpose to be the *purpose* of the statute, whereas the express mechanisms by which Congress seeks to bring about these goals is best understood as the statute's *intent*.[36] To understand the FDA and judiciary's differing interpretations of the ODA, this Comment analyzes the language of the Statute, recent ODA litigation, FDA's promulgated regulations, as well as recent response to pharmaceutical companies' increase in designation requests for orphan drugs.[37]

Ultimately, this Comment strives to determine whether or not the ODA can effectively achieve the goals Congress set forth in 1983.[38] This Comment conducts a statutory analysis of the ODA and closely examines how courts, the FDA, and litigant pharmaceutical compa-

28. *See* Mark Terry, *Debate Over the Orphan Drug Act Heats Up*, BIOSPACE (Aug. 30, 2018), https://www.biospace.com/article/-ppl4-debate-over-the-orphan-drug-act-heats-up/ [https://perma.cc/Y6XT-UQDG].

29. *See* Tribble & Lupkin, *supra* note 18.

30. *See id*. *See also* Gusovsky, *supra* note 19 ("Drugs approved by the Food and Drug Administration as 'orphan drugs' have seen sales increase from $46.6 billion in 2014 to $54 billion this year in the U.S. alone and are projected by drug industry consultant EvaluatePharma to reach above $60 billion in 2016. Worldwide, orphan drug sales are forecast to total $102 billion this year and $178 billion by 2020.").

31. *See* Orphan Drug Act of 1983, Pub. L. No. 97-414, § 1(b)(5)–(6), 96 Stat. 2049, 2049 (1983).

32. *See* Tribble & Lupkin, *supra* note 18 ("[O]rphan drugs like Cerezyme often have very few competing drugs that could help them drive down the cost . . . . '[T]he pharmaceutical company can set the price and [the pharmacies] have to be a price acceptor.'").

33. When italicized as *purpose*, the Comment refers to the "ultimate goal" of the ODA: treating victims of orphan diseases. *See infra* Part III.A.

34. When italicized as *intent*, the Comment refers to Congress's express authorization to create lucrative financial incentives for drugs that have earned orphan drug designation. *See infra* Part III.B.

35. *See infra* Part III.B.

36. *See infra* Part III.B.

37. *See infra* Parts IV–V.

38. *See infra* Part III.

nies interpret the Statute differently.[39] This Comment argues that Congress's *intent* in passing the ODA was to create lucrative incentives for the development of drugs for orphan diseases.[40] But, Congress's *purpose* in drafting the ODA was to ensure the drugs became available to patients.[41] The incentives serve as a tool to achieve the *purpose* of the ODA: to treat patients suffering from rare diseases.[42] This Comment concludes that to better effectuate this *purpose*, Congress must amend the ODA or pass other legislation empowering the FDA to promulgate regulations that alter the schedule and administration of the ODA's lucrative "basket of goodies."[43]

Part I analyzes the history of the ODA and discusses how the Administrative Procedure Act ("APA") and *Chevron v. NRDC* relate to ODA litigation.[44] Part II analyzes *Depomed v. HHS*, an important recent case involving the ODA, to illustrate the conflict between the legislative *intent* and the social *purpose* of the ODA.[45] This Section focuses on distinguishing between the *purpose* of the Statute as articulated in the FDA's guidance and the ODA itself, with the *intent* of the ODA as revealed through litigation in federal courts.[46] Part IV examines whether the FDA's recently promulgated regulations will aid in effectuating the ODA's goals and survive scrutiny in court.[47] Part V proposes a series of amendments to the ODA to reconcile the differences between its *intent* and *purpose*.[48] Additionally, Part V argues that "salami-slicing," the re-marketing of existing drugs for ODA purposes, does not violate the *intent* of the ODA, but it thwarts the *purpose* of the ODA.[49] The Comment concludes with a prediction of what future litigation will yield if Congress does not amend the ODA.[50]

II. BACKGROUND

A. *History and Design of the ODA*

In drafting the ODA, the authors specifically contemplated diseases like amyotrophic lateral sclerosis ("ALS") and Tourette's syndrome.[51] Because of the small population of Americans affected by diseases

39. *See infra* Part III.
40. *See infra* Part III.B.
41. *See infra* Part III.A.
42. *See infra* Part III.
43. *See* Tribble & Lupkin, *supra* note 18 ("In a 2009 webinar, an FDA official referred to the incentive package as 'our basket of goodies.'").
44. *See infra* Part II.
45. *See infra* Part III.
46. *See infra* Part III.
47. *See infra* Part IV.
48. *See infra* Part V.
49. *See infra* Part V.A.
50. *See infra* Part VI.
51. *See* Orphan Drug Act of 1983, Pub. L. No. 97–414, § 1(b)(1), 96 Stat. 2049, 2049 (1983). *See also* Tribble & Lupkin, *supra* note 18 ("Meyers[,] . . . an angry mother who wouldn't put up with pharmaceutical companies ignoring her son's ill-

like ALS and Tourette's syndrome, pharmaceutical companies maintained a low likelihood of recuperating the costs of developing drugs for such diseases.[52] To address the lack of motivation for pharmaceutical companies or "sponsors" to invest in developing orphan drugs, Congress sought to provide financial incentives to spur their development.[53] The Statute calls these diseases "rare diseases or conditions."[54] Accordingly, a rare disease or condition refers to any disease for which a company has no reasonable expectation of recovering its costs of development from sales.[55]

As intended, the ODA reduces the financial barrier preventing orphan drugs from making it to market.[56] Presumably, if a sufficiently profitable market could sustain a sponsor's drug, it would never require or receive the orphan drug benefits.[57] As the Statute notes, the FDA will designate a drug as an orphan drug after "the Secretary finds that [the] drug for which [the] request is submitted . . . is being or will be investigated for a rare disease or condition and . . . an application for such drug is approved."[58] Congress expressly delegated this determination to the FDA to ensure that the ODA incentives are used to effectuate the Statute's goals.[59]

To protect the sponsor's development of an orphan drug, the ODA guarantees a seven-year period of exclusivity if the statutory obligations are met.[60] This period of exclusivity is subject to two exceptions.[61] First, the FDA may approve another drug for the same disease and condition as the first if the "holder of the approved application or of the license cannot assure the availability of sufficient quantities of the drug to meet the needs of persons with the disease or condition for which the drug was designated."[62] Second, if the holder provides written consent for the "approval of other applications or the issuance of other licenses before the expiration of [the] seven-year period," the FDA may violate the original sponsor's exclusivity period and grant a new drug status as an orphan drug.[63] These statutory exceptions sug-

ness, Tourette syndrome . . . mobilized advocates, lawmakers[,] and even TV actor Jack Klugman . . . to persuade Congress to pass the Orphan Drug Act.").

52. *See* Orphan Drug Act of 1983 § 1(b)(1).

53. *See* 21 U.S.C. §§ 360aa, 360ee (2012).

54. 21 U.S.C. § 360bb (2012).

55. 21 U.S.C. § 360bb(a)(2)(A) (defining a "rare disease or condition" as one "affect[ing] less than 200,000 persons in the United States").

56. Orphan Drug Act of 1983 § 1(b)(5).

57. *See id.* § 1(b)(3).

58. 21 U.S.C. § 360bb.

59. *Id*.

60. *See* 21 U.S.C. § 360bb(a)(1)(A)–(B) (outlining that the drug must be designated to treat or investigate an orphan disease and its application be approved under 21 U.S.C. § 355 or 42 USC § 262 and noting that once it has been, the "Secretary shall designate the drug as a drug for such disease or condition").

61. *See* 21 U.S.C. § 360cc(b) (2012).

62. 21 U.S.C. § 360cc(b)(1).

63. 21 U.S.C. § 360cc(b)(2).

gest that Congress prioritizes making viable drugs available in the market above determining which sponsor gets to bring that drug to market.[64]

The ODA does not secure market exclusivity by patents, though it works similarly in effect.[65] Periods of exclusivity are more valuable benefits than patents, however, because they are self-effectuating.[66] That is, when administered correctly, periods of exclusivity are distinguishable from patents in that a sponsor should not need to litigate for them to become effective.[67] Thus, when a sponsor qualifies for seven years of marketing exclusivity, they receive a substantial financial benefit, potentially foreclosing other companies' ability to market generic drugs for the same disease.[68] The Statute also extends tax credits for companies to test expenses of orphan drugs[69] and credit for qualified clinical testing expenses.[70] Sponsors also benefit from "defraying the costs of qualified testing expenses incurred in connection with the development"[71] of orphan drugs. Taken together, these statutory protections of the development and distribution of orphan drugs provide strong incentives for pharmaceutical companies to market orphan drugs.[72]

B. *Judicial Doctrines*

1. The Administrative Procedure Act

Congress grants administrative agencies authority to engage in expressly delegated quasi-legislative functions.[73] The APA outlines the process that holds administrative agencies accountable for the regulations Congress authorizes them to promulgate.[74] Congress authorizes agencies like the FDA, the Department of Justice, and the Environmental Protection Agency to engage in rulemaking to effectuate a

64. *Id.*

65. *See* Robin Feldman, *Regulatory Property: The New IP*, 40 COLUM. J.L. & ARTS 53, 60 ("Although marketing rights are focused on selling in the market, they are somewhat stronger than ordinary patent rights. First, patent rights are not self-executing. No district attorney, no federal agency will step forward to champion a patent holder's rights. If a patent holder wishes to exercise its right to exclude someone from selling the product, the patent holder must bring a lawsuit. In contrast, when a company receives marketing rights, the FDA enforces those rights by refusing to grant approval to any other company.").

66. *Id.*

67. *Id.*

68. *Id.* at 60–61.

69. *See* I.R.C. § 45C (2012) ("Clinical testing expenses for certain drugs for rare diseases or conditions.").

70. I.R.C. § 45C(c).

71. 21 U.S.C. § 360ee(a) (2012).

72. *See* I.R.C. § 45C ("Clinical testing expenses for certain drugs for rare diseases or conditions").

73. *See, e.g.*, INS v. Chadha, 462 U.S. 919 (1983).

74. *See* Administrative Procedure Act, Pub. L. No. 79-404, 60 Stat. 237 (1946).

given statute.[75] Section 706 of the APA grants judicial oversight to agency actions to protect the public from agencies that stray from Congress's goals in passing a law.[76] Specifically, any agency action, determination, or promulgated rule is subject to judicial review under section 706.[77] Thus, if pharmaceutical companies object to the FDA's actions related to the ODA, they may bring challenges under the APA. As such, companies may claim that the FDA violates the APA by improperly interpreting the meaning of specific language in the ODA.[78] However, it is settled law that "a court is not to substitute its judgment for that of the agency."[79] In reviewing these APA claims, federal courts engage in what is known as a *Chevron* analysis.

2. *Chevron* Analysis

In *Chevron v. NRDC*, the U.S. Supreme Court set forth a two-part test to evaluate whether to defer to a government agency's interpretation of a statute that the agency administers.[80] In *Chevron*, the Court recognized that the judicial branch should not evaluate the wisdom or merits of congressional action, including authority delegated to agencies.[81] The Court held that "[only] if the statute is silent or ambiguous with respect to the specific issue, [will the court ask] whether the agency's answer is based on a permissible construction of the statute."[82] In the face of a challenge to administrative action, the Court held that the role of the judiciary is to determine whether or not Congress created a space where the agency needed to "elucidate a specific provision of the statute by regulation."[83]

Under the first step of *Chevron*, courts use basic tools of statutory construction to review whether Congress addressed the issue in question.[84] Courts will give effect to the plain language of a statue when it is unambiguous and are reluctant to construe ambiguities in a way that offends congressional intent.[85] When no ambiguity is found, the reviewing court and the agency must give effect to the unambiguously

75. *See, e.g.*, 5 U.S.C. § 551(4) (2012).

