IN THE UNITED STATES DISTRICT COURT
FOR THE SOUTHERN DISTRICT OF FLORIDA

CATALYST PHARMACEUTICALS, INC.,
355 Alhambra Circle, Suite 1250
Coral Gables, FL 33134

Plaintiff,

v.

ALEX AZAR, Secretary of Health and Human Services
200 Independence Avenue, SW
Washington, DC 20201;

U.S. DEPARTMENT OF HEALTH AND HUMAN SERVICES
200 Independence Avenue, SW
Washington, DC 20201;

STEPHEN M. HAHN, M.D., Commissioner of Food and Drugs
10903 New Hampshire Avenue
Silver Spring, MD 20993; and

U.S. FOOD AND DRUG ADMINISTRATION
10903 New Hampshire Avenue
Silver Spring, MD 20993,

Defendants.

Case No. 1:19-cv-22425-Bloom/Louis

Judge Beth Bloom

PLAINTIFF'S OPPOSITION TO DEFENDANTS' MOTION TO STRIKE AND CROSS-MOTION TO COMPLETE AND/OR SUPPLEMENT THE ADMINISTRATIVE RECORD

Plaintiff Catalyst Pharmaceuticals, Inc., (Catalyst) hereby opposes Defendants' Motion to Strike, ECF No. 48, and respectfully moves for an order requiring the Defendants to complete and/or supplement the Administrative Record by adding to it the documents identified in Exhibit A, attached to the accompanying Declaration of Ryan S. Baasch, along with certain related

materials. Catalyst brought this suit under the Administrative Procedure Act (APA). Under the APA, agency action is evaluated on the basis of the Administrative Record compiled by the defendant agency. An Administrative Record must include any information that was considered by the agency when it issued the decision under review. As is evident from the accompanying memorandum and attached documents (which FDA was ordered to produce under the Freedom of Information Act in other litigation), Defendant the Food and Drug Administration (FDA) actually considered all of the information in question in reaching the decision challenged in this case. But FDA omitted all of that information from the Administrative Record it has produced in this case. Catalyst respectfully requests that the Court issue an order directing FDA to incorporate these documents into the Administrative Record.

Pursuant to Local Rule 7.1(a)(3)(A), I hereby certify that Counsel for Catalyst conferred with Counsel for Defendants and Counsel for Jacobus on January 30 and 31, 2020. Defendants oppose as to the documents Catalyst attached to its motion for summary judgment, and are currently unable to take a positon as to the other documents attached to the accompanying Declaration of Ryan S. Baasch, but will respond in due course. Jacobus opposes this motion.

Respectfully submitted,

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PLAINTIFF'S MEMORANDUM IN SUPPORT OF OPPOSITION TO MOTION TO STRIKE AND CROSS-MOTION TO SUPPLEMENT AND/OR COMPLETE THE ADMINISTRATIVE RECORD

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INTRODUCTION

Plaintiff Catalyst Pharmaceuticals, Inc. (Catalyst) brought this Administrative Procedure Act (APA) suit to challenge FDA's approval of Intervenor-Defendant Jacobus's competing drug Ruzurgi. This case relates principally to the straightforward plain language of 21 U.S.C. § 360cc, governing the seven year period Congress enacted for "Orphan Drug Exclusivity" ("ODE" or "exclusivity") under the Federal Food, Drug, and Cosmetic Act (FDCA). If FDA approves a new drug application for a drug previously designated as an "orphan drug" for treatment of a rare disease, the text mandates that FDA "may not approve another [drug] application . . . for the ***same drug*** for the ***same disease or condition***" for any other applicant "***for seven years***." 21 U.S.C. § 360cc(a) (emphasis added).

FDA admits that it granted Catalyst's drug Firdapse this seven-year period of exclusivity, which expires on November 28, 2025. *See* FDA Cross-Motion for Summary Judgment at 3-4, ECF No. 47 (FDA Cross-MSJ). FDA also admits that Jacobus's competing drug is in fact the "***same drug***" as Firdapse, and acknowledges that both drugs are for the "***same disease or condition***"—Lambert Eaton Myasthenic Syndrome ("LEMS").[1] Although 21 U.S.C. § 360cc specifies the precise and limited circumstances when this straightforward "same drug/same disease" test might not apply, *e.g.*, § 360cc(b)(1) (drug shortage); § 360cc(c) (finding of "clinical superiority"), FDA also does not claim that any of those circumstances are present here. FDA

[1] *See* FDA Cross-MSJ at 14 ("Catalyst's drug and Jacobus's drug are the '**same drug**' for purposes of the statutory orphan drug exclusivity provisions." (emphasis added)); *see also* FDA Answer ¶ 6, ECF No. 22 ("Defendants admit that, for the purposes of orphan drug exclusivity, Ruzurgi is the **same drug** (contains the same active moiety) as Firdapse." (emphasis added)); Jacobus Cross-Motion for Summary Judgment at 11 n.3, ECF No. 46 (Jacobus Cross-MSJ) (acknowledging that FDA "recognized that LEMS was a **single disease**" (emphasis added)); FDA Cross-MSJ at 15-16 (acknowledging that "LEMS" is the only disease or condition at issue; and that Firdapse and Ruzurgi are both approved for a "**subset of that disease or condition**" (emphasis added)).

instead argues that its own policy preferences allow it *discretion* to create what it admits is an unprecedented and unwritten additional exception to the straightforward statutory "same drug/same disease" text. *See* FDA Answer ¶ 54 (admitting this is an unprecedented approval). But, as multiple other courts have held, if FDA desires to create a new exception to the statutory text, it must go to Congress—indeed, this is exactly what FDA did three years ago to address a different policy concern it had about ODE in a different case.[2]

