UNITED STATES DISTRICT COURT
SOUTHERN DISTRICT OF NEW YORK

CHARLES SEIFE,

Plaintiff,

v.

FOOD AND DRUG ADMINISTRATION and DEPARTMENT OF HEALTH AND HUMAN SERVICES,

Defendants,

and

SAREPTA THERAPEUTICS, INC.,

Defendant-Intervenor.

1:17-cv-3960 (JMF)

DEFENDANTS' MEMORANDUM OF LAW IN SUPPORT OF MOTION FOR SUMMARY JUDGEMENT

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Defendants the United States Food and Drug Administration ("FDA") and the Department of Health and Human Services ("HHS") (collectively "Defendants" or the "Government") respectfully submit this memorandum of law in support of their motion for summary judgment with respect to the Complaint filed by Plaintiff Charles Seife.

PRELIMINARY STATEMENT

The narrow issue before the Court in the parties' cross-motions for summary judgment is whether the FDA properly withheld from release, pursuant to FOIA Exemption 4, certain information regarding clinical studies submitted by Defendant-Intervenor Sarepta Therapeutics, Inc. ("Sarepta") to the FDA in connection with its new drug application for Exondys 51. Specifically, the FDA redacted granular-level details of Sarepta's clinical studies, detailed study result information, certain study end-point information, and certain adverse event information. Although Sarepta had publicly released some information regarding its clinical studies, the withheld information has not been publicly released. Release of the withheld information would cause Sarepta significant harm in the highly competitive market for developing a drug to treat Duchenne muscular dystrophy because it would allow Sarepta's competitors to reap the benefits of Sarepta's clinical studies and Sarepta's significant resources invested in those studies by using proprietary information from these studies to develop competing therapies without the same level of investment. Moreover, release of the information would provide Sarepta's competitors insight into Sarepta's future research plans. Accordingly, the information was properly withheld pursuant to FOIA Exemption 4, and Defendants' motion for summary judgment should be granted.

BACKGROUND[1]

This action pertains to a Freedom of Information Act ("FOIA") request received by Defendants from Plaintiff on December 14, 2016, that sought records related to FDA's September 19, 2016, approval of the drug eteplirsen manufactured by Sarepta and marketed as Exondys 51 for treatment of Duchenne muscular dystrophy ("DMD"). *See* ECF No. 1 ("Compl.") ¶¶ 1-3 & Ex. A; *see also* Declaration of Sarah Kotler ("Kotler Decl.") ¶¶ 11, 13 & Ex. A. The requested documents included, *inter alia*, materials submitted by Sarepta to FDA to support the approval application for the drug,[2] as well as internal Government communications and deliberations regarding the approval. *See* Kotler Decl. Ex. A. All of the information sought by the request was maintained by FDA's Center for Drug Evaluation and Research ("CDER"). *Id.* ¶ 13.

Plaintiff initiated this lawsuit on May 25, 2017, after FDA denied his appeal for expedited processing. *See* ECF. No. 1. Plaintiff then filed a motion for partial summary judgment seeking expedited processing on June 21, 2017. *See* ECF No. 15. Plaintiff and the Government reached agreement on a production schedule for the Government to produce documents responsive to the FOIA request (as refined by Plaintiff during the course of the Parties' negotiations), which was entered by the Court on June 27, 2017, and also resolved the pending motion for partial summary

[1] The Government is not submitting a Local Rule 56.1 statement as "the general rule in this Circuit is that in FOIA actions, agency affidavits alone will support a grant of summary judgment" and a Local Rule 56.1 statement "would be meaningless." *Ferguson v. FBI*, 89 Civ. 5071 (RPP), 1995 WL 329307, at *2 (S.D.N.Y. June 1, 1995), *aff'd*, 83 F.3d 41 (2d Cir. 1996); *N.Y. Times Co. v. DOJ*, 872 F. Supp. 2d 309, 314 (S.D.N.Y. 2012); *ACLU v. Office of the Dir. of Nat. Intelligence,* 10 Civ. 4419 (RJS), 2011 WL 5563520, at *1 n.1 (S.D.N.Y. Nov. 15, 2011).

