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March 4, 2016

Via email to http://www.accessdata.fda.gov/scripts/foi/FOIRequest/index.cfm

United States Food and Drug Administration
5630 Fishers Lane, Room 1035
Rockville, Maryland 20857
Division of Freedom of Information
Office of the Executive Secretariat, OC

RE: FOIA Request Concerning Ionis Pharmaceuticals, Inc. and Biogen Pharmaceuticals leading to the development of the compound Nusinersen

Dear Executive Secretariat:

This is a Freedom of Information Act (FOIA) request for information concerning the Food and Drug Administration (FDA) process with respect to Ionis Pharmaceuticals, Inc. and Biogen Pharmaceuticals leading to the development of the compound Nusinersen as a potential treatment for spinal muscular atrophy (SMA) from January 1, 2015 to March 3, 2016.

Set forth here is information to aide in providing the response to this request.

- SMA is a severe motor-neuron disease. It is designated a rare orphan disease.
- Ionis Pharmaceuticals, Inc., changed its corporate name in late 2015 that was formerly Isis Pharmaceuticals, Inc. Hereinafter, Ionis.
- Ionis is headquartered in Carlsbad, California.
- Ionis developed and owns Nusinersen as a potential drug compound therapy treatment.
- Nusinersen is formerly known as ISISRX and IONIS-$SMN_{RX.}$
- Nusinersen is an antisense drug discovered in collaboration with Cold Spring Harbor Laboratory. SMA occurs from a deletion or mutation of a

gene responsible for producing a protein critical for normal cellular function. Nusinersen is designed to increase the production of this protein by modulating the splicing of a closely related gene, thereby compensating for the underlying genetic defect.

- Biogen currently licenses Nusinersen and is associated with previous and current clinical trials of the therapy.
- Biogen is headquartered in Cambridge, Massachusetts.
- Nusinersen is currently in phase III status.
- Nusinersen is designated an Investigational New Drug.

Given the above details, this FOIA request is for the following information:

1. Please provide any documentation; minutes of meetings; emails; correspondence; or other records as to whether, as of March 3, 2016, either Ionis or Biogen has made an application, written request, or discussed with FDA officials formally or informally any request for approval to provide or otherwise make available Nusinersen to SMA patients within the United States.
2. Please provide any documentation; minutes of meetings; emails; correspondence; or other records as to whether, from January 1, 2015 through March 3, 2016, either Ionis or Biogen has discussed formally or informally a program with the FDA to establish an expanded therapy(ies) access program, also known as a compassionate care access or single patient access program, for SMA patients not currently participating in a clinical trial investigation.
3. Please provide copies of meeting minutes; data; charts; graphs; findings, results; statements; correspondence; or other records provided by Ionis and Biogen to the FDA with respect to SMA and Nusinersen from January 1, 2015 through March 3, 2016.
4. Please provide copies of any Federal Register notices issued by the FDA with respect to SMA-related regulatory and other federal agencies (e.g., the National Institutes of Health, Department of Commerce) from January 1, 2015 through March 3, 2016.
5. Please provide copies of any Federal Register notices issued by the FDA with respect to SMA research; clinical trials; Investigational New Drug policies; Orphan Drug designation; or other specifically related information to SMA and/or Nusinersen from January 1, 2015 through March 3, 2016.
6. Please provide copies of any data; meeting notes; pre-meeting agenda; transcripts; or other documentation specifically related to any teleconferences, in-person meetings, email or other correspondence between the FDA and Ionis

and Biogen related to SMA and/or Nusinersen from January 1, 2015 through March 3, 2016.

7. Whether the FDA, from January 1, 2015 through March 3, 2016, has denied any request to establish an expanded access policy by Ionis or Biogen, and, if so, please provide any written documentation explaining the FDA's position or determination of such request.
8. Please provide the names, titles, and contact information of each FDA official authorized to determine whether and at what stage a potential therapy SMA treatment in phase III status may be considered for an expanded access approval by the FDA from January 1, 2015 through March 3, 2016.
9. Please provide any documentation as to whether the FDA may or will review next (e.g., March 1, 2016 through December 31, 2016) a change in status of Nusinersen. Considering the dynamic nature of medical research notwithstanding, please provide any written documents, emails, or other documentation related to whether the FDA has indicated a date, specific or general, it may or will consider a review-point, end-point, or milestone timeline indicating whether the FDA will or may consider the status of Nusinersen.
10. Please provide any other information; records; minutes; emails; and correspondence between the FDA and any other Federal agency, privately held corporation, institution of higher education, research facility, or other entity related to SMA and Nusinersen from January 1, 2015 through March 3, 2016.

I am able to pay up to one-hundred dollars ($100.00) to obtain the information requested. Please contact me if the fees amount to more than one-hundred dollars. In any event, please provide the information up to the cost of one-hundred dollars. My child is afflicted with SMA and I am trying to advance access to Nusinersen for her and other children. Thank you for this information.

Sincerely,

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