In reviewing Catalyst's claims, the Court reviews FDA's Administrative Record. *Florida Fruit & Vegetable Ass'n v. Brock*, 771 F.2d 1455, 1459 (11th Cir. 1985). Courts are authorized, however, to order an agency to incorporate additional documents in that Record that were before the agency at the time and considered by decision-makers, but inappropriately omitted from the Record. *See BBX Capital Corp. v. FDIC*, 2018 WL 6531601, at *1 (S.D. Fla. Aug. 15, 2018); *see also infra* at 4-6.[3]

Here, two overlapping sources of documentation that should have been in FDA's Administrative Record are now at issue: (1) certain information supplied to the Court in two declarations attached to Catalyst's motion for summary judgment (which FDA moved to strike); and (2) a production of redacted Freedom of Information Act (FOIA) documents FDA made to Catalyst's counsel pursuant to an October 21, 2019 Order in *Latham & Watkins LLP v. U.S. FDA*, No. 1:19-cv-1867-TJK (D.D.C.). Below, Catalyst describes each attached subset of such documentation, and cites applicable case law explaining precisely why FDA should have included

[2] See *Eagle Pharm., Inc. v. Azar*, 2018 WL 3838265, at *2-3 (D.D.C. June 8, 2018) (discussing this history); FDA Reauthorization Act of 2017, Pub. L. No. 115-52, § 607(a), § 527(c)-(d), 131 Stat. 10005, 1049 (codified at current 21 U.S.C. § 360cc(c), establishing prospective authority for FDA's "clinical superiority" review).

[3] In its Motion to Strike (at 8-9), FDA itself suggested that if Catalyst wishes for the Court to consider additional information with the summary judgment briefing, Catalyst should move for FDA to complete or supplement the Administrative Record.

unredacted versions of this information in its Administrative Record from the outset—subject to the applicable Protective Order, ECF No. 30, as necessary.[4] In short, each set of attached documents includes information FDA personnel *actually considered* in rendering the administrative decision at issue in this case, and each is directly relevant to Catalyst's claims and FDA's defenses in this case. *See BBX Capital*, 2018 WL 6531601, at *1.

BACKGROUND

Catalyst filed its Complaint in this suit on June 12, 2019. On September 18, 2019, the Court ordered Defendants to produce "the full and accurate transcript of the entire record of proceedings relating to this case (*i.e.*, the complete administrative record)" by October 8, 2019. ECF No. 26. On October 8, 2019, Defendants filed an incomplete and redacted Administrative Record. ECF No. 27. The parties then engaged in a lengthy meet and confer process, including multiple phone calls, to discuss completing the Administrative Record. *See* Decl. of Andrew D. Prins ¶ 4, ECF No. 38-2. As a result of these discussions, Defendants agreed to produce a revised Administrative Record with additional documents, and to remove redactions if the Court entered a protective order. Joint Mot. to Enter Protective Order & Alter the Scheduling Order ¶ 6, ECF No. 28. This Court then entered a Stipulated Protective Order and an Amended Scheduling Order directing Defendants to transmit the complete and unredacted Revised Administrative Record to Catalyst. ECF No. 29. Unfortunately, Catalyst was able to tell from the face of certain documents in the Revised Administrative Record that it remained incomplete, necessitating Defendants updating the Revised Administrative Record multiple times. ECF Nos. 31, 44.

[4] In a very limited number of the documents described below, it appears redactions were applied to cover the names or other personal identifying information of LEMS patients. As stated in the proposed order filed concurrently with this motion, Catalyst does not wish to disclose individual patient names in any context and is ***not*** requesting that this information be unredacted.

Catalyst's counsel also filed a separate suit against FDA on June 25, 2019 because FDA failed to respond to two FOIA requests regarding the Ruzurgi and Firdapse approvals by the mandatory FOIA statutory deadline. *Latham & Watkins LLP v. U.S. FDA*, No. 1:19-cv-1867-TJK (D.D.C.). On September 10, 2019, FDA agreed to "make a preliminary determination regarding the number of a subset of documents responsive to one specific aspect of" one of the FOIA requests (generally, documents showing comparison, or other actions pertaining to both of, Firdapse and Ruzurgi). *Latham & Watkins LLP*, Joint Status Report ¶ 9, ECF No. 9. In a follow-up report to the Court on October 18, 2019, FDA claimed there were "roughly 1000" responsive pages in this subset of documents, and proposed to complete production of those documents "[o]n or before February 18, 2020." *Latham & Watkins LLP*, Joint Status Report ¶¶ 2, 4, ECF No. 10. Catalyst's counsel indicated that these documents should be produced by January 17, 2020, specifically because Catalyst needed them to properly brief its summary judgment motion in *this* case. *See id.* ¶ 9. On October 21, 2019, the court in that action ordered FDA to produce these documents *on a timeline to coincide with the summary judgment briefing here*—by January 17, 2020. *See* Minute Order dated October 21, 2019 (Kelly, J.).[5] Catalyst's counsel received the production on that date.