[2] As part of a manufacturer's application for approval to market a new drug, the manufacturer must submit substantial and detailed information about the drug, including data concerning the drug's safety and effectiveness obtained through both preliminary research and clinical trials (*i.e.*, trials conducted in humans). *See* 21 U.S.C § 355(b)(1); 21 C.F.R. § 314.50. The results of those clinical trials are submitted as part of the new drug application when the manufacturer seeks marketing approval for the product. *See* 21 C.F.R. part 314.

judgment. *See* ECF No. 39. On September 15, 2017, Sarepta moved to intervene as a defendant. *See* ECF Nos. 43 & 44. Sarepta's motion was granted on September 18, 2017. *See* ECF No. 47.

Pursuant to 21 C.F.R. § 20.61, FDA consulted with Sarepta regarding the confidentiality of certain information Sarepta had submitted to the FDA in connection with the New Drug Application ("NDA") for Exondys 51 Injection (eteplirsen). *See* Declaration of Nancy B. Sager ("Sager Decl.") ¶¶ 25-26 & Exs. K-N. Specifically, on July 28, 2017, FDA sent Sarepta copies of the Clinical Study Reports for Study 201 and 202 ("CSRs") that it had submitted to FDA in the NDA and which totaled 35,479 pages. *Id.* ¶ 25. A CSR is a full report of an individual study of a drug conducted in human patients. The report discusses how the critical design features of the study were chosen and includes enough information on the plan, methods, and conduct of the study so that there is no ambiguity in how the study was carried out. The report also contains detailed information concerning individual patient data and analytical methods to allow replication of the critical analyses. *See Guideline for Industry: Structure and Content of Clinical Study Reports*, available at https://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM073113.pdf (last accessed Mar. 21, 2018). FDA provided the CSRs to Sarepta to give the company—which had created the reports and conducted the studies—an opportunity to identify any proprietary information. *See* Sager Decl. ¶ 25. Sarepta proposed redactions to the two CSRs and provided a draft index to FDA explaining the bases of the proposed redactions. *See* Sager Decl. ¶¶ 28, 31. Prior to producing the materials to Plaintiff, FDA reviewed the proposed redactions and made certain changes. *See id.* ¶¶ 29, 32.

In accordance with the agreed upon production schedule, between July 24, 2017, and December 8, 2017, FDA made nine productions and produced a total of approximately 45,000 pages of documents in response to Plaintiff's FOIA request. *See* Sager Decl. ¶¶ 13, 15-27. On

December 8, 2017, FDA made its final production of documents in response to Plaintiff's FOIA request. *Id.* ¶ 23. Thereafter, the parties engaged in a number of discussions in an effort to narrow the issues that Plaintiff raised regarding FDA's productions. *See* ECF Nos. 49, 63 & 66. In December 2017 and January 2018, in response to specific questions raised about the productions, FDA re-produced certain documents with certain redactions removed. *See* Sager Decl. ¶ 23.

The only redactions Plaintiff has indicated he intends to challenge pertain to certain information from the Study 201 and Study 202 CSRs that was withheld pursuant to FOIA Exemption 4 because the release of the information would cause competitive harm to Sarepta. *See* ECF No. 66 at 1.[3] The specific redactions in the CSRs Plaintiff has advised he will challenge are reflected on the *Vaughn* index. *See* Sager Decl. Ex. O. The general categories of redacted information include: (1) granular-level details of Sarepta's clinical studies; (2) patient-level data regarding study results and patient characteristics; (3) certain adverse event information; and (4) certain exploratory endpoint information. *See* Sager Decl. ¶ 35; Ex. O.

Plaintiff does not challenge the adequacy of the Government's search, nor does he challenge any withholdings from any other documents in the production. *See* ECF No. 66 at 2.