76. 5 U.S.C. § 706 (2012).

77. *Id.* ("[H]old unlawful and set aside agency action, findings, and conclusions found to be—(A) arbitrary, capricious, an abuse of discretion, or otherwise not in accordance with law.").

78. *See, e.g.*, Depomed, Inc. v. U.S. Dep't of Health & Human Servs., 66 F. Supp. 3d 217 (D.D.C. 2014).

79. Motor Vehicle Mfrs. Ass'n of U.S. v. State Farm Mut. Auto. Ins. Co. 463 U.S. 29, 42–43 (1983).

80. Chevron v. Nat. Res. Def. Council, 467 U.S. 837, 865–66 (1984); *see also* David J. Barron & Elena Kagan, *Chevron's Nondelegation Doctrine*, 2001 SUP. CT. REV. 201.

81. *See Chevron*, 467 U.S. at 866.

82. *Id*. at 843.

83. *Id.* at 843–44.

84. *See id.* at 842–43.

85. Schafer v. Astrue, 641 F.3d 49, 54 (4th Cir. 2011) (noting that "[t]he plain language of the statute is the most reliable" indicator of congressional intent).

expressed intent of Congress.[86] Moreover, because "[j]udges are not experts in the field, and are not part of either political branch of government,"[87] courts will only engage in the second level of a *Chevron* analysis ("*Chevron* II") when an ambiguity requires such analysis.[88]

Under the second step of *Chevron*, courts determine "whether the agency's action is based on a permissible construction of the statute."[89] Often, so long as the agency's interpretation represents a "reasonable accommodation of conflicting policies that were committed to the agency's care by the statute," the court will afford the agency what is known as *Chevron* deference.[90] Agencies entitled to *Chevron* deference can exercise discretion in the space of congressional silence or ambiguity unless a court finds their interpretations unreasonable.[91] This Comment considers several instances involving ODA litigation and corresponding *Chevron* analysis.[92]

III. THE LEGISLATIVE *INTENT* OF THE ODA CONFLICTS WITH ITS *PURPOSE*

Interpreting the legislative intent of a statute often proves a complex task.[93] Scholars, judges, and agencies can choose from a variety of approaches to interpret a statute's meaning.[94] Some approaches seek to unpack a bill's legislative history to arrive at the meaning of the statutory text.[95] Other approaches consider Congress's general intent or what the political context suggests about the statute's purpose.[96] In contrast, some textualist and plain meaning approaches refuse to consider anything but the simplest sources of guidance—like dictionaries.[97] The Supreme Court's *Chevron* framework safeguards judges from complex statutory analysis when the statute's language is unambiguous on its face.[98] Moreover, if a *Chevron* II analysis is re-

86. *See Chevron*, 467 U.S. at 842.
87. *Id.* at 865.
88. *Id.* at 842–43.
89. *Id.* at 843.
90. Otsuka Pharm. Co. v. Burwell, No. GJH-15-852, 2015 U.S. Dist. LEXIS 68230, at *35 (D. Md. May 27, 2015).
91. *See Chevron*, 467 U.S. at 842; *see generally* Barron & Kagan, *supra* note 80.
92. *See infra* Part III.A.
93. *See generally* WILLIAM N. ESKRIDGE, JR. ET AL., LEGISLATION AND STATUTORY INTERPRETATION, 214 (1st ed. 2000) ("The trouble starts when you try to determine what is meant by legislative intent . . . .").
94. *See id.* at 211–12, 223.
95. *Id.* at 211.
96. *Id.* at 221 ("Purposivism attempts to achieve the democratic legitimacy of other intentionalist theories in a way that renders statutory interpretation adaptable to new circumstances. Purposivism sets the originalist inquiry at a higher level of generality. It asks, 'What was the statute's goal?' rather than 'What did the drafters specifically intend?'").
97. *Id.* at 228.
98. *See Chevron*, 467 US. at 842–43 ("When a court reviews an agency's construction of the statute which it administers, it is confronted with two questions. First,

quired, agencies receive extensive deference when reasonably interpreting their own regulations, so long as the interpretation is not wholly inconsistent with the statute.[99]

Interpretation of a statute's meaning is important because it can reveal a discrepancy between a statute's legislative *intent* and social *purpose*.[100] As discussed above, this Comment considers a statute's ultimate goal or social purpose to be the *purpose* of the statute, whereas the express mechanisms by which Congress seeks to bring these goals about is best understood as the *intent*.[101] A variety of statutory interpretation methods demonstrate differences between *intent* and *purpose*.[102] Textualist methods of statutory analysis provide the most predictable and straightforward ways to examine the meaning of statutes.[103] However, these methods of analysis can neglect the ultimate goals of the legislation.[104] Often, analysis of a statute's text alone will only reveal the literal *intent* evident in the language of the statute, neglecting to consider the legislation's broader aims, or *purpose*.[105]

Other approaches, known as intentionalist frameworks, consider contextual factors and evidence outside the four corners of the law.[106] Purposivism, for example, seeks to determine if there is a difference between a statute's apparent goal and what the drafters intended.[107] The purposivism approach to statutory analysis relies less on a strict construction of the statute's text and credits the relative context at the enactment of the legislation.[108] This approach affords the statute's reader latitude in addressing new or unforeseen circumstances.[109] Especially in situations where a statute's *intent* may not accord with its *purpose*, the purposivism approach makes analyzing a statute's effec-

always, is the question whether Congress has directly spoken to the precise question at issue. If the intent of Congress is clear, that is the end of the matter; for the court, as well as the agency, must give effect to the unambiguously expressed intent of Congress.").

99. *See* Bowles v. Seminole Rock & Sand Co., 325 U.S. 410, 414 (1945) (noting that, in construing administrative regulations, "the ultimate criterion is the administrative interpretation, which becomes of controlling weight unless it is plainly erroneous or inconsistent with the regulation."); *see also* Thomas Jefferson Univ. v. Shalala, 512 U.S. 504, 512 (1994) (noting that "an agency's construction of its own regulations is entitled to 'substantial deference'").

100. *See* ESKRIDGE, JR. ET AL., *supra* note 93, at 221.

101. *See infra* Part III.

102. *See* ESKRIDGE, JR. ET AL., *supra* note 93, at 211–12, 223.

103. *Id.* at 223.

104. *Id.*; *see also* Bradley Silverman, *Statutory Ambiguity in King v. Burwell: Time for a Categorical Chevron Rule*, 125 YALE L.J. F. 44, 53 (2015) (suggesting that at times where a statute's text and legislative history are equally clear and still in conflict, a categorical "agency wins" rule advances "*Chevron*'s purpose of empowering agencies with broad policymaking latitude").

105. *See* ESKRIDGE, JR. ET AL., *supra* note 93, at 223.

106. *Id.* at 214.

107. *Id.* at 221.

108. *See id.*

109. *Id.*

tiveness more feasible.[110] In addition, this approach provides greater emphasis on what this Comment refers to as the statute's *purpose*, whereas the textualist methods of statutory analysis tend to only reveal a statute's literal *intent*.[111] However, the argument that the statutory *purpose* better captures a drafter's goals relies on an essential foundation: that the goal of the statute is discernable from the congressional record.[112] In this case, the *purpose* of the ODA is largely uncontroversial.[113]

A. *The Purpose of the ODA is to Treat Patients Suffering from Rare Diseases*

The distinction between the *intent* and *purpose* of the ODA explains much of the recent controversy surrounding the Statute. As designed, the ODA operates by creating lucrative incentives to spur development of remedies for orphan diseases and to provide therapies or cures to patients suffering from rare diseases.[114] The *intent* of the Statute is to provide these financial incentives to pharmaceutical companies and inspire them to take on otherwise unprofitable drug development.[115] The development of these orphan drugs ultimately achieves the *purpose* of the ODA: providing treatments for patients suffering from orphan diseases.[116] The congressional record and the language of the Statute confirm this ultimate goal, or *purpose*.[117]

The FDA understands the ODA's ultimate *purpose* well.[118] In June 2017, the FDA released the Orphan Drug Modernization Plan ("Modernization Plan" or the "Plan").[119] The Plan seeks to assure the public that the FDA is aware of the recent backlog in orphan drug petitions and will actively "enable continued progress toward more treatments

110. *See id.* at 221–22.

111. *See id.* at 223.

112. *See id.* at 221.

113. *See infra* Part III.A; *see also* Matthew Herder, *What Is the Purpose of the Orphan Drug Act?*, PLOS MED. (Jan 3, 2017), http://journals.plos.org/plosmedicine/article/file?id=10.1371/journal.pmed.1002191&type=printable [https://perma.cc/3ZSF-VZAH] (arguing that the "purpose" of the ODA is what this Comment refers to as the *intent*, the "[redistribution] of resources to medical needs that would otherwise be marginalized by market forces").

114. *See, e.g.*, 21 U.S.C. § 360ee(a) (2012).

115. *Id.*

116. *See* Orphan Drug Act of 1983, Pub. L. No. 97-414, § 1(b) 96 Stat. 2049, 2049 (1983).

117. 21 U.S.C. § 360ee(a).

118. *See* U.S. FOOD & DRUG ADMIN., *supra* note 9; *see also* Orphan Drug Regulations, 78 Fed. Reg. 35,117, 35,122 (to be codified at 21 C.F.R. pt. 316) (noting that the "FDA continues to believe that the current framework is the best means for giving effect to the intent of the Orphan Drug Act, [and] to provide incentives for sponsors to develop promising drugs for rare diseases and conditions that would not otherwise be developed and approved").

119. *See* U.S. FOOD & DRUG ADMIN., *supra* note 9.

and even potential cures for rare diseases."[120] Throughout the Plan, the FDA clearly articulates that the Statute's goal is to "[pursue] treatments for rare diseases," and to continue "progress for the millions of patients who are affected by one of these disorders."[121] Thus, the FDA accepts that the *purpose* of the ODA focuses on providing treatment to patients who suffer from rare diseases.