ARGUMENT

In Administrative Procedure Act cases, the defendant agency's action is usually evaluated on the basis of the administrative record it produces. *See Florida Fruit & Vegetable*, 771 F.2d at 1459. This makes it critical that the administrative record contain all relevant information about the agency action. A properly assembled administrative record must, at a minimum, include "all documents and materials *directly or indirectly* considered by the agency." *See Citizens for Smart*

[5] The Minute Order states, in relevant part, "Upon consideration of the parties' Joint Status Report, it is hereby ORDERED that Defendant shall process and produce the subset of documents referenced in the parties' Joint Status Report, ECF No. 10 at 1, by January 17, 2020."

Growth v. Peters, 2008 WL 11331898, at *3 (S.D. Fla. Sept. 24, 2008) (emphasis added) (citing *Bar MK Ranches v. Yuetter*, 994 F.2d 735, 739 (10th Cir. 1993)) *see also BBX Capital Corp. v. FDIC*, 2018 WL 6531601, at *2 (S.D. Fla. Aug. 15, 2018) (same). It must also "include all materials that '***might have influenced*** the agency's decision,'" regardless of whether the agency ultimately relied on the information. *See Amfac Resorts, LLC v. U.S. Dep't of the Interior*, 143 F. Supp. 2d 7, 12 (D.D.C. 2001) (quoting *Nat'l Courier Ass'n v. Bd. of Governors*, 516 F.2d 1229, 1241 (D.C. Cir. 1975)). As the Department of Justice (DOJ) has explained in its internal guidance for federal agencies, a proper administrative record should include:

- "Documents and materials which were available to the decision-making office at the time the decision was made";
- "E-mail[s]";
- "communications the agency received from . . . the public, and any responses to those communications"; and
- "information that supports *or opposes* the challenged agency decision."

See Guidance to Client Agencies on Compiling the Administrative Record, 48 U.S. ATTY'S BULLETIN no. 1 (Feb. 2000).[6]

In cases where an agency omits the type of material the DOJ guidelines identify, courts recognize that the agency has "failed to properly assemble the record," and that additional information must be considered. *See Miccosukee Tribe of Indians v. United States*, 2008 WL 2967654, at *4 (S.D. Fla. July 29, 2008) ("A court, however, may go beyond the administrative record in certain circumstances."); *Citizens for Smart Growth*, 2008 WL 11331898, at *3; *Sierra Club, Inc. v. Leavitt*, 488 F.3d 904, 914 n.16 (11th Cir. 2007); *see also Walter O. Boswell Mem'l*

[6] https://www.justice.gov/sites/default/files/usao/legacy/2006/06/30/usab4801.pdf.

Hosp. v. Heckler, 749 F.2d 788, 792 (D.C. Cir. 1984) ("To review less than the full administrative record might allow a party to withhold evidence unfavorable to is case.").[7] Ultimately, "[i]f a court is to review an agency's action fairly, it should have before it neither more nor less information than did the agency when it made its decision." *Id.* at 792. Where, as here, a plaintiff moves for the agency to include records that were *actually* before the agency (as opposed to documents that allegedly should have been before the agency), the plaintiff must only show "(1) when the documents were presented to the agency; (2) to whom; (3) and under what context." *See BBX Capital*, 2018 WL 6531601, at *1 (explaining that where these factors are satisfied the record must be completed to include these records); *see also Citizens for Smart Growth*, 2008 WL 11331898, at *3 (ordering agency to complete record with documents "obtained through public records requests"); *Alabama-Tombigbee Rivers Coal. v. Kempthorne*, 477 F.3d 1250, 1260-62 (11th Cir. 2007) (considering evidence obtained by the plaintiff through public records requests).

As demonstrated below, Catalyst has assembled a discrete series of documents—attached at Tabs 1-5—that should have been included in the Administrative Record under applicable case law. Certain of these documents are publicly available and were attached to Catalyst's prior declarations. *See* Decl. of Daniel Brennan, ECF No. 38-1. The remaining documents were produced in redacted form in the separate FOIA action.[8] As explained below, even the highly redacted portions of the FOIA documents at issue here demonstrate that FDA *actually considered* those materials when it made the decision challenged in this case. Catalyst requests that the Court

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[7] Courts may also review extra-record material without considering a formal motion to complete and/or supplement where the plaintiff independently possesses and submits the information. *See Bundorf v. Jewell*, 142 F. Supp. 3d 1138 (D. Nev. 2015) (APA case; court reviewed information in plaintiff declaration submitted in connection with summary judgment brief over agency's motion to strike); *Oceana, Inc. v. Pritzker*, 126 F. Supp. 3d 110 (D.D.C. 2015) (similar).

[8] Pursuant to the most recent order in the FOIA case, the parties will submit a joint status report regarding further proceedings by February 3, 2020.

deny FDA's motion to strike and also order FDA to include the unredacted versions of the documents described herein in the Administrative Record for this matter, subject to the Protective Order.

For purposes of clarity, we have organized the attached documents into four subject matter categories:

(1) Documents showing that FDA actually considered information on drug pricing and a letter from Senator Bernie Sanders in the context of reaching the challenged decision, despite the fact FDA readily admits it lacked authority to do so;

(2) Documents showing that FDA reached a decision that the very small number of pediatric patients would still have access to LEMS medication under "Expanded Access" programs even if Jacobus's drug was not approved—which specifically rebuts FDA and Jacobus's arguments that FDA had to exercise its "discretion" in this case to ensure pediatric access to LEMS medication;

(3) Documents showing that FDA recognized and intended that Jacobus's drug would be sold "off-label" to adult LEMS patients even if approved only for the very small number of pediatric LEMS patients (1-2% of the LEMS patient population), demonstrating that FDA was intending to and did act to undermine Catalyst's statutory exclusivity.