ARGUMENT

A. Legal Standards for Summary Judgment in FOIA Actions

The Freedom of Information Act, 5 U.S.C. § 552, represents a balance struck by Congress "'between the right of the public to know and the need of the Government to keep information in

[3] As the parties explained in their March 22, 2018, joint letter, FDA also withheld information in the documents on the basis of patient privacy pursuant to FOIA Exemption 6. At this time, Plaintiff does not challenge the Exemption 6 withholdings. In the event the Court were to rule in Plaintiff's favor on the Exemption 4 withholdings, the parties would meet and confer on redactions that would ensure patient privacy before the documents are released. All parties have reserved their rights to raise issues related to Exemption 6 in the event that they are unable to reach agreement. *See* ECF No. 66 at 1-2.

confidence.'" *John Doe Agency v. John Doe Corp.*, 493 U.S. 146, 152 (1989) (quoting H.R. Rep. No. 1497, 89th Cong., 2d Sess., 6 (1966)); *New York Times Co. v. DOJ*, 872 F. Supp. 2d 309, 314 (S.D.N.Y. 2012). Thus, while FOIA requires disclosure under certain circumstances, the statute recognizes "that public disclosure is not always in the public interest," *CIA v. Sims*, 471 U.S. 159, 166-167 (1985), and mandates that records need not be disclosed if "the documents fall within [the] enumerated exemptions." *DOI v. Klamath Water Users Protective Ass'n*, 532 U.S. 1, 7 (2001) (citations omitted); *see also John Doe Agency*, 493 U.S. at 152 (FOIA exemptions are "intended to have meaningful reach and application"); *Martin v. DOJ,* 488 F.3d 446, 453 (D.C. Cir. 2007) ("Recognizing, however, that the public's right to information was not absolute and that disclosure of certain information may harm legitimate governmental or private interests, Congress created several exemptions to FOIA disclosure requirements.").[4]

Most FOIA actions are resolved by summary judgment. *See Carney v. DOJ*, 19 F.3d 807, 812 (2d Cir. 1994); *N.Y. Times v. DOJ*, 915 F. Supp. 2d 508, 531 (S.D.N.Y. 2013). Summary judgment is warranted if "there is no genuine dispute as to any material fact and the movant is entitled to judgment as a matter of law." Fed. R. Civ. P. 56(a). A defendant is entitled to summary judgment in a FOIA case when it demonstrates that its search was adequate and that any withheld documents fell within an Exemption to FOIA. *See Carney*, 19 F.3d at 812. The defendant agency bears the burden to demonstrate that any information it withheld is exempt from disclosure. *See* 5 U.S.C. § 552(a)(4)(B). The agency can satisfy this burden using "[a]ffidavits or declarations supplying facts indicating that the agency has conducted a thorough search and giving reasonably

[4] Courts in this Circuit frequently cite FOIA decisions from the D.C. Circuit, "a jurisdiction with considerable experience on FOIA matters." *Main Street Legal Servs., Inc. v. Nat'l Sec. Council,* 13 Civ. 948 (ENV), 2013 WL 4494712, at *2 (E.D.N.Y. Aug. 7, 2013) (citing cases).

detailed explanations why any withheld documents fall within an Exemption." *See Carney,* 19 F.3d at 812.

FDA supports this motion with declarations from two FDA employees: Sarah Kotler, Director of the Division of Freedom of Information ("DFOI"), FDA's Office of the Executive Secretariat; and Nancy B. Sager, Director of the Division of Information Disclosure Policy ("DIDP"), in FDA's Center for Drug Evaluation and Research ("CDER") and accompanying *Vaughn* index, as well as the Declaration of Sarepta employee, Ian Estepan, *see* ECF No. 72 ("Estepan Decl."). [5] These declarations establish that FDA's withholding of the redacted information from the Study 201 and Study 202 CSRs as confidential commercial information pursuant to FOIA Exemption 4 is appropriate.

B. The FDA Properly Withheld Confidential Information Submitted by Sarepta Pursuant to FOIA Exemption 4

Exemption 4 protects "trade secrets and commercial or financial information obtained from a person [that is] privileged or confidential." 5 U.S.C. § 552(b)(4). This exemption covers two distinct categories of information: (1) trade secrets; and (2) information that is (a) commercial or financial, and (b) obtained from a person, and (c) is privileged or confidential. *Nadler v. FDIC*, 92 F.3d 93, 95 (2d Cir. 1996). In this case, the second category applies. Plaintiff does not dispute that the withheld information is commercial or financial; nor does he dispute that it was obtained