It is unpersuasive to suggest that the goal of the ODA is to provide subsidies to financially enrich pharmaceutical companies.[122] The language and legislative history of the Statute support the accuracy of the FDA's interpretation of the ODA.[123] The Statute explicitly refers to the important public interest in reducing costs of developing drugs for orphan diseases.[124] Additionally, the House of Representatives expressly intended for ODA benefits to help orphan drug sponsors to "recoup the cost of development by capturing all revenues from the sale of the drug for the rare disease."[125]

However, some critics of the ODA assert its *purpose* extends beyond the goal of treating rare diseases.[126] These critics suggest that the general motivation of the ODA is to "redistribute resources to medical needs that would otherwise be marginalized by market forces."[127] Such a purpose encompasses more than treating the most rare and costly of diseases and their various sub-classifications.[128] But this broader interpretation of the ODA's *purpose* is inconsistent with the Statute.[129] The name of the Statute and its opening remarks suggest a narrower *purpose* of the law, which expressly addresses concerns facing the market for rare diseases.[130] A broad interpretation that allocates some resources to unmet medical needs—like diseases that disproportionately affect the world's poor—might be noble, but it is inconsistent with the *purpose* of the ODA.[131]

Under purposivism, it is essential to clearly define the ODA's objective from the outset to determine if the Statute is being effectuated as designed.[132] Though the ultimate *purpose* of the ODA is relatively unambiguous, problems emerge when courts adjudicate claims arising

120. *Id.*
121. *Id.*
122. *See* Tribble & Lupkin, *supra* note 18.
123. Mut. Pharm. Co. v. Ivax Pharm., Inc., 459 F. Supp. 2d 925, 929–30 n.1 (C.D. Cal 2006) (quoting H.R. REP. NO. 99-153, reprinted in 1985 U.S.C.C.A.N. 301, 303.).
124. Orphan Drug Act of 1983, Pub. L. No. 97-414, § 1(b)(3)–(6), 96 Stat. 2049, 2049 (1983).
125. *See Ivax Pharm.*, 459 F. Supp. 2d at 292–30 n.1 (citations omitted).
126. *See* Herder, *supra* note 113, at 2.
127. *Id.*
128. *Id.*
129. *See supra* Part III.
130. Orphan Drug Act of 1983, Pub. L. No. 97-414, § 1(b)(1)-(3), 96 Stat. 2049, 2049 (1983).
131. *See id.*; Herder, *supra* note 113, at 2.
132. *See* ESKRIDGE, JR. ET AL., *supra* note 93, at 221.

out of the language of the Statute. Applying the *Chevron* framework, courts must initially undertake a literal and textualist interpretation of the language of the Statute. As discussed, the *Chevron* framework permits agency interpretation of ambiguous statutory language.[133] However, when parts of a statute are unambiguous and rigidly constructed without consideration of the circumstances of the broader context of the law, the statute's true *purpose* will suffer at the hands of the narrowly constructed *intent*.[134] In the context of the ODA, in some cases, the rigidly construed *intent* of the Statute makes it difficult for the public to benefit from the Statute in accordance with the *purpose* of the ODA.[135]

B. *The Intent of the ODA is to Create Lucrative Financial Incentives to Develop Remedies for Rare Diseases and Conditions*

Blind adherence to the explicit *intent* of the ODA makes it difficult to achieve the ODA's *purpose*. Courts tend to adopt textualist frameworks and construe the language of statutes narrowly to arrive at the most likely objectives of the law. Poorly written laws can create challenges for judges trying to reconcile tension between a statute's *intent* and *purpose*.

1. *Depomed* Evidences Tension Between the the ODA's Intent and Purpose

The *purpose* of a statute is distinguishable from its *intent* in that the purpose is the goal or the objective of the law while intent is narrower.[136] In *Depomed, Inc. v. United States HHS*, Depomed brought suit alleging that the FDA abused its discretion in denying the drug Gralise[137] a seven-year period of market exclusivity after having "satisfied [the only] two statutory requirements: (1) designation by the [FDA] as a so-called 'orphan drug' . . . and (2) receipt of FDA approval to be marketed to the public."[138] In *Depomed*, the United States District Court for the District of Columbia addressed whether the ODA required the FDA to extend a seven-year period of marketing exclusivity to Gralise, a designated orphan drug.[139] The FDA denied Gralise exclusivity because Neurontin and other generic, non-

133. *See* Chevron v. Nat. Res. Def. Council, 467 U.S. 837 (1984).

134. *See* ESKRIDGE, JR. ET AL., *supra* note 93, at 221.

135. *See generally infra* Part III.

136. *See* ESKRIDGE, JR. ET AL., *supra* note 93, at 220.

137. Depomed, Inc. v. U.S. Dep't of Health & Human Servs., 66 F. Supp. 3d 217, 220 (D.D.C. 2014) (noting that Gralise is used to treat a rare condition known as postherpetic neuralgia ("PHN")).

138. *Id.*

139. *Id.* at 221.

orphan designated drugs already existed in the market to treat postherpetic neuralgia ("PHN")––the disease that Gralise treats.[140]

Essentially, the FDA determined that because Gralise was the "same drug" as Neurontin and other generic drugs already treating PHN, the public would not benefit from the extension of the ODA's lucrative financial protections to Gralise.[141] Therefore, to obtain FDA approval classifying Gralise as an orphan drug, the FDA required Depomed to demonstrate that Gralise was "clinically superior" to Neurontin and other PHN drugs.[142] A drug is considered clinically superior when it is shown to provide a significant therapeutic advantage over the existing drugs that treat the same disease or condition.[143] Petitioners can demonstrate their drug's clinical superiority by showing its greater effectiveness, greater safety (including the reduction in adverse side effects), or by some other "major contribution to patient care."[144] The FDA reasoned that the seven-year period of marketing exclusivity was unwarranted without a showing of Gralise's clinical superiority because:

> No rationale [supports awarding] taxpayer monies [to the] clinical development of an identical product for an identical indication as one which has been approved after the most thorough evaluation possible. [And this] point remains valid even when the rare disease product initially approved to market was never designated as an Orphan product.[145]

Accordingly, the FDA denied multiple petitions to designate Gralise as an orphan drug when Depomed failed to demonstrate that the drug was clinically superior to the existing PHN treatments.[146] The FDA

140. *Id.* at 224.

141. *Id.*

142. *Id.* at 226 (noting that a showing of clinical superiority is required prior to seven-year exclusivity period).

143. 21 C.F.R. § 316.3(b)(3)(i)–(iii) (2018).

144. *Id.*

145. *See Depomed*, 66 F. Supp. 3d at 224.

146. *See id.* at 225 (noting that the stated reason for the denial was the lack of evidence of clinical superiority) (internal quotation marks removed). After Depomed's first petition was denied, Abbott Labs ("Abbott") acquired the rights to Gralise and submitted an application for marketing rights to the FDA. *Id.* The FDA conditionally granted a renewed request for the designation of Gralise as an orphan drug in 2010, pending Abbott's ability to demonstrate the drug was "clinically superior." *Id.* at 225–26. In that time, the FDA renewed the petition with an argument for how it was clinically superior to Neurontin and other generic drugs already treating PHN. *Id.* Consequently, the FDA designated Gralise for treatment of an orphan disease in January of 2011 but denied the seven-year period of exclusivity because Abbott had not proven that Gralise was clinically superior to Neurontin. *Id.* at 226. Depomed reacquired the rights to Gralise and sued the FDA under the APA for refusing to recognize exclusivity that was required by a plain reading of the statute. *Id.* Gralise ultimately submitted documents asserting why Gralise was clinically superior to Neurontin, but these were rejected by the FDA. *See id.*

ultimately designated Gralise as an orphan drug but refused to extend the seven-year period of marketing exclusivity.[147]

The FDA's refusal to extend financial benefits to Gralise further demonstrates the FDA's interpretation of the *purpose* of the Statute: ODA benefits are only useful when they improve patient care.[148] Therefore, the FDA's refusal to grant Gralise's market exclusivity additionally serves to highlight the discord between the Statute's *intent* and *purpose*. The FDA's extra requirement of clinical superiority furthers the *purpose* of the Statute. With this higher standard of clinical superiority, the FDA can limit the ODA financial benefits to makers of drugs that would otherwise not be able to recuperate their development costs or whose drugs will dramatically improve patient care.[149] Therefore, drugs like Gralise would not merit the extension of the seven-year period of exclusivity because the FDA does not recognize a need for additional drugs to treat orphan diseases already treatable by existing drugs in the market.[150] Depomed argued that the FDA abused its discretion in violation of the APA when it constructed this extra-statutory requirement of uniqueness, or special clinical superiority.[151]

The *Depomed* Court did not consider the validity of the FDA's determination of what makes a drug clinically superior.[152] However, it did consider whether this FDA-promulgated[153] requirement of uniqueness strayed from the discretion Congress afforded to the FDA under the ODA.[154] Ultimately, the *Depomed* Court determined that the FDA's administration of ODA benefits did not comply with a plain reading of the Statute.[155] Depomed first contested the FDA's denial of the seven-year period of exclusivity by asserting that the FDA owed Gralise its exclusivity period because existing drug treatments for PHN never received orphan drug approval.[156] To this, the FDA responded that the only relevant factor in denying Gralise was that it had not made a showing of "clinically superiority."[157] Accordingly, whether the existing treatments held designations as orphan

147. *See id.*
148. *See id.*
149. *See id.* at 221.
150. *Id.* at 224.
151. *Id.* at 220.
152. *Id*. at 229 ("[T]the Court finds no need to proceed beyond *Chevron*'s step one, meaning that the Court's analysis need not, and does not, address Depomed's argument that the FDA's interpretation of the Act to permit regulations that require clinical superiority was unreasonable.").
153. The FDA promulgated regulations known as the "Final Rule" in 2013 that largely clarified the meaning behind clinical superiority. *See* 21 C.F.R. § 316.3(b)(3) (2018).
154. *See Depomed*, 66 F. Supp. 3d at 237.
155. *See id.*
156. *Id.* at 224.
157. *Id.* at 225.

drugs was not dispositive in determining Gralise's orphan drug approval.[158] Thus, even though Gralise had received designation and approval as an orphan drug, the FDA argued that denying Gralise a period of exclusivity was within its discretion because it was not "clinically superior" to Neurontin.[159]

In response, Depomed asserted that the FDA had no discretion to withhold a period of exclusivity once a drug earned designation as an orphan drug.[160] It was on this argument that Depomed ultimately prevailed against the FDA and Gralise earned its exclusivity period.[161] The court granted Depomed's motion for summary judgment, finding that "the plain language of the [ODA] unambiguously requires the FDA to recognize that any drug that has been both designated as an orphan drug . . . and also approved for marketing is entitled to an exclusivity period."[162]

To determine whether the FDA owed Depomed an exclusivity period for Gralise, the *Depomed* Court closely examined the statutory language of the ODA.[163] The court found that the plain language of the Statute was unambiguous, and a *Chevron* II analysis was unnecessary.[164] Therefore, the court held that the ODA's exclusivity provision "does not permit or invite any discretion on the part of the FDA regarding whether or not to continue authorizing new such drug marketing once an orphan drug has been so designated and approved."[165] Finding the language of the Statute to be unambiguous, the District of D.C. interpreted a narrow *intent* of the ODA: that Congress expressly sought for all drugs that earn ODA approval to receive every ODA benefit.[166] Accordingly, *Depomed* suggests that the FDA does not retain discretion to determine the degree to which ODA incentives

158. *See id.*

159. *Id.* at 219–20.

160. *Id.* at 226.

161. *See id.* at 237.

162. *Id.* at 220.

163. *Id.* at 230.

164. *Id.* at 229 ("As explained further below, this Court concludes that the plain language of the Orphan Drug Act requires the FDA to recognize exclusivity for Gralise. Consequently, the Court finds no need to proceed beyond *Chevron*'s step one, meaning that the Court's analysis need not, and does not, address Depomed's argument that the FDA's interpretation of the Act to permit regulations that require clinical superiority was unreasonable."); *see also* Otsuka Pharm. Co. v. Burwell, No. GJH-15-852, 2015 U.S. Dist. LEXIS 68230, at *37 (D. Md. May 27, 2015) (noting that the ODA, specifically section 360cc, is unambiguous).

165. *Depomed*, 66 F. Supp. 3d at 231.

166. *See id.* at 230 ("the text . . . makes clear that the incentive Congress intended to create in the orphan drug context is not a thing to be 'conveyed' to drug manufacturers at all; rather, it is a restriction of the FDA's ability to approve the marketing of other such drugs for the same rare disease or condition . . . when a drug that has been designated as an orphan drug is approved for marketing.").

should be administered to any drug once designated as an orphan drug.[167]

Expressing its frustration with the *Depomed* decision, the FDA almost immediately issued a public statement qualifying the holding and affirming its commitment to the "clinical superiority" standard.[168] Shortly thereafter, the FDA promulgated new regulations to distinguish the *Depomed* facts as unique in anticipation of future litigation.[169] Through the regulations, the FDA sought to clarify that it would not approve drugs that it determines to be the "same" and not clinically superior to their pre-approved counterparts.[170] Thus, the FDA reaffirmed its commitment to the *purpose* of the ODA: that exclusivity is owed only to those orphan-designated drugs worthy of ODA financial support. In so doing, the FDA argued that the *Depomed* decision failed to effectuate the *purpose* of the ODA when it granted Depomed its seven-year period of exclusivity.