(4) Documents showing that FDA realized that the statutory regime in 21 U.S.C. § 360cc specified only a small number of limited circumstances in which the plain language of Catalyst's ODE did not apply, and showing that FDA recognized those circumstances were not applicable to the Jacobus application.

In reviewing the following materials, the following background information is relevant about FDA's decision-making process. The disputed regulatory action in this case was FDA's admittedly unprecedented decision to issue a new drug approval to Jacobus, despite Catalyst's

exclusivity. *See* FDA Answer ¶ 54. As indicated (*supra* at 1 & n.1), there is no dispute that FDA's approval for Jacobus was for the "same drug" and the "same disease" on which FDA had previously granted Catalyst seven years of ODE under 21 U.S.C. § 360cc. FDA's rationale is that it nevertheless had *discretion* to do so because Jacobus's drug would only be labeled to treat the tiny number of "pediatric" patients with LEMS (Jacobus estimates about 15)—although FDA anticipated that Jacobus's drug would be prescribed off-label for adults. *See* FDA Cross-MSJ at 14-15.

The principal body within FDA charged with evaluating Catalyst's Firdapse and Jacobus's Ruzurgi applications was FDA's Center for Drug Evaluation and Research (CDER);[9] in addition, CDER's "Exclusivity Board" played a role in the determination whether Firdapse's ODE blocked FDA from approving Ruzurgi. *See* FDA Cross-MSJ at 4-5 (describing role of the Exclusivity Board); *see also* 21 C.F.R. § 10.75 (providing the Exclusivity Board's decision can be overruled by any supervisor (the CDER Director) or the Commissioner). The existing Administrative Record, along with the attached materials (Tab 5), identify many FDA personnel as key players in the decision-making process, including the following:

- Kelley Nduom: Senior FDA Regulatory Counsel who coordinated the "CDER Exclusivity Board discussions around the Jacobus application for Ruzurgi," FDACDER000511,[10] and signed the official memorandum explaining FDA's legal rationale for approving Ruzurgi. *See* ECF No. 27-1, FDA0474-483; *id.* at FDA0483 (Nduom signature); *see also* FDA Cross-MSJ at 5.

[9] U.S. FDA, CENTER FOR DRUG EVALUATION AND RESEARCH: CDER, https://bit.ly/2Gxerra.

[10] All documents identified as "FDACDER[XXXXXX] are part of the FOIA production.

- CDER's "Deputy Director for Regulatory Programs," FDACDER000416, in charge of "overseeing the regulation of research, development, manufacture, and marketing or prescription, over-the-counter, and generic drugs in the United States."[11]
- The Director of CDER's Office of Medical Policy.[12]
- CDER Personnel described in FDA documents as "essential folks" in the Ruzurgi and Firdapse exclusivity discussions (FDCADER000520): Teresa Buracchio (Clinical Team Leader, CDER's Division of Neurology Products, Office of Drug Evaluation-I, Office of New Drugs, *see* ECF No. 27-1, FDA0107; ECF No. 27-2, FDA0632), Dr. Ellis Unger (Director of CDER's Office of Drug Evaluation-I, Office of New Drugs[13]), Colleen LoCicero (Associate Director for Regulatory Affairs at CDER's Office of Drug Evaluation I, Office of New Drugs[14]), and James Myers (Director of CDER's Office of New Drug Policy's Regulatory Policy Division).
- Jay Sitlani: Senior Regulatory Counsel, CDER's Office of Regulatory Policy (as of 2016)[15] who was responsible for "working on getting the Jacobus [application] on the agenda for . . . exclusivity board meeting to start the discussion about Catalyst's orphan exclusivity." FDACDER000403.

I. TAB 1: DOCUMENTS SHOWING FDA CONSIDERED FIRDAPSE'S PRICE WHEN IT APPROVED RUZURGI

As FDA has repeatedly admitted, it is not allowed to consider drug prices as part of its analysis of drug approvals and exclusivities. *See Orphan Drug Regulations*, 57 Fed. Reg. 62,076, 62,079 (Dec. 29, 1992) ("FDA has no authority over drug pricing *or any authority to consider it in drug approval*." (emphasis added)); FDA Cross-MSJ at 11 (FDA recognizing it "*d[oes] not*

[11] https://bit.ly/2RPyKFe.

[12] https://bit.ly/2U2Vgxh.

[13] https://bit.ly/2t4Dy1f.

[14] U.S. FDA, *Activity Outline, FDA Drug Topics: An Overview of FDA's Expanded Access Program with a Focus on Individual Patient Expanded Access* 2, https://bit.ly/2GvUPnc.

[15] CDER, *Application Number: 125545Orig1s000, Administrative and Correspondence Documents* 13, https://bit.ly/2O7iFd3.