[5] Courts in this District and elsewhere have accepted and considered declarations from the third-party submitters of information at issue in Exemption 4 cases. *See, e.g.*, *NRDC v. U.S. Dep't of Interior*, 36 F. Supp. 3d 384, 401 n.10 (S.D.N.Y. 2014) (finding that such declarations are "relevant and based on personal knowledge," and thus that "[t]he Court is . . . at liberty to consider the declarations"); *Pub. Citizen v. HHS*, 66 F. Supp. 3d 196, 208 (D.D.C. 2014) (Defendant-Intervenor's "declarants make a strong case as to why the information contained in these documents could be used to cause substantial commercial harm."); *Utah v. U.S. Dep't of Interior*, 256 F.3d 967, 970 (10th Cir. 2001) (considering declarations from the two private entities whose information was sought to be disclosed).

from persons. *See* ECF No. 66 at 1. The only question before the Court is whether the information is confidential.

"[I]nformation is confidential for the purposes of Exemption 4 if its disclosure would have the effect either: '(1) of impairing the government's ability to obtain information—necessary information—in the future, or (2) of causing substantial harm to the competitive position of the person from whom the information was obtained.'" *Inner City Press/Cmty. On the Move v. Bd. of Governors of the Fed. Res. Sys.,* 463 F.3d 239, 244 (2d Cir. 2006) (quoting *Cont'l Stock Transfer & Trust Co. v. SEC,* 566 F.2d 373, 375 (2d Cir. 1977) (adopting standard set forth in *Nat'l Parks & Conservation Ass'n v. Morton,* 498 F.2d 765, 770 (D.C. Cir. 1974)).[6] The legislative history of FOIA "firmly supports the inference that [Exemption 4] is intended for the benefit of persons who supply information as well as the agencies which gather it." *Nat'l Parks*, 498 F.2d at 767-70. In this case, the FDA has withheld certain information from the CSRs because its release would cause substantial competitive harm to Sarepta. *See* Sager Decl. ¶¶ 34-36 & Ex. O; *see also generally* Estepan Decl.

To establish substantial harm to the competitive position of the person from whom the information was obtained, courts do not require a sophisticated economic analysis of the likely effects of disclosure nor a showing that releasing the information would cause "actual competitive

[6] Since the *National Parks* decision, the D.C. Circuit has developed an additional test for withholding of information that is voluntarily provided to the government, which provides that such voluntarily-provided information should be withheld pursuant to Exemption 4 if it "would customarily not be released to the public by the person from whom it was obtained." *Critical Mass Energy Project v. Nuclear Regulatory Comm'n*, 975 F.2d 871, 878-79 (D.C. Cir. 1992) (en banc). To date, the *Critical Mass* test has not been adopted by the Second Circuit. *See Inner City Press*, 463 F.3d at 245 n.6. In any event, the information at issue contained in the CSRs is information that Sarepta was required to submit in connection with its NDA. *See* 21 U.S.C. § 355(b)(1) ("Such person shall submit to the Secretary as a part of the application . . . full reports of investigations which have been made to show whether or not such drug is safe for use and whether such drug is effective in use. . . . ").

harm." *See Pub. Citizen Health Research Grp. v. FDA*, 704 F.2d 1280, 1291 (D.C. Cir. 1983). Rather, "[t]o establish competitive harm, the Government must show that 'the person who submitted the information faces both (1) actual competition and (2) a likelihood of 'substantial' competitive injury if the information were released." *NRDC*, 36 F. Supp. 3d at 402 (quoting *Inner City Press v. Bd. of Governors*, 380 F. Supp. 2d 211, 219 (S.D.N.Y. 2005), *aff'd* 463 F.3d 239 (2d Cir. 2006)). In determining whether the "competitive injury" prong has been satisfied, "the Court need only exercise its judgment in view of the nature of the material sought and competitive circumstances in which the submitter does business, but no actual adverse effect on competition need be shown." *Customs & Int'l Trade Newsletter v. U.S. Customs & Border Prot.*, 588 F. Supp. 2d 51, 55 (D.D.C. 2008) (internal quotations and citations omitted).[7]