In *Baker Norton Pharms. v. FDA*, the District of D.C.'s plain reading of the Statute similarly furthered the ODA's *intent* to the detriment of its *purpose*.[171] In evaluating the extent to which the ODA is ambiguous in its use of the word "drug," the court reasoned that the "the Orphan Drug Act seeks to provide a meaningful financial incentive for the development of orphan drugs."[172] However, as this Comment articulates, the *purpose* of the ODA is broader than to simply extend financial benefits to drug companies.[173] The *purpose* is to ensure that patients gain access to novel pharmaceutical treatments for orphan diseases.[174] When the *Baker* Court discussed the ODA's financial incentives,[175] it described what *Depomed* called the "unambigu-

167. *Id.*

168. Policy on Orphan Drug Exclusivity; Clarification, 79 Fed. Reg. 76,888, (Dec. 23 2014) ("In consideration of any uncertainty created by the court's decision in *Depomed*, the Agency is issuing this statement. It is the Agency's position that, given the limited terms of the court's decision to GRALISE, FDA intends to continue to apply its existing regulations in part 316 to orphan-drug exclusivity matters. FDA interprets section 527 of the FD&C Act and its regulations (both the older regulations that still apply to original requests for designation made on or before August 12, 2013, as well as the current regulations) to require the sponsor of a designated drug that is the 'same' as a previously approved drug to demonstrate that its drug is 'clinically superior' to that drug upon approval in order for the subsequently approved drug to be eligible for orphan-drug exclusivity.").

169. *See* Orphan Drug Regulations, 78 Fed. Reg. 35,117, 36,118 (June 12, 2013) (amending 21 C.F.R. pt. 316).

170. *Id.*

171. Baker Norton Pharm., Inc. v. Food & Drug Admin., 132 F. Supp. 2d 30, 35–36 (D.D.C. 2001).

172. *Id.* at 35.

173. *See supra* Part II.A.

174. *See* Orphan Drug Act of 1983, Pub. L. No. 97-414, § 1(b)(1), 96 Stat. 2049, 2049 (1983).

175. *Baker Norton Pharm.*, 132 F. Supp. 3d at 38.

ous intent" of the Statute.[176] In both cases, the District of D.C.'s strict interpretation of the Statute effectuates the *intent* of the ODA.[177] Thus, similar to the *Depomed* holding, the *Baker* holding misrepresents the true *purpose* of the ODA.

In *Depomed*, the court did not engage in a *Chevron* II analysis or grant the FDA *Chevron* deference because it interpreted the Statute to expressly require that the FDA confer Gralise the full benefits of the ODA.[178] In *Depomed*, the FDA had to adhere to the court's strict interpretation of Congress's *intent* because the FDA designated Gralise as an orphan drug that could be marketed for that purpose.[179] When the *Depomed* court found that Gralise earned designation as a new orphan drug designed to treat a rare disease or condition, the court held that the Statute required the FDA to extend marketing exclusivity to Depomed for the marketing and development of Gralise.[180] Therefore, the FDA improperly denied Gralise marketing exclusivity in the first place because it met the statutory qualifications of earning designation of and approval for marketing as an orphan drug.[181] The FDA did not immediately approve Gralise because other drugs were on the market to treat PHN.[182] While the FDA ultimately determined that Gralise was clinically superior, the FDA's refusal to extend marketing exclusivity thereafter suggests it does not believe Gralise's clinical superiority deserved full ODA protections.[183]

Depomed clarifies the FDA's position that the award of marketing exclusivity is only owed to companies that devote resources and capital into the development of *new* orphan drugs.[184] This notion seems consistent with the express *purpose* of the Statute: to create lucrative incentives that can help companies recuperate the costs of developing otherwise unprofitable orphan drugs.[185] But, implied in that grant of incentives is that the drug would otherwise not be profitable.[186] That is, absent the ODA's financial protections, the drug would not exist.[187]

176. Depomed, Inc. v. U.S. Dep't of Health & Human Servs., 66 F. Supp. 3d 217, 224 (D.D.C. 2014).

177. *See id.* at 229 (stating that "[a]n examination of any statute for indicia of ambiguity under *Chevron* must begin (and may end) with an analysis of the statutory text."); *Baker Norton Pharm.*, 132 F. Supp. 2d at 34 (stating that "the Court starts with the relevant statutory language of the Orphan Drug Act.").

178. *See Depomed*, 66 F. Supp. 3d at 229.

179. *Id.* at 233.

180. *Id.* at 233–34 (noting that Gralise is used to treat PHN).

181. *Id.* at 230.

182. *Id.*

183. *See id.* at 226.

184. *See id.*

185. *See* Orphan Drug Act of 1983, Pub. L. No. 97-414, § 1(b)(3)–(5), 96 Stat. 2049, 2049 (1983).

186. *See id.*

187. *See id.*

Whether Gralise could have been profitable without the ODA protections is hard to determine.[188] However, the FDA's initial reluctance to award marketing exclusivity to Gralise tends to illustrate the FDA's belief that the Statute did not justify awarding Gralise the ODA's financial incentives.[189] However, because of the way that Congress originally drafted the ODA, if the FDA approves a drug for orphan designation, the FDA will be required to extend all ODA benefits to the drug, including market exclusivity.[190] Extension of market exclusivity is necessary even if the FDA finds that the drug is not in need of ODA financial support.[191] As the ODA was written, the FDA had to grant the ODA's financial incentives to any drug that demonstrated clinical superiority.[192] Thus, granting ODA benefits to all drugs that earn designation tends to effectuate the *intent* of the ODA at the expense of its true *purpose*.

However, the *Depomed* holding arguably effectuates the *purpose* of the ODA: that sponsors develop more orphan drug remedies.[193] When the *Depomed* court read the Statute to be unambiguous and required the FDA to approve Gralise, the market gained another orphan drug.[194] Patients suffering from PHN gained access to the convenience and flexibility of Gralise's slow-release Gabapentin product.[195] Congress expressly intended this result: more orphan-drug remedies available on the market.[196]

Other courts have adjudicated claims regarding the ODA with similar results.[197] In *Berlex Lab v. FDA*, the District of D.C. deferred to the FDA's interpretation of its regulations relating to the ODA's financial incentives.[198] The court found that the FDA had an adequate basis upon which to consider the petitioner's drug, Avonex, clinically superior to an existing drug, Betaseron, which treated the same orphan disease.[199] To arrive at this conclusion, the FDA determined that the substantial reduction in side effects of the newer petitioning drug, Avonex, justified invalidating the older remedy's market exclusivity.[200] As the court noted:

188. *See Depomed*, 66 F. Supp. 3d at 219 (discussing FDA's contention that exclusivity was not necessary for Gralise to recuperate costs).
189. *See id.* at 224.
190. *Id.* at 230.
191. *See id.*
192. *See id.* at 222–23.
193. *See id.* at 237.
194. *Id.*
195. *Id.* at 224.
196. *See* Orphan Drug Act of 1983, Pub. L. No. 97-414, § 1(b)(3)–(5), 96 Stat. 2049, 2049 (1983).
197. *See* Berlex Labs., Inc. v. Food & Drug Admin., 942 F. Supp. 19, 22 (D.D.C. 1996).
198. *Id.* at 24.
199. *Id.*
200. *Id.* at 23.

> The statute does permit FDA approval of a drug that treats the same condition as did the original orphan drug if FDA determines that the two drugs are not the same. FDA's implementing regulations provide that a new drug will not be considered the same as a previously approved drug if the new drug is "clinically superior."[201]

Accordingly, the *Berlex* court held that the FDA did not arbitrarily nullify Betaseron's orphan drug exclusivity after finding that Avonex was different and clinically superior.[202]

However, while the *Berlex* and *Depomed* decisions brought new orphan drugs to consumers, this outcome alone does not fulfill the *purpose* of the Statute.[203] Like in *Depomed*, the *Berlex* court's construction of the ODA resulted in another orphan drug entering the market.[204] However, unlike in *Depomed*, the petitioning drug Avonex was a substantial improvement over Betaseron relative to Gralise's purported superiority over Neurontin.[205] Arguably, the District of D.C. in *Berlex* served dual public interests.[206] First, the public benefitted from gaining access to another orphan drug remedy.[207] Additionally, the public benefitted in having the resources of the ODA tactfully used to incentivize and develop Avonex, a substantially improved medication.[208]

Depomed is further distinguishable from *Berlex* in that Neurontin and the other generic drugs already treating PHN were not substantially less effective than Gralise.[209] Thus, after Gralise received market exclusivity, the public benefited from having an additional drug on the market, even though the FDA never believed that Gralise's clinical superiority warranted ODA benefits.[210] The critical difference between the FDA's clinical superiority determinations in *Depomed* and *Berlex* is that in *Berlex* the FDA argued that the degree of Avonex's superiority over Betaseron warranted extension of ODA benefits.[211] Contrarily, in *Depomed*, the FDA contended that Gralise never offered a major contribution to patient care justifying market exclusiv-

201. *See id.* (quoting 21 C.F.R. § 316(b)(13)(ii) (2018)).

202. *See id.* at 22 (noting that the "FDA . . . [based] its conclusion on the substantially less frequent occurrence of the death of skin tissue in the injection area, or injection site necrosis, associated with Avonex" and that Avonex users benefit from an 81% reduction in injection site reactions as compared to Betaseron).

203. *See* Depomed, Inc. v. U.S. Dep't of Health & Human Servs., 66 F. Supp. 3d 217, 237 (D.D.C. 2014); *Berlex Labs.*, 942 F. Supp. at 27.

204. *See Berlex Labs.*, 942 F. Supp. at 27.

205. *See id.* at 24.

206. *See id.* at 27.

207. *Id.*

208. *Id.* at 24.

209. *See* Depomed, Inc. v. U.S. Dep't of Health & Human Servs., 66 F. Supp. 3d 217, 224 (D.D.C. 2014).

210. *See* Part III.A (discussing the social purpose of the ODA); *see also* Orphan Drug Act of 1983, Pub. L. No. 97-414, § 1(b)(5)–(6), 96 Stat. 2049, 2049 (1983).

211. *See Berlex Labs.*, 942 F. Supp. at 24.

ity.[212] In both cases, the market gained a new orphan drug remedy.[213] However, in *Depomed*, the FDA determined that ODA benefits were unnecessary and unwarranted because Gralise was not clinically superior to the same degree as Avonex over Betaseron.[214] Thus, it seems that unless a drug furthers the social *purpose* of the ODA by demonstrating substantial clinical superiority over other drugs already treating the same rare disease or condition, the FDA intends to withhold marketing exclusivity.