have authority to consider price under the Orphan Drug Act" (emphasis added)). And, under the APA, "[a]gency action[] [is] arbitrary and capricious when the agency has relied on factors which Congress has not intended it to consider." *See Georgia Dep't of Educ. v. U.S. Dep't of Educ.*, 883 F.3d 1311, 1315 (11th Cir. 2018).[16] Yet, despite the many redactions, the documents that Catalyst has obtained show that key decision-makers at all levels of leadership at FDA *actually considered* drug pricing in the course of their decision-making process. At a key point in time, one FDA decision-maker remarked: "This is a difficult case, but more so perhaps because we know about the competition and price issues." FDACDER000419. An FDA senior regulatory counsel responsible for this issue remarked: "Firdapse is the drug that is raising the ire of Senator Sanders and others in Congress, in part because of the cost of the drug . . . So, our exclusivity discussions, while technical and legalistic as they always are, will by necessity have to occur against this backdrop." FDACDER00485-86. Senator Sanders sent a letter to FDA complaining about pricing and specifically mentioning a past case known as "Makena," suggesting that case was a model and that FDA should exercise its discretion not to enforce Catalyst's exclusivity against Jacobus. FDACDER000611-12 & n.8. In the context of evaluating the exclusivity issue here, FDA had specifically discussed multiple options to allow Jacobus or other LEMS medication to stay on the market despite Catalyst's exclusivity, including the "Makena" approach,, FDACDER000412-16; FDA decision-makers evaluated Ruzurgi as a potential solution after CDER leadership circulated an article about pricing issues.[17] Indeed, documentation about these pricing concerns was widely

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[16] *See Sierra Club v. Zinke*, 2018 WL 3126401, at *4 (N.D. Cal. June 26, 2018) ("The administrative record needs to be whole so that [the Court] can ultimately determine whether [the agency] has relied on factors which Congress has not intended it to consider.").

[17] As Catalyst explained in its summary judgment brief (ECF No. 38 at 8-9), this pricing controversy was factually misguided: Ruzurgi was ultimately priced at approximately $200,000 per year, and Catalyst established a comprehensive patient financial assistance/insurance navigation program which allowed most patients to pay $10 or less per month for Firdapse out of

circulated within FDA to decision-makers up and down the chain, including to the FDA Commissioners' Office. FDACDER000532.

To demonstrate that FDA should have included these documents in the Administrative Record in unredacted form, we provide more detail on each document in Tab 1 here:

- In December 2018 (shortly after Firdapse was approved on November 28, 2018), CDER's Deputy Director for Regulatory Programs circulated a news article about Firdapse's high price, and claimed that FDA was "in the middle of working up a proposal to address high priced drugs." The email chain discusses exclusivity and options to approve Jacobus's Ruzurgi. Multiple key FDA decision-makers, including James Myers and Colleen LoCicero, are copied and provided feedback on the issue. *See* FDACDER000412-416.
- In January 2019, Colleen LoCicero emailed James Myers and multiple FDA decision-makers with questions about Ruzurgi's possible approval. The email chain is heavily redacted, but in one unredacted excerpt the Director of CDER's Office of Medical Policy states: "This is a difficult case, but more so perhaps because we know about the competition and price issues." FDACDER000419.
- In February 2019, Senator Sanders announced that he would be investigating Firdapse's price. *See* Press Release, https://bit.ly/2PtdRjk. An FDA staffer forwarded a news article discussing Sanders' campaign to CDER management. The response from CDER's Director was: "Do you know where we are with Jacobus?" Colleen LoCicero, James Myers, and Drs. Unger and the Director of CDER's Office of Medical Policy —all subordinates—later received this email. FDACDER000563-66.
- In February 2019, Kelley Nduom, Colleen LoCicero, Jay Sitlani, and others were conferring regarding Firdapse and Ruzurgi exclusivity issues. Sitlani wrote, "Firdapse is the drug that is raising the ire of Senator Sanders and others in Congress, in part because of the cost of the drug. . . So, our exclusivity discussions, while technical and legalistic as

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pocket. Catalyst also committed to distribute the drug for no cost to patients who are unable to obtain insurance coverage, subject to applicable regulatory requirements. At least one reason why Ruzurgi was likely less expensive is because, while FDA never "provided funding to Catalyst for Firdapse," *it did fund a study which apparently served to reduce Jacobus' costs and thus allow it to offer a lower price.* FDACDER000840.

they always are, will by necessity have to occur against this backdrop." FDACDER00485-86.

- In February 2019, multiple FDA decision-makers, including Colleen LoCicero and Teresa Buracchio, held a "meeting to discuss talking points for addressing questions regarding the unavailability of the Jacobus amifampridine and the price of Firdapse." FDACDER000523-25.
- In February 2019 Kelley Nduom wrote multiple FDA decision-makers, including Jay Sitlani and Teresa Buracchio, to note that the Ruzurgi/Firdapse "issue has been heating up in the news." FDACDER000508-11.
- In February 2019, Colleen LoCicero sent James Myers, Dr. Unger, and others, a social media link discussing "the Catalyst/Jacobus [drug] saga." FDACDER000495.
- In February 2019, Colleen LoCicero wrote to the Director of CDER's Office of Medical Policy, James Myers, and others that FDA "is receiving ***a lot*** of inquiries, as well as complaints, from individuals, physicians, the ***Commissioner's Office***, the Patient Affairs Staff, [CDER management] and others . . . about the price of the Catalyst product." FDACDER000528-35 (emphasis added).
- At the end of February, Senator Sanders directly asked FDA to do something about Firdapse's price, even if that meant allowing an unapproved drug to stay on the market. FDACDER000611-12. Senator Sanders specifically identified "agency precedent" for this practice: a product known as "Makena." FDACDER000612 n.8. Makena is a case where FDA exercised discretion not to enforce against a competing unapproved compounded drug, and FDA specifically noted in December 2018 that this precedent could be a solution here. FDACDER000413-16.
- FDA decision-makers wrote and sent multiple emails discussing Senator Sanders' letter and preparing how to respond. Colleen LoCicero took a leadership role in preparing the response. FDACDER000613-18. She discussed the response with key decision-makers including the Director of CDER's Office of Medical Policy, and Dr. Unger, James Myers, and Teresa Buracchio. FDACDER000594-95; FDACDER000720-23; FDACDER000728-36; *see also* FDACDER000737-39 (fully redacted accompanying letter); FDACDER000723 (referencing, but omitting, attached file); FDACDER001137-39(response to Sanders; FDACDER000619-21 (fully redacted); FDACDER000622-28

(heavily redacted document indicating CDER "consulted OCC on the general matter of [REDACTED]"; subject line: "Discussion of Sanders Letter Response").