1. The Market for New Drugs Is Extremely Competitive

The pharmaceutical industry is highly competitive. *See Pub. Citizen Health Research Group v. NIH*, 209 F. Supp. 2d 37, 47 (D.D.C. 2002) (recognizing that the "pharmaceutical industry is a highly competitive market where companies routinely attempt to discover a possible advantage over their competitors"); *Citizens Comm'n on Human Rights v. FDA*, No. 92CV5313, 1993 WL 1610471, at *7–9 (C.D. Cal. May 10, 1993), *aff'd in part, remanded in part sub nom. Citizens Comm'n on Human Rights v. FDA*., 45 F.3d 1325 (9th Cir. 1995) ("Actual competition in the drug manufacturing business is evident from the record before the Court. Drug manufacturers must invest enormous time and capital to 'pioneer' a new drug . . . . After a new drug is approved for

[7] FDA has also satisfied its obligation to provide plaintiff with "[a]ny reasonably segregable portion" of the requested records "after delet[ing those] portions which are exempt . . . ." 5 U.S.C. § 552(b); *see, e.g., Billington v. Dep't of Justice*, 233 F.3d 581, 586 (D.C. Cir. 2000); *Fla. Immigrant Advocacy Ctr. v. National Security Agency,* 380 F. Supp. 2d 1332, 1337 (S.D. Fla 2005). Indeed, after discussions with Plaintiff, FDA did a secondary review, and released additional previously redacted information. *See* Sager Decl. ¶ 23.

marketing, actual competition also exists among manufacturers seeking approval to market the drug in a 'generic' form.").

Sarepta has invested a significant amount of time and expense in developing Exondys 51. *See* Estepan Decl. ¶¶ 11, 14-21. Sarepta submitted an Investigational New Drug application ("IND") to the FDA for eteplirsen in 2007. *Id.* ¶ 14. It conducted clinical studies in 2011 and 2012. *Id.* ¶¶ 15-16. Finally, it submitted an NDA for eteplirsen in 2015. *Id.* ¶ 20. Although Exondys 51 is currently the only exon-skipping therapy[8] approved by the FDA for DMD in the United States, Sarepta's competitors are working to develop their own DMD treatments—including treatments that use exon-skipping. *See* Estepan Decl. ¶¶ 44-49. As Sarepta details in its declaration, since Sarepta's clinical development of eteplirsen was initiated, between early 2016 and late 2017, three other drug manufacturers have been developing antisense oligonucleotides for DMD. *See id.* ¶¶ 45, 47-49. Additionally, six companies are pursuing development of drug therapies to cause DMD patients to produce dystrophin. *See id.* ¶ 46. There are dozens of planned trials for other exon-shipping DMD treatments and 27 DMD assets in clinical development with expected US market approval between 2020 and 2027. *See id.* ¶ 58-59.

The information provided in Sarepta's declaration regarding the names and activities of Sarepta's competitors developing competing treatments establishes that actual competition exists for the development of a DMD drug. *See, e.g., Gov't Accountability Project v. FDA*, 206 F. Supp. 3d 420, 439 (D.D.C. 2016) (holding that "[i]t is hard to imagine what more information the FDA and [Defendant-Intervenor] could have provided to demonstrate . . . actual competition" when

[8] Exon skipping is a molecular biological process by which the cellular machinery is instructed to "skip over" a certain part of gene sequence when reading it. In the context of muscular dystrophy, exon skipping can cause the body to skip over the mutation to produce a functional, shorter form of the dystrophin gene, thereby mitigating the impact of the disease. *See* Estepan Decl. ¶ 9.

declarations that named specific competitors, explained the structure of the market and/or provided specific examples of competing products across drug classes); *People for Ethical Treatment of Animals v. U.S. Dep't of Agric.*, No. CIV. 03 C 195-SBC, 2005 WL 1241141, at *6 (D.D.C. May 24, 2005) (declaration that "describes the number of competitors" in the industry and is based on declarant's years of experience in industry was sufficient to establish actual competition); *Citizen's Comm'n on Human Rights*, 1993 WL 1610471, at *8.

2. Release of The Redacted Information Would Likely Cause Substantial Competitive Injury to Sarepta

In the highly competitive market of new drug development and manufacturing, the release of the withheld information would provide an advantage to Sarepta's competitors and cause substantial competitive injury to Sarepta. Courts have recognized that if information is improperly released from the materials that drug manufacturers submit to the FDA for drug approval, other companies could make use of that information to "eliminate much of the time and effort that would otherwise be required to bring to market a product competitive with the product for which" the submitting company filed the application. *Judicial Watch, Inc. v. FDA*, 449 F.3d 141, 148-49 (D.C. Cir. 2006) (quoting *Pub. Citizen Health Research Group v. FDA,* 185 F.3d 898, 905 (D.C. Cir. 1999); *see also Webb v. HHS*, 696 F.2d 101, 103 (D.C. Cir. 1982) ("If a manufacturer's competitor could obtain all the data in the manufacturer's NDA, it could utilize them in its own NDA without incurring the time, labor, risk, and expense involved in developing them independently.").