The FDA's view of the ODA's *purpose* is consistent with how many critics of the ODA see the Statute's primary goals.[215] Critics of the ODA are not concerned with whether orphan drugs make it to market.[216] Rather, the issue is whether the orphan drugs that are ultimately brought to market are prohibitively expensive for consumers and insurance companies.[217] Getting the drugs on the market is insufficient to fulfill the Statute's *purpose* if they are too expensive for patients to use.[218] Thus, the presence of orphan drugs in the market alone will not satisfy the ODA's goals when the new drug expends valuable ODA resources on development that may have never required the financial support. The FDA itself asserted that Gralise did not deserve any orphan drug protections.[219] Consequently, extending marketing exclusivity to Gralise resulted in the misuse of taxpayer resources.[220]

Depomed illustrates a unique situation where an orphan drug that gains approval from the FDA likely does not further the goals of the Statute. The *Depomed* litigation highlights that the *intent* and the *purpose* of the ODA are in conflict.[221] Because the District of D.C. interpreted the ODA to be unambiguous, the *Chevron* doctrine rendered the FDA unable to use its own discretion in administering ODA benefits without additional congressional authorization.[222] Fortunately, Congress amended the ODA in 2017, so the FDA can keep drugs like Gralise from frustrating the *purpose* of the ODA.[223] Though, Congress failed to fully address the tension between the Statute's *intent* and *purpose* with its amendment in 2017.

212. *See Depomed*, 66 F. Supp. 3d at 224.
213. *Id.* at 237; *Berlex Labs.*, 942 F. Supp. 3d at 27.
214. *See Depomed*, 66 F. Supp. 3d at 224.
215. *See* Tribble & Lupkin, *supra* note 18.
216. *See id.*
217. *See id.*
218. *See id.*
219. *See Depomed*, 66 F. Supp. 3d at 224.
220. *Id.*
221. *See id.*
222. *See* Chevron v. Nat. Res. Def. Council, 467 U.S. 837, 842–43 (1984).
223. *See infra* Part IV.

IV. FDA-Promulgated Regulations Will Spark Litigation, Not Relieve the ODA's Conflicting Intent and Purpose

On December 23, 2014, shortly after the District of D.C. decided *Depomed*, the FDA published new regulations.[224] Therein, the FDA justified its stringent interpretation of what qualifies as a "same drug."[225] The FDA sought to qualify the *Depomed* holding and provide an explanation for how it will determine a drug's "sameness" in future orphan drug petitions.[226] Specifically, the FDA communicated its "[intent] to continue to apply its existing regulations . . . to orphan-drug exclusivity matters."[227] According to the FDA, a sponsor for a drug that is determined to be the "same" as a previously approved drug will need to demonstrate "clinical superiority" in order to be eligible for orphan-drug exclusivity.[228] The FDA maintained that a drug is the "same" as another if it uses the same "active moiety" as another existing drug.[229] Purportedly, this rationale is not in conflict with *Depomed*.[230]

However, United Therapeutics Corporation ("UTC") filed a lawsuit in the summer of 2017, suggesting that the FDA's decision to maintain this posture with respect to a drug's sameness is erroneous.[231] UTC filed a complaint on August 4, 2017, alleging that the FDA "unlawfully denied granting the company a period of orphan drug exclusivity . . . for Orenitram (treprostinil)" after the FDA designated it as an orphan drug in December 2013.[232] Orenitram treats pul-

224. Orphan Drug Regulations, 78 Fed. Reg. 35,117 (June 12, 2013).

225. *See* 21 C.F.R. §§ 316.3(b)(12), 316.31(a) (2018).

226. *See* Policy on Orphan-Drug Exclusivity; Clarification, 79 Fed. Reg. 76,888 (Dec. 23, 2014).

227. *Id.* at 76,888.

228. Orphan Drug Regulations, 78 Fed. Reg. at 35,117–18.

229. Otuska Pharm. Co v. Price 869 F.3d 987, 989 (D.C. Cir. 2017) ("A drug's active moiety has long played a key role in determining its eligibility to receive marketing exclusivity: to be entitled to exclusivity, a drug must either contain a previously unapproved active moiety or use an approved moiety in a new way. In approving [another drug], the FDA staked out the position that a drug's active moiety not only determines its eligibility for marketing exclusivity, but also defines the field of drugs subject to that exclusivity.").

230. *See* Policy on Orphan-Drug Exclusivity; Clarification, 79 Fed. Reg. at 76,888 ("In consideration of any uncertainty created by the court's decision in Depomed, the Agency is issuing this statement. It is the Agency's position that, given the limited terms of the court's decision to GRALISE, FDA intends to continue to apply its existing regulations in part 316 to orphan-drug exclusivity matters.").

231. Kurt R. Karst, *United Therapeutics Sues FDA After Agency Denies Orphan Drug Exclusivity for ORENITRAM*, FDA L. Blog (Aug. 9, 2017), http://www.fdalawblog.net/2017/08/united-therapeutics-sues-fda-after-agency-denies-orphan-drug-exclusivity-for-orenitram/ [https://perma.cc/ULY2-HV8G].

232. *Id.*; *see also* Press Release, United Therapeutics, United Therapeutics Announces FREEDOM -EV Study of Orenitram® To Continue As Planned Following Interim Analysis (Sept. 7, 2017, 8:00 PM ET), https://www.prnewswire.com/news-releases/united-therapeutics-announces-freedom-ev-study-of-orenitram-to-continue-as-

monary arterial hypertension ("PAH"), a disease that satisfies the ODA's definition of a rare disease.[233] UTC's complaint analogized to claims in *Depomed*, asserting that the "FDA has given itself authority found nowhere in the statute to withhold the statutory orphan drug exclusivity Congress utilized to incentivize the development of these drugs."[234] UTC argued that the FDA's actions directly conflicted with the express language of the ODA and the court's holding in *Depomed*.[235] This dispute highlights the tension between the ODA's legal *intent* and social *purpose*. Like the court in *Depomed*, UTC maintains that the ODA strictly requires the FDA to provide seven years of market exclusivity to any drug that has received designation as an orphan drug, including Orenitram.[236]

When UTC petitioned the FDA to designate Orenitram as an orphan drug, UTC already owned two existing drugs that treat PAH: Remodulin and Tyvaso.[237] Patients take Remodulin intravenously, and Tyvaso requires use of an inhalation device.[238] Orenitram contains the same active moiety as Remodulin and Tyvaso (treprostinil), but Orenitram is administered orally once per day.[239] Because all three drugs contain the same "active moiety" to treat the same disease or condition, the FDA considers them the "same" as defined by the Statute.[240] Therefore, the FDA denied UTC's petition to designate Orenitam as an orphan drug because "UTC had not provided an adequate hypothesis that . . . treprostinil is clinically superior . . . ."[241] Like the drug Gralise in *Depomed*, Orenitram ultimately received designation as an orphan drug, but the FDA withheld the accompany-

planned-following-interim-analysis-300516055.html [https://perma.cc/94W9-VRZF] ("Orenitram is an extended-release, oral tablet form of treprostinil, which was launched commercially in the United States during the second quarter of 2014. Orenitram is the only FDA approved, orally administered prostacyclin analogue, and is the only oral PAH prostacyclin class therapy approved in the United States that is titratable to tolerability, without a dose ceiling. Orenitram was approved by the FDA in December 2013 for treatment of PAH patients to improve exercise capacity.").

233. Complaint for Declaratory and Injunctive Relief, at 2–3, United Therapeutics Corp. v. U.S. Dep't of Health & Human Servs., 1:117-cv-01577 (D.D.C. Aug. 4, 2017) [hereinafter United Therapeutics Complaint].

234. *Id.* at 8.

235. *Id.* at 9 ("The FDA continues to act in direct conflict with both the [ODA] and Judge Jackson's holding in *Depomed*.").

236. *See* Depomed, Inc. v. U.S. Dep't of Health & Human Servs., 66 F. Supp. 3d 217 (D.D.C. 2014) (holding that once the two-prong statutory test has been met, the FDA must provide marketing exclusivity to the petitioning pharmaceutical company).

237. *See* United Therapeutics Complaint, *supra* note 233, at 9.

238. *Id.* at 11.

239. *Id.* at 9–10.

240. 21 C.F.R. § 316.3(b)(2) (2018) (defining "active moiety" as "the molecule or ion, excluding those appended portions of the molecule that cause the drug to be an ester, salt (including a salt with hydrogen or coordination bonds), or other noncovalent derivative (such as a complex, chelate, or clathrate) of the molecule, responsible for the physiological or pharmacological action of the drug substance.").

241. United Therapeutics Complaint, *supra* note 233, at 11.

ing period of market exclusivity.[242] Similar to Depomed, UTC challenged the FDA's decision to withhold the period of marketing exclusivity after Orenitam earned designation as an orphan drug.[243]

According to UTC, the FDA rejected the contention that Orenitram is clinically superior because UTC failed to "[demonstrate] . . . by means of greater efficacy, greater safety or a major contribution to patient care."[244] UTC challenged this exact requirement as violating the APA for "[exceeding the FDA's] statutory authority [and] otherwise not in accordance with the law."[245] Currently, the UTC litigation is stayed pending a ruling by the D.C. Circuit in *Eagle Pharmaceuticals, Inc. v. Azar*, a case considering similar issues.[246]

A. *The Modernization Plan Ignored the Discrepancy Between the Intent and Purpose of the ODA*

As previously discussed, the FDA published the Modernization Plan in June 2017.[247] The Plan describes how the FDA intended to resolve the backlog of existing designation applications that poses challenges for timely and accurate review of orphan drug designation requests.[248] The Plan promised that the FDA would have completely reviewed older orphan drug designations by the end of September 2017.[249] Additionally, the Plan committed the FDA to responding to every new orphan drug designation request no later than ninety days after receiving them.[250] The ambitious set of changes includes minimizing discretionary work, implementing a streamlined review template, and improving collaboration with other offices that jointly review the petitions.[251]

In September 2017, Scott Gottlieb, the commissioner of the FDA, authored a press release that discussed the Plan and the FDA's future strategy for the ODA.[252] The press release described how the FDA completed the Plan ahead of schedule and noted various adjustments, such as designing a new process to improve efficiency in the orphan drug designation process.[253] More importantly, however, Gottlieb addressed the designation process itself and explained how the FDA in-

242. *Id.* at 12. Unlike in *Depomed*, however, the FDA initially refused to recognize Orenitram's status as an orphan drug in 2011; it took until 2016 for the FDA to recognize Orenitram's orphan drug status. *See id.*
243. *Id.* at 13–14.
244. *Id.* at 12.
245. *Id.* at 13.
246. *See* Brief for the Federal Appellants, Eagle Pharma., Inc. v. Azar (No. 18-5207).
247. *See* U.S. FOOD & DRUG ADMIN., *supra* note 9, at 1.
248. *Id.* at 3.
249. *Id.*
250. *Id.*
251. *Id.*
252. *See* Gottlieb, *supra* note 8.
253. *Id.*

tended to reconsider the way it makes ODA incentives available.[254] Specifically, Gottlieb noted that the FDA will "be taking new policy steps to make sure that the incentives offered by the ODA are granted by FDA in a way that's consistent with the manner Congress intended."[255] Gottlieb's new focus for the FDA was to align the designation and review process more closely with the goals of the Statute.[256]

Gottlieb's statement was timely, as UTC filed its lawsuit challenging the FDA's interpretation of the ODA one month prior.[257] Gottlieb publicly acknowledged that the FDA had been actively seeking to interpret the goals of the ODA and "to get input on complex scientific and regulatory issues such as those raised by molecularly targeted drugs . . . and the appropriate application of orphan incentives in that paradigm."[258] Such language strongly implies that the FDA intended to determine the most appropriate way to administer the various financial incentives of the ODA.[259] This demonstrates a commitment to the post-*Depomed* regulations: no drug will earn ODA market exclusivity unless it demonstrates clinical superiority.[260] And it further suggests the gap between the FDA's interpretation of the *purpose* of the Statute and Congress's *intent* will persist. As alleged by UTC and as held by the District of D.C. in *Depomed*, Congress never granted the FDA discretion to withhold ODA benefits.[261]

As discussed, Congress's expressed *intent* in passing the ODA was for the entire lucrative "basket of goodies" to be afforded to every orphan drug for the treatment of a "rare disease or condition."[262] Gottlieb articulated that the FDA would continue to disagree with *Depomed*'s holding because it frustrated the *purpose* of the ODA.[263] His press release suggests that to preserve the *purpose* of the ODA, the FDA must continue to wield discretion to determine which drugs deserve ODA exclusivity.[264] The FDA's intention to sort through its backlog without giving merit to the *Depomed* holding will surely generate lawsuits and plaintiffs who, relying on the *Depomed* holding, will charge that the FDA violates the APA.[265]

254. *Id.*
255. *Id.*
256. *Id*.
257. *See* United Therapeutics Complaint, *supra* note 233, at 8.
258. *See* Gottlieb, *supra* note 8.
259. *Id.*
260. *See supra* Part III (discussing regulations promulgated after *Depomed*).
261. Depomed, Inc. v. U.S. Dep't of Health & Human Servs., 66 F. Supp. 3d 217, 219 (D.D.C. 2014).
262. *See* Tribble & Lupkin, *supra* note 18 ("In a 2009 webinar, an FDA official referred to the incentive package as 'our basket of goodies.'").
263. *See* Gottlieb, *supra* note 8 (FDA should examine "aspects of how the agency grants designations, to make sure they continue to reflect . . . the goals intended by Congress.").
264. *See id.*
265. *See* Karst, *supra* note 231.