- After FDA approved Ruzurgi, it authored and circulated internally a (heavily redacted) slide deck presenting positive press coverage and seeming to applaud the negative impact the Ruzurgi approval had on Catalyst's *share price*. *See* FDACDER001000-01; *see also* FDACDER000947-51 (key FDA decision-maker responding to the press about Catalyst pricing and Jacobus approvals: "Good Press! Thanks for sending. I like being crafty… Too bad the Catalyst lawyers will be on our doorstep soon""); *see also infra* at 17.[18]

II. TAB 2: DOCUMENTS SHOWING PEDIATRIC LEMS PATIENTS COULD RECEIVE TREATMENT WITHOUT A RUZURGI APPROVAL

Both Jacobus and FDA have made policy arguments in their summary judgment briefs suggesting that the Ruzurgi approval was justified to provide a treatment option for pediatric patients. *See* Jacobus Cross-MSJ at 7 (arguing "no purpose would be served by creating a vacuum under which no entity could provide a drug with valuable applications to populations, such as the pediatric LEMS patients here"); *cf.* FDA Cross-MSJ at 1 (emphasizing that Ruzurgi is the "only FDA-approved drug to treat pediatric LEMS patients"). Catalyst explained in its summary judgment brief (at 10) that Ruzurgi or Firdapse could still be available to pediatric patients under "expanded access" programs, even if Ruzurgi was not approved. The documents below further

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[18] FDA argues that "Catalyst has not pointed to any evidence or made a colorable argument that FDA actually considered any" material related to price, and that Catalyst has offered only a "conclusory assumption" that the agency "approved Ruzurgi in order to undercut Catalyst's high price for Firdapse." FDA Motion to Strike at 6, 7. But these documents now provide more than ample evidence that FDA considered Firdapse's price. *See also infra* Tab 3. In light of these documents, FDA can no longer suggest that it did not "directly or indirectly" consider this factor. *See Fund for Animals v. Williams*, 391 F. Supp. 2d 191, 199 (D.D.C. 2005) (agencies "cannot justify the exclusion of . . . adverse documents from the administrative record simply by claiming that the documents were not directly or indirectly considered by the agency").

In addition, the core material that FDA has moved to strike, ECF No. 38-1, Exs. B (Senator Sanders letter), and C (FDA letter to patients), are in the FOIA documents that Catalyst is now moving to include in the Administrative Record. *See* FDACDER000611-12 (Senator Sanders letter); FDACDER000952-53 (FDA letter to patients) (in Tab 3).

demonstrate that FDA personnel were aware during the decision-making process that the very small number of pediatric LEMS patients (Jacobus estimates "less than 15" such patients)[19] *would never lose access to Jacobus' drug even if Ruzurgi was not approved*. FDA informed Jacobus it could continue to provide its drug to pediatric LEMS patients under expanded access programs. *See* FDACDER000587-89. And FDA was aware, before approving Ruzurgi, that Catalyst was already developing data in 2018 to support Firdapse's use in pediatrics, FDACDER000239, which has since also been provided to FDA. Of course, under FDA's own argument, FDA Cross-MSJ at 22-23, Catalyst's approved drug could also be prescribed "off label" to pediatric patients. And it could also have been delivered to pediatric LEMS patients through Catalyst's own expanded access program.

"Agency actions are arbitrary and capricious when the agency . . . offer[s] an explanation for its decision that runs counter to the evidence before the agency." *See Georgia Dep't of Educ.*, 883 F.3d at 1314 (internal citation omitted); *Genuine Parts Co. v. EPA*, 890 F.3d 304, 312 (D.C. Cir. 2018) ("[A]n agency cannot ignore evidence that undercuts its judgment; and it may not minimize such evidence without adequate explanation."). FDA should include the following documents from Tab 2 (and any other documents in their possession related thereto) in the Administrative Record in unredacted form showing that FDA considered pediatric access to LEMS medication and *knew it was not a problem necessitating approval of Ruzurgi*.

- In March 2019, Colleen LoCicero and Teresa Buracchio received emails stating that "*we have advised Jacobus* that they may continue to provide their product to physicians for treatment use under expanded access for . . . pediatric LEMS patients." FDACDER000587-89 (emphasis added); *see also* FDACDER000576-77 (same).

[19] Ed Silverman, *Laura Jacobs hopes to provide her newly approved drug at 'little or no' cost to patients*, STAT+ (May 7, 2019), https://bit.ly/2RUAOvS.

- In addition, FDA has fully redacted the official instruction it directly provided to Jacobus. *See* FDACDER000460-66.
- The Director of CDER's Office of Medical Policy, Colleen LoCicero, and others also discussed a fully redacted memorandum on "Policy Implications for Catalyst/Jacobus Products and Expanded Access Use." FDACDER000394-97.

FDA documents also show that Catalyst was developing the same pediatric data that Jacobus had, and that Catalyst would likely be approvable even for the very few pediatric patients using the exact same rationale FDA employed for Jacobus. FDACDER000239-44; FDACDER000325-27. Indeed, under FDA's logic, Catalysts' drug could also be prescribed "off-label" for those very few pediatric patients.