As Sarepta explains in its declaration and as is detailed in the *Vaughn* index, release of the withheld information would allow Sarepta's competitors to do just that—thereby allowing them to reap the benefits of Sarepta's clinical studies without investing their own time and resources into producing their own studies. *See* Estepan Decl. ¶¶ 22-28; Sager Decl. Ex. O. Moreover, the

information at issue has not been shared outside Sarepta and is closely controlled within the company—dissemination of study results is limited to certain members of the clinical development, regulatory, biostatistics, and data management functions, select reporting functions, as well as certain members of the executive committee. *See* Estepan Decl. ¶ 19, 22. Notably, while other pharmaceutical companies have requested access to Sarepta's clinical trial data and testing methodologies, Sarepta has opted to maintain the confidentiality of this information. *Id.* ¶ 52.

a. Granular-Level Detail Regarding Clinical Studies

The FDA has withheld certain portions of the CSRs because release of the information would reveal granular-level detail of Sarepta's clinical study procedures—including information regarding the timing of when Sarepta performed certain tests and details regarding the tests themselves, dosing information, the proper method to quantify dystrophin and patient selection criteria. *See* Sager Decl. Ex. O at 2-18; *see also* Estepan Decl. ¶¶ 23-28. Due to the lack of information and understanding regarding DMD—including the lack of previously recognized endpoints and poorly characterized, diverse rates of disease progression—even the most elementary aspects of a clinical trial require a significant investment of resources to develop. *See id.* ¶¶ 22, 24. Sarepta invested significant resources in developing its clinical studies and over three years perfecting its clinical study procedure. *Id.* ¶ 22. While some details of Sarepta's test protocols have been made available, the withheld information has remained non-public. *See id.* ¶ 23.

Release of the redacted information would allow Sarepta's competitors to copy Sarepta's study design, or selectively modify it, without having invested the time and resources into producing their own studies. *Id.* ¶¶ 22, 24. Specifically, Sarepta's competitors could bypass the

years of trial and error that Sarepta undertook to develop successful dosing approaches or to develop validation techniques to quantify dystrophin. *See id.* ¶¶ 25-26. Essentially competitors could "retrospectively" design their clinical trials, providing them a higher likelihood of success, and shortening their timeline for FDA approval. *Id.* ¶ 28. The release of this information regarding specific aspects of study protocols could be applied to other exon skipping drugs—thereby providing further advantage to Sarepta's competitors. *See id.* ¶ 22.

Such a competitive advantage to Sarepta's competitors would cause a competitive disadvantage to Sarepta because the company has invested significant time and resources into developing the clinical studies. *Id.* ¶¶ 22, 30. *See Worthington Compressors, Inc. v. Costle*, 662 F.2d 45, 51 (D.C. Cir. 1981) ("Because competition in business turns on the relative costs and opportunities faced by members of the same industry, there is a potential windfall for competitors to whom valuable information is released under FOIA. If those competitors are charged only minimal FOIA retrieval costs for the information, rather than the considerable costs of private reproduction, they may be getting quite a bargain. Such bargains could easily have competitive consequences. . . ."); *Pub. Citizen v. HHS*, 66 F. Supp. 3d 196, 214 (D.D.C. 2014) ("A competitor's ability to obtain the information at virtually no cost would cause competitive harm to Pfizer, since it could be used affirmatively by those competitors to challenge Pfizer's place in the market and exploit any vulnerability revealed through the [document's] content."); *cf. Webb*, 696 F.2d at 103.