1. The FDA's Intentions with the Plan are Supported by the Promulgation of the "Final Rule" in 2013

Before *Depomed*, the FDA finalized revisions to a proposed rule from 2011 that sought to clarify section 316.3 of the Code of Federal Regulations, the promulgated regulations defining general provisions of the ODA.[266] With the promulgation of the Final Rule, the FDA sought to "clarify the existing regulation" and reaffirm that orphan drug approval will only occur if no prior drug has been approved for the same use.[267] Moreover, the FDA clarified that sponsors "may have to demonstrate clinical superiority to obtain orphan-drug designation . . . and [that it] will recognize orphan-drug exclusivity as long as clinical superiority to the previously approved drug is demonstrated."[268] The FDA expressly communicated its rationale for these requirements, noting that "these revisions will clarify, streamline, and improve the orphan-drug designation process [and confirmed that these] amendments are fully consistent with the Orphan Drug Act."[269] The FDA invited comments to the Final Rule and responded to each in turn.[270] Critics of the clinical superiority requirement commented that "more liberal granting of orphan-drug designation[s] without changing orphan-drug exclusivity requirements [furthers] the intent of the Orphan Drug Act."[271] The FDA responded to such comments simply by claiming that:

> [T]he current framework is the best means for giving effect to the intent of the Orphan Drug Act, to provide incentives for sponsors to develop promising drugs for rare diseases and conditions *that would not otherwise be developed and approved*, including drugs that are *potentially safer or more effective than already approved drugs*.[272]

To support this assertion, the FDA referenced a House Report from 1982 that noted that "the legislative history is replete with references to the fundamental need to provide treatment for presently untreated patients."[273] The FDA quoted this legislative history to emphasize that the *purpose* of ODA resources is to support patients who have no available treatments for their disease.[274]

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266. *See* Orphan Drug Regulations, 78 Fed. Reg. 35,117 (June 12, 2013) (amending 21 C.F.R. § 316).

267. *Id.* at 34,118.

268. *Id.*

269. *Id.*

270. *Id.*

271. *Id.* at 35,121.

272. *See id.* at 35,122 (emphasis added).

273. *See id.* (quoting Genentech Inc. v. Bowen, 676 F. Supp. 301, 312 (D.D.C. 1987)).

274. Genentech, Inc. v. Bowen, 676 F. Supp. 301, 312 (D.D.C. 1987).

With this background, the FDA's recent Modernization Plan is especially relevant to the pending UTC litigation.[275] Some critics may argue that amending the ODA is unnecessary because the FDA's modernization plan will resolve the conflicts between the Statute's intent and purpose. Commentators suggest that the FDA welcomed UTC's challenge on *Depomed* grounds for an opportunity to go before the District of D.C. once more.[276] These commentators suggest that the FDA sought a more favorable ruling recognizing the FDA's discretion to determine whether a drug is clinically superior before providing ODA benefits.[277] Despite the FDA's willingness to engage in litigation with UTC, the FDA's stance is clear: the ODA's benefits should be reserved for drugs that advance the *purpose* of the ODA.[278] The FDA will likely remain steadfast in the UTC litigation, maintaining the position that the only drugs that effectuate the ODA's *purpose* are new drugs that improve health of patients suffering from orphan diseases.[279] The sponsors will only earn orphan drug designation by demonstrating their drugs have a clinical superiority or make a "major contribution to patient care" over the pre-approved counterparts.[280] Nothing in the FDA's promulgated rules, published commentary, or litigation with Depomed suggests that the FDA believes that Congress intended for ODA benefits to extend to new drugs that do not improve patient care.[281] However, the FDA's Modernization Plan and its commitment to this determination of which drugs deserve orphan drug exclusivity could not resolve the underlying issue of the tension between the *intent* and the *purpose* of the ODA. Even the *Depomed* court suggests that the FDA should "[fashion] regulations to prevent such abuse"[282] in the designation of exclusivity phase to resolve the *Depomed* problem. But, the Plan and the Final Rule suggest that the FDA believes the only way it can effectuate the social *purpose* and ultimate goal of the ODA is to deny marketing exclusivity to drugs that do not help patients in a meaningful way.[283] This is an insufficient

275. *See* Orphan Drug Regulations, 78 Fed. Reg. 35,117 (June 12, 2013) (amending 21 C.F.R. § 316); *see* United Therapeutics Complaint, *supra* note 233, at 8.

276. Kurt R. Karst, *How Effective is a "Depomed Threat" at Resolving an Orphan Drug Clinical Superiority Dispute?*, FDA L. BLOG (Sept. 27, 2015), http://www.fdalawblog.net/fda_law_blog_hyman_phelps/2015/09/how-effective-is-a-depomed-threat-at-resolving-an-orphan-drug-clinical-superiority-dispute.html [https://perma.cc/8E3R-5AF5].

277. *Id.*

278. *See* Orphan Drug Regulations, 78 Fed. Reg. 35,117 (June 12, 2013) (amending 21 C.F.R. § 316).

279. *See* Depomed, Inc. v. U.S. Dep't of Health & Human Servs., 66 F. Supp. 3d 217, 221 (D.D.C. 2014); Orphan Drug Regulations, 78 Fed. Reg. 35,117 (June 12, 2013) (amending 21 C.F.R. § 316).

280. *See supra* Part III.B (discussing the "clinical superiority").

281. *See supra* Part III.B.

282. *See Depomed*, 66 F. Supp. 3d at 235.

283. Orphan Drug Regulations, 78 Fed. Reg. 35,117 (June 12, 2013) (amending 21 C.F.R. § 316).

solution because courts will continue to engage in *Chevron* analyses to determine if the language of the ODA is ambiguous.

When courts hold the language of the ODA to be unambiguous, like in *Depomed*, they find that the FDA lacks the discretion to deny drugs market exclusivity once they receive orphan drug designation. The *Depomed* decision highlights a sort of stalemate where companies whose drugs meet the two-prong requirement[284] and are qualified as an orphan drug will continue to petition the FDA for exclusivity. But, absent a showing of clinical superiority, the FDA will continue to deny these drugs market exclusivity. Therefore, the Modernization Plan alone could not resolve the most pressing issue involving the ODA: handling situations where drugs met the intended statutory requirements of earning orphan-drug approval but failed to advance the *purpose* of the ODA.

V. Congress Should Amend the Statute and Provide the FDA More Discretion in the Administration of ODA Benefits

Central to the *Depomed*,[285] *United Therapeutics*,[286] and *Berlex* cases is the challenge to the FDA's interpretation of the ODA under the APA.[287] In each case, the sponsors questioned the legitimacy of the FDA's administration of the ODA.[288] This Comment argues that the tension between the *intent* and the *purpose* of the Statute explains why the FDA denies drug petitions that do not demonstrate clinical superiority.[289] To resolve this tension, Congress had to amend the Statute to empower the FDA with more discretion to administer ODA benefits. Until Congress expressly provided the FDA with such discretion, lawsuits like *Depomed* and *United Therapeutics* were to continue.[290]

Depomed prevailed against the FDA because the Statute did not expressly outline a method for the FDA to evaluate the degree to which a sponsor's drug deserves ODA benefits.[291] As *Depomed* made clear, the FDA must award a sponsor's drug ODA benefits if it earns approval and designation for treatment of a rare disease or condition.[292] This is a problem when the drug that receives these ODA ben-

284. *See Depomed*, 66 F. Supp. 3d at 228 (noting that once a drug has received designation as an orphan drug and earned marketing approval for such a purpose, the FDA must reward it with seven years of marketing exclusivity).

285. *Id.*

286. United Therapeutics Complaint, *supra* note 233, at 12–13.

287. Berlex Labs., Inc. v. Food & Drug Admin., 942 F. Supp. 19, 26 (D.D.C. 1996).

288. *See Depomed*, 66 F. Supp. 3d at 228; *Berlex Labs.*, 942 F. Supp. at 26; United Therapeutics Complaint, *supra* note 233, at 12–13.

289. *See supra* Part III (discussing the difference between the *intent* and *purpose* of the statute).

290. *See supra* Part IV (discussing the likely outcome of the UTC litigation).

291. *See, e.g.*, *Depomed*, 66 F. Supp. 3d at 229–30 (noting that the FDA exceeded its authority granted by the ODA).

292. *Id.*

efits, like Gralise in *Depomed*, does not further the *purpose* of the Statute. As discussed, the FDA's reluctance to approve Gralise demonstrates its belief that its addition to the market would not further the *purpose* of the ODA.[293] The FDA recognizes that some drugs can satisfy the strict qualifications of becoming an orphan drug but may not be deserving or in need of the ODA's financial protections.[294] To avoid situations like *Depomed* and *United Therapeutics*, Congress had to explicitly grant the FDA greater discretion in how it administers ODA benefits to reconcile the divergent *intent* and *purpose* of the Statute.[295]

Congress has effectively already determined that any drug that treats fewer than 200,000 patients is unlikely to recuperate the costs of production and is therefore deserving of ODA benefits.[296] The ODA also permits a sponsor to demonstrate that recuperating the costs of development through sales alone is not possible even if the disease affects more than 200,000 people.[297] However, at the time of the *Depomed* and *United Therapeutics* litigation, no equivalent process existed for the FDA to assess and determine whether a drug that qualifies for ODA benefits does not require financial incentives to make it to market.[298]

The affordability of the drugs when they arrive on the market is important because, as Kaiser Health News alleges, many orphan-drug remedies are prohibitively expensive for patients.[299] The ODA fails to achieve its *purpose* when drugs that do not require financial support make it to market and are prohibitively expensive.