- Colleen LoCicero, Teresa Burachhio, and other decision-makers also knew in October 2018 that Catalyst "recently started a study to collect [pediatric] data." FDACDER000239-44. Therefore, before Ruzurgi was approved, FDA was aware that Catalyst very shortly would have had all the information (and more) that the agency wanted in order to approve Firdapse for pediatric patients.
- In October 2018, Colleen LoCicero stated, "Catalyst may make an argument that their product could have been approved in patients [REDACTED] because it contains the adult data needed for the pediatric evidence/dosing extrapolation and that FDA should have initiated/suggested/raised this with Catalyst, because we have done this sort of thing in the past." FDACDER000325-27. But she also notes that FDA told Catalyst it could not extrapolate. *Id.* In other words, Catalyst was told *not* to do what FDA *did* allow Jacobus to do.

III. TAB 3: DOCUMENTS SHOWING THAT FDA INTENDED THAT RUZURGI BE SOLD OFF-LABEL TO ADULT PATIENTS

Similar to pricing, FDA also cannot base drug approvals on intended off-label use. *See* FDA Cross-MSJ at 22 (acknowledging Jacobus is not allowed to promote its drug off-label). ODE is a statutory exclusivity established by Congress to incentivize drug developers to invest money

in drugs that do not treat large populations. *See Eagle*, 2018 WL 3838265, at *1. And Catalyst invested over $100 million in developing and obtaining approval for Firdapse. Complaint ¶ 3, ECF No. 1. But the documents indicate that FDA *actually considered* the fact that a pediatric approval, for an incredibly small number of patients, would allow physicians to prescribe Jacobus's drug for the vastly larger adult LEMS population off label (thereby creating price competition): According to FDA personnel directly involved in decision-making: "[W]hile the Jacobus product, once approved, won't be labeled for use in adults with LEMS, healthcare providers will be able to prescribe it for adults off label under the practice of medicine. ***The Jacobus approval should, therefore, take care of the situation***." FDACDER000923 (emphasis added); *see also* FDACDER000924-26.[20] Another FDA official involved in the decision-making reviewed a critical press article about Catalyst pricing and ability to prescribe Jacobus' product for adults "off label" and commented: *"Good press, thanks for sending. I like being crafty... Too bad the Catalyst lawyers will be on our doorstep soon."* FDACDER000947 (emphasis added); *see also* FDACDER000948-51.[21] FDA also specifically issued public FAQs and sent letters to patients suggesting, in red text, that their healthcare providers could prescribe the Jacobus drug off label. FDA should include these documents from Tab 3 in the Administrative Record in an unredacted form.

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[20] Note that Catalyst has instituted special programs to ensure patient access at minimal co-pay price levels for patients who lack adequate insurance coverage or resources to cover the cost of the drug. *See supra* n. 17. Likewise, since FDA's approval, Catalyst has provided Firdapse for hundreds of patients, and has successfully worked with physicians and patients on Firdapse to address any issues regarding pricing, access dosage, and any questions or concerns regarding the drug. *See also* FDACDER000586; FDACDER000594-95 (Tab 4) (discussing patient access, pricing, and other issues).

[21] FDA also issued a public "tentative" approval for Ruzurgi in adults, which might have been perceived as a suggestion that the drug was safe and effective for off-label use in adults. *See* ECF No. No. 27-1, FDA0511-12.

- In March 2019, Colleen LoCicero received an email containing copies of complaints from LEMS patients. Those complaints include "Catalyst[] price gouging" and that "[t]he price for firdapse is insane." The complaints also referred to treatment issues with Firdapse. LoCicero responded, "*[W]hile the Jacobus product, once approved, won't be labeled for use in adults with LEMS, healthcare providers will be able to prescribe it for adults off label under the practice of medicine. The Jacobus approval should, therefore, take care of the situation.*" FDACDER000923-26 (emphasis added).
- Within 24 hours of the Ruzurgi approval, an FDA press officer circulated a news article indicating that the Ruzurgi approval was an "unexpected twist to a simmering controversy over a rare disease drug that earlier this year briefly became a poster child for high-priced medicines." The article also stated that "Catalyst stock was down as much as 44%" and that if Ruzurgi is "on the market for children, it can be prescribed for adults." Teresa Buracchio, Colleen LoCicero, Kelley Nduom, Jay Sitlani, Dr. Unger, and others received the email. Dr. Unger responded: "***Good Press! Thanks for sending***. ***I like being crafty***… ***Too bad the Catalyst lawyers will be on our doorstep soon***." FDACDER000947-51 (emphasis added).
- FDA issued FAQs and sent multiple letters to patients and others suggesting that Ruzurgi could be prescribed off-label to adults, and in multiple of those communications to adult patients specifically emphasized that point by highlighting that text IN RED. *See* FDACDER000872-75 (indicating "patients potentially [could] use either drug treatment off-label"); FDACDER000952-53 (response to complaint about Firdapse informing patient that Ruzurgi was just approved and to "talk with your health care provider about the right treatment option for you"); FDACDER001076-77 (same); FDACDER001101 (similar, instructing patient: "Please talk to your health care provider about the right treatment option for you."); FDACDER001112 (same); FDACDER001080-81 (pharmaceutical CEO asking whether there is any restriction on Ruzurgi "marketing this approved pharmaceutical for **adults**"; FDA responded: "[t]he practice of medicine is not regulated by FDA, and the decision to treat a patient with a drug for an unapproved use is up to the treating health care professional").
- There is also a completely redacted document at FDACDER000790-95.