b. Patient-Level Data Regarding Study Results and Patient Characteristics

FDA also withheld: granular-level descriptions of the study results; data tables included in the CSRs; and data tables attached to the CSRs as appendices. *See* Sager Decl. Ex. O at 4-10, 12-18. All of these documents provide individual patient-level indicators and statistical analysis of their significance. *See id; see also* Estepan Decl. ¶¶ 28-33. Release of such information would

cause Sarepta competitive harm because it would allow competitors to use the results in their own research, to inform their competitive decisions, or to further their own sales or marketing campaigns by claiming that their product is superior to Exondys 51. *See* Estepan Decl. ¶¶ 29, 32. As such, release of detailed information regarding the results of the clinical studies would give Sarepta's competitors the benefit of having conducted those studies without having incurred the costs of such studies. *See id.* ¶ 29. Moreover, it would give Sarepta's competitors a free control data set—the very type of control dataset that Sarepta spent years and millions of dollars developing. *See id.* ¶ 31, 33. Indeed, the value that Sarepta would be deprived of is not speculative—if it chose to do so, Sarepta could now sell its data to competitors for use as a control set. *Id.* ¶ 33.

c. Information Regarding Endpoints

FDA also withheld non-public information regarding clinical study endpoints from the CSRs. *See* Sager Decl. Ex. O at 1, 3, 4, 10-12, 14, 16-18. "Endpoints" define what is being investigated in a study and measure and evaluate drug efficacy. Estepan Decl. ¶ 35.[9] "Clinical" endpoints consider the direct effects on the patient, such as appearance (or not) of certain symptoms or performance on certain tests; "surrogate" endpoints use lab measurements to track the presence or absence of a marker that is affiliated with the disease under study. *Id.* As Sarepta explains in its declaration, because DMD is not a well-characterized disease, and involves a very small population, the ideal outcomes for the patient population are not well-known. *See* Estepan Decl. ¶ 34, 38. Sarepta invested millions of dollars to further understand the DMD's characteristics and to define what would be the effect of a "successful" treatment. *Id.* ¶ 34.

[9] *See also Multiple Endpoints in Clinical Trials: Guidance for Industry* (draft guidance), January 2017, available at https://www.fda.gov/downloads/drugs/guidancecomplianceregulatory information/guidances/ucm536750.pdf (last accessed April 5, 2018).

Not all endpoints explored in a particular study are ultimately pursued (and published) in relation to that study and a company may choose to conduct further research on a particular exploratory endpoint in a future study. *Id.* ¶ 37. With knowledge of the endpoints that Sarepta pursued in its clinical studies and the results Sarepta obtained, Sarepta's competitors would have an advantage that Sarepta did not have when conducting its study—sparing them the significant investment of time and resources that Sarepta incurred in individually considering, testing and analyzing multiple potential clinical endpoints that Sarepta did not ultimately utilize to demonstrate the efficacy of eteplirsen and allowing competitors to bring their competing products to market faster. *Id.* ¶ 39.

Revealing information regarding non-public exploratory endpoints would reveal to Sarepta's competitors information about which endpoints Sarepta is currently pursuing. Competitors could then use such information either to mirror Sarepta's approach or to predict areas in which Sarepta may focus its future research. *Id.* ¶ 37.

d. Information Regarding Nonpublic Adverse Events

The CSRs also describe adverse events that occurred during the studies. *See id.* ¶ 40. As part of the clinical studies, Sarepta made a determination as to which subset of the adverse events were in fact adverse reactions associated with the action of the drug. *Id.* Those adverse reactions are disclosed on the drug's FDA-approved label, whereas unrelated adverse events are not. *See* Sager Decl. Ex. O at 1, 14. FDA redacted mention of adverse events that had not previously been publicly disclosed from the CSRs. *Id.* It also redacted data related to the analysis of adverse events. *Id.* at 9, 14; *see also* Estepan Decl. ¶ 42.

Release of information regarding nonpublic adverse events would benefit Sarepta's competitors. Because distinguishing unrelated adverse events from adverse reactions related to

the drug requires significant investment of time and resources, other companies could use this information about potential adverse reactions and events in their own studies without investing the necessary resources into making these determinations. *See* Estepan Decl. ¶¶ 40-41, 43.

The detailed information in Sarepta's declaration and the *Vaughn* index establishes that release of the redacted information would cause competitive harm to Sarepta.

CONCLUSION

For the foregoing reasons, Defendants' motion for summary judgment should be granted.

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