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293. *See* Memorandum of Law in Support of Defendants' Response to Plaintiff's Motion for Summary Judgment & Cross-Motion at 2, United Therapeutics Corp. v. U.S. Dep't of Health & Human Servs., No. 1:17-cv-01577 (D.D.C. Dec. 22, 2017) ("Consistent with the statute's purpose, FDA has, for more than twenty-five years, interpreted the Orphan Drug Act to confer a seven-year period of exclusivity to only the *first* drug approved as an orphan drug (meaning a drug with a new active ingredient or that is clinically superior). This interpretation is both reasonable and deserving of deference. Indeed, Congress recently affirmed this interpretation in enacting the FDA Reauthorization Act of 2017. Under this interpretation, UTC is not entitled to continue its monopoly, because Orenitram is neither novel nor clinically superior to the previously-approved versions of treprostinil. Orenitram should be denied exclusivity, as FDA correctly decided. An alternative result would be anathema to the Orphan Drug Act's underlying purpose, and would create a windfall for UTC to the detriment of patients with a rare disease.").

294. *See supra* Part III.A.

295. *See, e.g.*, *Depomed*, 66 F. Supp. 3d at 230 (noting that the FDA exceeded its authority granted by the ODA).

296. *See* 21 U.S.C. § 360bb(a)(2) (2012) (defining a "rare disease or condition" as one "affect[ing] less than 200,000 persons in the United States"); *see* Gottlieb, *supra* note 8 ("[A] rare disease [is] defined as a disease which generally affects fewer than 200,000 people in the United States . . . .").

297. *See* 21 U.S.C. § 360bb(a)(2).

298. *See Depomed*, 66 F. Supp. 3d at 229–30.

299. *See* Tribble & Lupkin, *supra* note 18.

For example, imagine a situation where a brand new drug, "Drug A," earns ODA designation and is granted ODA exclusivity and benefits for the treatment of rare disease, "Disease X." At the time of its approval, Drug A is unique and a completely new remedy to Disease X that has thus far never had a pharmaceutical treatment. At this point, the *intent* of the ODA has undoubtedly been achieved: a novel remedy made it to market and was developed because of the ODA's financial benefits. Then, in the third year of its seven-year period of exclusivity, Drug A breaks even and the pharmaceutical sponsor recuperates its costs of developing and producing the drug. Thereafter, each new unit of sale earns the pharmaceutical company a profit.

If "Drug B" also treats Disease X and seeks FDA designation as an orphan drug, the FDA would deny the drug designation for the remaining four years of Drug A's exclusivity period under the ODA unless Drug B demonstrates clinical superiority.[300] This situation matches the *Depomed* and *Berlex* cases.[301] In situations where Drug A has become profitable after recuperating its costs of production, thwarting Drug B's entrance into the market frustrates the *purpose* of the ODA. This frustrates the *purpose* of the ODA because the introduction of competitor Drug B could only drive-down prices of Drug A and provide alternative treatments for patients suffering from Disease X. The reduction in price and the increase in alternative treatments are both of substantial benefit to patients suffering from orphan diseases.[302] Such outcomes would be consistent with the *purpose* of the ODA. However, the ODA does not permit the FDA to examine the extent of a sponsor's financial need after it receives approval as an orphan drug and received ODA benefits.[303] In situations where Drug A recuperates its costs, Congress should empower the FDA to reduce Drug A's exclusivity period only if it is shown that the prices of Drug A are so prohibitive that the drug is not reasonably available to patients with Disease X.

A. *Congress Must Permit the FDA to Consider Market Factors to Selectively Administer ODA Benefits*

In 2017, Congress passed the FDA Reauthorization Act of 2017 ("FDARA").[304] FDARA clarified that when the FDA determines a petitioner seeks a new period of exclusivity for a drug that has previously enjoyed ODA benefits, the FDA should deny the same drug a new period of exclusivity.[305] Essentially, FDARA resolved the *De-*

300. *See, e.g.*, *Depomed*, 66 F. Supp. 3d at 222; Berlex Labs., Inc. v. Food & Drug Admin., 942 F. Supp. 19, 22 (D.D.C. 1996).
301. *Depomed*, 66 F. Supp. 3d at 226; *Berlex Labs.*, 942 F. Supp. at 22.
302. *See* Tribble & Lupkin, *supra* note 18.
303. *See Depomed*, 66 F. Supp. 3d at 229–30.
304. FDA Reauthorization Act of 2017, Pub. L. No. 115-52, 131 Stat. 1005 (2017).
305. *See* 21 U.S.C. § 360cc (2012 & Supp. 2018).

pomed situation and suggests that Congress ultimately agreed with the FDA's interpretation of the ODA.[306] FDARA grants the FDA some additional discretion in determining whether a drug deserves a period of exclusivity.[307]

Though FDARA resolves some ambiguity in the ODA,[308] Congress should further expand the FDA's ability to parse the benefits of the ODA. Currently, no provision of the ODA contemplates the price at which orphan drugs should be made available.[309] Ideally, orphan drugs would be affordable after the ODA subsidies supported their development. However, Congress's focus in 1983 was developing new remedies, not the prices of the drugs themselves.[310] Fortunately, policymakers have begun revisiting the effectiveness of the ODA.[311] As recently as February 2019, Senator Chuck Grassley and the Senate Finance Committee called for a closer look at exorbitant drug prices in the United States.[312] The Senate Finance Committee panel questioned CEOs from big-name companies like Pfizer, Johnson & Johnson, and Merck about the increasingly high costs of drug prices.[313] Congress has also sought drug pricing documents from twelve companies about various prescription drugs, more than half of which are orphan drugs with periods of market exclusivity.[314] The congressional scrutiny will draw due attention to the remaining issues with the

306. *See* Defendant's Response to Plaintiff's Motion for Summary Judgment and Cross-Motion at 26, United Therapeutics Corp. v. U.S. Dep't of Health & Human Servs., (No. 1:17-cv-01577) ("Simply put, the FDARA clarifies what should have always been evident: the old version of the Orphan Drug Act was ambiguous, and FDA properly interpreted that ambiguity to preclude the kind of automatic serial exclusivity that UTC seeks to achieve in this case.").

307. *Id.*

308. *Id.* at 19.

309. *See* Orphan Drug Act of 1983, Pub. L. No. 97-414, § 1(b)(5)–(6), 96 Stat. 2049, 2049 (1983).

310. *Id.*

311. *See* Sarah Jane Tribble, *FDA Moves to Rein in Drugmakers' Abuse of Orphan Drug Law*, NPR (Sept. 13, 2017, 1:20 PM ET), https://www.npr.org/sections/health-shots/2017/09/13/550700062/fda-moves-to-rein-in-drugmakers-abuse-of-orphan-drug-law [https://perma.cc/EQ3W-FTWP] (noting that "Gottlieb became commissioner in May, a few months after three key Republican senators called for a federal investigation into potential abuses of the Orphan Drug Act. In March, the Government Accountability Office agreed to investigate").

312. *See* Sarah Karlin-Smith, *'It's Finally Pharma's Turn': Drug CEOs face Capitol Hill reckoning*, POLITICO (Feb. 25, 2019, 10:11 AM), https://www.politico.com/story/2019/02/25/drug-prices-hearing-congress-1182283 [https://perma.cc/N8DQ-ETPE].

313. *Id*.

314. Press Release, Elijah Cummings, Chairman, House Committee on Oversight and Reform, House of Representatives, Oversight Committee Launches Sweeping Drug Price Investigation (Jan. 14, 2019), https://oversight.house.gov/sites/democrats.oversight.house.gov/files/documents/_Drug%20Price%20Investigation%20Letters%20Recipients.pdf [https://perma.cc/8Y7F-DGFM] (follow "Click here for a list of companies and drugs that are the subject of today's letters" hyperlink) [https://perma.cc/T9C8-8FVH]; *see also Search Orphan Drug Designations and Approvals,* U.S FOOD & DRUG ADMIN., https://www.accessdata.fda.gov/scripts/opdlisting/oopd/index.cfm [https://perma.cc/29EA-BVSL] (by typing the name of the drug in to the

ODA, and could precipitate additional amendments. By authorizing the FDA to selectively administer the benefits of the ODA, Congress can ameliorate policy concerns like the price of orphan drugs, outlined by Senator Grassley, KHN, and other ODA critics.[315]

Congress must grant the FDA greater discretion to go "behind the petition" and request that the sponsor provide a more comprehensive showing of why a drug still requires ODA protections after recuperating its costs. This additional amendment will better align the *intent* and *purpose* of the ODA because Congress expressly designed the Statute to facilitate the development of drugs that would otherwise never be developed because of financial constraints.[316] Thus, when an orphan drug promises to be especially successful, or it recuperates its costs of production and marketing before the seven-year period of exclusivity, the FDA should be permitted to limit or alter the schedule of benefits administered. Amending the Statute to permit the FDA this extra discretion is consistent with the ODA's legislative history.[317] This amendment would ensure that ODA resources flow towards more potential orphan drugs that genuinely need the financial support or have yet to be developed. The challenge for Congress will be to further amend the Statute such that the FDA cannot completely eviscerate the ODA's strongest incentive: the seven-year period of market exclusivity.[318] Put differently, the FDA should only infringe on periods of exclusivity when market circumstances, including prohibitively expensive drugs or the availability of clinically superior substitutes, justifies ending market exclusivity.

Thus, returning to hypothetical Disease X, after Drug A recuperates all of its costs, if the price of the drug is prohibitively expensive, then Congress should authorize the FDA to reevaluate the original drug's need for exclusivity. Congress should permit the FDA to shorten the period of exclusivity to facilitate the development of generic drugs to drive down prices. Doing so would resolve many of KHN's criticisms, including the prohibitive price issue.[319] The FDA could also link the amount of the award given for a new orphan-drug petition to the degree to which it improves patient care.[320] For example, if Drug B only reduces side effects by a few percentage points or changes the way the drug is administered, to avoid the *Depomed* or

search query, the website shows whether the drug is an orphan drug, whether it has been approved for a period of market exclusivity, and how much time it has left)

315. *See* Tribble & Lupkin, *supra* note 18.

316. *See* Orphan Drug Act § 1(b)(1)(6), 96 Stat. at 2049.

317. *See* Genentech, Inc. v. Bowen, 676 F. Supp. 301, 312 (D.D.C. 1987) (suggesting that Congress's priority in designing the ODA was to make novel orphan-drugs available for the first time).

318. *See* 21 U.S.C. § 360cc (2012) (discussing the purpose of the ODA financial incentives).

319. *See* Tribble & Lupkin, *supra* note 18.

320. *Cf.* Depomed, Inc. v. U.S. Dep't of Health & Human Servs., 66 F. Supp. 3d 217 (D.D.C. 2014) (discussing patient care as a factor in clinical superiority).

Berlex situations, the FDA could grant the new drug *some* ODA benefits to recuperate some of the costs of development. Because the market benefits from competition that reduces prices, unless there is a genuine need for the full seven years of exclusivity,[321] the FDA should be permitted to withhold this benefit on a case-by-case or piecemeal basis.

1. Amending the Statute Will Resolve Many of Public Policy Concerns Cited by KHN

Congress designed the tax incentives and periods of marketing exclusivity to be financially lucrative.[322] However, Congress should further amend the Statute because the very exclusivity that breathes life into orphan drugs might also be making the drugs prohibitively expensive and not reasonably available.[323] Here, "reasonably available" means more than simply present in the market. Products are only reasonably available when their intended consumers can afford them. The KHN reports focus critically on the high prices of orphan-designated drugs[324] because the *purpose* of the ODA cannot be achieved if the drugs that rely on ODA benefits are too expensive for patients to use. Therefore, the FDA can only effectuate the *purpose* of the ODA if drugs that otherwise could not have made it to the market will be reasonably available to consumers.