IV. TAB 4: DOCUMENTS SHOWING THAT FDA REALIZED THERE WERE ONLY VERY LIMITED CIRCUMSTANCES SPECIFIED IN THE STATUTE FOR DEPARTING FROM THE "SAME DRUG, SAME DISEASE" REQUIREMENT

The Orphan Drug Act provides very limited circumstances where drugs that are the "same drug" and for the "same disease or condition" can be approved. 21 U.S.C. § 360cc(b), (c). FDA was aware that it had to consider whether a Ruzurgi approval would fit any of these circumstances. The documents below from Tab 4 reflect that FDA *did* consider these circumstances. But then FDA ignored the carefully crafted statutory scheme and simply purported to create a new exemption from the clear "same drug/same disease or condition" bar. *See* FDA Answer ¶ 54; *Georgia Dep't of Educ.*, 883 F.3d at 1314 (agency must "consider [each] important aspect of the problem") (internal citation omitted).

First, the Orphan Drug Act and FDA regulations provide that Ruzurgi could have been approved lawfully if FDA found it "clinically superior" to Firdapse. 21 U.S.C. § 360cc(c); 21 C.F.R. § 316.3(b)(3), (14). In early January 2019 James Meyers emailed other decision-makers to notify them that "orphan exclusivity would block approval of [Ruzurgi], unless [Ruzurgi] is proven to be clinically superior to [Firdapse]." FDACDER000412-16. He also concluded Ruzurgi was "*not*" clinically superior to Firdapse. *Id.* The Commissioner's office remained invested in identifying viable differences between the two drugs to support a clinical superiority finding. *See* FDACDER000519-22. Thus, Kelley Nduom asked Teresa Buracchio, Jay Sitlani, and other decision-makers whether "Jacobus has made any claims of clinical superiority" or if any "other factors" supported a clinical superiority finding. FDACDER000515-18. And subsequent documents show that FDA never identified a viable difference.

Second, the Orphan Drug Act provides that FDA could have approved Ruzurgi if Catalyst could "not ensure the availability of sufficient quantities of [Firdapse] to meet the needs of" LEMS patients. 21 U.S.C. § 360cc(b)(1). FDA also investigated this circumstance. *See*

FDACDER000578-82. But Catalyst explained to the agency that there were no supply issues, and that it was offering multiple programs to ensure all LEMS patients were able to obtain Firdapse. FDACDER000583-86. And the evidence before FDA showed that there was not "a single patient who missed even a single dose in their transition to commercially available Firdapse." FDACDER000594-95.

There are also multiple *fully* redacted documents that appear to be highly relevant to FDA's legal considerations and that should be included in the Administrative Record.

- In February 2019 Colleen LoCicero also emailed a colleague apparently describing what FDA's Office of Chief Counsel ("OCC") is concluding about exclusivity and related issues. FDACDER000558. But the email is completely redacted.
- There is also a completely redacted memorandum on whether "the orphan drug exclusivit[y] the FDA recognized for Firdapse . . . block[s] the approval of Ruzurgi." FDACDER000859-69.
- In February 2019 Colleen LoCicero emailed Teresa Buracchio and others for feedback on a "background document" for the Exclusivity Board on Firdapse and Ruzurgi. FDACDER000490-92. The background document is completely redacted. But, in response to LoCicero's document, Buracchio stated that "the indication [for Ruzurgi] will be the same as Firdapse other than the age" and that "the wording of the indication statement is not a relevant issue for consideration in the exclusivity discussion." This document must be added to the Administrative Record, along with any other documents provided to the Exclusivity Board, an identification of who sat on the Exclusivity Board, and any other emails not produced that show transmission of information to or from the Exclusivity Board regarding Firdapse and Ruzurgi. *See, e.g.*, FDACDER490-92.

V. FDA MUST COMPLETE AND/OR SUPPLEMENT THE ADMINISTRATIVE RECORD WITH UNREDACTED DOCUMENTS

As indicated above, there is no question that FDA should be required to provide *unredacted* versions of these documents. *See* Stipulated Protective Order ¶ 6, ECF No. 30; *see also supra* n.4. Redactions are "not appropriate" if they frustrate APA review. *See Int'l Longshoremen's Ass'n,*

AFL-CIO v. Nat'l Mediation Bd., 2006 WL 197461, at *4 n.4 (D.D.C. Jan. 25, 2006). FDA may claim its redactions are necessary to protect trade secrets or other information that should not become public. But the Court's Protective Order, ECF No. 30, already addresses this problem. *See id.*; *see also Public Emps for Envtl. Responsibility v. Beaudreau*, 2012 WL 12942599, at *8 (D.D.C. Nov. 9, 2012) (providing agency may "seek to amend the protective order governing this case" if it is concerned about improper public disclosure of documents); *Food & Water Watch v. USDA*, 325 F. Supp. 3d 39, 58-59 (D.D.C. 2018) ("If any portions of this completed Administrative Record contain financial information that the defendants believe should be protected from public dissemination, the defendants may seek appropriate protective measures.").

CONCLUSION

The Court should deny FDA's motion to strike, and order FDA to complete and/or supplement the Administrative Record with unredacted versions of the documents discussed in this memorandum and attached to the accompanying Declaration of Ryan S. Baasch.

Dated: January 31, 2020

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CERTIFICATE OF SERVICE

I HEREBY CERTIFY that on January 31, 2020, a true and correct copy of the foregoing was served via CM/ECF on the following counsel:

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