When companies earn marketing exclusivity, they enjoy effective monopolies over their corner of the drug market, demanding any price they choose without meaningful competition.[325] Congress intentionally designed the ODA with such enticing monopoly power to encourage the development of these high-cost drugs.[326] Eliminating this strong incentive would threaten the *intent* of the Statute; however, expanding the FDA's discretion would permit the FDA to introduce competitor orphan drugs when prices of existing drugs are prohibitively high.[327] The amended Statute should continue to incentivize sponsors who seek to provide novel remedies for orphan diseases that have yet to market a pharmaceutical treatment.[328] This Comment endorses the FDA's assessment that ODA resources should be prioritized to address untreated orphan disease populations.[329] Thus, if

321. *See* Henry G. Grabowski & John M. Vernon, *Brand Loyalty, Entery, and Price Competition in Pharmaceuticals After the 1984 Drug Act*, 35 J.L. & ECON. 331 (1992).
322. *See* Orphan Drug Act of 1983, Pub. L. No. 97-414, § 1(b)(5)–(6), 96 Stat. 2049, 2049 (1983) (discussing the purpose of the ODA financial incentives).
323. *See* Tribble & Lupkin, *supra* note 18.
324. *Id.*
325. *See id.*
326. *Id.*
327. *See supra* Part II.B.
328. *See* Orphan Drug Act of 1983, Pub. L. No. 97-414, § 1(b)(5)-(6), 96 Stat. 2049, 2049 (1983).
329. *See* Genentech, Inc. v. Bowen, 676 F. Supp. 301, 312 (D.D.C. 1987).

wielded appropriately, the FDA can use this new discretion to reconcile the *intent* and *purpose* of the Statute.[330]

2. Amending the Statute can Drive-Down Prices and Address Salami-Slicing

Many ODA critics are frustrated with a practice called "salami-slicing."[331] Salami-slicing occurs when pharmaceutical companies repurpose existing drugs that treat mainstream diseases to treat rare diseases and conditions.[332] FDARA resolves some serial exclusivity issues but does not specifically address salami-slicing.[333] Salami-slicing also occurs when sponsors earn approval of a drug in multiple subtypes of a disease.[334] Humira provides a classic example of salami-slicing and is one of the twelve drugs that was reviewed by the Senate Finance Committee in February 2019.[335] In 2002, the FDA initially approved Humira to treat rheumatoid arthritis, a disease affecting around 1.5 million people in the United States.[336] Later, in 2008, the FDA approved Humira to treat pediatric rheumatoid arthritis, an orphan disease.[337] When Humira obtained its orphan designation, Humira had already become the top-selling drug in the world, not in need of the ODA financial protections.[338]

When a sponsor engages in salami-slicing, it takes advantage of the fact that one type of orphan disease will often develop into multiple varying subtypes over time, allowing for an indefinite monopoly over

330. *Id.*

331. *See* Michael Mezher, *FDA Analyst Counters Critiques of ODA*, REGULATORY AFF. PROF. SOC'Y (Oct. 18, 2017), http://raps.org/Regulatory-Focus/News/2017/10/18/28713/FDA-Analyst-Counters-Critiques-of-Orphan-Drug-Act/ [https://perma.cc/A274-LPQL].

332. *Id.*

333. *See* Defendant's Response to Plaintiff's Motion for Summary Judgment and Cross-Motion at 26, United Therapeutics Corp. v. U.S. Dep't of Health & Human Servs. (No. 1:17-cv-01577) ("Simply put, the FDARA clarifies what should have always been evident: the old version of the Orphan Drug Act was ambiguous, and FDA properly interpreted that ambiguity to preclude the kind of automatic serial exclusivity that UTC seeks to achieve in this case.").

334. Mezher, *supra* note 331.

335. *See* Sarah Jane Tribble & Sydney Lupkin, *Drugs For Rare Diseases Have Become Uncommonly Rich Monopolies*, NPR (Jan. 17, 2017, 4:49 AM ET) https://www.npr.org/sections/health-shots/2017/01/17/509506836/drugs-for-rare-diseases-have-become-uncommonly-rich-monopolies [https://perma.cc/S5MU-TCJ6]; *see also* Press Release, Cummings, *supra* note 314.

336. *See What is Rheumatoid Arthritis?*, ARTHRITIS FOUND., http://www.arthritis.org/about-arthritis/types/rheumatoid-arthritis/what-is-rheumatoid-arthritis.php (last visited Jan. 29, 2019) [https://perma.cc/MVL8-JQNF]; *see also* Tribble & Lupkin, *supra* note 335.

337. Tribble & Lupkin, *supra* note 335.

338. *Id.*

treating a disease.[339] FDA analysts reject the premise that salami-slicing alone is responsible for high orphan-drug prices.[340] However, when the same drug sponsor accrues multiple consecutive seven-year exclusivity periods on the same drug, there is no competition to drive down the drug's price.[341] Critics suggest that by permitting salami-slicing, the ODA thwarts innovation and the creation of novel treatments while protecting older orphan drug remedies.[342]

Because Congress's priority in passing the ODA has always been to incentivize development of drugs for diseases with no existing pharmaceutical treatment, salami-slicing does not further the *purpose* of the ODA.[343] For example, Humira did not need ODA exclusivity to be re-marketed for juvenile rheumatoid arthritis because Humira had already become the top-selling drug in the world.[344] Humira's sponsor could have afforded to market Humira to the juvenile patient population without ODA status.[345] Thus, the recent spike in ODA petitions suggests that sponsors are pursuing orphan-drug designation because of the substantial financial reward in obtaining the designation.[346] Although the Statute incentivizes sponsors to pursue the financial benefits of the ODA, so long as salami-slicing is permissible, the ODA's *purpose* remains unachieved because ODA resources do not flow to novel remedies.[347]

To resolve the salami-slicing issue, the FDA could implement sliding scales that reward varying degrees of financial support for a sponsor's drug. In the amended Statute, Congress could permit the FDA to award a degree of ODA benefits linked to the market need and the

339. Ryan Marling, *Salami-Slicing, Precision Medicine, and the Orphan Drug Act*, CHRISTIANSEN INST. (Feb. 23, 2017), https://www.christenseninstitute.org/blog/salami-slicing-precision-medicine-orphan-drug-act/ [https://perma.cc/X4PH-UCWN].

340. *See* Mezher, *supra* note 331.

341. *See* Marling, *supra* note 339.

342. *Id.*

343. *See* Genentech, Inc. v. Bowen, 676 F. Supp. 301, 312 (D.D.C. 1987).

344. *See* Tribble & Lupkin, *supra* note 335.

345. *Id*.

346. *Id*.

347. *See id.* ("More than 80 other orphans won FDA approval for more than one rare disease, and in some cases, multiple rare diseases. For each additional approval, the drugmaker qualified for a fresh batch of incentives. Botox, stocked in most dermatologists' offices, started out as a drug to treat painful muscle spasms of the eye and now has three orphan drug approvals. It's also approved as a mass market drug to treat a variety of ailments, including chronic migraines and wrinkles. Altogether, KHN's investigation found that about a third of orphan approvals by the FDA since the program began have been either for repurposed mass market drugs or drugs that received multiple orphan approvals."). *See also* Baker Norton Pharm., Inc. v. Food & Drug Admin., 132 F. Supp. 2d 30, 32 (D.D.C. 2001) ("Because the drug is designated as an orphan drug before it is approved, more than one applicant may receive orphan designation for what later may be deemed the same 'drug' for treatment of the same disease or condition. Once the drug is designated an orphan drug, it goes through the approval process for orphan drug exclusivity under 21 U.S.C. § 360cc.").

likelihood of recuperating costs.[348] As discussed, the FDA already requires a sponsor to demonstrate that there is no reasonable expectation that the cost of developing and making the drug will be recovered in the United States.[349] The amended Statute should allow the FDA to reevaluate a sponsor's financial need after the drug has entered the market. This amendment would motivate sponsors to continue to innovate and improve their orphan drug, ultimately improving competition and driving down prices. With lower prices, the availability of orphan drugs increases, advancing the original *purpose* of the Statute.

Because the sole intention of the exclusivity period is to help companies recuperate production costs and incentivize development, once that has occurred, the financial benefit of exclusivity becomes more of a privilege than a need.[350] Undoubtedly, periods of exclusivity should be extended to sponsors when circumstances warrant: like when prohibitive development costs would otherwise stymie the development of a treatment for an orphan disease. Furthermore, when an orphan drug is reasonably available to patients and there are no alternative treatments promising any major contribution to patient care, the initial sponsor should maintain its full seven-year period of exclusivity.[351] The FDA should only reduce the original sponsor's seven-year exclusivity period when more promising treatment alternatives emerge that also require ODA financial incentives to offset prohibitive development costs.[352] The FDA could reduce the exclusivity period of an existing drug when the target patient population would be substantially improved, either through cheaper alternative orphan drugs or safer, more effective orphan drugs.[353]

VI. Conclusion

Congress did not intend for the ODA to be a pathway for pharmaceutical companies to improve their profitability.[354] Rather, the goal of the ODA is clear: to remove financial barriers to the development of treatments for rare diseases and conditions so that patients can get access to pharmaceutical remedies for the first time.[355] Thus, when a sponsor recuperates the orphan drug's costs of production and makes

348. This ensures consistency with the original *purpose* and *intent* of the ODA.

349. *See* Tribble & Lupkin, *supra* note 335.

350. *See* Orphan Drug Act of 1983, Pub. L. No. 97-414, § 1(b)(5)–(6), 96 Stat. 2049, 2049 (1983).

351. *See id*. (supporting the assertion that the *intent* of the Statute is to incentivize the development of orphan drugs with seven-year periods of market exclusivity).

352. *See* Depomed, Inc. v. U.S. Dep't of Health & Human Servs., 66 F. Supp. 3d 217, 234 (D.D.C. 2014); Berlex Labs., Inc. v. Food & Drug Admin., 942 F. Supp. 19, 23 (D.D.C. 1996).

353. *Berlex Labs.*, 942 F. Supp. at 23.

354. Orphan Drug Act of 1983, Pub. L. No. 97-414, § 1(b)(5)–(6), 96 Stat. 2049, 2049 (1983); *see also* Tribble & Lupkin, *supra* note 335.

355. *See* Genentech, Inc. v. Bowen, 676 F. Supp. 301, 312 (D.D.C. 1987).

it to market, or even becomes profitable, the *intent* of the ODA has been satisfied.[356] The *purpose* might still remain unfulfilled, however, if the drugs are so prohibitively expensive that patients cannot purchase or use them.[357]

To effectuate the *purpose* of the ODA, Congress must permit the FDA to forecast a drug's financial success and exercise greater discretion over the administration of ODA benefits.[358] In so doing, Congress can remedy the issue of salami-slicing and the prohibitively high prices of some orphan drugs. The challenge for Congress will be to resolve the salami-slicing problem without destroying the ODA's strongest incentive: seven years of market exclusivity. However, the FDA routinely exercises discretion in construing definitions of what makes a drug the "same" or how it demonstrates a major contribution to patient care, so this additional authority is not without precedent.[359] With such authority, the FDA can reconcile the *intent* and *purpose* of the ODA to ensure patients suffering from orphan diseases have access to reasonably affordable orphan drugs.

356. *See supra* Part II.B.
357. *See supra* Part II.A.
358. *See supra* Part II.A.
359. *See, e.g.*, Depomed, Inc. v. U.S. Dep't of Health & Human Servs., 66 F. Supp. 3d 217 (D.D.C. 2014); Berlex Labs., Inc. v. Food & Drug Admin., 942 F. Supp. 19 (D.D.C. 1996).